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SUPPLEMENT

Poster Abstracts

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The AMCP Poster Abstract Program provides a forum for authors to share their research with the managed care pharmacy community. Authors submit their abstracts to AMCP, and each abstract is reviewed by a team of peer reviewers and editors. All accepted abstracts are presented as posters at AMCP's Annual and Nexus meetings. These abstracts are also available through the AMCP meeting app. This JMCP supplement publishes all abstracts that were peer reviewed and accepted for presentation at Nexus 2024. Abstracts submitted in the Student and Encore categories did not undergo peer review; therefore, these abstracts are not included in the supplement.

ABSTRACT REVIEW PROCESS

Eighty-five reviewers and 4 JMCP editors completed the review process for Nexus 2024. Each abstract was reviewed and scored using a 1-5 scale with the following 5 criteria (15 rating scores per abstract), which are used by JMCP to evaluate manuscripts for publication:

• Relevance • Originality • Quality
• Bias • Clarity

Each of the reviewers also made an independent accept/reject recommendation. The 15 rating scores and 3 accept/reject recommendations for each abstract were reviewed by a JMCP editor, who made an accept/reject decision. These decisions were reviewed and finalized by the JMCP editor-in-chief. The mean rating scores were used to award Platinum, Gold, Silver, and Bronze medals for the best abstracts submitted. The abstract reviewers for Nexus 2024 were as follows:

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Medal-Winning Abstracts

Each abstract was assessed by reviewers using a 1-5 scale on the following 5 criteria: relevance, originality, quality, bias, and clarity. These are the same criteria used by *JMCP* to evaluate manuscripts. The abstract's mean score on the 5 criteria was used to award Platinum, Gold, Silver, or Bronze medals.

Brandon T. Suehs [C40] Experience with an outcomes-based rebate agreement for apalutamide in the treatment of prostate cancer

Daniel Gratie [U17] Does the maximum fair price accurately represent the value of multi-indication products?

Jonathan James [U25] Access to specialty medicines with alternative funding programs: A descriptive survey of patient experiences

Molly Beinfeld [U34] Trends in US health plan coverage for adalimumab products

William B. Wong [C10] Real-world costs associated with upfront biomarker testing in advanced non–small cell lung cancer and metastatic colorectal cancer

Shweta Kamat [C30] Health care costs associated with prophylactic neurokinin-1 receptor antagonist use among women with invasive breast cancer

Alina Liang [D17] Evaluating the 10-year impact of an immunoglobulin utilization management and dose optimization program in the managed care setting

Danielle Baird [E21] Barriers to growth hormone access in pediatric patients at an academic medical center

Jiayuan Wang [E29] Assessing sociodemographic differences between adherent and nonadherent patients with obesity on glucagon-like peptide 1 medications

Elizabeth Brunner [F28] Impact of centanafadine, a novel nitrosamine drug substance–related impurity, on quality of life in adolescents with attention-deficit/hyperactivity disorder

Swapna Karkare [G1] Budget impact of subcutaneous efgartigimod PH20 for chronic inflammatory demyelinating polyneuropathy from a US payer perspective

Ralph Quimbo [K1] Development of a claims-based artificial intelligence algorithm for identifying inflammatory bowel disease flares

Kasey Estenson [K3] LEAP-002 update: Lenvatinib + pembrolizumab vs lenvatinib + placebo as first-line therapy for patients with advanced hepatocellular carcinoma after an additional 12 months of follow-up

Jeanine A. Flanigan [M17] Influence of clinical scenarios on cost effectiveness model results for biosimilar denosumab in women with postmenopausal osteoporosis

Medal-Winning Abstracts

Shannon Grabich [M22] Real-world treatment patterns of phosphorodiamidate morpholino oligomer therapies in patients with Duchenne muscular dystrophy: An administrative claims–based analysis

Shaila Yoshida [U9] The role of patient experience data in payer decision-making

Debra Carlson [U30] Medical drug coding and adjudication review

Zahra Majd [C61] Treatment adherence and persistence among patients with chronic lymphocytic leukemia/small lymphocytic lymphoma receiving first-line Bruton tyrosine kinase inhibitors

Giovanna Tedesco Barcelos [D7] Real-world health care resource utilization and costs for children with sickle cell disease in the United States: Retrospective Medicaid analysis

Bertha A. De Los Santos [E7] Trends in diabetes cost of illness

Landon Z. Marshall [E42] Real-world adherence and persistence to glucagon-like peptide-1 receptor agonists at 2 years among commercially insured adults with obesity without diabetes

Scott Leslie [G2] Real-world case series study of pediatric patients with spinal muscular atrophy treated with onasemnogene abeparvovec (Zolgensma)

Malgorzata Ciepielewska [G4] Health care resource utilization of oral edaravone–treated patients with amyotrophic lateral sclerosis enrolled in an administrative claims database

Pooja Gokhale [G43] Cost-effectiveness analysis of ubrogepant, rimegepant, and zavegepant compared with each other and usual care for the acute treatment of migraine

Steven Sherman [H3] Economic benefit of aflibercept 8 mg vs faricimab in the treatment of patients with neovascular age-related macular degeneration or diabetic macular edema in the United States

Tuan Huynh [I4] Pharmacist-led statin improvement project in a multipayer care organization

Nihar R. Desai [I16] Health care resource utilization among patients with paroxysmal supraventricular tachycardia

A. Mark Fendrick [L14] Brand-to-brand nonmedical switching among interleukin-17 inhibitors or other biologics: Implications of a formulary change

Michael York [U13] Finding a niche? Role of real-world evidence in US Food and Drug Administration expedited development and review pathways

Brandon T. Suehs [U29] Health-related social needs and quality measure attainment among dual-eligible Medicare Advantage beneficiaries

Medal-Winning Abstracts

Alexjandro Daviano [C59] Impact of social risk factors on first-line treatment patterns among Medicare Advantage members with newly diagnosed multiple myeloma

Nehir Yapar [E36] Effect of weight loss medications on cardiovascular risk among active US military personnel

Matt Sidovar [F14] Long-term metabolic change associated with KarXT (xanomeline and trospium) in patients with schizophrenia and pre-existing metabolic conditions: Interim results from pooled, long-term safety studies EMERGENT-4 and EMERGENT-5

Richard A. Brook [F21] Medical, prescription, disability, and absence cost for employees with major depressive disorder: Developing real-world models using shorter pre-index time periods for Charlson Comorbidity Indexes and smaller sample sizes

Alexandra G. Hames [G14] Impact of maximum out-of-pocket spending on affordability of high-cost treatments for Alzheimer disease

Michael J. Doane [G47] Clinical, economic, and humanistic burden associated with narcolepsy: Results from a systematic literature review

Karen C. Thomas [R2] Pharmacist involvement in safety screening for patients starting immune-modulating therapies: Results from a multisite, prospective, observational cohort study

Nicole Grevenitz [U20] Promoting health equity through enhanced, personalized digital outreach

Jennifer Malinowski [U22] Variation in payer prior authorization rates is an unequal barrier to patient care

Platinum Award-Winning Abstracts

C40 Experience with an outcomes-based rebate agreement for apalutamide in the treatment of prostate cancer

Brandon T. Suehs, Bart Brown, MS
Humana Healthcare Research, Inc, 500 W. Main Street, Louisville, KY 40202; bsuehs6@humana.com

BACKGROUND: Humana Inc. and Janssen Biotech Inc. entered into an innovative outcomes-based rebate agreement (OBA) for apalutamide in 2019. The OBA offered financial rebates for the on-label utilization of apalutamide based on clinical performance of apalutamide (trade name: Erleada) for treatment of prostate cancer among certain individuals enrolled in Humana Medicare Advantage prescription drug (MAPD) plans.

OBJECTIVE: To summarize the implementation of the OBA and the outcomes reported during the implementation of the OBA.

METHODS: This analysis included individuals enrolled in eligible MAPD plans newly initiating apalutamide treatment from July 2019 through June 2023. Patients that were prescribed any oral androgen receptor inhibitors (OAIs) prior to initial dispensing of apalutamide were excluded. In order to assess clinical performance, 2 prostate-specific antigen (PSA) laboratory test results were required, one during the baseline period prior to apalutamide treatment initiation (up to 91 days prior to dispensing) and a second PSA test result between +56 and +112 days after the date of initial dispensing. Treatment response was defined as reduction of >50% in PSA level. Descriptive analyses of attrition and treatment response were conducted.

RESULTS: A total of 4,042 individuals were identified initially with a dispensing of at least 60 days supply of apalutamide. Among those, 1,556 individuals were new to OAI therapy and eligible for inclusion in the clinical performance analysis. Baseline PSA results were available for 42.2% (n = 656) of individuals, and both baseline and post–OAI initiation PSA test results were available for 19.5% (n = 303) of new initiators. A further 20 individuals were excluded from the clinical performance analysis owing to a lapse in continuous enrollment between the first PSA test and the postinitiation retest. Among individuals included in the clinical performance assessment (n = 283), 67.5% (n = 191) demonstrated PSA reduction of >50%.

CONCLUSIONS: Pharmacy OBA arrangements represent an innovative way to tie price concessions on utilization of products to outcomes-oriented metrics. Challenges include availability of the necessary clinical data for the health plan to fully implement performance-based measures. Strategies to improve clinical data availability to support such agreements, such as medical record reviews or other tactics, should be considered.

SPONSORSHIP: None.

U17 Does the maximum fair price accurately represent the value of multi-indication products?

Daniel Gratie, PharmD, MS, Ami Buikema, MPH, Sarah Bandy, PharmD, PhD, Elizabeth Brooks, Erin Hulbert, MS, MBA, Kristin Moore, PhD, MPH, Cristina Masseria, PhD, MSc
AESARA, PO Box 4266, Chapel Hill, NC 27515; dan@aesara.com

BACKGROUND: The Inflation Reduction Act (IRA) of 2022 allows Medicare to negotiate prices for high-cost prescription drugs. Current guidance estimates average drug expenditure across dosages and formulations; no information has been provided on how variability across indications will be considered and the impact this will have on the inherent value of a treatment.

OBJECTIVE: To investigate high-spend Medicare drugs with multiple indications that are eligible for IRA negotiations in 2025 and to estimate the distribution of dosage/formulations tied to different indications.

METHODS: Using 2022 Medicare expenditures, we identified high-spend drugs with more than 4 indications covered by the Centers for Medicare & Medicaid Services, not previously considered for IRA negotiations (XTANDI, JAKAFI, TAGRISSO, REPATHA, COSENTYX, OTEZLA, VRAYLAR). Claims data from the Optum Research Database of Medicare Advantage with Part D (MAPD) enrollees from January 1 to December 31, 2022, were used. Patients were eligible for inclusion with at least 1 fill for a potential drug (index date), enrollment for at least 1 day, and a related diagnosis code. The distribution of dosage/formulations by drug (National Drug Code-9 numbers) and

label-consistent indications based on *International Classification of Diseases, Tenth Revision* codes within 1 year pre-index were described and analyzed.

RESULTS: There were 5,586,290 MAPD beneficiaries with at least 1 day of eligibility and a health care encounter (visit or prescription) in 2022. Samples of patients with a prescription fill in 2022 ranged from 992 (TAGRISSO) to 34,466 (REPATHA), with an average of 2.71 (SD = 1.38) dosage and formulations compared with 4.14 (SD = 0.38) indications per drug. For example, weighting XTANDI (n = 2,625) by formulation would show 28.5% of patients were prescribed 40-mg capsules, 53.7% 40-mg tablets, and 17.8% 80-mg tablets. However, this would not show that 69.7% of these patients were being treated for metastatic disease compared with 30.3% for nonmetastatic disease, as dosage did not vary widely by metastatic status.

CONCLUSIONS: Our findings highlight variability in drug utilization based on dosage/formulation and indications. Medicare should consider a weighting approach reflecting the clinical value of medications across different indications, beyond just formulation options. The value of these drugs depends on the unmet needs and clinical outcomes for each indication. These results demonstrate that the current IRA calculations may not accurately represent a product's clinical value when establishing a maximum fair price.

SPONSORSHIP: None.

U25 Access to specialty medicines with alternative funding programs: A descriptive survey of patient experiences

Jonathan James, Sarah N. Gibbs, MPH, Irina Yermilov, MD, MPH, MS, Lori Bienvenu, MS, LPC, William B. Wong, PharmD, MS
Hope Charities, 37325 Market Place Dr. Suite B, Prairieville, LA 70769; jonathan@hope-charities.org

BACKGROUND: Alternative funding programs (AFPs) attempt to lower plan sponsor costs by excluding specialty medicines from a beneficiary's plan coverage and obtaining those medicines through alternative sources (typically, manufacturer patient assistance programs) via a third party (ie, AFP vendor). Patients' experiences with and access to medicines through these programs have not been previously described.

OBJECTIVE: To describe patients' experiences and medication access with AFPs.

METHODS: A US national survey consisting of optional single- and multiple-choice questions with branching logic was administered (October to December 2023) to patients who had reported experience with AFPs. Patients were recruited online from a patient panel and patient advocacy group. Broadly, the survey evaluated patients' (1) awareness of AFPs, (2) experience with the patient assistance program application process via the AFP vendor, and (3) timeliness of medication access if granted, and/or the health impact of any delay in medication access. All responses were analyzed descriptively (proportions, means) and reported only for patients who responded to the question(s).

RESULTS: A total of 227 patients were included in the final sample. Most (61%) did not learn about AFPs through their employer and instead first learned about them as part of their health plan benefit when trying to obtain their specialty medication. More than half of patients (54%) reported being uncomfortable with the representative from the AFP vendor, including feeling hesitant providing them with sensitive information. Patients reported a mean wait time of approximately 2 months (68.2 days) to receive their medication and a negative impact on their health (24% reported waiting worsened their condition and 64% reported that it led to stress and/or anxiety). Those who reported waiting led to a negative impact on their health considered leaving or had left their job at a rate 3-5 times higher than those who did not. Eighty-eight percent of patients reported being stressed or anxious because of the medication coverage denial and the uncertainty of being able to obtain their medication.

CONCLUSIONS: Most patients obtaining their specialty medicines via AFPs reported being uncomfortable with the process and had delays in obtaining their medication, which may be linked to worse mental well-being, worsening disease progression, and consideration of a job change. Employers should consider delays in medication access along with the potential downstream impacts on employee retention and employee-employer relationships when considering implementing an AFP into their health plans.

SPONSORSHIP: Genentech, Inc.

U34 Trends in US health plan coverage for adalimumab products

Molly Beinfeld, James Chambers, Fariel C.A. LaMountain
Center for the Evaluation of Value and Risk in Health, Institute for Clinical Research and Health Policy Studies, Tufts Medical Center 33 Kneeland St., Boston, MA 2111; molly.beinfeld@tuftsmedicine.org

BACKGROUND: The first adalimumab biosimilar launched in the United States in January 2023, ending Humira's market exclusivity. Throughout 2023, 7 additional biosimilars entered the market, with more expected in 2024. Despite the increase in competition and potential cost savings,

there are concerns about the limited uptake of adalimumab biosimilars, with health plans playing a key role.

OBJECTIVE: To examine the frequency with which US commercial health plans have granted Humira preferred coverage since the introduction of adalimumab biosimilars.

METHODS: We created a dataset of adalimumab coverage policies issued by 15 large US commercial health plans at 3 timepoints in 2023, using the Tufts Medicine Specialty Drug and Evidence database. The dataset included 8 biosimilars and Humira. We analyzed each US Food and Drug Administration–approved indication separately, considering only coverage decisions where payers covered both Humira and at least 1 biosimilar. We categorized each decision as follows: (1) "Non-preferred: ≥1 Biosimilar Preferred Over Humira," meaning the plan covered 1 or more biosimilars as a preferred treatment prior to granting access to Humira, (2) "Copreferred: Humira Same Line as ≥1 Biosimilar," indicating the plan covered 1 or more biosimilars AND Humira as preferred treatments, or (3) "Preferred: Humira Sole Preferred," where the plan covered Humira as the sole preferred treatment before granting access to biosimilars. We examined the frequency that plans granted Humira preferred status and how it changed over 2023.

RESULTS: The number of coverage policies for adalimumab products increased from 88 in April to 126 in December 2023 as additional biosimilars became available. Plans increasingly preferred biosimilars alongside or over Humira, while the frequency of granting Humira "sole-preferred" status declined. In April, 27% of policies included Humira as the sole-preferred treatment, but by December, none did. By December, 14 of 15 plans copreferred Humira and 1 or more biosimilars and 1 plan preferred at least 1 biosimilar over Humira.

CONCLUSIONS: As adalimumab biosimilar competition has grown, health plan coverage has evolved to offer patients a choice between multiple biosimilars and Humira. The low utilization of adalimumab biosimilars so far may be attributed to patient and physician preference for brand-name products given the option, as well as declines in the net price of Humira. Further research on the relationship between prices, utilization, and coverage may provide more insights into future trends in the US biosimilars market.

SPONSORSHIP: None.

Professional Reviewed Abstracts

A00-B99 Certain Infectious and Parasitic Diseases

(eg, hepatitis C, HIV)

A2 Real-world outcomes of patients with recurrent *Clostridioides difficile* infection administered REBYOTA (fecal microbiota, live-jslm)

Khanna S[1], *Goldenberg D*[2], *Seo S*[3], *Yang M*[4], *Garcia-Horton V*[4], *Gao Y*[4], *Kim H*[4], *Ormenaj L*[4], *Guo A*[3]; *khanna.sahil@mayo.edu*; *sanghyuk.seo@ferring.com*
[1]*Mayo Clinic*; [2]*Stanford University School of Medicine*; [3]*Ferring Pharmaceuticals, Inc.*; [4]*Analysis Group, Inc.*

BACKGROUND: Recurrent *Clostridioides difficile* infection (rCDI) is a common bacterial infection with symptoms ranging from diarrhea to life-threatening sepsis. Up to 65% of patients with rCDI experience subsequent recurrences. REBYOTA (fecal microbiota, live-jslm [RBL]) is the first US Food and Drug Administration–approved fecal microbiota–based product for the prevention of recurrences after antibiotic treatment in adults with rCDI.

OBJECTIVE: To assess the real-world outcomes of patients with rCDI who were administered RBL at home or in a clinic in the United States.

METHODS: Adults with rCDI who received RBL either at home or in a clinic setting in the United States with a minimum follow-up period of 8 weeks were included. The primary outcome was treatment success, defined as the absence of CDI recurrence within 8 weeks of RBL administration, similar to the definition of treatment success in RBL clinical trials. Treatment success was assessed in the overall patient sample as well as within subgroups stratified by age (<65 or ≥65 years), number of prior CDI recurrences (<3 or ≥3), prior bezlotoxumab use, and RBL administration setting (home vs clinic). Treatment success rates were summarized using counts and proportions.

RESULTS: Between July 2023 and May 2024, 155 patients with rCDI received RBL, of whom 108 had at least 8 weeks of follow-up and reported CDI recurrence status. The overall treatment success rate at week 8 was 83.3%. Overall, 50% of patients were aged 65 years and older and had a numerically lower treatment success rate compared with those aged younger than 65 years (79.6% vs 87.0%, $P = 0.30$). Patients who experienced fewer than 3 prior CDI recurrences (22 [20.4%]) had a numerically higher treatment success rate at week 8 compared with those with 3 or more prior recurrences (90.9% vs 81.4%, $P = 0.36$). A total of 10.6% of patients had prior treatment with bezlotoxumab for their rCDI, with similar treatment success rates observed among those who received bezlotoxumab and those who did not (81.8% vs 82.8%, $P = 1.00$). Patients who received RBL at home (81 [75%]) in our sample had a significantly higher treatment success rate compared with patients who received RBL in a clinic (88.9% vs 64.0%, $P < 0.05$).

CONCLUSIONS: RBL was highly effective in preventing CDI recurrences in this real-world setting. The effectiveness was also observed among high-risk subgroups such as patients aged 65 years and older and patients with 3 or more prior CDI recurrences. RBL was associated with a numerically higher treatment success rate among those who received it at or before their second recurrence.

SPONSORSHIP: Ferring Pharmaceuticals, Inc.

B1 Impact of provider prescriptions on herpes zoster vaccine uptake in older US adults

Gatwood J[1], *Zhu Y*[2], *Le L*[2], *Steffens A*[2], *Gallagher S*[2], *Stempniewicz N*[3]; *justin.d.gatwood@gsk.com*
[1]*GlaxoSmithKline*; [2]*Optum Life Sciences*; [3]*GSK*

BACKGROUND: In the United States, a majority of the recombinant zoster vaccine (RZV) doses are given in community pharmacies, which have become a convenient alternative to traditional medical clinics for adults to receive recommended immunizations. However, shifting vaccine administration to occur outside the physician office introduces additional potential barriers to vaccination. Although not generally required, generating prescriptions for vaccines may ultimately lead to vaccination, but evidence identifying the extent to which that happens is lacking.

OBJECTIVE: To describe RZV uptake following a prescription for vaccination among US adults aged 50 years and older

METHODS: This retrospective cohort study used administrative claims with linked electronic health record data from commercial or Medicare Advantage with Part D health plan members (November 2016 to March 2023). To be included, patients must have been aged 50 years or older

and received a prescription for RZV and to have had 12 months of continuous enrollment prior to the initial RZV prescription (index date), no evidence of prior RZV vaccination, and full demographic information. Comparisons between patients who did or did not receive RZV following a prescription were assessed descriptively and by multivariable logistic regression.

RESULTS: Of 8,715 patients with an RZV prescription, 71.0% received an RZV dose; 39.1% were vaccinated on the same day as the prescription (median time = 14 days [IQR = 0-266]). The majority of the prescriptions (67.0%) were written by primary care providers and during a wellness or preventive care visit (73.0%). Few patients (n = 8.5%) were coprescribed an additional vaccine, most commonly Td/Tdap (45.2%). A notably high proportion of patients aged 65-69 years were vaccinated following prescription (77.2%), and the proportions of those receiving RZV after being prescribed tended to be higher among those with more education and higher household income. Conversely, Black (62.9%) and Hispanic (61.5%) patients and those residing in the Northeast (54.2%) had numerically lower proportions of RZV vaccination following prescription. No differences in RZV vaccination were observed across areas by pharmacy density.

CONCLUSIONS: Prescriptions for RZV are seldom employed in clinical practice, but given the rate of observed uptake, they may be an effective tool to improve vaccination rates in adults aged 50 years and older. Future studies should consider investigating the factors hindering vaccination even when a documented recommendation has been given.

SPONSORSHIP: GSK (VEO-000693)

B7 Overall and age-specific estimation of the annual incremental health care resource use and costs of HIV compared with non HIV population

Cohen J[1], *Anupindi R*[2], *Doshi R*[2], *Yeaw J*[2], *Zhou X*[2], *Christoph M*[3], *Trom C*[3], *Chen M*[3], *Zachry W*[3]; *joshua.parsons.cohen@gmail.com*; *woodie.zachry@gilead.com*
[1]*Self-employed*; [2]IQVIA *Inc.*; [3]*Gilead Sciences, Inc.*

BACKGROUND: Past studies have shown that people living with HIV (PWH) had substantially higher all-cause annual costs than individuals without HIV. However, with the changing HIV therapy landscape and recent innovations improving life expectancy of PWH, there is a need for updated analyses regarding the incremental costs of HIV in the United States.

OBJECTIVE: To describe and compare cumulative annual health care resource use (HCRU) and direct health care costs among adult PWH and those without HIV, overall and by age groups in the United States.

METHODS: For this matched cohort study, adult PWH (year of first HIV diagnosis = index year) from January 2018 to September 2023 with evidence of antiretroviral treatment and continuous enrollment in medical and pharmacy benefits during each index year were identified from the IQVIA PharMetrics Plus adjudicated claims database. Patients without HIV (controls) were selected with exact matching (1:3 ratio) on age, sex, region, and health plan type. All-cause HRU and costs (computed from allowed amount) were assessed among patients aged 25-74 years and compared between the matched cohorts.

RESULTS: A total of 45,465 (2018), 47,482 (2019), 51,460 (2020), 49,869 (2021), 51,592 (2022), and 54,530 (2023) PWH and matched controls were identified. Mean all-cause annual and cumulative costs were significantly higher among PWH than controls for each age group and among older age groups ($38,308 vs $3,665 [18-24 years], $40,387 vs $3,602 [25-29 years], $42,604 vs $4,457 [30-39 years], $48,238 vs $6,304 [40-49 years], $52,636 vs $9,104 [50-59 years], $57,433 vs $12,245 [60-69 years], and $62,359 vs $15,254 [70-74 years]; all $P < 0.0001$). Mean annual pharmacy, inpatient, outpatient medical (including telehealth), and emergency department (ED) costs were significantly higher in PWH compared with controls (all $P < 0.0001$), with pharmacy costs accounting for majority of total costs among PWH. Similarly, the proportion of patients with at least 1 prescription claim, inpatient, outpatient (including telehealth), and ED visit was significantly higher in PWH compared with controls each year (all $P < 0.0001$).

CONCLUSIONS: This study of a large representative sample of commercially insured US adults found that PWH had significantly higher health care costs than those without HIV from 2018 to 2023 in all utilization categories, with a higher cost burden observed among older PWH. Higher HCRU among PWH highlights the need for HIV prevention and screening, in addition to rapid initiation of antiretroviral therapy and retention in care.

SPONSORSHIP: Gilead Sciences

B8 Higher real-world adherence and persistence with long-acting cabotegravir plus rilpivirine compared with oral antiretroviral therapy among people with HIV in the United States: The ABOVE study

Garris C[1], *Desai R*[2], *Chang R*[2], *Clear L*[3], *Chen Z*[4], *Liu D*[2], *DerSarkissian M*[4], *Teichner P*[1], *Overton E*[1]; *cindy.p.garris@viivhealthcare.com*
[1]*ViiV Healthcare*; [2]*Analysis Group, Inc*; [3]*Analysis Group*; [4]*Analysis Group, Inc.*

BACKGROUND: Long-acting cabotegravir plus rilpivirine (CAB+RPV LA) is the only complete long-acting regimen for treatment of virologically suppressed people with HIV (PWH) and may alleviate adherence challenges with daily oral therapy. Treatment adherence and persistence are critical for the long-term success of HIV treatment.

OBJECTIVE: To evaluate real-world adherence and persistence to CAB+RPV LA vs remaining on oral antiretroviral therapy (ART) regimens.

METHODS: ABOVE was a retrospective US cohort study using Symphony Health Solutions Integrated Dataverse administrative claims database from January 1, 2020, to August 31, 2023. PWH aged 12 years and older on stable guideline-recommended oral ART were categorized into those initiating CAB+RPV LA and those remaining on oral ART. Index date was defined as first injection between January 1, 2021, and March 1, 2023 (CAB+RPV LA cohort) or imputed for the oral ART cohort. PWH were required to have at least 12 months of follow-up after index. Standardized mortality ratio weights were generated based on propensity scores to balance baseline characteristics between cohorts. Adherence (percentage of PWH with proportion of days covered ≥0.9 over 12 months following index) and persistence (days from index to the earliest of treatment discontinuation or end of follow-up) to the index regimen were compared. A doubly robust logistic regression model was used to estimate the adjusted odds ratio and 95% CI for adherence.

RESULTS: A total of 442,091 PWH were identified during the study period. After applying eligibility criteria, 1,245 in the CAB+RPV LA cohort (mean age 47 years, 24% female) and 58,644 in the oral ART cohort (mean age 50 years, 23% female) were included. The majority of CAB+RPV LA dosing was every 2 months only (58%) or switched from monthly to every 2 months (29%). After standardized mortality ratio weighting, key baseline characteristics were balanced. In the weighted sample, a higher proportion of PWH in the CAB+RPV LA cohort was adherent (74% vs 30%, $P < 0.001$; median [IQR] proportion of days covered = 1.00 [0.89-1.00] vs 0.80 [0.51-0.92]) and had higher persistence (median [IQR] = 424 [201-537] vs 393 [174-431] days, $P < 0.001$) compared with the oral ART cohort. PWH in the CAB+RPV LA cohort had significantly higher odds of being adherent over 12 months compared with the oral ART cohort (adjusted odds ratio = 8.06, 95% CI = 6.62, 9.81, $P < 0.001$).

CONCLUSIONS: These data demonstrate that, among US PWH previously on stable oral ART, switching to long-acting ART resulted in significantly higher 12-month adherence and persistence over the follow-up compared with remaining on oral ART.

SPONSORSHIP: ViiV Healthcare

B9 The incentive structure in the United States must change to motivate action to End the HIV Epidemic

Riley P[1], *Pan A*[1], *Brogan A*[2], *Walko S*[2], *Hogue S*[1]; *phoenix.riley@aesara.com*; *andrew.p.brogan@viivhealthcare.com*
[1]AESARA; [2]*ViiV Healthcare*

BACKGROUND: Efforts to end the HIV epidemic reflect a global commitment to curb the transmission and acquisition of HIV. Ending the HIV Epidemic (EHE) in the United States launched in 2019 and is coordinated by the US Department of Health and Human Services. The goal of EHE is to reduce new HIV acquisitions by 75% by 2025 and 90% by 2030. EHE focuses on diagnosis, treatment, prevention, and outbreak response. Although progress has been made, significant challenges remain.

OBJECTIVE: To understand US payer perspectives of, and their role in, EHE.

METHODS: Qualitative in-depth interviews were conducted with 6 executive-level payers across the United States covering commercial, Medicare, and Medicaid lives.

RESULTS: Half of the payers interviewed were unaware of the EHE initiative. Once explained, all agreed meeting EHE goals will be challenging. All agreed they have a role in EHE, but disparities exist regarding the depth of payer involvement. EHE will require standardized strategies across stakeholders focusing on prevention, diagnosis, and lifetime engagement in care. Despite this, affordability issues persist, and utilization management was emphasized to control costs within their plans. Payers noted that the lack of incentives to make progress on EHE, such as HIV quality measures (QMs), hinder efforts toward achieving EHE goals; 83% of payers endorsed implementation of appropriate HIV QMs. QMs are crucial for tracking and benchmarking EHE progress, budget planning, and engaging stakeholders. QMs should focus on increasing

HIV testing, adherence, persistence, viral suppression, and preexposure prophylaxis utilization to achieve the desired impact. Payers interviewed recognized the importance of providers actioning HIV guidelines and screening recommendations. Payers acknowledged that there is a continued need for HIV education. However, direct payer outreach to members is limited because of opposition stemming from HIV-related stigma and prior experiences with member/employer pushback (eg, HPV vaccine advertising and education campaigns). Payers acknowledged the need to address social determinants of health and their impact on health outcomes and EHE.

CONCLUSIONS: HIV parallels other public health crises (eg, opioid epidemic, COVID-19), underscoring the importance of collective action and public health mandates to drive progress toward EHE. Achieving EHE goals by 2030 will require collaboration between manufacturers, payers, providers, and communities to better use existing resources, overcome stigma, and implement new incentives such as appropriate HIV QMs to stimulate action toward EHE goals.

SPONSORSHIP: ViiV Healthcare

B10 Treatment patterns and health care resource utilization of heavily treatment-experienced people with HIV

Gupta S[1], *Cappell K*[2], *Coaquira Castro J*[3], *Mezzio D*[4], *Bonafede M*[5], *Gruber J*[4], *Price K*[4], *Navadeh S*[4], *Sedgley R*[2], *Segal-Maurer S*[6]; *sgupta1@iu.edu*; *jeanpierre.coaquira@gilead.com*
[1]*Indiana University School of Medicine*; [2]*Real World Evidence, Veradigm*; [3]*Gilead Sciences Inc*; [4]*Gilead Sciences, Inc.*; [5]*Veradigm*; [6]*New York-Presbyterian Queens*

BACKGROUND: Heavily treatment-experienced people with HIV (HTE PWH) have limited antiretroviral therapy (ART) options owing to drug resistance and intolerance, potential loss of/changes to insurance, and/or concomitant medication interactions. HTE PWH have been characterized using a large US-based real-world database (Segal-Maurer S, et al. ACTHIV 2024).

OBJECTIVE: To present treatment patterns and health care resource utilization (HCRU) for HTE PWH.

METHODS: HIV treatment–experienced individuals were identified from the Veradigm Network electronic health record (EHR)–linked claims database during the study period, January 2015 to December 2022. HTE PWH met at least 1 of 4 specific HTE-defining criteria based on ART indication and exposure, evidence of viremia, and/or ART resistance. Other eligibility criteria were as follows: aged 18 years or older, HIV diagnosis, continuous claims enrollment, and EHR activity. A line of therapy was defined as the continuous period during which individuals had supply of a treatment regimen. Treatment patterns, HCRU, and cost findings were reported for the 6-month period after the index date (earliest date on which HTE criteria were met). The Mann-Whitney U-test (2-sided) was used to compare median health care costs between HTE PWH and a comparator group of treatment-experienced (but not HTE) PWH directly matched on age group, sex, race, ethnicity, geographic region, year of index date, and payor type.

RESULTS: Over a 6-month follow-up for 2,836 HTE PWH meeting HTE criteria, 902 (31.0%) experienced 1 regimen change, 442 (15.2%) experienced 2 regimen changes, 126 (4.3%) experienced 3 regimen changes, and 70 (2.4%) experienced 4 or more regimen changes. The proportion of HTE PWH on 1 pill/day decreased from line of therapy 1 to 5 (36.9% to 25.7%), whereas the proportion on 2 or 3+ pills/day increased or remained consistent (26.3% to 37.1% and 36.8% to 37.1%, respectively). For HCRU analysis, 2,710 HTE PWH (93%) were matched with non-HTE PWH. Median total costs for HTE PWH vs non-HTE PWH during the 6-month follow-up were as follows: all-cause health care (medical + pharmacy), $23,082 vs $18,440; all-cause medical, $3,823 vs $2,935; HIV-related health care, $17,799 vs $14,678; HIV-related medical, $1,308 vs $975 (all comparisons, $P<0.05$). The largest factors contributing to increased costs for HTE PWH were pharmacy use and outpatient services.

CONCLUSIONS: Across the HTE PWH treatment journey, regimens were observed as becoming more complicated over time. Compared with their non-HTE counterparts, meeting HTE PWH criteria was associated with a greater financial burden, including higher HIV-related and all-cause HCRU.

SPONSORSHIP: Gilead Sciences, Inc.

B14 Characteristics associated with diagnosis of 1 or more risk factors for severe respiratory syncytial virus disease by age 50 years among adults in the United States

Horn E[1], *Singer D*[1], *La E*[1], *Booth A*[2], *Saiontz-Martinez C*[2], *Berger A*[2]; *emily.k.horn@gsk.com*
[1]GSK; [2]*Evidera PPD*

BACKGROUND: Respiratory syncytial virus (RSV) causes substantial disease burden in older adults and adults with certain underlying medical conditions. The adjuvanted RSV prefusion F protein (RSVPreF3) vaccine is currently recommended by the US Centers for Disease Control and Prevention for use in adults aged 60 years and older using shared clinical decision-making and is under review by the

US Food and Drug Administration for use in adults aged 50-59 years at increased risk of lower respiratory tract disease caused by RSV.

OBJECTIVE: To explore characteristics associated with being diagnosed with at least 1 risk factor (RF) for severe RSV disease by age 50 years among US adults.

METHODS: A retrospective cross-sectional analysis of pooled National Health and Nutrition Examination Survey data from 4 survey waves (2011 to March 2020) was conducted. Survey weights were applied to extrapolate to all noninstitutionalized adults in the United States. Age at diagnosis of the following 10 relevant RFs was ascertained via self-report: chronic obstructive pulmonary disease, asthma, congestive heart failure, coronary heart disease, stroke, angina pectoris, myocardial infarction, diabetes, renal disease, and liver disease. Among respondents aged 50 years or older, a multivariable logistic regression model was developed to assess independent associations of selected respondent characteristics (ie, sex, race and ethnicity, poverty income ratio, routine place for health care, smoking status, and body mass index) with diagnosis of at least 1 RF for severe RSV disease by age 50 years.

RESULTS: Among the 58,999,617 adults aged 20 years and older with at least 1 RF for severe RSV disease, 60.0% were diagnosed by age 50 years. Hispanic (odds ratio [OR] = 1.6 [95% CI = 1.3-2.0]) and non-Hispanic Black (OR = 1.3 [1.1-1.7]) adults had significantly higher likelihoods of being diagnosed with at least 1 RF for severe RSV disease by age 50 years vs non-Hispanic White adults. Adults without a routine place for health care were significantly more likely to be diagnosed with at least 1 RF by age 50 years (OR = 2.1 [1.4-3.2]) vs adults whose routine place was doctors' offices. Smoking and obesity were also significantly associated with diagnosis of at least 1 RF by age 50 years.

CONCLUSIONS: After adjusting for respondent characteristics, adults from certain racial and ethnic minority groups and adults with poorer health care access were significantly more likely to be diagnosed with RFs for severe RSV disease by age 50 years. Understanding characteristics associated with diagnosis of at least 1 RF for severe RSV disease at an earlier age can help identify adults who could benefit most from RSV vaccination.

SPONSORSHIP: GSK (Study identifier: VEO-000686)

B15 Characteristics associated with respiratory syncytial virus vaccination among adults aged 60 years and older in the United States

La E[1], *McGuiness C*[2], *Singer D*[1], *Yasuda M*[2], *Chen C*[2]; *liz.m.la@gsk.com*
[1]GSK; [2]IQVIA

BACKGROUND: Older adults and adults with certain chronic conditions are at increased risk for severe respiratory syncytial virus (RSV) disease. In the United States, the Centers for Disease Control and Prevention's Advisory Committee on Immunization Practices recommends RSV vaccination among adults aged 60 years and older using shared clinical decision-making.

OBJECTIVE: To identify characteristics associated with likelihood of RSV vaccination among US adults aged 60 years and older.

METHODS: A retrospective database analysis was conducted using IQVIA's open-source pharmacy (LRx) and medical (Dx) claims data linked to consumer attribute data to evaluate RSV vaccination uptake from August 2023 to February 2024. The analysis included patients aged 60 years and older with at least 1 claim for any encounter from January 2023 to February 2024. A multivariable logistic regression model explored factors associated with RSV vaccination, including patient age, sex, race and ethnicity, annual household income, payer type, educational attainment, US census region, urbanicity, Area Deprivation Index, pharmacy density, Charlson Comorbidity Index, presence of potential risk factors for severe RSV disease, and receipt of at least 1 non-RSV vaccine from August 2023 to February 2024.

RESULTS: RSV vaccination uptake was 13.1% (n = 6,503,598/49,543,319) among adults aged 60 years and older who were included in the model. Odds of RSV vaccination were 23 times higher for those who received at least 1 non-RSV vaccine from August 2023 to February 2024 vs those who had not (odds ratio [OR] = 23.24 [95% CI = 23.17-23.32]). Adults in older age groups had higher odds of RSV vaccination vs adults aged 60-64 years, with ORs ranging from 1.14 (1.13-1.14) for those aged 65-69 years to 1.39 (1.39-1.40) for those aged 75-79 years. Higher odds were also observed among those with Medicare vs commercial insurance (OR = 2.44 [2.43-2.45]), as well as with higher annual household income and education. Compared with non-Hispanic White adults, odds of RSV vaccination were lower among Hispanic (OR = 0.70 [0.70-0.71]), Black (0.80 [0.79-0.80]), and Asian/other (OR = 0.86 [0.86-0.87]) adults. Presence of chronic pulmonary disease was the clinical risk factor with the highest observed OR vs those without (1.27 [1.27-1.28]).

CONCLUSIONS: Results highlight disparities in older adult RSV vaccination uptake in 2023-2024, with RSV vaccination varying by receipt of other vaccines, age, race and ethnicity, and other social determinants of health. Additional efforts are needed to ensure equitable access to RSV vaccines among disadvantaged groups and those who are at highest risk for severe RSV disease.

SPONSORSHIP: GSK (Study identifier: VEO-000828)

C00-D49 Neoplasms

(eg, breast cancer, lung cancer, melanoma, multiple myeloma)

C4 Total cost-of-care analysis of immune-oncology treatments for patients with unresectable hepatocellular carcinoma in the United States

Sun L[1], *Genestier V*[2], *Sah J*[1], *Anderson R*[1]; *li.sun3@astrazeneca.com*; *rye.anderson@astrazeneca.com*
[1]*AstraZeneca*; [2]*Amaris Consulting*

BACKGROUND: Hepatocellular carcinoma (HCC) is the third leading cause of cancer death worldwide; most patients present with unresectable HCC (uHCC). In the phase 3 IMbrave150 study (NCT03434379), atezolizumab+bevacizumab (A+B) improved overall and progression-free survival vs sorafenib in patients with uHCC. The phase 3 HIMALAYA study (NCT03298451) showed that tremelimumab+durvalumab (STRIDE) improved overall survival vs sorafenib in patients with uHCC. A+B and STRIDE are approved for first-line treatment of patients with uHCC in the United States (National Comprehensive Cancer Network Guidelines, Hepatocellular Carcinoma. Version 1.2023).

OBJECTIVE: To estimate the total economic burden of treating patients with newly diagnosed uHCC with STRIDE vs A+B from the Medicare (US) perspective.

METHODS: We developed a cost-of-care model that followed patients with uHCC eligible for first-line systemic therapy over the course of 1 year, considering direct medical costs. In accordance with American Society of Clinical Oncology guidelines (2020) for A+B, eligible patients were those with newly diagnosed uHCC and either Barcelona Clinic Liver Cancer stage C with Child-Pugh A or Barcelona Clinic Liver Cancer stage B with Child-Pugh A and ineligible for locoregional treatment. Direct medical costs included treatment acquisition and administration and health care resource use (ie, inpatient, outpatient, monitoring and endoscopy costs, and costs of treating grade ≥3 treatment-emergent adverse events [TEAEs]). Health care resource rates and respective costs were sourced from real-world evidence studies. Grade 3 and higher TEAEs and mean duration of treatment were derived from the HIMALAYA and IMbrave150 trials. Unit costs were collected from Medicare sources, valued in 2023 US dollars.

RESULTS: STRIDE treatment resulted in an annual average cost saving of $42,992 per patient vs A+B treatment from the Medicare perspective. The main driver of cost savings with STRIDE vs A+B was treatment acquisition (–$30,104, –21%). Lower costs with STRIDE vs A+B were also seen for TEAE management (–$5,738, –70%), monitoring (–$4,135, –83%), treatment administration (–$2,254, –68%), and endoscopy (–$760, –100%). The incremental cost offset for a 1-million-member Medicare plan (based on 70 treated patients per year) was $3,025,252 per year with STRIDE vs A+B.

CONCLUSIONS: Findings from this study indicate that STRIDE offers cost savings compared with A+B for uHCC treatment in the US health care system. These insights offer critical guidance for health care decision-makers aiming to optimize resource allocation. Further real-world evidence studies are needed to understand long-term survival benefits and associated treatment costs.

SPONSORSHIP: AstraZeneca

C5 Molecular testing in biliary tract cancer to inform treatment options: A targeted literature review

Dayyani F[1], *Bridgewater J*[2], *Valle J*[3], *Su W*[4], *Sabater J*[5], *Mettam S*[5], *Fan L*[4], *Bowditch S*[6], *Ahuja A*[7], *Sharma V*[7], *Paisley S*[8], *Smith M*[8], *Kim R*[9]; *fdayyani@hs.uci.edu*
[1]*UCI Health Chao Family Comprehensive Cancer Center, Department of Medical Oncology*; [2]*UCL Cancer Institute, Department of Medical Oncology*; [3]*Cholangiocarcinoma Foundation*; [4]*Jazz Pharmaceuticals*; [5]*Jazz Pharmaceuticals*; [6]*Jazz Pharmaceuticals, Health Economics and Outcomes Research Europe and International*; [7]*Lumanity*; [8]*Lumanity*; [9]*Moffitt Cancer Center*

BACKGROUND: Biliary tract cancer (BTC) is a rare and aggressive group of malignancies. Confirmation of molecular subtyping is essential for targeted therapy. Clinical guidelines make recommendations for the detection of molecular alterations, but standardized protocols across molecular subtypes, test modalities, and sampling methods are not yet established.

OBJECTIVE: To describe diagnostic methods for molecular testing in unresectable, locally advanced and metastatic BTC.

METHODS: Medline, Embase, and supplementary web searches, restricted to the last 10 years, were undertaken in July 2023. Studies were selected according to prespecified criteria.

RESULTS: Several studies focused on molecular profiling in BTC using different test modalities. Next-generation sequencing was identified as a means of concomitantly profiling multiple molecular targets, with tests such as immunohistochemistry (IHC) and fluorescence in situ hybridization (FISH) used to assess single gene alterations. Few studies compared, as the primary study aim, the diagnostic performance and concordance of different tests for the detection of molecular alterations. In studies profiling human epidermal growth factor receptor 2 status, concordance between IHC and FISH was 94.7% in one study and 41.7% and 91.7% for IHC2+ and IHC3+ cases, respectively, in another. In a study profiling fibroblast growth factor receptor 2 rearrangements, the sensitivity and specificity of FISH was 100% and 99%, respectively, and concordance between FISH and RNA sequencing was 92%. Comparisons of test performance across studies were difficult owing to confounding factors such as sample types, testing methods, types of molecular alteration, and BTC anatomical subtypes. The challenges of obtaining adequate tissue samples were raised in multiple studies. One study assessed the tissue sample failure rate as being 26.8%. Several studies compared tissue-based vs liquid-based samples. The detection rate of plasma-based circulating-tumor DNA (ctDNA) samples was similar compared with tissue biopsies but dependent on molecular alteration (IDH1 87% concordance, BRAF V600E 100%, fibroblast growth factor receptor 2 18%). In a study comparing bile-based ctDNA with tissue and plasma-based ctDNA with tissue, bile and tissue concordance was higher (80%) than that of plasma and tissue (42.9%).

CONCLUSIONS: Variation in testing modalities and sampling methods is an important consideration in interpreting the evidence base on molecular testing in BTC. With recent advances in targeted therapy, investigation into best practice is needed to support optimal treatment.

SPONSORSHIP: Jazz Pharmaceuticals

C6 Real-world cost of care for commercially insured and Medicare Advantage patients with metastatic pancreatic ductal adenocarcinoma treated with first-line FOLFIRINOX or gemcitabine + nab-paclitaxel

Dennen S[1], *Masek M*[1], *Cockrum P*[2], *Noshad S*[1], *Nagelhout E*[1], *Paluri R*[3]; *syvartdennen@gmail.com*; *paul.cockrum@ipsen.com*
[1]*Genesis Research Group*; [2]*Ipsen Biopharmaceuticals, Inc.*; [3]*Atrium Health Wake Forest Baptist*

BACKGROUND: FOLFIRINOX (FFX) and gemcitabine + nab-paclitaxel (GnP) have traditionally been standard first-line (1L) treatments for metastatic pancreatic ductal adenocarcinoma (mPDAC). Each regimen is associated with distinct toxic effects. FFX may be modified (mFFX) with dose reductions to increase tolerability. Although the cost of the component drugs of FFX is less than those of GnP, previous research has shown the total cost of care (TCoC) of the 2 therapies is similar owing to high supportive care costs associated with 1L FFX. Further evidence on costs by payer type, including distinguishing FFX from mFFX, is needed to inform decision-making.

OBJECTIVE: To measure TCoC and key cost components during 1L therapy for commercially insured and Medicare Advantage patients treated with 1L FFX, mFFX, or GnP.

METHODS: This retrospective observational study used Optum Market Clarity claims data. Adults diagnosed with mPDAC between January 1, 2015, and May 31, 2023, who initiated treatment with 1L FFX, mFFX, or GnP –14 to +90 days from metastatic diagnosis were included. Patients censored during 1L therapy were excluded. Costs were measured until death, initiation of a new line, or 28 days after line end, whichever was earliest. FFX was defined as receiving both 5FU bolus and infusion in the first cycle, whereas mFFX was defined as receiving 5FU infusion only.

RESULTS: Among 1L commercially insured patients, 536 were treated with FFX, 673 with mFFX, and 494 with GnP. Average TCoC for FFX, mFFX, and GnP was $137,813, $120,109, and $133,042, respectively. FFX and mFFX had a lower average cost for component drugs than GnP ($10,916, $7,653, and $60,466, respectively). FFX and mFFX had a higher average granulocyte colony-stimulating factor (G-CSF) cost compared with GnP ($38,074, $27,823, and $4,029, respectively). Among 1L Medicare Advantage patients, 201 were treated with FFX, 317 with mFFX, and 894 with GnP. Average TCoC for FFX, mFFX, and GnP was $110,788, $98,667, and $110,211, respectively. FFX and mFFX had a lower average cost for component drugs vs GnP ($8,028, $6,016, and $49,263, respectively). FFX and mFFX had a higher average G-CSF cost compared with GnP ($30,535, $24,596, and $2,412, respectively).

CONCLUSIONS: In a real-world cohort of patients with 1L mPDAC, FFX, mFFX, and GnP all had similar TCoC. Relative to 1L GnP, TCoC was slightly higher for FFX patients and lower for mFFX. The TCoC of FFX and mFFX were driven by G-CSF use, whereas the TCoC of GnP was driven by the cost of the regimen. Further evaluation of the relationship between price, clinical outcomes, and patient quality of life is warranted.

SPONSORSHIP: Ipsen Biopharmaceuticals, Inc.

C7 Prevalence and cost of chemotherapy-induced myelosuppression in metastatic colorectal cancer: A claims data analysis

Barzi A[1], *Brouwer E*[2], *Ostojic H*[3], *Marian M*[4], *Ramsey S*[2], *Keating K*[4]; *afsaneh.barzi@myaccesshope.org*
[1]*Access Hope*; [2]*Curta*; [3]*Bayer Pharmaceuticals*; [4]*Bayer*

BACKGROUND: Chemotherapy regimens for metastatic colorectal cancer (mCRC) carry an associated risk of myelosuppression and related adverse events.

OBJECTIVE: To characterize the occurrence and cost of myelosuppression in patients with mCRC receiving chemotherapy treatment, by line of therapy (LOT) and chemotherapy treatment regimen.

METHODS: Using Optum claims data, this retrospective cohort study identified adult patients with a primary diagnosis of mCRC and at least 2 codes for treatments with chemotherapy from July 2016 through June 2022. Patients were grouped by regimen received (single agent, FOLFOX, FOLFIRI, FOLFOXIRI, CAPOX, and trifluridine/tipiracil). Myelosuppression was defined as at least 1 claim of the following: diagnosis of neutropenia or febrile neutropenia, blood transfusion, or treatment (ie, nonprophylactic use) with G-CSF. Differences in characteristics between patients with and without myelosuppression were determined using a multivariate regression model adjusting for age, sex, race, geographic region, and insurance type.

RESULTS: Of 6,929 patients starting 1L chemotherapy, the average age was 64 years (SD = 13) and 44% of patients were female; 2,169 patients continued to 2L and 742 to 3L+. Myelosuppression occurred in 45% of all patients, including neutropenia (30% of all patients), thrombocytopenia (21%), anemia (9%), and febrile neutropenia (3%). The percentage of patients with any myelosuppression increased across LOTs (1L: 45%; 2L: 60%; 3L+: 68%). Although febrile neutropenia was the least prevalent, neutropenia rates were higher and varied among regimens (24%-58%); they were highest for patients receiving FOLFOXIRI, followed by trifluridine/tipiracil and FOLFOX. Patients who experienced myelosuppression spent significantly more time on treatment in 1L (7.4 vs 5.9 months, $P < 0.001$) and in 2L (4.8 vs 3.8 months, $P < 0.001$). Patients who experienced myelosuppression also had significantly higher health care costs (adjusted difference 1L: $74,811; 2L: $44,185; 3L+: $127,789, $P < 0.01$).

CONCLUSIONS: The prevalence of myelosuppression in patients with chemotherapy-treated mCRC was high across all LOTs, greater in later LOTs, and varied little by treatment regimen. Although the incidence of febrile neutropenia was low, any neutropenia was associated with significantly longer time on treatment and higher health care costs, likely because of myelosuppression treatment and delays in chemotherapy administration. Treatment alternatives with lower risks of neutropenia and equivalent outcomes should be considered in later LOTs for mCRC to address myelosuppression and its associated cost burden.

SPONSORSHIP: Bayer

C8 Rates of hematologic adverse events and excess cost among Medicare beneficiaries receiving first-line therapies for metastatic pancreatic ductal adenocarcinoma

Bhatt P[1], *Dieguez G*[1], *Cockrum P*[2], *Hirsch J*[1], *Kim G*[3]; *prachi.bhatt@milliman.com*; *paul.cockrum@ipsen.com*
[1]*Milliman, Inc.*; [2]*Ipsen Biopharmaceuticals, Inc.*; [3]*George Washington University*

BACKGROUND: Metastatic pancreatic ductal adenocarcinoma (mPDAC) treatments are associated with hematologic adverse events (hAEs) and additional costs.

OBJECTIVE: To examine real-world hAE incidence and costs among Medicare fee-for-service beneficiaries with mPDAC receiving first-line FOLFIRINOX (FFX) or gemcitabine/ nab-paclitaxel (Abraxane) (gem/abrax).

METHODS: We identified patients with mPDAC in the 2018-2022 Medicare Parts A, B, and D 100% research identifiable files. Diagnosis required at least 2 malignant neoplasm of pancreatic duct diagnosis codes AND at least 1 metastases code. Patients were assigned cohorts based on drugs within a 2-day period of first chemo infusion cycle: (1) FFX with all 4 components (oxaliplatin, irinotecan, leucovorin, 5-FU bolus and infusion), (2) FFX with no 5-FU bolus, and (3) gem/abrax. Total cost of care (TCOC) and incidence rates of clinically relevant hAEs were determined for each cohort. Patients without hAEs were assigned a shadow hAE date. Excess costs for hAEs were determined by comparing per-administration costs for patients with and without an hAE.

RESULTS: We included 25,700 patients: 13% received FFX with all 4 components (TCOC $7,142), 19% received FFX with no 5-FU bolus (TCOC $6,595), and 67% received gem/abrax (TCOC $6,505). Compared with the FFX with all 4 components cohort, patients receiving FFX with no 5-FU bolus had lower rates of neutropenia (–18%), febrile neutropenia (–30%), anemia (–11%), and thrombocytopenia (–15%). Patients receiving gem/abrax had higher rates of anemia (27%) but lower rates of neutropenia (–40%), febrile neutropenia (–37%), and thrombocytopenia (–8%). The per-administration excess cost associated with hAEs was $4,563 for neutropenia, $5,699 for anemia, and $5,495

for thrombocytopenia. Patients developing febrile neutropenia experienced $8,551 in excess costs, an additional $3,987 in excess of neutropenia costs. Mean per-administration excess cost associated with any of the hAEs studied was $5,993, largely driven by inpatient admissions, which accounted for 55% ($3,296) of excess costs.

CONCLUSIONS: Among patients receiving 1L therapies, hAEs occur more often in patients receiving FFX with all 4 components, except anemia, which occurred more often in those receiving gem/abrax. Patients receiving FFX with no 5-FU bolus had consistently lower rates of hAEs than patients receiving all 4 components of FFX. The occurrence of an hAE led to excess costs of $5,993. The largest cost contributor was inpatient admissions, indicating serious hAEs requiring hospitalization.

SPONSORSHIP: Ipsen Biopharmaceuticals

C9 Rates of nonhematologic adverse events and excess cost among Medicare beneficiaries receiving first-line therapies for metastatic pancreatic ductal adenocarcinoma

Bhatt P[1], Dieguez G[1], Cockrum P[2], Hirsch J[1], Kim G[3]; prachi.bhatt@milliman.com; paul.cockrum@ipsen.com
[1]Milliman, Inc.; [2]Ipsen Biopharmaceuticals, Inc.; [3]George Washington University

BACKGROUND: Metastatic pancreatic ductal adenocarcinoma (mPDAC) treatments are associated with nonhematologic adverse events (nhAEs) and additional costs.

OBJECTIVE: To examine real-world nhAE incidence and costs among Medicare fee-for-service (FFS) beneficiaries with mPDAC receiving first-line FOLFIRINOX (FFX) or gemcitabine/nab-paclitaxel (Abraxane) (gem/abrax).

METHODS: We identified patients with mPDAC in the 2018-2022 Medicare Parts A, B, and D 100% research identifiable data files. Diagnosis required at least 2 malignant neoplasm of pancreatic duct diagnosis codes AND at least 1 metastases code. Patients were assigned cohorts based on drugs within a 2-day period of their first chemo infusion cycle: (1) FFX with all 4 component drugs (oxaliplatin, irinotecan, leucovorin, 5-FU bolus and infusion), (2) FFX with no 5-FU bolus, but with 5-FU infusion, and (3) gem/abrax. Incidence rates of clinically relevant nhAEs for each cohort were determined. Patients without nhAEs were assigned a shadow nhAE date. Excess costs for patients experiencing an nhAE were determined by comparing the per-administration costs for patients with and without an nhAE. Total cost of care (TCOC) represents total allowed costs (payer costs plus patient cost sharing).

RESULTS: We included 25,700 patients: 13% received FFX with all 4 components (TCOC $7,142), 19% received FFX with no 5-FU bolus (TCOC $6,595), and 67% received gem/abrax (TCOC $6,505). Using the FFX with all 4 components cohort as comparator, patients receiving FFX with no 5-FU bolus had lower rates of diarrhea (–1%), abdominal pain (–8%), nausea/vomiting (–6%), and fatigue (–9%) but higher rates of neuropathy (7%). Patients receiving gem/abrax had lower rates of diarrhea (–41%), abdominal pain (–2%), and nausea/vomiting (–19%) but higher rates of fatigue (17%) and neuropathy (15%). The per-administration excess cost associated with nhAEs was $4,499 for diarrhea, $4,176 for abdominal pain, and $3,443 for nausea/vomiting, $4,005 for fatigue, and $2,999 for neuropathy. Mean per-administration excess cost associated with any of the nhAEs studied was $3,665, largely driven by inpatient admissions, which accounted for 61% ($2,236) of excess costs.

CONCLUSIONS: Among patients receiving 1L therapies, nhAEs occur more often in patients receiving FFX with all 4 components, except fatigue and neuropathy, which occurred more often in those receiving gem/abrax. The occurrence of an nhAE led to excess costs of $3,665. The largest cost contributor was inpatient admissions, indicating serious nhAEs requiring hospitalization.

SPONSORSHIP: Ipsen Biopharmaceuticals

C10 Real-world costs associated with upfront biomarker testing in advanced non–small cell lung cancer and metastatic colorectal cancer

Wong W, To T; wong.william@gene.com
Genentech

BACKGROUND: Although guidelines recommend biomarker testing to optimize outcomes (via targeted therapy) for patients with advanced non–small cell lung cancer (aNSCLC) and metastatic colorectal cancer (mCRC), state policymakers considering laws to facilitate testing have concerns about the cost. Additional data are needed to assess the cost of testing relative to the total cost of care (TCOC) and understand the value of genomic testing in these tumors.

OBJECTIVE: To describe costs of biomarker testing in the context of the TCOC for a patient with aNSCLC or mCRC.

METHODS: A retrospective analysis (2018-2022) of medical and pharmacy claims using the IQVIA PharMetrics Plus database was conducted to assess upfront biomarker testing (≤90 days of index date [the aNSCLC or mCRC diagnosis]), receipt of targeted therapy, biomarker test costs, and total costs. Patients' biomarker test status was categorized as having claims for multigene panel tests (MGPT), single gene test only (SGT), or no biomarker test. Biomarker testing costs

were estimated as the allowed amounts associated with the test claim. The TCOC was the sum of allowed amounts in all claims from the index date until the end of follow-up. Costs were analyzed descriptively as means/medians and as a proportion of the total cost of care.

RESULTS: The study included 24,828 patients, with 11% receiving upfront MGPT, 67% receiving upfront SGT only, and 22% having no evidence of upfront testing (including 13% with no testing at any time). Among those with upfront testing, more patients with upfront MGPT received targeted therapy vs those with upfront SGT only (12.9% vs 8.0%, $P < 0.01$). The mean cost of all upfront biomarker tests for those with upfront MGPT, including the cost of some SGTs, was $4,400 (SD = $3,360), whereas the mean cost for those with SGT only was $1,318 (SD = $2,168). The mean TCOC per patient across both cohorts was $248,561 (SD = $278,282) and varied by follow-up time (<1 year of follow-up: $149,553 [SD = $138,421); >1 year of follow-up: $323,953 [SD = $329,659]). The cost of upfront biomarker testing as a proportion of the TCOC was 2.8% for those with upfront MGPT (median follow-up: 0.95 years) and 0.9% for those with upfront SGT only (median follow-up: 1.10 years). Among those with upfront MGPT, the cost of genomic testing was 3.8% for those patients with less than 1 year of follow-up and 1.7% for those patients with more than 1 year of follow-up.

CONCLUSIONS: Upfront MGPT increased identification of patients who are eligible for targeted therapy while representing a small portion of the TCOC. Further efforts are needed to facilitate access to comprehensive testing, such as state laws requiring coverage consistent with guidelines.

SPONSORSHIP: Genentech, Inc.

C17 A unique approach to precision medicine: Racial disparities in non–small cell lung cancer biomarker testing

Safran J, Londono D, Robbins R, Kane G, Mendes M; jared.safran@gmail.com PurpleLab

BACKGROUND: Health care is evolving into precision medicine or what the US Food and Drug Administration (FDA) refers to as personalized medicine. This targeted approach is critical for organizations like pharmaceutical, life science, health plans, and providers to have the tools needed to make safe decisions for their patient populations. Precision medicine can assist clinicians, thereby reducing their need for educated guesses, improving patient outcomes, and helping them make more informed decisions regarding available treatment options. According to the American Lung Association, people of color with lung cancer face worse outcomes compared with their White American counterparts.

OBJECTIVE: To determine whether a racial disparity exists in biomarker testing for patients with non–small cell lung cancer (NSCLC).

METHODS: PurpleLab created a crosswalk (mapping) that leveraged laboratory testing codes from Quest Diagnostics and Labcorp and mapped them to procedure (Current Procedural Terminology [CPT]4) codes. The clinical team reviewed biomarkers listed in the FDA biomarker drug labeling table and linked them to the appropriate medications that biomarkers target based on diagnosis (*International Classification of Diseases [ICD], Tenth Revision, Clinical Modification*) and CPT codes. PurpleLab reviewed CPT claims for the respective biomarkers for NSCLC including ALK, EGFR, BRAF, KRAS G12C, ROS1, MET, NTRK, RET, ERBB2 (HER2), and PD-L1 from January 2022 through April 2024 for adults aged 18 years and older. Biomarker testing claims from the PurpleLab database were compared with 2020 US Cancer Statistics (USCS) Data Visualizations incidence rate per 100,000 people for lung and bronchus cancers.

RESULTS: According to the PurpleLab claims database, 1,913,773 individuals had an associated CPT claim for biomarker testing for NSCLC: 84.5% of the patients were White, 11.4% were Black or African American, 3.8% were Asian, and 0.3% listed their race as Other. Per US Cancer Statistics, the rate of new cancers by race per 100,000 people for lung and bronchus cancer were 51.4 White, non-Hispanic; 47.7 Black, non-Hispanic; and 28.2 Asian and Pacific Islander, non-Hispanic.

CONCLUSIONS: Given that the incidence rates for new lung and bronchus cancers among White and Black individuals are similar (51.4% and 47.7%, respectively), the biomarker test is disproportionately given to White patients, and thus, a potential testing disparity exists. Currently no specific diagnosis code for NSCLC exists. PurpleLab leveraged CPT, ICD, and Pharmacy codes (National Drug Code, Healthcare Common Procedure Coding System), which when combined serve as a proxy for precision medicine for NSCLC. Further research is needed to test additional methods and strategies of communication to non-White patients to increase biomarker testing.

SPONSORSHIP: PurpleLab

C18 US health care resource utilization and costs in non–small cell lung cancer after initiating first-line maintenance therapy following platinum-based chemotherapy plus pembrolizumab

Solem C[1], *Burns S*[2], *Kalayjian A*[3], *Gouldin W*[3], *Liao A*[1], *Shah M*[4]; *caitlyn.t.solem@gsk.com*
[1]GSK; [2]GSK; [3]GSK; [4]GSK

BACKGROUND: Despite improved treatments for advanced/metastatic non–small cell lung cancer (a/mNSCLC), outcomes for patients with a/mNSCLC, without driver alterations, remain poor.

OBJECTIVE: To describe demographic and clinical characteristics, health care resource utilization (HCRU), and costs among US patients with a/mNSCLC who initiated first-line (1L) maintenance (1LM) pembrolizumab (pembro) or 1LM pembro-pemetrexed.

METHODS: In this retrospective cohort study using the US Merative MarketScan commercial database, eligible patients were aged 18 years or older at index (defined as 1LM initiation date); had at least 2 claims with an NSCLC diagnosis (*International Classification of Diseases, Tenth Revision, Clinical Modification* code: C34x; January 1, 2017, to October 31, 2023); and initiated 1LM pembro±pemetrexed after 4-6 cycles of platinum-based chemotherapy–pembro±pemetrexed (a proxy for achieving stable disease or response). Because staging data were not available, treatment with platinum-based chemotherapy–pembro, which was only approved for a/mNSCLC at the time of this study, was used to identify patients with a/mNSCLC. Patients were excluded if they did not have continuous medical and pharmacy coverage for at least 6 months before and at least 1 month after index, if they received lung cancer surgery, or if they used durvalumab or targeted therapies (January 1, 2017, to index). HCRU was measured across medications and settings (emergency department, inpatient, outpatient, skilled nursing facility, other) after 1LM initiation and summarized as total visits per patient per month (PPPM). Costs were totaled and described PPPM.

RESULTS: Among 1,111 eligible patients (mean age, 62.6 years; 48.4% female), 44.1% received 1LM pembro, 55.9% received 1LM pembro-pemetrexed, 28.4% had brain metastases (BM) at baseline, and 36.4% had BM at study end. Patients had a median of 9.0 months (IQR = 4.9-17.0) of follow-up. A greater percentage of patients who received 1LM pembro-pemetrexed had earlier index dates, were female, and had commercial (vs Medicare [Advantage]) insurance (vs 1LM pembro). All patients had at least 1 outpatient visit during follow-up (mean, 3.6 visits PPPM). Mean total cost was $27,652 PPPM across all medications and settings, with medications driving 70% of the total cost. Mean total all-cause cost was $8,361 PPPM higher for patients treated with 1LM pembro-pemetrexed (vs 1LM pembro), primarily driven by medication and outpatient costs.

CONCLUSIONS: Patients with a/mNSCLC without driver alterations, which included 36.4% who had BM, incurred substantial HCRU and costs, largely because of medication. Patients who received 1LM pembro-pemetrexed incurred numerically greater costs PPPM (vs 1LM pembro), primarily driven by medication and outpatient cost differences. These results show a continued economic burden and unmet need for patients with a/mNSCLC.

SPONSORSHIP: GSK

C19 Using a Delphi method to develop an operational definition of first-line maintenance therapy for patients with advanced/metastatic non–small cell lung cancer

Tapan U[1], *Shah M*[2], *Bell K*[3], *Liao A*[3], *Yang H*[4], *Lee S*[5], *Korrapati L*[5], *Reinmuth N*[6]; *Umit.Tapan@bmc.org*; *manasee.v.shah@gsk.com*
[1]*Section of Hematology & Medical Oncology, Boston University Chobanian & Avedisian School of Medicine, and Boston Medical Center*; [2]GSK; [3]GSK; [4]*Analysis Group, Inc.*; [5]*Analysis Group*; [6]*Thoracic Oncology Department, Asklepios Lung Clinics Munich-Gauting*

BACKGROUND: For patients with advanced/metastatic non–small cell lung cancer (a/mNSCLC), without actionable genomic alterations, first-line (1L) maintenance therapy (1LMT) recommendations vary by geographic region, 1L induction therapy, and clinical and tumor characteristics. Furthermore, agents used for 1LMT are similar to those used for subsequent lines of therapy. With these challenges, it may be difficult to reliably identify patients with a/mNSCLC who are receiving 1LMT in real-world datasets (eg, electronic medical record and claims data).

OBJECTIVE: To develop an operational definition for 1LMT in real-world datasets for patients with a/mNSCLC without actionable genomic alterations using a Delphi method.

METHODS: Consensus statements related to an operational definition for 1LMT were developed based on a literature review and input from 2 clinical experts (U.T., N.R.). Using an iterative Delphi method, a survey was then administered over 3 rounds (June 30, 2023, to January 9, 2024) to oncologists actively treating patients with a/mNSCLC in France, Germany, the United Kingdom, and the United States. The survey consisted of consensus-seeking statements with responses (5-point Likert scale of agreement, multiple choice [select one], and free text). Consensus was defined

as achieving similar response from at least 75% of panel members. In each round, the survey was updated based on aggregated results from the prior round.

RESULTS: Participating oncologists (rounds 1 [n=15], 2 [n=13], and 3 [n=12]) reached consensus that a therapy/regimen (eligible as 1LMT) that was initiated 60 days or less after completing 4-6 rounds of 1L induction therapy (including continuation maintenance) should be considered 1LMT; a therapy/regimen initiated more than 120 days after 1L induction therapy completion should be considered second-line (2L) therapy. There was no consensus on how to classify therapy that was initiated 61-120 days after 1L induction therapy completion. Consensus was reached that if a patient initiated a chemotherapy agent different from that used in 1L induction therapy, it should be considered 2L and not 1LMT, regardless of the initiation time frame; however, if pemetrexed was used, there was no consensus on whether it should be considered switch 1LMT or 2L. These definitions held true regardless of histology.

CONCLUSIONS: Oncologists treating patients with a/mNSCLC agreed on 1LMT and 2L therapy definitions if initiated 60 days or less or more than 120 days after 1L induction therapy completion, respectively. When using real-world datasets, these operational definitions may be valuable to discern 1LMT from 2L therapy, particularly when comprehensive clinical data or decision rationale are lacking.

SPONSORSHIP: GSK

C20 Modeled costs of adverse event management in patients with treatment-naive epidermal growth factor receptor non–small cell lung cancer with exon 19 deletion or exon 21 L858R mutations treated with amivantamab plus lazertinib or osimertinib plus chemotherapy or osimertinib

Vadagam P[1], *Lin I*[1], *Musci R*[2], *Wang L*[2], *Zou D*[2]; *pvadagam@its.jnj.com*
[1]*Janssen Scientific Affairs, LLC, a Johnson & Johnson company*; [2]*Evidera*

BACKGROUND: Adverse event (AE) or side-effect management is one component that drives treatment decision-making in patients with locally advanced or metastatic non–small cell lung cancer (a/m NSCLC). New 1L therapies for a/m NSCLC with epidermal growth factor receptor (EGFR) exon 19 deletion or exon 21 L858R substitutions have demonstrated efficacy in recent phase 3 trials. However, the therapeutic value of these regimens may vary when considering AE management costs. This study evaluated AE costs using phase 3 randomized controlled trials (RCTs) for 2 new 1L regimens and the current standard of care, osimertinib (osi).

OBJECTIVE: To estimate grade 3 and 4 AE costs of 1L amivantamab plus lazertinib (ami+laz), osimertinib plus platinum-based chemotherapy (osi+chemo), or osi in common EGFR mutation+ a/m NSCLC from the US Commercial and Medicare Advantage payer perspectives.

METHODS: Grade 3 and 4 AEs identified from phase 3 RCTs, occurring in at least 5% of patients in ami+laz (MARIPOSA), osi+chemo (FLAURA2), or osi (MARIPOSA), were included. A specialist visit cost was applied to aspartate transaminase/alanine transaminase increase, fatigue, hypermagnesemia, hypoalbuminemia, paronychia, proteinuria, rash, and stomatitis; inpatient costs were applied to anemia, dermatitis acneiform, diarrhea, dyspnea, hypertension, hypokalemia, hyponatremia, infusion-related reaction, interstitial lung disease, lymphopenia, neutropenia, thrombocytopenia, and venous thromboembolism (VTE). Specialist visit costs were from InHealth and Centers for Medicare & Medicaid Services (CMS) 2024 Physician Fee schedule for commercial and Medicare, respectively. Inpatient costs were from Healthcare Cost and Utilization Project 2020 data inflated to 2024 USD (commercial) and CMS 2024 Acute Inpatient Prospective Payment System (Medicare). AEs were applied as a one-off cost at the time of treatment initiation.

RESULTS: The estimated total AE management cost per patient per treatment course was $4,847 for ami+laz, $7,282 for osi+chemo, and $2,761 for osi for commercial patients and $3,101 for ami+laz, $4,024 for osi+chemo, and $1,677 for osi for Medicare patients. The most frequent AEs were rash (27%), venous thromboembolism (11%), and paronychia (11%) with ami+laz; anemia (20%), neutropenia (13%), and thrombocytopenia (7%) with osi+chemo; and hyponatremia (6%), hypermagnesemia (5%), and VTE and dyspnea (4% each) with osi.

CONCLUSIONS: Although newer 1L regimens may offer better overall efficacy, the cost of AE management was estimated to be lower in patients treated with 1L ami+laz compared with osi+chemo. The implications of differing AE profiles and the impact on total cost of care among regimens is a relevant consideration for assessment of therapeutic value by US health care stakeholders.

SPONSORSHIP: Janssen Scientific Affairs, LLC, a Johnson & Johnson company

C21 A retrospective real-world evaluation of docetaxel-based treatments after standard of care for patients with nonsquamous advanced non–small cell lung cancer

Apple J[1], *Zou L*[1], *Damineni S*[1], *Welch E*[2], *Shah R*[3], *Shah A*[1], *Wu C*[3], *Wang E*[3]; *jon.apple@astrazeneca.com*
[1]*Astrazeneca*; [2]*Panalgo*; [3]*Daiichi Sankyo, Inc*

BACKGROUND: The standard-of-care (SOC) treatment for patients with nonsquamous (NSQ) advanced non–small cell lung cancer (aNSCLC) is immunotherapy (IO), typically given either sequentially or as a combination with platinum-based chemotherapy (PBC). If the patient harbors a known actionable genomic alteration, SOC is typically targeted therapy followed by PBC. Upon failure of SOC, there is a paucity of effective treatment options. Prior real-world studies have shown that docetaxel, either alone or in combination with ramucirumab, is the most frequently used regimen after failing SOC treatment.

OBJECTIVE: To describe the real-world effectiveness of docetaxel with ramucirumab and docetaxel monotherapy after prior treatment with SOC.

METHODS: Adult patients diagnosed with NSQ stage IIIB/C or IV NSCLC were identified retrospectively from the Flatiron electronic medical record US database from January 1, 2018, to May 31, 2023. Patients were included if they received subsequent docetaxel with or without ramucirumab after prior SOC treatments (IO and PBC or targeted therapy and PBC). Patients were followed from the initiation date of docetaxel with ramucirumab or docetaxel monotherapy (index date) until the earliest of death, lost to follow-up, or end of data availability (November 30, 2023). Median real-world overall survival (rwOS), real-world time to treatment discontinuation (rwTTD), and real-world time to next treatment (rwTTNT) were reported using Kaplan-Meier analysis.

RESULTS: Of the 1,938 patients with NSQ aNSCLC who received SOC therapy, 519 (26.8%) received subsequent docetaxel-based treatment. Of the docetaxel-based regimens, 359 (69.2%) received docetaxel with ramucirumab and 160 (30.8%) received docetaxel monotherapy. For docetaxel with ramucirumab and docetaxel monotherapy, respectively, the median age was 66 and 67 years, 62.1% and 61.3% were White, 48.8% and 54.4% were female, 85.5% and 87.5% had a history of smoking, 88.0% and 84.4% were treated in community centers, and 64.9% and 65.6% had an Eastern Cooperative Oncology Group performance status of less than or equal to 1. The median (95% CI) rwOS, rwTTD, and rwTTNT for docetaxel with ramucirumab was 6.41 months (5.39-7.52), 2.76 months (2.30-3.25), and 5.03 months (4.47-5.49), respectively. The median (95% CI) rwOS, rwTTD, and rwTTNT for docetaxel monotherapy was 5.39 months (4.30-6.80), 1.61 months (1.38-2.23), and 3.91 months (3.42-4.60), respectively.

CONCLUSIONS: Treatment with docetaxel with ramucirumab or docetaxel monotherapy following SOC showed limited clinical benefit in this real-world dataset based on rwOS, rwTTD, and rwTTNT. More effective treatment options are urgently needed to improve real-world survival outcomes in this challenging post-SOC setting.

SPONSORSHIP: AstraZeneca and Daiichi Sankyo

C22 Classification of risk factors associated with reduced real-world clinical outcomes in patients with common epidermal growth factor receptor–mutated non–small cell lung cancer treated with frontline standard of care

Spira A[1], *Ran T*[2], *Lin I*[2], *Chen C*[2], *He A*[2], *Belani N*[3], *Du S*[2]; *samantha.fanning@lumanity.com*
[1]*Virginia Cancer Specialists*; [2]*Janssen Scientific Affairs, LLC, a Johnson & Johnson company*; [3]*Temple University Health System*

BACKGROUND: Osimertinib (osi), a third-generation tyrosine kinase inhibitor, is the current frontline (1L) standard of care (SOC) for common epidermal growth factor receptor–mutated (cEGFRm) non–small cell lung cancer (NSCLC), but limited evidence exists regarding mutations and patient types associated with reduced real-world clinical outcomes.

OBJECTIVE: To describe overall survival (OS), time to next treatment (TTNT), and time to discontinuation (TTD) among patients with advanced/metastatic (a/m) cEGFRm NSCLC receiving osi monotherapy as 1L therapy and identify patient groups at risk for reduced outcomes.

METHODS: This real-world retrospective cohort study used patient clinical and genomic data from the Flatiron Clinico-Genomic Database. Univariate Cox proportional hazards models identified risk factors associated with reduced OS, TTNT, and TTD. Outcomes were described overall and by risk factor via Kaplan-Meier.

RESULTS: Of 703 patients identified, 67% were female, 53% were White, 44% had a history of smoking, and 70% were confirmed to have an Eastern Cooperative Oncology Group (ECOG) performance status of less than or equal to 1. Mean age, follow-up, and duration of therapy were 69 years, 19.6 months, and 14.9 months. Overall, median OS, TTNT, and TTD were 29.8, 17.6, and 15.9 months. For context, the FLAURA trial showed 38.6, 25.5, and 20.8 months. Risk factors associated with significantly reduced OS were baseline liver metastases (mets; 18.8 months for mets vs 30.0 months for no mets, $P<0.0001$), bone mets (23.5 vs 36.8 months, $P<0.0001$), central

nervous system mets (21.6 vs 32.3 months, $P=0.0005$), ECOG greater than or equal to 2 (19.3 vs 31.2 months, $P<0.0001$), and mutations of EGFR L858R (24.2 vs 35.8 months, $P<0.0001$), TP53 (24.6 vs 35.8 months, $P=0.013$), and CDK4 (20.2 vs 29.7 months, $P=0.042$). Descriptive OS were reported for mutations of PIK3CA (16.5 vs 29.7 months, $P=0.055$), RB1 (21.5 vs 29.8 months, $P=0.12$), and EGFR amplification (23.5 vs 30.5 months, $P=0.07$). Risk factors associated with significantly reduced TTNT were liver mets (11.5 vs 19.2 months, $P<0.0001$), bone mets (14.7 vs 20.4 months, $P=0.01$), CNS mets (14.1 vs 19.6 months, $P=0.02$), ECOG greater than or equal to 2 (11.9 vs 18.0 months, $P=0.013$), and EGFR L858R mutations (16.4 vs 18.2 months, $P=0.005$). Risk factors for reduced TTD were similar. Based on identified risk factors, 90% (632/703) of the overall population had at least 1 risk factor for reduced OS, 71% (446/632) of whom were ECOG less than or equal to 1. Similarly, 83% (587/703) had at least 1 risk factor for reduced TTNT, with 70% (413/587) ECOG less than or equal to 1.

CONCLUSIONS: Overall, a large majority of patients with cEGFRm a/m NSCLC treated with current 1L SOC had risk factors associated with reduced real-world survival (90%) or TTNT (83%). Key individual risk factors included metastases of the central nervous system, liver, or bone and mutations of TP53, CDK4, or L858R. These findings highlight the substantial unmet need for novel treatments for those patients.

SPONSORSHIP: Janssen Pharmaceuticals

C23 Optimizing the use of oncology biomarker testing using health plan best practices and quality measures

Brixner D[1], *Matthews N*[2], *Richardson T*[3], *Pritchard D*[4], *Fox J*[5]; *diana.brixner@utah.edu*
[1]*University of Utah College of Pharmacy*; [2]AMCP; [3]*Impact Education, LLC*; [4]*Personalized Medicine Coalition*; [5]*Illumina*

BACKGROUND: Actionable molecular biomarkers and matched targeted therapies have significantly improved outcomes for patients with cancer. However, many patients do not receive biomarker testing, or the results are not returned prior to initiating therapy. For health plans, precision medicine offers a promising approach to enhance quality and manage costs, utilization, and waste. The realization of these benefits depends on addressing known barriers that affect appropriate biomarker testing practices.

OBJECTIVE: To identify best practices, conceptualize biomarker testing quality measures, and assess the feasibility of a national quality metric based on the AMCP's Market Insights program, which identified 7 critical areas where payers can influence biomarker testing in the non–small cell lung cancer precision oncology pathway.

METHODS: A targeted literature review was supplemented by 8 expert interviews to verify the widespread application of precision medicine via biomarker testing across various cancers. AMCP conducted 4 unstructured interviews with experts in oncology, quality measurement, and biomarker testing and 4 structured interviews with professionals in managed care pharmacy and health care delivery systems. Best practices and real-world examples were extracted from these discussions.

RESULTS: The interviews delineated 13 unmet needs for professional and specialty societies, molecular diagnostic and laboratory companies, care delivery systems, and health plans to address. Twenty-three best practices for health plans were identified and organized into a framework to enhance precision oncology in 7 focal areas: Biomarker Test Ordering, Testing Performance, Result Reporting, Treatment Decision, Quality Improvement and Measurement, Cost-Effectiveness, and Health Disparities. Additionally, 4 real-world examples of quality measures in biomarker testing were highlighted.

CONCLUSIONS: Health plans and care delivery systems are well positioned to support effective biomarker testing and are crucial in developing strategies to reduce disparities in cancer care. Establishing a national biomarker testing quality measure is complex owing to the diversity in cancer care and health plans in the United States; thus, a simplified approach is recommended. Additionally, expert guidance has outlined numerous best practices for health plans to consider if seeking to improve the quality of cancer care through biomarker testing.

SPONSORSHIP: AbbVie, Illumina, Amgen, Genentech

C24 Lower rate of discontinuation of sonidegib compared with vismodegib in a real-world analysis of patients with basal cell carcinoma

Lebwohl M[1], *Rigel D*[1], *Eroglu Z*[2], *Squittieri N*[3], *Gupta D*[4], *Zanardo E*[5], *Huynh L*[6], *Yenikomshian M*[6], *Barghout V*[7], *Ferro T*[3], *Patel K*[3]; *lebwohl@aol.com*
[1]*Department of Dermatology, Icahn School of Medicine at Mount Sinai*; [2]*Department of Cutaneous Oncology, The Moffitt Cancer Center and Research Institute*; [3]*Sun Pharmaceutical Industries, Inc.*; [4]*Analysis Group, Inc.*; [5]*Analysis Group, Inc.*; [6]*Analysis Group, Inc.*; [7]*Viver Health LLC*

BACKGROUND: Sonidegib (ODOMZO) and vismodegib (Erivedge) are the only 2 Hedgehog pathway inhibitors (HHIs) approved for patients with advanced basal cell carcinoma (BCC) not amenable to curative surgery or radiotherapy. Few studies have compared real-world rates of HHI persistence or discontinuation.

OBJECTIVE: To investigate real-world treatment patterns of patients with BCC who received sonidegib or vismodegib.

METHODS: This real-world, retrospective, longitudinal cohort study used claims from the Komodo Health Claims Database of patients who initiated sonidegib or vismodegib between January 1, 2016, and March 31, 2023; the first HHI claim in this period was the index date. Patients were adults (aged ≥18 years) with at least 1 diagnosis of BCC prior to index, at least 2 claims for sonidegib or vismodegib, and at least 3 months of clinical activity or continuous enrollment before and after index. Subgroup analyses included patients without surgery 60 days pre- or post-index. Discontinuation was defined as a gap twice the US Food and Drug Administration–recommended days supply of sonidegib (60 days) or vismodegib (56 days). Kaplan-Meier analyses estimated time to treatment discontinuation. Multivariable Cox proportional hazards models and logistic regressions were conducted.

RESULTS: Overall, 3,766 patients with BCC who received sonidegib (n = 374) or vismodegib (n = 3,392) were identified. Most patients were male (sonidegib [62.8%] and vismodegib [65.0%]), had a mean age of 66-67 years, and had BCC of the face (40.9% and 47.4%, respectively). Mean ± SD follow-up was 28.1 ± 18.4 and 33.0 ± 21.3 months for sonidegib- and vismodegib-treated patients, respectively. Median time to treatment discontinuation was 138 days (4.5 months) for sonidegib and 127 days (4.2 months) for vismodegib ($P = 0.041$ by log-rank test). After adjusting for baseline characteristics, sonidegib-treated patients were 23% ($P = 0.036$) and 32% ($P = 0.013$) less likely to discontinue treatment at 6 and 9 months, respectively, and had an 11% lower rate of discontinuation ($P = 0.037$) compared with vismodegib-treated patients. Among patients without surgery, after adjusting for baseline characteristics, sonidegib (n = 268) was associated with a lower likelihood of discontinuation at 6 (29% less likely, $P = 0.016$) and 9 (44% less likely, $P = 0.001$) months, with a 14% lower discontinuation rate overall ($P = 0.030$) compared with vismodegib (n = 2,458).

CONCLUSIONS: Patients with BCC treated with sonidegib remained on treatment significantly longer, were less likely to discontinue, and had lower discontinuation rates than those treated with vismodegib. These findings were consistent in patients without surgery in the 60 days before or after HHI initiation.

SPONSORSHIP: Sun Pharma

C25 Real-world regorafenib use among patients with advanced gastrointestinal stromal tumors in the United States

Denu R[1], *Appukkuttan S*[2], *Hocum B*[3], *Liao N*[2], *Katta A*[4], *Heo J*[3], *Schroader B*[5], *Singh R*[5], *Babajanyan S*[6], *Somaiah N*[7]; *radenu@mdanderson.org*; *sreevalsa.appukkuttan@bayer.com*; *bridgette.schroader@xcenda.com*
[1]*Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center*; [2]*Bayer US LLC*; [3]*Bayer*; [4]*Bayer US*; [5]*AmerisourceBergen*; [6]*Bayer Healthcare U.S, LLC*; [7]*UT MD Anderson Cancer Center*

BACKGROUND: Gastrointestinal stromal tumors (GISTs) are the most common gastrointestinal soft-tissue sarcomas. Regorafenib is an approved/guideline-preferred multikinase inhibitor used in third-line advanced GIST (aGIST) treatment. Real-world regorafenib utilization is unknown.

OBJECTIVE: To characterize the patient population and describe treatment utilization in patients with commercial insurance or Medicare with aGISTs who initiated regorafenib.

METHODS: Retrospective cohort analysis of Merative MarketScan database (April 2002 to June 2023) of patients with at least 1 regorafenib pharmacy claim during the identification period (October 1, 2015, to May 31, 2023), at least 1 *International Classification of Diseases, Tenth Revision* GIST code pre-index, and at least 12 months of continuous enrollment pre-index and at least 28 days post-index. The index date was the first regorafenib prescription claim date. Primary endpoints were duration of therapy (DOT) from index date to end of therapy and time to next therapy (TTNT) from index date to start of new GIST therapy. DOT was time from index date to date of last claim date + regorafenib days supply. Discontinuation was an at least 60-day gap in claims data.

RESULTS: One hundred sixty-six patients were included; mean (SD) age was 57.6 (15.2) years, 57.2% were male, and 73.5% had commercial insurance. Twenty (12%) patients had pre-index imatinib or sunitinib only, 75 (45.2%) had prior imatinib and sunitinib, 15 (9%) had prior pazopanib, and 3 (1.8%) had prior ripretinib. After a median (IQR) follow-up of 221.5 days (109-416), median regorafenib DOT was 95 days (36-177). Thirty-four patients switched therapy (median [IQR] TTNT = 142 days [77-191]). Patients with prior imatinib or sunitinib alone (n = 20) had a numerically longer median DOT than those who received both in baseline (n = 75) (142.5 days [87-257.5] vs 95 days [53-192], respectively). Patients on low-dose regorafenib (<160 mg/day on 21/28 day cycle; n = 44) had a numerically longer median DOT (103 days

[41.5-210.5]) than standard dose (n = 122; 94.5 days [35-171]) but similar median TTNT (n = 11; 143 days [70-293]; n = 23; 141 days [77-191], respectively). Common follow-up period medications were ripretinib (21 [12.7%]), imatinib (18 [10.8%]), sunitinib (10 [6.0%]), nilotinib (4 [2.4%]), dasatinib, sorafenib, pazopanib (3 [1.8%] each), and everolimus (2 [1.2%]).

CONCLUSIONS: Utilization of regorafenib varied compared with guideline recommendations. Patients on low-dose regorafenib had longer DOT than those on standard dose but similar TTNT. Regorafenib initiated after imatinib or sunitinib alone showed a numerically better DOT, suggesting potential benefit for early initiation of regorafenib. Generalizability may be limited because of limitations of claims data and number of patients.

SPONSORSHIP: Bayer HealthCare

C26 Extrapolating outcomes in pediatric patients with locally advanced or metastatic TRK fusion–positive infantile fibrosarcoma treated with larotrectinib or standard-of-care chemotherapy

Suh K[1], *Kang A*[2], *Appukkuttan S*[3], *Grossman J*[3], *Liao N*[3], *Sullivan S*[4]; *kas551@pitt.edu*
[1]*University of Pittsburgh*; [2]*Curta Health*; [3]*Bayer US LLC*; [4]*University of Washington*

BACKGROUND: Infantile fibrosarcomas (IFSs) are rare tumors that generally occur in young children. Larotrectinib is an approved, targeted therapy for cancers with NTRK gene fusion, and IFS is a tumor type that has a high prevalence (>90%) of the NTRK gene fusion. A recent comparative effectiveness study assessed larotrectinib to an external historical control group treated with a chemotherapy-based standard-of-care (SOC) regimen in pediatric patients with locally advanced or metastatic IFS. In this analysis, baseline characteristics between the 2 treatment groups were adjusted for using inverse probability of treatment weighting.

OBJECTIVE: To compare expected lifetime life-years (LYs) and quality-adjusted life-years (QALYs) between larotrectinib and SOC based on the aforementioned analysis.

METHODS: We developed a partitioned survival model to project long-term outcomes. Larotrectinib survival data were derived from an updated July 2022 analysis of 49 pediatric patients with IFS from the larotrectinib clinical trials program (NCT02122913, NCT02637687, and NCT02576431). The external SOC control arm included 42 patients, 40 of whom had a documented NTRK gene fusion from a French database. Progression-free survival and overall survival curves were estimated using several parametric survival distributions (Exponential, Weibull, Log-logistic, and Log-normal), then selected based on goodness-of-fit statistics, visual fit, and clinical plausibility. QALYs were estimated by adjusting the time spent in the preprogression and postprogression health states by utility values from the literature. A discount rate of 3% was applied to LYs and QALYs. Model uncertainty was evaluated using one-way and probabilistic sensitivity analyses.

RESULTS: Exponential curves were used to extrapolate results. The larotrectinib model estimated 33.14 LYs (95% credible interval [CrI] = 18.06-36.41), which translated to 11.47 QALYs (95% CrI = 2.24-30.55). LYs and QALYs in the SOC arm were projected to be 22.51 (95% CrI = 4.67-34.05) and 7.19 (95% CrI = 0.79-26.64), respectively. Compared with SOC, larotrectinib produced additional gains of 10.63 LYs and 4.28 QALYs.

CONCLUSIONS: In pediatric patients with locally advanced or metastatic TRK fusion–positive IFS, larotrectinib may produce substantial life expectancy and QALY gains compared with SOC chemotherapy. Additional real-world studies would further inform our results.

SPONSORSHIP: Bayer US LLC

C27 Perceptions and experiences of medical oncologists on biomarker testing and treatment of early breast cancer: Qualitative findings

Li Q[1], *Howell L*[2], *Kashine N*[3], *Noval A*[2], *Xu X*[4]; *sandy.li1@astrazeneca.com*
[1]*AstraZeneca*; [2]*Oracle Life Sciences*; [3]*Oracle*; [4]*AstraZeneca Oncology Outcomes Research*

BACKGROUND: In the evolving treatment landscape of early breast cancer (eBC), clarity is needed on best clinical practices regarding biomarker testing and treatment selection.

OBJECTIVE: To elicit the perceptions and experiences of medical oncologists to understand drivers and barriers to biomarker testing and treatment decision-making in eBC.

METHODS: Semistructured telephone interviews were conducted in the United States from October 9 to 24, 2023, with medical oncologists from both academic (n = 8) and community (n = 8) settings.

RESULTS: Oncologists said that all their patients with eBC were reflexively tested for estrogen receptors, progesterone receptors, and human epidermal growth factor receptor 2 at diagnosis/biopsy. Although some oncologists routinely tested all patients for breast cancer gene (BRCA), the majority (n = 12) only ordered testing for patients with certain conditions (family history, age, and triple-negative breast cancer). Patients' refusal (eg, fear, family implication, or inconvenience) was identified as a primary barrier for BRCA

testing. In addition, some oncologists ensured proper insurance coverage before ordering testing. Although timing of BRCA testing was inconsistent across practice settings, several oncologists highlighted the value of knowing BRCA status before surgery to inform both surgical and systemic therapy decisions and suggested that surgeons may have an essential role in ordering testing to enable timelier treatment decisions. Academic institutions were more likely to have a streamlined approach for integrating genetic counseling into their treatment paradigms, whereas community clinics tended to use it on a case-by-case basis. Although most oncologists agreed that there is no clear consensus of a high-risk definition, some indicated a certain single factor (eg, stage III) was sufficient to determine whether patients are at high-risk for recurrence. Most oncologists agreed that Lynparza (olaparib) would be their preferred treatment choice for patients with BRCA mutant hormone receptor–positive/human epidermal growth factor receptor 2–negative eBC and hinted interest in concurrent or sequential use of olaparib with other therapies in patients with BRCA mutant early triple-negative breast cancer.

CONCLUSIONS: Coverage requirements and patient decisions are 2 barriers to overcome to ensure timely implementation of BRCA testing. Timely adoption of the recent testing guidelines for both surgeons and medical oncologists, streamlined genetic counseling, and education of the predictive value of early BRCA testing in treatment decisions are lingering unmet needs to address to promote best practices in eBC.

SPONSORSHIP: AstraZeneca and Merck & Co., Inc.

C28 Impact of delaying disease recurrence on economic burden in patients with human epidermal growth factor receptor 2–positive early-stage breast cancer

Mehta S[1], *Faherty E*[1], *Princic N*[2], *Moynihan M*[2], *Henriques C*[2]; *sandhya.mehta@daiichisankyo.com*
[1]*Daiichi Sankyo, Inc.*; [2]*Merative*

BACKGROUND: There is a gap in understanding the impact of early disease recurrence on cumulative cost burden in human epidermal growth factor receptor 2–positive (HER2+) early breast cancer (eBC).

OBJECTIVE: To assess neoadjuvant (neo) and adjuvant (adj) treatment (tx) patterns, recurrence rates, and impact of recurrence timing on cumulative cost burden among patients with HER2+ eBC.

METHODS: The US health care claims in the Merative MarketScan Commercial and Medicare Database were used to identify adults newly diagnosed with BC between January 1, 2017, and September 30, 2022, and at least 1 HER2 targeted treatment following the BC date. Surgery (ie, mastectomy or lumpectomy) within a year of the BC date delineated neo and adj periods, before and after the surgery date, respectively, in which treatment patterns were described. Recurrence was reported during the postsurgery period as defined by evidence of additional chemotherapy treatment (after a 90-day gap following discontinuation or outside of the 120-day adjuvant window), metastasis, or end-of-life care. A generalized linear model (γ distribution and log link) adjusting for patient demographics and treatment type was used to assess the impact of disease recurrence on cumulative 3-year total all-cause costs during the post-surgery period.

RESULTS: A total of 3,745 patients with HER2+ eBC were included in the study (mean age 53.7 years): 57.4% (n = 2,151) with adj tx only, 40.2% (n = 1,504) both tx (neo and adj), 1.9% (n = 70) surgery only, and 0.5% (n = 20) neo tx only. Among those with neo tx (n = 1,524), carboplatin+docetaxel+trastuzumab (T)+pertuzumab (P) (68.5%) was the most used. And among those with adj tx (n = 2,151), the use of paclitaxel+T (31.5%) was common, followed by T alone (14.7%) and T+P (12.1%). During the follow-up (median duration after surgery: 2 years), the recurrence rate was highest for surgery only (70.0%) and similar for adj only (16.0%) and both tx (14.3%) cohorts. A generalized linear model showed that cumulative cost burden following surgery was higher among patients who experienced recurrence in less than 12 months vs no recurrence ($348,834 vs $265,279). Patients with chemo only as adj tx had a higher cumulative cost burden (relative risk = 1.28; $P < 0.001$) vs those with HER2 targeted treatment, and patients with neo tx had a lower cost burden (relative risk = 0.85; $P < 0.001$) compared with those with no neo tx.

CONCLUSIONS: Delays in disease recurrence were associated with a lower cumulative cost burden. The study findings highlight that use of HER2 targeted treatment (associated with longer event-free survival) earlier in the treatment pathway may improve patient outcomes and reduce the health care burden associated with BC.

SPONSORSHIP: Daiichi Sankyo, Inc., and AstraZeneca, LLC

C29 Treatment patterns, clinical outcomes, and health care resource utilization among patients with hormone receptor–positive human epidermal growth factor receptor 2–low or immunohistochemistry 0 metastatic breast cancer: Data from an integrated delivery network

Mehta S[1], *Barman H*[2], *Iyengar A*[3], *Rangarajan N*[4], *Faherty E*[1], *Wagner T*[5], *Simsek S*[6]; *sandhya.mehta@daiichisankyo.com*
[1]*Daiichi Sankyo, Inc.*; [2]*nference*; [3]*nference Inc.*; [4]*nference, Inc.*; [5]*nference, Inc.*; [6]*nference, Inc*

BACKGROUND: Limited treatment and outcomes data exist for patients with human epidermal growth factor receptor 2 (HER2)–low (immunohistochemistry [IHC] 1+ or IHC 2+ and ISH–) or IHC 0 metastatic breast cancer (mBC) treated via an integrated delivery network (IDN).

OBJECTIVE: To assess treatment patterns, outcomes, and health care resource utilization (HCRU) for patients with HER2-low or IHC 0 mBC treated at an IDN.

METHODS: We retrospectively analyzed deidentified structured and unstructured electronic health record data (January 2018 to June 2022) from an IDN in the United States (locations in Minnesota, Florida, and Arizona). Patients aged 18 years and older at diagnosis of hormone receptor (HR)+/HER2-low or HR+/IHC 0 mBC with at least 2 clinic visits and receipt of at least 1 line of therapy after mBC diagnosis were included; those receiving systemic therapy for another primary cancer, participating in a clinical trial during the observation period, or with documented HER2+ status prior to initiation of first-line (1L) therapy were excluded. Patient characteristics, treatment utilization, and HCRU are described; real-world time to treatment discontinuation (rwTTD) and time to next treatment (rwTTNT) were determined via Kaplan-Meier methods.

RESULTS: Of 1,300 patients with mBC meeting cohort selection criteria, 871 (67%) were HER2-low (HR+, n = 790) and 429 (33%) were IHC 0 (HR+, n = 352). Among patients with HR+ HER2-low mBC (n = 790; mean age: 61.4 years; White race: 88.7%), 373 patients (47%) progressed to second-line (2L) and 190 (24%) progressed to third-line (3L) therapy during the study follow-up period (median follow-up starting from 1L: 16.7, 2L: 15.2, and 3L: 10.8 months). Of patients receiving 1L, 2L, and 3L therapy, 16%, 17%, and 13%, respectively, discontinued therapy or died. In 1L, the majority of patients received a hormone therapy (HT)–based regimen (n = 579; 38% HT alone, 33% CDK4/6 inhibitor + HT, 2% other targeted treatment [OTT; mTOR/PARP inhibitor/PIK3CA] + HT) followed by chemotherapy (16%). Common 2L/3L therapies were HT alone (33%-35%) or CDK 4/6 inhibitor + HT (16%-19%), chemotherapy (20%-21%), or OTT+HT (9%-10%). The most followed treatment pathway was 2 sequences of an HT-based regimen followed by chemotherapy. From 1L to 3L, rwTTD and rwTTNT decreased from 11.0 and 15.4 months to 6.9 and 8.4 months, respectively. Hospitalization rates for 1L-3L were 9.5%-16.5%. Treatment patterns were similar but clinical outcomes shorter in HR+ IHC 0 vs HER2-low disease.

CONCLUSIONS: Among those with HR+ HER2-low mBC, treatments varied, with many patients receiving multiple lines of therapy shortly after 1L and experiencing diminishing time on treatment, highlighting a need for effective therapies that extend the duration of clinical benefit earlier in the disease management pathway.

SPONSORSHIP: Daiichi Sankyo & Astra Zeneca

C30 Health care costs associated with prophylactic neurokinin-1 receptor antagonist use among women with invasive breast cancer

Kamat S[1], *Brown B*[2], *Cohen S*[2], *Vyas A*[2]; *shwetakamat@openhealthgroup.com*
[1]*Open Health*; [2]*University of Rhode Island*

BACKGROUND: In April 2012, the American Society of Clinical Oncology joined the "Choosing Wisely" (CW) initiative to reduce the use of low-value oncology services. Neurokinin-1 receptor antagonists (NK1-RAs) are a class of expensive antiemetics and are not recommended for patients who receive low– to moderate–emetic risk chemotherapy

OBJECTIVE: To examine differences in mean total costs reimbursed by third-party payers and patient out-of-pocket (OOP) costs between those who received prophylactic NK1-RAs and those who received other antiemetics among women with invasive breast cancer.

METHODS: Using Optum's deidentified Clinformatics Data Mart Database for 2013-2018 (post-CW period), a retrospective cohort study was conducted for women aged 18 years and older with breast cancer who initiated low/minimal/moderate–emetic risk chemotherapy (classified per the National Comprehensive Cancer Network guidelines). All approved prophylactic NK1-RAs (aprepitant, fosaprepitant, rolapitant, netupitant/palonosetron) were included. A generalized linear model and 2-part model with γ distribution and log link were used to model costs, respectively, controlling for baseline patient sociodemographic characteristics.

RESULTS: Out of a total of 12,068 women, 11.2% (n = 1,356) received NK1-RAs. The mean total third-party payer costs for women who received NK1-RAs was the highest ($4,519; 95% CI = $3,538-$5,500), followed by serotonin-receptor antagonists (5HT3-RAs) with or without steroids ($3,095; 95% CI = $2,343-$3,847). The adjusted mean total

third-party payer costs for women who received steroids only, first-generation 5HT3-RAs with or without steroids, or second-generation 5HT3-RAs with or without steroids were found to be significantly lower by 97.8%, 95.0%, and 42.1% ($P = 0.023$), respectively, when compared with those who received NK1-RAs. There was no significant difference in the mean total third-party payer costs between women who received 5HT3-RAs with or without steroids and those who received NK1-RAs ($P = 0.450$). The mean OOP costs to patients were higher for those who received prophylactic NK1-RAs ($30; 95% CI = $27-$32), followed by those who received second-generation 5HT3-RAs with or without steroids ($15; 95% CI = $11-$18).

CONCLUSIONS: The mean total costs reimbursed by payers were significantly higher for those who received NK1-RAs as compared with other prophylactic antiemetics, except for those who received 5HT3-RAs with or without steroids. Judiciously using NK1-RAs per guideline recommendations could result in significant cost avoidance for patients and payers.

SPONSORSHIP: None

C34 Impact of coverage policy complexity and restrictiveness on supportive care receipt in a real-world cohort of patients with cancer

Rivers Z[1], *Ben-Shachar R*[2], *Klein I*[3], *Belinson S*[3], *Levine A*[4], *Chambers J*[5], *Beinfeld M*[5]; *zachrivers50@gmail.com*
[1]*Tempus AI, Inc*; [2]*Tempus AI, Inc*; [3]*Tempus AI, Inc*; [4]*Tufts Medical Center*; [5]*Center for the Evaluation of Value and Risk in Health, Institute for Clinical Research and Health Policy Studies, Tufts Medical Center*

BACKGROUND: Real-world data (RWD) are a valuable data source for oncology drug development and regulatory decision-making. Heterogeneity in insurance coverage by payer can impact patient care.

OBJECTIVE: To leverage 2 novel measures of insurance coverage of supportive medications for oncology treatment–related toxicities to characterize access to care in RWD cancer cohorts.

METHODS: Using Tempus AI, Komodo, and Tufts Center for the Evaluation of Value and Risk in Health data, we linked patient-level clinical and health claims data to temporally accurate coverage policies. We evaluated patients with non–small cell lung cancer (NSCLC), colorectal cancer (CRC), and breast cancer (BC) treated with chemotherapy. Evaluable patients had 1 year of continuous insurance coverage after their first chemotherapy treatment. We assessed coverage policies for specialty medications used to treat nausea and vomiting (NV) and neutropenia per National Comprehensive Cancer Network toxicity guidelines in the year following treatment initiation. We used 2 metrics of coverage: the Coverage Restriction Summary Score (range 0-8 measuring restrictions above the US Food and Drug Administration label accounting for restrictions based on patient subgroups, combination therapy, prescriber qualifications, and step therapy) and the Complexity Score (range 0-10 measuring word count, line breaks, documentation requirements, and number of policy documents). A higher score represents higher restriction or complexity. We explore the impact of these scores on receipt of the National Comprehensive Cancer Network–recommended treatment, accounting for toxicity type, using Fisher's exact test.

RESULTS: The cohort included 9,545 patients (4,211 NSCLC, 2,368 CRC, and 2,966 BC) across 12 payers. We considered 3 medications for NV and 7 medications for neutropenia. Neutropenia treatments had a wider complexity range and SD (0-10, SD = 3.0) compared with NV medications (0-8, 2.2), whereas NV medications had a wider restrictiveness range (0-4, 1.2) than neutropenia meds (0-3, 1.0). Complexity, but not restrictiveness, drove a statistically significant difference in receipt of antiemetics ($P < 0.05$ for each medication). Four of the 7 treatments for neutropenia were significantly impacted by complexity, whereas restriction impacted 2 of 7.

CONCLUSIONS: Clinicians are faced with complex and restrictive policies when providing care. We show that policy complexity impacts receipt of guideline-recommended treatments for neutropenia and NV. Future work should explore whether the relationships identified impact clinical outcomes including persistence and survival.

SPONSORSHIP: Tempus AI, Inc

C36 Biosimilar persistence of trastuzumab after conversion from reference product: An integrated health system claims database analysis

Ghaibi S, Yep T; *shadi.x.ghaibi@kp.org*; *Tracy.D.Yep@kp.org*
Kaiser Permanente Washington

BACKGROUND: Biologic medications are generally large, complex molecules approved by the US Food and Drug Administration (FDA) to treat and cure diseases and medical conditions. They are often high-cost treatments in numerous therapeutic categories. FDA-approved biosimilars and reference products are highly similar to each other with no clinically meaningful differences between the two. Use of biosimilars is estimated to lower health care costs by 15%-35% compared with reference products. Kaiser Permanente Washington evaluates each reference biologic product and its biosimilars to implement strategies that drive affordable care. Members on Herceptin were transitioned to the preferred trastuzumab biosimilar in September 2019.

Determining biosimilar persistence after an initial conversion from reference product provides insight to ongoing utilization of the most cost-effective treatment option.

OBJECTIVE: To determine 3-, 6-, and 12-month persistence of trastuzumab biosimilar use after conversion from Herceptin.

METHODS: A retrospective claims analysis was completed as part of quality improvement. Medical benefit claims for all trastuzumab products from September 2019 through December 2023 were collected for analysis. Completion of trastuzumab therapy was assumed for patients who no longer had claims during the study period. The primary endpoint of patient persistence on trastuzumab biosimilar was calculated through 2023 for those who converted from Herceptin between September 2019 and August 2020 (index year).

RESULTS: A total of 127 patients received any trastuzumab infusion within the index year. Of those, 42 patients were converted from Herceptin to a biosimilar within that time frame. Two patients (5%) switched back to Herceptin after confirmed infusion reaction following initial doses of trastuzumab biosimilar (1 and 2 doses, respectively). Overall, 12 (28.6%) patients remained on biosimilar for less than 3 months; 10 (23.8%) patients remained on biosimilar for 3-6 months; 7 (16.7%) patients remained on biosimilar for 7-12 months; and 13 (30.9%) patients remained on biosimilar for 13 months or longer.

CONCLUSIONS: Kaiser Permanente Washington claims data suggest a majority of patients (n = 40, 95%) were persistent on a trastuzumab biosimilar after conversion from Herceptin until completion of therapy.

SPONSORSHIP: None

C38 Disparities in real-world treatment patterns and outcomes among patients with metastatic castration-resistant prostate cancer: A PRECISION (PRostatE Cancer dISease observatION) data platform study

Shore N[1], *George D*[2], *Sartor O*[3], *Wei X*[4], *Nguyen J*[5], *Patel J*[5], *Bollu V*[6], *Sawhney A*[5], *Kang B*[5], *Fallick M*[6], *Byrne C*[7], *Tang J*[7], *LaVallee C*[7], *Heath E*[8]; *nshore@gsuro.com*
[1]*Carolina Urologic Research Center*; [2]*Duke Cancer Institute, Duke University School of Medicine*; [3]*Mayo Clinic*; [4]*Dana-Farber Cancer Institute*; [5]*Novartis Pharmaceuticals Corporation*; [6]*Novarits Pharmaceuticals Corporation*; [7]*Asclepius Analytics*; [8]*Karmanos Cancer Center*

BACKGROUND: Prostate cancer management is associated with known disparities by race, geographic location, education, occupational status, and socioeconomic level, but how these impact patients with end-stage disease is poorly understood.

OBJECTIVE: To examine the associations between patient characteristics, treatment initiation, and outcomes in patients with metastatic castration-resistant prostate cancer (mCRPC) treated in the United States.

METHODS: This retrospective cohort study using the PRECISION data platform—a validated, harmonized real-world dataset from predominantly community urology centers, as well as from academic and community oncology centers—included patients with clinician-documented mCRPC (index date) between 2018 and 2023. Patient characteristics and treatment patterns were evaluated descriptively; time to treatment initiation (TTTI) and overall survival (OS) were assessed via time-to-event analyses. Multivariate analyses were used to assess relationships between patient characteristics, TTTI, and OS.

RESULTS: A total of 13,367 patients with mCRPC were included; 88% were aged ≥65 years, 62% were White, and 14% were Black. Overall, 9% of patients had been treated at oncology practices and 91% at urology sites. Approximately 70% of patients initiated a first-line (1L) therapy at or after mCRPC diagnosis, and median TTTI was 2.2 months. Most (55%) patients initiated androgen receptor pathway inhibitors in the 1L. Patients who were unemployed or who had not completed high school had significantly lower rates of 1L therapy initiation. An age of 85 years and older and treatment at urology sites were associated with significantly longer TTTI. Median OS was 23.1 months for patients with mCRPC treated at oncology practices and 29.2 for those treated at urology practices.

CONCLUSIONS: This analysis highlights potential undertreatment of patients with mCRPC in clinical practice, with 30% of patients not receiving a 1L therapy. In this real-world study, median OS following mCRPC diagnosis was only 2 years for patients treated at oncology practices in the United States. This study showed that, especially among patients with markers of lower socioeconomic status, disparities exist in the management of patients with mCRPC. Given that the current data are predominantly obtained from community urology sites, future studies are warranted, looking at patient characteristics and key disparities within urology and oncology sites of care, to identify actionable insights.

SPONSORSHIP: Novartis Pharmaceuticals Corporation

C39 Real-world treatment patterns and clinical outcomes in chemotherapy-naive patients with metastatic castration-resistant prostate cancer in the US Surveillance, Epidemiology, and End Results–Medicare population

McKay R[1], *Patel J*[2], *Kang B*[2], *Sawhney A*[2], *Wilkinson S*[3], *Byrne C*[4], *Tang J*[4], *Runeckles K*[4], *Nguyen J*[2]; *rmckay@ucsd.edu*
[1]*Moores Cancer Center, University of California San Diego*; [2]*Novartis Pharmaceuticals Corporation*; [3]*Novartis Pharmaceuticals Corporation*; [4]*Asclepius Analytics*

BACKGROUND: Although advances in metastatic castration-resistant prostate cancer (mCRPC) treatment have emerged with the approval of new agents, impacts on real-world treatment sequencing and outcomes in chemotherapy-naive patients with mCRPC are not yet well understood in the Medicare setting.

OBJECTIVE: To describe the characteristics, treatment patterns, and clinical outcomes of patients with mCRPC who were chemotherapy-naive and received 1 prior androgen receptor pathway inhibitor (ARPI).

METHODS: This retrospective, observational study used US Surveillance, Epidemiology, and End Results–Medicare data from January 1, 2009, to December 31, 2019. Adult men with mCRPC who had previously received 1 ARPI before receiving a second ARPI (ARPI cohort) or a taxane (taxane cohort) were included. The index date was the date of initiation of the second ARPI or initiation of a taxane. Patients who had received poly(ADP-ribose) polymerase inhibitors, immunotherapy, radiation, biologics, or chemotherapy within the past 12 months were excluded. Treatment patterns were evaluated descriptively; inverse probability of treatment weighting–adjusted Kaplan-Meier analysis was used to assess overall survival (OS).

RESULTS: A total of 923 patients were included in the final study population; 714 (77%) and 209 (23%) were in the ARPI and taxane cohorts, respectively. Available patient characteristics (ie, race, ethnicity, and geographic region) were broadly comparable between the 2 cohorts, although the ARPI cohort was significantly older than the taxane cohort (mean age 75 vs 71 years; $P < 0.0001$). Time from PC diagnosis to mCRPC diagnosis was longer in the taxane cohort compared with the ARPI cohort (median 469 vs 399 days; $P = 0.0350$). The taxane cohort had a significantly longer treatment duration compared with the ARPI cohort (3.6 vs 2.4 months; $P = 0.0500$) and a higher rate of advancement to a subsequent therapy (54% vs 33%). After discontinuation with ARPI and taxane, patients were more likely to receive taxanes in later lines. Overall, 63% of patients did not receive any subsequent treatment. Median OS was 12.8 months in the ARPI cohort and 11.8 months in the taxane cohort.

CONCLUSIONS: The results of this study show that many patients with mCRPC were not receiving any treatment in later lines, highlighting the unmet need for better therapies than those currently available. Median OS in chemotherapy-naive patients with mCRPC with 1 prior ARPI treatment remained low regardless of whether patients switched to another ARPI or a taxane. Overall, there is an unmet need for more effective therapies for patients with mCRPC.

SPONSORSHIP: Novartis Pharmaceuticals Corporation

C40 Experience with an outcomes-based rebate agreement for apalutamide in the treatment of prostate cancer

Brown B[1], *Suehs B*[2]; *bsuehs6@humana.com*
[1]*Humana, Inc., Louisville, KY*; [2]*Humana Healthcare Research, Inc, Louisville, KY*

BACKGROUND: Humana Inc. and Janssen Biotech Inc. entered into an innovative outcomes-based rebate agreement (OBA) for apalutamide in 2019. The OBA offered financial rebates for the on-label utilization of apalutamide based on clinical performance of apalutamide (trade name: Erleada) for treatment of prostate cancer among certain individuals enrolled in Humana Medicare Advantage Prescription Drug (MAPD) plans.

OBJECTIVE: To summarize the implementation of the OBA and the outcomes reported during the implementation of the OBA.

METHODS: This analysis included individuals enrolled in eligible MAPD plans newly initiating apalutamide treatment from July 2019 through June 2023. Patients that were prescribed any oral androgen receptor inhibitors (OAIs) prior to initial dispensing of apalutamide were excluded. To assess clinical performance, 2 prostate-specific antigen (PSA) laboratory test results were required; one during the baseline period prior to apalutamide treatment initiation (up to 91 days prior to dispensing) and a second PSA test result between +56 and +112 days after the date of initial dispensing. Treatment response was defined as PSA reduction of greater than 50% in PSA level. Descriptive analyses of attrition and treatment response were conducted.

RESULTS: A total of 4,042 individuals were identified initially with a dispensing of at least 60 days supply of apalutamide. Among those, 1,556 individuals were new to OAI therapy and eligible for inclusion in the clinical performance analysis. Baseline PSA results were available for 42.2% (n = 656) of individuals, and both baseline and post–OAI initiation

PSA test results were available for 19.5% (n = 303) of new initiators. A further 20 individuals were excluded from the clinical performance analysis owing to a lapse in continuous enrollment between the first PSA test and the postinitiation retest. Among individuals included in the clinical performance assessment (n = 283), 67.5% (n = 191) demonstrated PSA reduction of greater than 50%.

CONCLUSIONS: Pharmacy OBA arrangements represent an innovative way to tie price concessions on utilization of products to outcomes-oriented metrics. Challenges include availability of the necessary clinical data for the health plan to fully implement performance-based measures. Strategies to improve clinical data availability to support such agreements, such as medical record reviews or other tactics, should be considered.

SPONSORSHIP: None

C41 Diagnostic performance of prostate cancer–specific clinical phenotypes identified using real-world databases: A systematic literature review

Vyas A[1], *Kamat S*[1], *Thomas S*[1], *Gambino C*[1], *Brown B*[1], *Raval A*[2]; *avyas@uri.edu*; *amitkumar.raval@bayer.com*
[1]*University of Rhode Island*; [2]*Bayer Pharmaceuticals*

BACKGROUND: Real-world databases (RWDs) often lack the ability to directly identify complex clinical conditions or outcomes. This often requires the development of computable phenotypes based on clinical reasoning–based algorithms or prediction models with validation through a reference standard such as chart review. Although there are studies reporting different phenotypes for key prostate cancer disease or outcomes, these have not been summarized systematically.

OBJECTIVE: To conduct a systematic review of the literature to summarize validation statistics on prostate cancer–specific phenotypes, including metastasis (any metastasis, bone metastasis, and lymph node metastasis), biochemical recurrence (BCR), castration-resistant prostate cancer (CRPC), hormone-sensitive prostate cancer, and performance status.

METHODS: We performed systematic searches in PubMed/Medline and Embase for published literature from 2012 to 2023. Studies reporting algorithms and prediction models for prostate cancer phenotypes based on structured data (claims, laboratory values, hospital records) were included. A summary of algorithms and prediction models, along with their respective estimates of diagnostic accuracy compared with reference standards (chart review) and/or measures of uncertainty, were provided. An area under the curve (AUC) greater than 0.7 was considered an acceptable phenotype.

RESULTS: Out of 7,427 retrieved citations, 29 unique retrospective studies (31 citations) were included, with a majority (n = 18) being conducted in the United States. Prediction models for any metastasis had AUC greater than 0.88, whereas the claims-based algorithms had high sensitivity and specificity values. Also, prediction models for bone metastasis had high AUC values (>0.7), and claims-based algorithms had high sensitivity and specificity values. Prediction models for BCR based on clinical variables and tumor-specific characteristics had AUC values greater than 0.7. One claims-based algorithm for metastatic CRPC had high sensitivity and specificity. Some studies developed algorithms to identify patients with hormone-sensitive prostate cancer but did not assess their diagnostic accuracy. Claims-based algorithms for performance status had at least 75% sensitivity and relatively high specificity.

CONCLUSIONS: Our systematic review of the literature highlights the acceptable accuracy of computable phenotypes for prostate cancer, including (bone) metastasis, BCR, and performance status within RWDs. In addition, further validation studies are needed for RWD-based phenotypes that do not yet have validation estimates.

SPONSORSHIP: Bayer Healthcare Pharmaceuticals

C42 Opportunities for product differentiation and evidence generation with a focus on health disparities information in the Academy of Managed Care Pharmacy Format for Formulary Submissions version 5.0

Pollack M[1], *Cadarette S*[1], *Stewart F*[1], *Dillaman M*[1], *Abresch L*[1], *Crawford B*[1], *Jung J*[1], *Gill M*[1], *Arregui M*[1], *Schroader B*[2]; *megan.pollack@cencora.com*; *bridgette.schroader@xcenda.com*
[1]*Cencora*; [2]*AmerisourceBergen*

BACKGROUND: The AMCP Format is the gold standard for manufacturers to communicate comprehensive clinical and economic evidence to US health care decision-makers to aid formulary, coverage, policy, and reimbursement decisions for new and existing products. A new area of focus in the AMCP Format version 5.0, released April 2024, is the inclusion of health disparities information related to social/demographic factors; however, it is unclear to what extent health disparities literature may be available for oncologic disease states.

OBJECTIVE: To describe available literature with health disparity information in prostate cancer (PC) and Hodgkin lymphoma (HL), proxies for general and rare oncologic disease states.

METHODS: In May 2024 we searched Medline and Embase via Ovid for US-based studies (2019-2024) that reported data on health disparities in people with PC or HL. Eligible

studies included any/no intervention; eligible study designs included systematic literature reviews, and interventional, retrospective, prospective, case-control, and cross-sectional studies. Social and demographic factors of interest included race, ethnicity, sex, income, geographic region, marital status, insurance status, socioeconomic status, sexual orientation, religion, disability, and age. Title and abstract screening results are presented here. Screening and data extraction were conducted by 1 researcher with a second researcher performing quality checks.

RESULTS: Initial searches in Medline and Embase yielded 995 abstracts, of which 255 met the inclusion criteria. Studies in PC made up the majority of the abstracts (n = 241), whereas abstracts in HL were more limited (n = 14). Across both oncologic disease states, the most frequently reported health disparity factors were race (PC: n = 193; HL: n = 7), ethnicity (PC: n = 64; HL: n = 7), and geographic region (PC: n = 53; HL: n = 3); health disparity data were extremely limited or not reported for sex, sexual orientation, religion, and disability (PC: n < 5 each; HL: ≤1 each).

CONCLUSIONS: The release of the AMCP Format 5.0 dossier guidance provides an opportunity for manufacturers to differentiate their products through inclusion of health disparity data. Using PC and HL as proxies for general and rare oncologic disease states, we observed multiple data gaps exist related to health disparities, particularly in the rare (HL) disease space. Therefore, there is an opportunity for manufacturers to generate new evidence to demonstrate how a product addresses unmet needs related to health disparities.

SPONSORSHIP: Cencora

C43 Real-world economic burden of disease recurrence in patients with muscle-invasive bladder cancer treated with radical cystectomy: A retrospective administrative claims analysis

Squires P[1], *Cook E*[2], *Song Y*[2], *Wang C*[1], *Jiang A*[2], *Zhang A*[2], *Li H*[1], *Rogiers A*[1], *Mamtani R*[3]; *patrick.squires@merck.com*; *ching-yu.wang@merck.com*

[1]*Merck & Co., Inc.*; [2]*Analysis Group, Inc.*; [3]*Abramson Cancer Center Perelman School of Medicine at the University of Pennsylvania*

BACKGROUND: Bladder cancer is a common cancer that results in significant mortality, morbidity, and economic cost. Radical cystectomy (RC) is the standard of care for patients with muscle-invasive bladder cancer (MIBC). Although RC is performed with curative intent, a significant proportion of patients experience cancer recurrence after RC. The economic impact of recurrence in a contemporary US cohort has not been recently described.

OBJECTIVE: To assess the economic impact of recurrence on health care resource utilization (HCRU) and cost among RC-treated patients with MIBC in the United States.

METHODS: This retrospective, observational analysis of Surveillance, Epidemiology, and End Results–Medicare data (2007-2020) included patients diagnosed with T2-T4aN0M0 or T1-T4aN1M0 MIBC, stratified by whether they experienced recurrence after RC. The index date for patients with recurrence (identified using diagnosis codes and/or treatment) was defined as 30 days prior to recurrence and was randomly assigned for patients without recurrence to match the time window between RC and the index date in the recurrence cohort. Patients were followed from the index date until the end of continuous enrollment or death. Rates of HCRU and mean health care costs (in 2022 USD) per patient per month (PPPM) were compared between the cohorts.

RESULTS: Demographic and clinical characteristics were generally similar between patients with (n = 503) and without recurrence (n = 602), with few exceptions. Patients with vs without recurrence were more likely to have had stage 3a disease (47.9% vs 32.7%) and cisplatin contraindications (54.1% vs 47.5%, both $P < 0.05$). Median follow-up time was 1.0 (IQR = 0.3-2.7) and 1.6 (0.6-3.3) years for patients with and without recurrence, respectively ($P = 0.07$). During follow-up, patients with vs without recurrence had higher adjusted incidence rates (aIRRs, all $P < 0.001$) of all-cause and MIBC-related HCRU, including hospitalization (all-cause: aIRR = 2.4; MIBC-related: aIRR = 4.1), emergency department visits (2.7; 3.5), and outpatient visits (2.0; 3.2). Adjusted all-cause and MIBC-related total medical costs were higher for patients with vs without recurrence (all-cause cost difference: $7,191 PPPM; MIBC-related cost difference: $3,073 PPPM), largely contributed by hospitalization cost (cost difference: $4,542 PPPM; $2,171 PPPM; all $P < 0.001$).

CONCLUSIONS: In this contemporary US cohort of patients with MIBC treated with RC ± perioperative chemotherapy, cancer recurrence is associated with high HCRU and cost, resulting in a significant economic burden and further underscoring the need for novel and effective risk-reducing therapies.

SPONSORSHIP: Merck Sharp & Dohme LLC, a subsidiary of Merck & Co., Inc., Rahway, NJ, USA

C44 Budget impact model for biosimilar denosumab in the US oncology population

Flanigan J[1], Chaplin S[2], van Stiphout J[2], Schauf M[3], Tindal M[1], Li E[1], Lyman G[4]; jeanine.flanigan_ext@sandoz.com
[1]*Sandoz*; [2]*Cencora*; [3]*Sandoz International GmbH*; [4]*Fred Hutchinson Cancer Center*

BACKGROUND: The first biosimilar approved in the United States was within the oncology therapeutic area; since then, multiple oncology biosimilars have become available and have decreased costs for the health care system. Denosumab is a bone-modifying agent used to prevent skeletal-related events across multiple tumor types. The potential savings after introduction of a biosimilar denosumab is currently unknown.

OBJECTIVE: To quantify the potential savings of a biosimilar denosumab for oncology indications from a Medicare perspective.

METHODS: The budget impact of biosimilar denosumab use for a hypothetical population of 1 million (M) patients over 5 years was assessed. Model inputs for skeletal-related event incidence, bone-modifying agent use, projected biosimilar uptake, health care resource utilization, and costs were derived from published literature and publicly available sources. For the cost of biosimilar denosumab, various cost scenarios relative to reference denosumab's payment limit were evaluated. In addition, different biosimilar utilization scenarios were analyzed. Total budget savings, as well as per patient per year (PMPY) and per patient per month (PMPM), were calculated.

RESULTS: For a population of 1 M, a total of 46,556 patients were estimated to be treated with denosumab over a period of 5 years. Twenty-five percent uptake of biosimilar denosumab by year 5 was estimated to yield a total of $9.1 M in net savings. This also resulted in a savings of $1.66 PMPY and $0.14 PMPM. Fifty percent uptake of biosimilar denosumab would result in $18.2 M total savings, with a savings of $3.32 PMPY and $0.28 PMPM. Lastly, 70% uptake of biosimilar denosumab would result in $31.1 M total savings, with a savings of $5.68 PMPY and $0.47 PMPM. Reduced biosimilar pricing resulted in estimated savings of $14.8 M to $133.3 M, assuming 50% uptake of biosimilar denosumab, over a 5-year period.

CONCLUSIONS: Following various scenarios, biosimilar denosumab utilization may contribute to significant cost savings to the health care system. Actualized savings will vary depending on many factors, including the cost of biosimilar denosumab as well as proportional biosimilar denosumab uptake.

SPONSORSHIP: Sandoz, Inc.

C45 Treatment patterns in first-line treatment of metastatic gastroenteropancreatic neuroendocrine tumors: Analysis of US claims data

Pavel M[1], Abraham I[2], Halfdanarson T[3], Princic N[4], Bernhardt D[5]; marianne.pavel@uk-erlangen.de
[1]*Department of Medicine 1, Friedrich Alexander University Erlangen-Nuernberg, University Hospital Erlangen*; [2]*Matrix45 / University of Arizona*; [3]*Mayo Clinic*; [4]*Merative*; [5]*ITM Pharmasolutions GmBH*

BACKGROUND: A rare disease, gastroenteropancreatic neuroendocrine tumors (GEP-NETs) are often characterized by an indolent disease course with variable symptoms dependent on tumor location and whether the tumor is functional and producing hormones. Two-thirds of patients are diagnosed at the locally advanced or metastasized stage. Guidelines of the National Comprehensive Cancer Network specify several first-line (1L) treatment options. Somatostatin analogs (SSAs) are among the most frequently advised drugs, particularly in low-grade NETs.

OBJECTIVE: To describe 1L treatment patterns of patients with newly diagnosed mGEP-NETs in the United States.

METHODS: Retrospective cohort study using the MarketScan Commercial and Medicare Database (July 1, 2017, to January 31, 2023). Included were patients with newly diagnosed mGEP-NETs with at least 1 inpatient claim or at least 2 nondiagnostic outpatient claims for NET treatment; at least 12 months of continuous enrollment prior to diagnosis to ensure patients being newly diagnosed; and at least 12 months of follow-up after index date. Data retrieved included age, sex, insurance, and comorbidities; treatments; and treatment discontinuation, death, or loss to follow-up (estimates generally rounded to nearest integer).

RESULTS: Included were a total of 2,002 patients with newly diagnosed mGEP-NETs (50% female; median age 60 years); 65% had commercial insurance vs 35% on Medicare plans. Comorbidities included hypertension (67%), type 2 diabetes (30%), heart disease/failure (27%), and kidney disease (18%). Approximately 50% had tumor surgery in the first year after diagnosis (intestinal lesions 24%, large intestine 22%, and/or small intestine 15%; other approximately 40%). A large proportion of patients (53%) did not receive systemic drug therapy in the first 2 years after diagnosis. Of those who did, 1L treatments included SSAs (62%), mainly in monoregimens (57%); chemo regimens (36%); everolimus (3%); and peptide receptor radionuclide therapy (0.5%). Median duration of SSA 1L treatment was only 11.3 months. Although 96% received an imaging scan within 3 months of diagnosis, only 18% received the National Comprehensive Cancer Network–recommended imaging every 3-6 months in the

first 3 years of treatment. Type of insurance did not have a relevant impact on care received.

CONCLUSIONS: Treatment patterns show significant variation in how patients newly diagnosed with mGEP-NETs are managed in 1L. Adherence to 1L drug treatment guidelines is limited, suggesting a marked need for clinician and patient education and engagement with patient advocacy organizations. Health care providers and payers must consider subsequent changes in treatment patterns in their planning.

SPONSORSHIP: ITM Pharma Solutions GmbH

C53 Hematological oral oncolytics: Adherence, health care cost, and utilization

Staskon F[1], *Khan K*[2], *Pfeifer A*[3], *Havern L*[4]; *francis.staskon@walgreens.com*; *khadijah.khan@walgreens.com*
[1]*Walgreen Co.*; [2]*Walgreen Co.*; [3]*AllianceRx Walgreens Pharmacy*; [4]*Walgreens*

BACKGROUND: Connected Care Oncology (CC-Onc) is a patient-centered clinical program that includes a set of 21 unique oral hematological oncolytics for affected oncology patients.

OBJECTIVE: To identify significant associations between a discontinuation adherence metric for the set of CC-Onc hematological oral oncolytics and total medical costs, hospitalizations, and total length of stay (LOS) for inpatients. This research was deemed exempt from HIPAA by a Walgreens Advarra IRB #39505.

METHODS: A retrospective cohort design of patients was used from the MarketScan Commercial Claims and Encounters for 2022. The sample selection required patients to have at least 2 fills of targeted medication from the 2022 files with a primary cancer diagnosis code in medical files for 2021 or 2022, to be continuously enrolled, and to be aged from 18 to 64 years. Exclusion criteria were presence of hospice care or organ transplants, and those starting medication therapy in last 45 days of 2022. Discontinuation was indicated by a gap exceeding 1.5×(prior days supply) on consecutive fills. General linear models predicted total medical costs, hospitalizations, and LOS (with γ or logit links). Predictors included discontinuation, 12 covariates (including COVID-19 indications, surgeries, Charlson index comorbidities [less cancer], combination therapy, inpatient and outpatient utilization levels, demographics, and insurance type) and their interaction terms. The economic valuation compares the model predictions by discontinuation status.

RESULTS: A total of 4,312 patients in 2022 met sample criteria, with 72.2% adherent. Predicted total medical costs significantly increased for nonadherent patients compared with adherent patients ($73,663 per patient per year [PPPY], $P<0.0001$). When considering the additional costs associated with pharmacy adherence, the predicted total medical and pharmacy costs remained increased for nonadherent patients by $19,193 PPPY ($P<0.46$). Compared with adherent patients, odds of hospitalization were significantly higher (odds ratio = 2.22, $P<0.0001$) and LOS was significantly longer for nonadherent patients (4 days, $P<0.0001$). When considering the difference in hospital admissions rate and LOS when admitted, the predicted inpatient medical spend was higher for the nonadherent patients by $22,691 PPPY. Details on all significant model covariates and outcomes as well as economic valuation comparisons are presented in the poster.

CONCLUSIONS: Being adherent to oral hematological oncolytics can lead to lower medical costs, odds of hospitalization, and LOS, after controlling for many other influences on these outcomes.

SPONSORSHIP: Walgreen Co.

C54 Epidemiological burden of multiple myeloma: A systematic literature review

Ailawadhi S[1], *Hasegawa K*[2], *Itani T*[2], *Giordana M*[2], *Granados E*[2], *Donelson S*[3], *Chan R*[3], *Kostic A*[3], *Kanters S*[4], *Zoratti M*[4], *Mateos M*[5]; *ken.hasegawa1@gilead.com*
[1]*Mayo Clinic Florida*; [2]*Kite, A Gilead Company*; [3]*Arcellx, Inc.*; [4]*RainCity Analytics*; [5]*University Hospital of Salamanca/IBSAL*

BACKGROUND: Multiple myeloma (MM) is a malignant neoplasm of plasma cells with a historically poor prognosis and high societal burden.

OBJECTIVE: To describe the epidemiological profile of MM across an international landscape to contextualize its societal impacts and the anticipated impact of emerging treatments.

METHODS: Searches in Medline and Embase were conducted (January 2012 to June 2023) for population-based studies of MM incidence and prevalence from the United States, Australia, Canada, the United Kingdom, Germany, Italy, Spain, France, and Japan. Statistics from population-based registries (GLOBOCAN; US Surveillance, Epidemiology, and End Results program) were also reviewed.

RESULTS: We included 59 accounts (56 unique studies) from the 8,067 records identified during the search, covering statistics from 1974 to 2020. The United States had the highest number of studies from one country (25 studies). Based on country-level estimates, incidence varied in the literature from a low of 1.5/100,000 (Japan; 2002) to a high of 7.7/100,000 (Canada; 2013). Across geographies, within-study

longitudinal data consistently pointed to incidence increasing over time. In studies with available age-stratified data, incidence increased markedly in individuals aged 65 years and older. Prevalence was less well reported, although longitudinal and sex-stratified estimates pointed to increasing rates over time and a larger proportion of male individuals affected. Per GLOBOCAN statistics, 187,952 new cases were diagnosed globally in 2022. Although the global age-standardized incidence rate (gASR) was 1.8/100,000, country variation was observed with higher rates in Canada (gASR 5.3) and the United States (gASR 4.8) compared with Japan (gASR 1.5). Sex differences were consistently reported, with higher rates in male individuals (gASR 2.1) than in female individuals (gASR 1.5). Per US Surveillance, Epidemiology, and End Results figures, the 2021 incidence was 7.7/100,000 with a January 2021 prevalence of 179,063. Incidence was highest among Black individuals (15.7/100,000 in 2021). A projected 35,780 new cases are expected in 2024 in the United States. The 5-year relative survival rates increased from 35.6% (2000) to 61.1% (2016), with a significant average absolute change of 0.4% modeled for 2015-2020 in the United States.

CONCLUSIONS: Despite geographical heterogeneity, the incidence of MM is increasing globally. Age (≥65 years), sex (male), and race (Black) were consistent risk factors across data sources. As survival rates improve and the number of cases rises worldwide, the MM-associated burden is anticipated to rise with a need for improved therapeutics to mitigate this burden.

SPONSORSHIP: Kite Pharma/Arcellx

C55 Ibrutinib dosage form and product strength optimization results in savings through a managed care pharmacist clinical management tool

Friedlander N, Curtis K, Gleason P;
nicholas.friedlander@primetherapeutics.com
Prime Therapeutics

BACKGROUND: Oral cancer therapy costs have continued to grow, underscoring the need for enhanced pharmacy clinical management programs to encourage cost-effective drug therapy. A drug therapy optimization savings opportunity exists for ibrutinib owing to differential pricing for the varying product strengths and dosage forms. Managed care pharmacist (MCP) outreach can result in optimized ibrutinib product selection to reduce unnecessary expenditures.

OBJECTIVE: To encourage that the most cost-effective per-milligram product formulation is used, we developed dispensing optimization logic targeting ibrutinib high-cost-per-milligram formulations and integrated this logic into an MCP web application tool designed to facilitate MCP-to-provider outreach.

METHODS: From the 14.5 million commercially insured members enrolled in the MCP outreach program, during September 2022 to April 2024, pharmacy claims review occurred weekly to identify ibrutinib utilizers and their most recently dispensed ibrutinib product. Cases involving members using a non–cost-optimized ibrutinib strength/dosage form were made available to MCPs through a web application. Once current dose and product selection was confirmed through claims history review, MCPs outreached to prescribers involved in member care to propose a change to the lowest-cost-per-milligram product. Case notes were documented in the web tool. Total savings were calculated based on the annualized cost of current therapeutic regimen vs annualized cost of adjusted regimen.

RESULTS: Seven hundred forty-three unique members with at least 1 paid pharmacy claim for ibrutinib between September 2022 and April 2024 were identified. Across these 743 members, 94 (12.7%) were found to have non–cost-optimized ibrutinib utilization and were reviewed by MCP. Thirty-nine (41.5%) of 94 identified cases resulted in validated savings, generating a total of $4.07 million in payer savings. Fourteen (14.9%) outreaches were performed without success and 24 (25.5%) were determined to not be true opportunities at the time of MCP review. An additional 8 (8.5%) cases are in progress with an estimated total savings of $692,200. Nine (9.6%) had a change in eligibility between the time of identification and MCP review.

CONCLUSIONS: Opportunities exist to encourage cost-effective ibrutinib therapy optimization through an MCP-to-provider outreach program. The program delivers ongoing pharmacy claims data surveillance and savings opportunity notification to MCPs. This program resulted in a 41% success rate in changing ibrutinib to a low-cost-per-milligram dosage form/strength, saving $4.1 million.

SPONSORSHIP: Prime Therapeutics LLC

C56 Comparative cost-per-responder analysis of ciltacabtagene autoleucel and real-world standard-of-care therapy in patients with lenalidomide-refractory multiple myeloma

Hansen D[1], *Castaneda Puglianini O*[1], *Zhang E*[2], *Usmani S*[3], *Sachdev R*[2], *Perciavalle M*[4], *Jagannath S*[5], *Lu X*[6], *De Wiest D*[7], *Huo S*[6], *Lee S*[8], *Qureshi Z*[9]; *doris.hansen@moffitt.org*
[1]*H. Lee Moffitt Cancer Center and Research Institute*; [2]*Evidera*; [3]*Memorial Sloan Kettering Cancer Center*; [4]*Legend Biotech*; [5]*Icahn School of Medicine at Mount sinai*; [6]*Johnson & Johnson*; [7]*Johnson and Johnson Innovative Medicine*; [8]*Johnson and Johnson*; [7]*Johnson and Johnson Innovative Medicine*

BACKGROUND: In the CARTITUDE-4 (NCT04181827) trial, ciltacabtagene autoleucel (cilta-cel) demonstrated improved efficacy vs trial-based standard of care (SOC).

OBJECTIVE: To assess—using a cost-per-responder (CPR) model—the treatment value of cilta-cel vs real-world SOC (RW-SOC).

METHODS: CPR analyses from a US mixed payer perspective were conducted comparing the direct medical cost (2024 USD) per patient receiving cilta-cel vs RW-SOC, based on US clinical practice (CancerMPact) and indirect treatment comparisons (ITC) availability. RW-SOC comprised daratumumab+pomalidomide+dexamethasone (25.1%), daratumumab+carfilzomib+dexamethasone (22.8%), bortezomib+dexamethasone (15.4%), daratumumab+bortezomib+dexamethasone (12.9%), carfilzomib+dexamethasone (11.9%), pomalidomide+dexamethasone (5.4%), selinexor+bortezomib+dexamethasone (4.8%), and elotuzumab+pomalidomide+dexamethasone (1.8%). For cilta-cel, progression-free survival (PFS), overall survival (OS), and complete response (CR) were from CARTITUDE-4. A standard parametric approach extrapolated survival beyond the trial period. For RW-SOC, clinical endpoints were derived via ITC vs cilta-cel. Resource use, drug acquisition, administration, monitoring, adverse event management, subsequent therapy, and terminal care costs were included. The base case assumed 100% inpatient cilta-cel infusion with 76.7% commercial and 23.3% Medicare payer mix over 3 years. Outcomes included total cost per treated patient, total cost per CR, and cost per month (CPM) in PFS.

RESULTS: Total cost per treated patient was estimated as $760,313 for cilta-cel and $740,794 for RW-SOC. Treatment acquisition and subsequent therapy were key cost drivers during PFS and postprogression survival, respectively. Total costs per CR and total CPM in PFS were estimated to be lower for cilta-cel ($1,027,451 and $25,122) than RW-SOC ($4,636,587 and $37,255). Assuming 30% outpatient cilta-cel infusion led to lower cost per CR and CPM in PFS for cilta-cel ($1,020,123 and $24,905). The CPM in PFS over 5 years was favorable for cilta-cel vs RW-SOC ($17,137 and $36,313). Payer mix with 31% commercial and 69% Medicare resulted in lower total costs per CR and total CPM in PFS for cilta-cel ($986,402 and $24,188) than RW-SOC ($4,396,770 and $35,074). As clinical inputs are based on multinational trials, generalizability to the US setting may be limited.

CONCLUSIONS: The cost per CR and CPM in PFS for cilta-cel were considerably lower than that for RW-SOC, demonstrating the substantial clinical and economic benefits cilta-cel offers patients with relapsed/refractory multiple myeloma.

SPONSORSHIP: Janssen Scientific Affairs, LLC, Legend Biotech USA Inc.

C57 Cost per median month of overall survival comparison of elranatamab-bcmm and teclistamab-cqyv in adult patients with relapsed or refractory multiple myeloma

Shah B[1], *Sandin R*[2], *Liu Y*[3], *Hu Y*[3], *Hughes D*[4], *Hart J*[4], *Hlavacek P*[4]; *bhavesh.shah@bmc.org*
[1]*Boston Medical Center*; [2]*Pfizer AB*; [3]*Cytel*; [4]*Pfizer Inc*

BACKGROUND: Elranatamab-bcmm (ELRA) and teclistamab-cqyv (TEC) are bispecific antibodies granted US Food and Drug Administration accelerated approval for the treatment of adult patients with relapsed or refractory multiple myeloma (RRMM) based on high and durable response rates. Clinical studies showed that median progression-free survival (PFS) and overall survival (OS) were 17.2 and 24.6 months with ELRA and 11.3 and 21.9 months with TEC, respectively.

OBJECTIVE: To compare the economic value of ELRA and TEC in terms of costs per median month of OS.

METHODS: Inputs were based on the most recent clinical data from MagnetisMM-3 (Cohort A) and MajesTec-1, US prescribing information, US government databases, and published literature. The comparison included primary drug costs and administration, allowing for switching to biweekly treatment as per label and literature. Costs of monitoring, medical resource use, grade 3/4 adverse events, all-grade cytokine release syndrome and immune effector cell–associated neurotoxicity syndrome, and subsequent treatment costs were also incorporated. Mean treatment duration (TD) was estimated using exponential extrapolation of the median TD from the clinical trials. Total primary drug cost and total cost of care over a 2-year time period was assessed. Costs per median month of OS for each treatment were calculated by dividing primary drug costs and total costs of treatment by the median OS from each trial,

respectively. Additionally, a scenario analysis was conducted with the assumption that both patients receiving ELRA and those receiving TEC were switched to biweekly dosing at week 25.

RESULTS: Primary drug acquisition costs ($332,835 vs $474,234) and total costs of care ($396,944 vs $558,663) were lower with ELRA vs TEC, explained by earlier switch to biweekly dosing, shorter TD, lower relative dose intensity, and lower hospitalization costs for step-up dosing. As a result, primary drug costs per median month of OS ($13,530 vs $21,655) and total costs of care per median month of OS ($16,136 vs $25,510) were lower for ELRA compared with TEC. In the scenario analysis, both total primary drug costs per median month of OS ($13,530 vs $15,744) and total costs of care per median month of OS ($16,136 vs $19,599) were lower with ELRA compared with TEC.

CONCLUSIONS: This analysis suggests ELRA may offer a lower cost of care per median month of OS compared with TEC, including a scenario with earlier biweekly dosing with TEC. However, the clinical effect of earlier dose switching with TEC is unknown from MajesTec-1.

SPONSORSHIP: Pfizer

C58 Health care resource utilization and costs associated with adverse events among commercially insured patients with chronic myeloid leukemia treated with tyrosine kinase inhibitors

Jabbour E[1], *Jadhav K*[2], *Yang H*[3], *Song W*[4], *Shi S*[4], *Li H*[4], *McDermott C*[5], *Yang D*[2], *Damon A*[2], *Sadek I*[2], *Ferreira G*[6], *Wei D*[2]; *ejabbour@mdanderson.org*
[1]*MD Anderson Cancer Center*; [2]*Novartis Pharmaceuticals Corporation*; [3]*Analysis Group, Inc.*; [4]*Analysis Group*; [5]*Novartis Ireland Ltd*; [6]*Novartis Pharma AG*

BACKGROUND: Despite the therapeutic benefits of first-generation (1G) and second-generation (2G) tyrosine kinase inhibitors (TKIs), approximately one-fifth of patients discontinue these treatments because of adverse events (AEs), potentially leading to increased health care resource utilization (HCRU) and costs.

OBJECTIVE: To estimate HCRU and costs associated with commonly observed AEs among patients with chronic myeloid leukemia (CML) receiving 1G or 2G TKIs across all lines of therapy.

METHODS: Patients with CML receiving 1G (imatinib) or 2G TKIs (dasatinib, nilotinib, or bosutinib) between July 2016 and March 2023 were selected from the IQIVIA PharMetrics Plus database. Demographic and clinical characteristics were assessed during the baseline period (6 months prior to treatment initiation). AEs, HCRU, and total costs (including medical and pharmacy costs) were examined for up to 6 months during the period when patients were receiving the index treatment (either 1G or 2G TKIs) as any line of therapy. Annualized incremental HCRU and costs comparing patients with vs without each AE of interest were estimated based on regression models adjusting for baseline characteristics and index TKIs.

RESULTS: A total of 2,546 patients were included, with a median age of 53.0 years (range: 18.0-86.0), and 43.8% were female. Approximately one-third of the patients received 1G TKIs (34.6%) and two-thirds 2G TKIs (45.0% dasatinib, 14.8% nilotinib, and 5.6% bosutinib) as the index treatment. More than 80% of the patients had mild (41.5%) or moderate (40.5%) Darkow disease complexity index (absence of severely complicating conditions) during the baseline period. Common AEs of any grade (≥5% prevalence) included abnormal glucose (20.8%), fatigue (18.7%), nausea/vomiting (11.7%), cardiac arrhythmias (11.3%), thrombocytopenia (11.1%), headache (8.5%), peripheral edema (8.2%), diarrhea (7.8%), coronary artery diseases (7.6%), rash (6.8%) and pleural effusion (6.4%). Annualized total incremental costs significantly associated with the above AEs ranged from $27,769 (diarrhea) to $91,526 (thrombocytopenia), which were mainly driven by difference in annualized medical costs ($26,587 for diarrhea and $95,614 for thrombocytopenia). Significant differences in the annualized number of inpatient admissions between those with and without the above AEs ranged from 0.16 (fatigue) to 0.76 (pleural effusion).

CONCLUSIONS: Substantial HCRU and costs were observed for AEs during treatment with 1G or 2G TKIs. The findings highlight the need for appropriate management of AEs and more tolerable treatment regimens to reduce the financial and humanistic burden on patients with CML.

SPONSORSHIP: Novartis Pharmaceuticals Corporation

C59 Impact of social risk factors on first-line treatment patterns among Medicare Advantage members with newly diagnosed multiple myeloma

Daviano A[1], *Xu Y*[2], *Suehs B*[2], *Wojcicki S*[3], *Harper J*[4], *Brunisholz K*[4]; *adaviano@humana.com*
[1]*Humana Healthcare Research, Inc.*; [2]*Humana Healthcare Research, Inc.*; [3]*Humana Inc.*; [4]*Johnson & Johnson*

BACKGROUND: Despite first-line treatment (1L Tx) advances in multiple myeloma, disparities in care continue to exist. Assessing the impact of social risk factors on Tx patterns is necessary to identify potential gaps in care and is aligned with Centers for Medicare & Medicaid Services (CMS) health equity priorities for reducing health disparities.

OBJECTIVE: To describe 1L Tx patterns among Medicare Advantage (MA) members with newly diagnosed multiple myeloma (NDMM) and the impact of dual eligibility and low-income subsidy (DE/LIS).

METHODS: This is a retrospective study using claims data from Humana Inc., the second largest MA insurer in the United States covering more than 8 million individuals. MA members aged 65-89 years and with NDMM were identified between January 1, 2017, and February 28, 2023. 1L was identified via a claims-based algorithm. Patient characteristics and 1L Tx patterns (regimens, time to initiation [TTI], duration of Tx [DOT]) were described. DE/LIS was used as a proxy measure of social risk. The relationships between DE/LIS status and race with Tx pattern variation were assessed.

RESULTS: A total of 4,483 members met the study criteria. The mean age was 75.2 years; 49.9% were female; 24.1% had DE/LIS; 37.5% were non-White race; and 72.8% had at least 1 MM symptom (hypercalcemia, renal impairment, anemia, and/or bone pain). Within 3 months of NDMM, 51.2% received 1L Tx; median TTI was 2.7 months. DE/LIS vs non-DE/LIS members had a lower rate of treatment within 3 months (44.9%, CI = 41.9%-47.9% vs 53.1%, CI = 51.5%-54.8%) and longer median TTI (6.6 months, CI = 3.7-17.7 months vs 2.2 months, CI = 1.9-2.6 months). DE/LIS status (hazard ratio [HR] = 0.80, CI = 0.72-0.87) and Black race (HR = 0.90, CI = 0.83-0.98, ref. White race) were associated with longer TTI after controlling for potentially confounding factors. A total of 2,523 (56.3%) members were treated within 12 months of NDMM; among them, 90.7% initiated Tx within 3 months, median TTI was 0.9 months, and median DOT was 10.5 months. From 2017 to 2022, 1L triple+ therapy increased from 59.0% to 76.8%, and there were no significant differences in 1L Tx regimen or DOT by race or DE/LIS status. Among these 2,523 individuals, Black race (HR = 0.85, CI = 0.78-0.93, ref. White race) but not DE/LIS was associated with longer TTI after controlling for confounding factors.

CONCLUSIONS: A longer TTI was observed for DE/LIS beneficiaries, indicating potential disparities. Among those receiving treatment within 12 months, no differences in 1L Tx regimen, DOT, or TTI were observed. These results may inform health policy decisions, quality improvement, resource deployment, and initiatives to address health disparities.

SPONSORSHIP: Johnson & Johnson

C60 Adverse events and payer health care costs in newly diagnosed older patients with chronic myeloid leukemia: A real-world data analysis of the Medicare Fee-for-Service population

Kota V[1], Wei D[2], Zhao Y[3], Singh S[3], Tomicki S[3], Shao A[4], Shrestha S[5], McDermott C[6], Marquez G[2], Palli S[7], Yang D[2], Damon A[2], Jadhav K[2]; vkota@augusta.edu; david-1.wei@novartis.com
[1]Wellstar MCGHealth; [2]Novartis Pharmaceuticals Corporation; [3]Genesis Research Group; [4]Genesis Research; [5]GRG; [6]Novartis Ireland Ltd; [7]Novartis Pharmaceuticals, Inc.

BACKGROUND: Tyrosine kinase inhibitors (TKIs) are standard therapy for patients with chronic myeloid leukemia (CML). The recent treatment landscape evolved with the approval of new TKIs. Despite the effectiveness of TKIs, little is known about real-world tolerance and cost of adverse events (AEs) in older patients with CML.

OBJECTIVE: To describe the prevalence of AEs and total payer health care cost in older (aged ≥65 years) patients with CML who received first-generation (1G) or second-generation (2G) TKIs as their first TKI in a real-world setting.

METHODS: This is a retrospective analysis in senior patients with CML who received their first TKI between January 1, 2017, and June 30, 2022, using the US Centers for Medicare & Medicaid Services 100% Medicare Fee-for-Service database. AE prevalences were described, and overall payer health care costs (medical and pharmacy) were compared among patients with and without AEs.

RESULTS: Overall, 1,846 patients with CML received 1 or more TKIs. Patients who received 1G TKI (n = 1,108) had a mean age (SD) of 75.4 (7.9) years, 53.2% were male, and 24.9% resided more than 25 miles from their prescribing provider. Of the 738 who received 2G TKI, the mean age (SD) was 73.6 (7.6) years, 55.6% were male, and 22.9% resided more than 25 miles from their prescribing provider. During the mean (SD) time on treatment of 18.5 (16.9) months for 1G TKI patients and 14.4 (14.9) months for 2G TKI patients, the most prevalent AEs were fatigue (1G: 41.1%; 2G: 34.8%), pleural effusion (1G: 18.7%; 2G: 34.6%), peripheral edema (1G: 41.0%; 2G: 26.2%), nausea/vomiting (1G: 26.8%; 2G: 20.5%), constipation (1G: 16.3%; 2G: 18.7%), diarrhea (1G: 26.8%; 2G: 18.4%), and renal insufficiency (1G: 21.6%; 2G: 17.8%). Total payer health care costs were higher for patients with an AE vs those without, including for pleural effusion (unadjusted mean cost difference: 1G: $40,970; 2G: $27,720), peripheral edema (1G: $20,183; 2G: $18,515), nausea/vomiting (1G: $20,087; 2G: $7,192), constipation (1G: $19,143; 2G: $24,030), diarrhea (1G: $9,978; 2G: $18,671), and renal insufficiency (1G: $54,726; 2G: $41,570). Patients with fatigue had similar total payer health care costs to patients without.

CONCLUSIONS: Across generations of TKIs, common AEs were associated with higher payer cost burden, suggesting a need for less toxic CML treatments and more effective AE management in newly diagnosed senior patients with CML receiving their first TKI.

SPONSORSHIP: Novartis Pharmaceuticals Corporation

C61 Treatment adherence and persistence among patients with chronic lymphocytic leukemia/small lymphocytic lymphoma receiving first-line Bruton tyrosine kinase inhibitors

Majd Z, Cheng N, Manthena S, Franceschini E, Bacchus S, Srivastava B; zahra.majd@abbvie.com
AbbVie, Inc.

BACKGROUND: Treatment for chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL) has evolved rapidly with approval of novel agents such as Bruton tyrosine kinase inhibitors (BTKis). Early adherence to ibrutinib (ibr) and acalabrutinib (acala) was previously shown to be similar; however, data related to treatment compliance to other BTKis are limited.

OBJECTIVE: To report real-world adherence and persistence to first-line (1L) ibr, acala, or zanubrutinib (zanu) in patients with CLL/SLL.

METHODS: Adult patients with CLL/SLL initiating 1L (index date) single-agent ibr, acala, or zanu were identified using Optum's deidentified Clinformatics Data Mart database between November 21, 2018, and September 30, 2023. Baseline characteristics were assessed during the 12 months of continuous eligibility (baseline period) prior to the index date. Adherence to 1L therapy was defined as a greater-than-or-equal-to 80% proportion of days covered (PDC) for the index treatment at 1, 2, and 3 months post-index date until initiation of second-line treatment, death, or end of continuous enrollment or data. Persistence was defined as no treatment gap of more than 30 days. The proportion of patients adherent and persistent to ibr, acala, or zanu were compared using logistic regression models controlling for age, sex, 1L regimen, Charlson Comorbidity Index (CCI), total all-cause health care costs (patient- + payer-paid), and baseline atrial fibrillation.

RESULTS: A total of 1,951 patients initiated 1L treatment (ibr: 955 patients; acala: 865 patients; zanu: 131 patients). In the ibr, acala, and zanu cohorts, 56%, 61%, and 60% of patients, respectively, were men; median age was 75 years, and median CCI score was 3 in all 3 cohorts. Median time of follow-up was 23.3 months (ibr), 14.6 months (acala), and 4.7 months (zanu). In patients who had at least 3 months of follow-up, mean PDC (1 month, 0.97, 0.98, 0.98; 2 months, 0.91, 0.89, 0.86; 3 months, 0.89, 0.87, 0.84, respectively), adherence (1 month, 99.9%, 99.8%, 100%; 2 months, 85.3%, 81.3%, 78.4%; 3 months, 82.6%, 77.9%, 72.8%), and persistence rates (1 month, 90.1%, 87.9%, 84.7%; 2 months, 84.7%, 81.4%, 80.6%; 3 months, 77.7%, 76.3%, 77.6%) varied across the 3 BTKis. In an adjusted logistic regression model, patients who received acala or zanu were less likely to be adherent at 3 months than those who received ibr (odds ratio [95% CI] = 0.76 [0.59-0.98] and 0.60 [0.36-0.99], respectively).

CONCLUSIONS: This real-world study provides insights into adherence and persistence in patients with CLL/SLL initiated on 1L single-agent BTKis. This study did not observe better treatment compliance with acala or zanu in comparison with ibr.

SPONSORSHIP: Pharmacyclics LLC, an AbbVie company

C62 Treatment of newly diagnosed Philadelphia chromosome–positive acute lymphoblastic leukemia with tyrosine kinase inhibitors in combination with chemotherapy: A patient-centered treatment comparison using outcomes from a discrete choice experiment and the PhALLCON study

Ashaye A[1], Thomas C[2], Krucien N[2], kota V[3], Marsh K[2]; ajibade.ashaye@takeda.com
[1]Takeda Development Center Americas, Inc.; [2]Evidera; [3]Wellstar MCGHealth

BACKGROUND: Prognosis for adults with Philadelphia chromosome–positive acute lymphoblastic leukemia (Ph+ ALL) has improved with tyrosine kinase inhibitors (TKIs) and chemotherapy. However, treatment involving TKIs was not US Food and Drug Administration (FDA) approved for frontline use until recently. In March 2024, the FDA granted accelerated approval to ponatinib in combination with chemotherapy for adults with newly diagnosed Ph+ ALL based on the PhALLCON study, the first phase 3 head-to-head trial of TKIs in this population. Understanding patients' preferences and trade-offs is important because of the varied benefit-risk profiles of TKIs.

OBJECTIVE: To evaluate preferences of patients with Ph+ ALL for frontline treatment and to quantify the impact of additional overall survival (OS) and duration of response (DOR) on patients' expected treatment choice.

METHODS: Two hundred one US adults (aged ≥18 years) with self-reported Ph+ ALL completed an online discrete choice experiment (DCE) between February and April 2020. Patients chose their preferred treatment option between 2 hypothetical profiles with varied levels of benefits and risk attributes, namely, OS (30-90 months), DOR (15-75 months), and risks of a major cardiovascular (CV) event risk (0%, 25%, 50%)

and myelosuppression (0%, 50%, 100%). A multinomial logit model was used to analyze preferences. A patient-centered treatment comparison was conducted using preferences elicited in the DCE and interim data from PhALLCON (ponatinib: 1.84% major CV event risk, 40.5% myelosuppression risk, DOR immature, OS immature; imatinib: 1.2% major CV event risk, 54.3% myelosuppression risk, DOR 22.32 months, OS immature). Given immature efficacy data, bivariate sensitivity analyses were conducted to assess the impact of DOR and OS on expected treatment choice.

RESULTS: Mean age was 44.8 ± 12.9 years. Patients prioritized increasing OS and DOR, placing less weight on reducing the risks of major CV events and myelosuppression. The patient-centered treatment comparison found ponatinib to be a significantly preferred TKI profile, relative to imatinib, for patients with Ph+ ALL on average, even in a scenario of equivalent DOR and OS (52.92% [95% CI = 52.47-53.38] probability of being selected). This is due to the 13.8% lower risk of myelosuppression with ponatinib, which outweighs the 0.64% higher risk of a major CV event from the patient perspective. For scenarios where ponatinib offers higher DOR and/or OS, the likelihood of ponatinib being the preferred option increases. For example, if ponatinib offered 15 months longer OS and 10 months longer DOR, there is a 68.27% (95% CI = 64.60-71.94) probability of ponatinib being selected.

CONCLUSIONS: Based on elicited preferences in a DCE and interim results from the PhALLCON study, a patient-centered treatment comparison finds ponatinib to be a significantly preferred TKI, relative to imatinib, for adults with Ph+ ALL.

SPONSORSHIP: Takeda Development Center Americas, Inc.

D2 Health care resource use in the United States for iron deficiency anemia among patients treated with intravenous iron replacement therapy

Engel-Nitz N[1], *Wang K*[2]; *nicole.engel-nitz@optum.com*
[1]*Optum Life Sciences*; [2]*Daiichi Sankyo US*

BACKGROUND: For patients with chronic diseases such as cancer, heart failure (HF), or chronic kidney disease (CKD), intravenous (IV) iron replacement therapy is often required for treatment of iron deficiency anemia (IDA). However, there are limited real-world data on treatment patterns associated with IV iron treatment.

OBJECTIVE: To assess resource use in patients with cancer, HF, or CKD treated with ferric carboxymaltose (FCM) or treated with low-dose IV iron (LDI) therapy.

METHODS: We studied commercial and Medicare Advantage patients from the Optum Research Database, a US administrative claims database. Patients were included if they had cancer, HF, or CKD, and an IDA diagnosis; index date was first treatment with IV iron therapy (2017-2019). Continuous enrollment was required for 6 months pre- and 12 months post-index (<12 months retained if due to death). Treatment cohorts were FCM and LDI (iron sucrose, iron dextran, or sodium ferric gluconate complex in sucrose). Health care utilization assessed included blood transfusions and IDA-related health care visits.

RESULTS: Patients with cancer (n = 10,763) included 5,892 FCM and 4,871 LDI patients. Pre-index, 21% of FCM and 25% of LDI received blood transfusions ($P < 0.001$). Over follow-up, 22% of FCM and 32% of LDI received blood transfusions ($P < 0.001$). The mean (SD) number of transfusions for FCM was 15 (23) vs 19 (26) for LDI ($P < 0.001$). IDA-related hospitalizations occurred among 22% of FCM vs 31% of LDI ($P < 0.001$). Patients with CKD (n = 10,617) included 4,377 FCM and 6,240 LDI patients. Pre-index, 21% of FCM and 23% of LDI received blood transfusions ($P = 0.005$). Over follow-up, 23% of FCM and 32% of LDI received blood transfusions ($P < 0.001$). The mean (SD) number of transfusions for FCM was 19 (29) vs 21 (32) for LDI ($P < 0.05$). IDA-related hospitalizations occurred among 28% of FCM vs 40% of LDI patients ($P < 0.001$). Patients with HF (n = 8,337) included 3,364 FCM and 4,973 LDI patients. Pre-index, 27% of FCM and 29% of LDI received blood transfusions ($P = 0.039$). Over follow-up, 27% of FCM and 37% of LDI received blood transfusions ($P < 0.001$). The mean (SD) number of transfusions for FCM was 20 (30) vs 22 (32) for LDI (NS). IDA-related hospitalizations occurred among 28% of FCM vs 40% of LDI patients ($P < 0.001$).

CONCLUSIONS: Patients with cancer, CKD, or HF treated with FCM had lower use of blood transfusions and IDA-related hospitalization after starting therapy compared with patients treated with LDI, suggesting FCM's impact on real-world health care utilization.

SPONSORSHIP: Daiichi Sankyo, Inc.

D4 A systematic literature review of clinical outcomes among patients with paroxysmal nocturnal hemoglobinuria

Waheed A[1], *Kaufhold S*[2], *Nellesen D*[2], *Chanpura M*[3], *Geevarghese A*[4], *Yen G*[4]; *glorian.yen@novartis.com*
[1]*Weill Cornell Medical Center*; [2]*Analysis Group, Inc.*; [3]*Novartis Pharmaceuticals Corporation*; [4]*Novartis Pharmaceutical Corp.*

BACKGROUND: Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, acquired hematopoietic stem cell disorder characterized by abnormal complement-mediated hemolysis, resulting in low hemoglobin (Hb) and high lactate dehydrogenase (LDH) levels, as well as symptoms including

fatigue, dyspnea, and pain. Various measures are used to assess treatment effectiveness in PNH.

OBJECTIVE: To systematically collect and summarize outcomes reported in clinical trials of patients with PNH.

METHODS: Systematic searches for English-language articles in Medline, Embase, and Cochrane libraries were initially conducted in January 2022 and then updated in April 2024 with no date restriction, supplemented by a manual search of recent major conferences. Studies were screened for relevance by 2 researchers independently, with disagreements adjudicated by a third reviewer. Studies were screened at 2 levels (title/abstract and full text) using the same prespecified Population, Intervention, Comparison, Outcomes, and Study criteria. This systematic literature review is registered with PROSPERO (CRD42022314640).

RESULTS: Overall, 2,119 unique publications were identified, among which 96 publications describing results from 26 clinical trials were relevant to this analysis. Both previously treated and treatment-naive patients with PNH were represented. Fourteen clinical trials evaluated US Food and Drug Administration–approved treatments for PNH (iptacopan, danicopan, pegcetacoplan, ravulizumab, eculizumab). Remaining trials assessed investigational agents or biosimilars. The majority (62%) of trials were phase 3, and the average sample size was 79 patients (range: 6-246). All trials were open-label except TRIUMPH (eculizumab vs placebo), ALPHA (danicopan vs placebo as add-on therapy), and 2 equivalence studies. Thirteen trials were randomized. Primary endpoints were heterogeneous, as were methods of reporting outcomes. Eleven trials had primary endpoints related to LDH levels, whereas 9 related to Hb levels. During study follow-up, mean LDH levels ranged from 150.4 to 2,419 U/L, and mean Hb levels ranged from 7.6 to 12.8 g/dL. Other methods of reporting outcomes included median laboratory values, ratios relative to the upper limit of normal, changes from baseline, and proportions of patients achieving a set threshold. Patient-reported outcomes were published for 20 trials, and transfusion data were published for 22 trials.

CONCLUSIONS: This systematic literature review has identified gaps and inconsistencies in the reporting of PNH outcomes across trials. Standardization of clinical assessment and reporting may facilitate a more comprehensive understanding of the burden associated with PNH.

SPONSORSHIP: Novartis

D5 Real-world treatment patterns among patients with paroxysmal nocturnal hemoglobinuria initiating eculizumab and ravulizumab: A US claims analysis

Lin C, Wang R, Tzeng E; lin.chiawei@gene.com
Genentech, Inc.

BACKGROUND: Eculizumab (ECU), ravulizumab (RAVU), and pegcetacoplan (PEG) are current drug treatment options for patients with paroxysmal nocturnal hemoglobinuria (PNH).

OBJECTIVE: To describe real-world treatment patterns for patients with PNH initiating ECU or RAVU in the United States.

METHODS: This is a retrospective cohort study using the IQVIA PharMetrics Plus claims database (December 21, 2018, to June 30, 2023). Eligible adults (aged ≥18 years) with at least 2 claims of ECU, RAVU, or PEG were identified. The index date was the earliest drug claim, with a 6-month prior period with no drug claims. Continuous medical and pharmacy coverage for 6 months before and 3 months after index were required. Patients with a non-PNH US Food and Drug Administration–approved diagnosis for ECU, RAVU, or PEG or who received an investigational treatment were excluded. PEG patients (n = 2) were excluded from the analysis owing to small sample size. Treatment discontinuations were defined as having a greater-than-42-day gap between infusions for ECU and a greater-than-112-day gap for RAVU in the maintenance phase (after Day 29 and Day 15 post-index for ECU and RAVU, respectively). ECU patients were considered to have dose escalation if they received more than 900 mg or had at least 2 infusions within 12 days. Dose escalation for RAVU was defined as having at least 2 infusions within 49 days or a dose increase of more than 300 mg. Patient characteristics and treatment patterns were summarized descriptively. The Kaplan-Meier method was used to evaluate time to treatment discontinuation from the index.

RESULTS: The study cohort consisted of 49 ECU and 69 RAVU patients, with a mean age of 42 years and 56% female. Approximately 70% of ECU patients discontinued at 1 year post-initiation (median time to discontinuation: 205 days). For RAVU, 13% and 30% discontinued treatment at 1 year and 2 years, respectively (median time to discontinuation: not reached). In the maintenance phase, 35% and 21% of ECU and RAVU patients experienced dose escalation. Among ECU infusions, 53% were at an escalated dose (>900 mg); 75% of infusions were given in 13-15 days, whereas 6% were given less than or equal to 12 days apart. Although most of the RAVU infusions (69%) were given 8 weeks apart, 26% of RAVU infusions were received at longer-than-label-recommended intervals

CONCLUSIONS: The majority (70%) of patients with PNH who initiated ECU discontinued the treatment within 1 year, and 35% experienced dose escalation. RAVU patients had fewer discontinuations, but 26% of RAVU infusions were administered at longer-than-label-recommended intervals, which may be due to scheduling issues and could potentially lead to inadequate disease control.

SPONSORSHIP: Genentech, Inc.

D6 Adults with sickle cell disease have increased health care resource utilization and costs compared with matched controls: Retrospective analysis of a Medicare and Medicaid database

Barcelos G[1], *Peixoto T*[2], *Alvir J*[3], *Lingohr-Smith M*[4], *Lin J*[4], *Baker C*[3]; *giovanna.barcelos@pfizer.com*
[1]*Pfizer AG*; [2]*Pfizer Portugal*; [3]*Pfizer Inc*; [4]*Novosys Health*

BACKGROUND: Sickle cell disease (SCD) is a lifelong inherited blood disorder, and patients typically have numerous complications throughout their lives. Increased health care resource utilization (HCRU) and costs have been reported for US patients with commercial insurance coverage, yet the majority of Americans with SCD are enrolled in Medicare and/or Medicaid.

OBJECTIVE: To compare HCRU and costs for US adults with SCD and matched controls without SCD enrolled in Medicare and/or Medicaid.

METHODS: Patients and controls (matched 1:1 for age, sex, and race) from the United States were retrospectively identified from the IBM MarketScan Medicaid and Medicare Database. Eligible patients were aged 18 years and older with at least 3 inpatient or outpatient SCD diagnoses (*International Classification of Diseases, Tenth Revision* codes D57.0-D57.219; D57.4-D57.819) during an index-identification period (July 1, 2016, to December 31, 2019; earliest code was the index; an index was randomly selected for controls). Continuous enrollment at least 6 months pre-index and at least 12 months post-index was required. All-cause HCRU and costs (inflated to 2020 US dollars) per patient per year (PPPY) were compared using analysis of variance.

RESULTS: For 5,597 patients and 5,597 matched controls, mean (SD) age was 35.1 (13.2) years and 60% were female. During the mean (SD) follow-up for the SCD cohort of 38.8 (15.3) months, 33.5% received hydroxyurea, 43.5% had at least 1 blood transfusion, and 85.2% were prescribed opioid pain medication. During follow-up, the most common acute and chronic complication of SCD, respectively, were vaso-occlusive crisis (80.7%) and avascular necrosis (26.8%). During the 12 months post-index, for patients vs controls, mean (SD) PPPY number of inpatient hospitalizations was 2.1 (3.6) vs 0.2 (0.5), length of stay 11.3 (22.7) vs 0.7 (3.2) days, outpatient claims 46.5 (69.8) vs 22.5 (60.5), emergency department (ED) visits 6.5 (13.4) vs 1.1 (2.8), and prescriptions 29.5 (39.7) vs 13.7 (30.8); all $P < 0.0001$. Mean (SD) costs PPPY for inpatient stays were $31,810 ($93,902) vs $1,700 ($14,532), outpatient including ED $9,625 ($17,080) vs $3,347 ($12,642), pharmacy $3,679 ($16,473) vs $992 ($17,178), and total costs of $45,114 ($101,927) vs $6,039 ($26,811); all $P < 0.0001$. Total out-of-pocket (OOP) payments PPPY were $205 ($886) for patients with SCD vs $32 ($192) for controls ($P < 0.0001$).

CONCLUSIONS: In this analysis of Medicare and Medicaid claims, adults with SCD had significantly increased HCRU vs matched controls. Payer and OOP costs were higher across all categories, with total costs for the SCD cohort greater than 7-fold those of controls. Overall, the results highlight the substantial clinical burden and economic impact of SCD in the United States.

SPONSORSHIP: Pfizer

D7 Real-world health care resource utilization and costs for children with sickle cell disease in the United States: Retrospective Medicaid analysis

Barcelos G[1], *Peixoto T*[2], *Alvir J*[3], *Lingohr-Smith M*[4], *Lin J*[4], *Baker C*[3]; *giovanna.barcelos@pfizer.com*
[1]*Pfizer AG*; [2]*Pfizer Portugal*; [3]*Pfizer Inc*; [4]*Novosys Health*

BACKGROUND: Sickle cell disease (SCD) is a lifelong, inherited blood disorder with complications leading to higher morbidity and mortality. Symptoms typically begin in the first year of life, yet limited information has been reported for socioeconomic pediatric burden of illness.

OBJECTIVE: To evaluate health care resource utilization (HCRU) and costs for US children with SCD and matched controls without SCD, using Medicaid data.

METHODS: US children with SCD and controls (matched 1:1 for age, sex, and race) were retrospectively identified from the IBM MarketScan Medicaid Database between January 1, 2016, and December 31, 2020. Eligible patients were aged younger than 18 years with at least 3 inpatient or outpatient SCD diagnoses (*International Classification of Diseases, Tenth Revision* codes D57.0-D57.219; D57.4-D57.819) during an index-identification period (July 1, 2016, to December 31, 2019; earliest code was the index; an index was randomly selected for controls) and continuous enrollment for at least 6 months pre-index and at least 12 months post-index. HCRU and costs (inflated to 2020 USD) per patient per year (PPPY) were compared using analysis of variance.

RESULTS: For 4,723 children with SCD and 4,723 matched controls, mean (SD) age was 8.8 (4.8) years and 48% were female; mean (SD) follow-up was 40 (15) months for patients

and 36 (15) months for controls. During follow-up, 46.7% of children with SCD received hydroxyurea, 31.1% at least 1 blood transfusion, and 79.9% an opioid pain medication. The most common acute and chronic SCD complications were vaso-occlusive crisis (79.7% vs 0% for controls) and scleral icterus (8.6% vs 0.1%), respectively. During the 12 months post-index, children with SCD had higher HCRU vs controls: the mean (SD) PPPY number of inpatient hospitalizations was 0.8 (1.5) vs 0.0 (0.2), length of stay 3.0 (8.1) vs 0.1 (2.5) days, outpatient claims 19.5 (25.6) vs 10.8 (24.8), emergency department (ED) visits 1.8 (2.3) vs 0.6 (1.2), and prescriptions 25.8 (23.0) vs 5.7 (11.3); all $P < 0.0001$. For the SCD cohort, mean (SD) costs PPPY were higher for all categories, with total costs $18,900 ($58,006) vs $2,127 ($13,029), inpatient costs $10,290 ($50,352) vs $260 ($5,042), outpatient including ED $4,670 ($9,785) vs $1,535 ($10,665), and pharmacy $3,940 ($16,734) vs $332 ($2,689); all $P < 0.0001$.

CONCLUSIONS: These results highlight the substantial clinical burden for children with SCD enrolled in Medicare and the corresponding economic impact for the health care system. Compared with matched controls, children with SCD had substantially increased HCRU, particularly inpatient hospitalizations and ED visits, and costs were higher across all categories, with total costs for the SCD cohort almost 9-fold those of controls.

SPONSORSHIP: Pfizer

D14 Achieve high-quality, cost-effective care for inherited bleeding disorders through meaningful connections between payers and providers

Faden R[1], *Schaefer N*[1], *Koulianos-Barkhouse K*[1], *Borrillo J*[2], *Dunn J*[3], *Casebeer S*[4], *Pangrace M*[4], *Richardson T*[4]; *jeffrey.d.dunn@comcast.net*
[1]*National Bleeding Disorders Foundation*; [2]*Hemophilia Alliance*; [3]*Cooperative Benefits Group*; [4]*Impact Education, LLC*

BACKGROUND: Launched in 2014, the Comprehensive Care Sustainability Collaborative (CCSC) supports the sustainability of the hemophilia treatment center (HTC) integrated medical home model of care by facilitating dialogue on both sides of the care management and cost risk equation. HTCs achieve optimal outcomes delivered through a medical home model that is recognized as the gold standard by providers; however, payers are largely unaware of their existence or do not understand their full value and scope of services.

OBJECTIVE: To identify barriers and overlapping opportunities between payers and hemophilia providers that can improve outcomes and lower costs.

METHODS: A strengths, weaknesses, opportunities, and threats (SWOT) analysis was conducted with input from 14 payer and 10 provider advisors. To further identify knowledge and communication gaps, a survey was fielded to 36 payer professionals with influence over formulary and/or medical policy decisions.

RESULTS: The SWOT analysis suggests payers view specialty pharmacy providers as an alternative to HTCs for factor replacement product distribution. Payers value HTC-based medical care for their members with bleeding disorders but often take issue with the payment model predicated on drug dispensation. Therefore, an alternative reimbursement model may be necessary to prevent HTCs from getting carved out from drug dispensation. The survey identified knowledge gaps in strategies including cost-effective medication strategies, best practice collaborations with HTCs, and patient out-of-pocket costs impact on access. Coverage policies are highly variable, with disease severity as the most common coverage criteria. Few payers are highly informed on hemophilia, and support for the HTC integrated care model is highly correlated with disease knowledge and HTC awareness. When asked about the potential impact of CCSC on payer's goals to positively impact outcomes, cost, and patient experience, 60% responded positive or highly positive.

CONCLUSIONS: There is not another care model that performs as well as the HTC for the medical management of hemophilia. However, centers need to capitalize on this advantage and disseminate associated messaging. CCSC has helped connect payers and providers to better manage members with bleeding disorders, but the need continues to increase understanding of the specific benefits of the HTC comprehensive care model.

SPONSORSHIP: Takeda, BioMarin, Biotechnology Innovation Organization, Bleeding & Clotting Disorders Institute, CSL Behring, Genentech, Inc., Gulf States Hemophilia and Thrombophilia Center, Louisiana Center for Bleeding and Clotting Disorders, Sanofi, and Spark Therapeutics

D15 Managed care and clinician insights on optimizing the management of inherited blood disorders in a changing market: Findings from AMCP Market Insights research

Rice M[1], *Matthews N*[2], *Richardson T*[3], *Haumschild R*[4], *Watkins J*[5]; *mrassociate@outlook.com*
[1]*Michelle Rice & Associates, LLC*; [2]AMCP; [3]*Impact Education, LLC*; [4]*Emory Healthcare*; [5]*Managed Care Perspectives, LLC*

BACKGROUND: Inherited blood disorders, including coagulation disorders like hemophilia and hemoglobinopathies such as sickle cell disease and β-thalassemia, significantly

impact blood cell formation, architecture, or function. These disorders, with well-understood genetic bases, are prime candidates for gene therapy. Concerns about the long-term effectiveness and high initial costs of gene therapies have tempered the enthusiasm for their transformative potential.

OBJECTIVE: To expand on the 2022 AMCP Market Insights on managing inherited blood disorders and identify additional managed care and clinician views on the clinical appropriateness of gene therapy, potential outcomes from value-based contracting, and the challenges patients face with health insurance coverage.

METHODS: In fall 2023, AMCP Market Insights engaged in 6 in-depth interviews with managed care experts overseeing more than 77 million individuals and conducted an online survey among hemophilia treatment centers in 18 states, serving approximately 13,000 patients.

RESULTS: The consensus among managed care professionals is that gene therapy for hemophilia is clinically appropriate and coverage will likely align with US Food and Drug Administration approvals. Expected value-based contracting outcomes include a reduction in regular clotting factor infusions and bleed frequency. For sickle cell disease, gene therapy is seen as suitable for all affected individuals interested in gene therapy. In severe β-thalassemia, gene therapy is appropriate for those requiring frequent transfusions or experiencing complications, aiming to reduce transfusion needs and enhance hemoglobin production. Survey results from 24 hemophilia treatment centers indicate that treatment customization is necessary to meet clinical and insurance coverage requirements, with 88% of respondents noting difficulties in insurance approval processes. Significant challenges include access to preferred health care providers (41%), mental health services (41%), and pain management (41%). Approximately 71% of participants felt that new therapies primarily improve patient outcomes, but 86% noted a deficiency in innovative reimbursement models, with 75% concerned about payer readiness to support cell and gene therapies.

CONCLUSIONS: Research indicates a strong agreement on the clinical benefits of gene therapy for inherited blood disorders among experts. However, widespread adoption faces obstacles such as insurance barriers, access to specialized care, and reimbursement models, which must be addressed to fully leverage these advanced treatments.

SPONSORSHIP: BioMarin, CSL Behring, Sanofi, Spark Therapeutics, and Takeda

D16 Budget impact of Injectafer (ferric carboxymaltose injection) for the treatment of iron deficiency in adult patients with heart failure and New York Heart Association class II/III to improve exercise capacity on a third-party US payer

Grace M[1], *Wang K*[2], *Graham C*[1]; *mgrace@rti.org*; *kevin.wang@daiichisankyo.com*
[1]*RTI Health Solutions*; [2]*Daiichi Sankyo US*

BACKGROUND: Ferric carboxymaltose injection's indication in the United States has been expanded to include treatment for iron deficiency (ID) in adult patients (aged ≥18 years) with heart failure (HF) and New York Heart Association (NYHA) class II/III to improve exercise capacity.

OBJECTIVE: To estimate the budget impact (difference in total and per-member per-month costs) to a third-party payer in the United States (eg, a commercial payer and/or Medicare) of managing ID in adult patients with HF with NYHA class II/III before and after the expansion of ferric carboxymaltose injection's indication.

METHODS: A budget impact model was developed in Excel to estimate the cost impact of the indication expansion. The population eligible for treatment consisted of adults who are iron deficient with HF and NYHA class II/III in need of treatment to improve exercise capacity. The scenarios compared were a market with expanded approval and a market without expanded approval. Costs considered were drug acquisition, drug administration, adverse events, and other health care resource utilization (HCRU) over a time horizon of 2 years. Model inputs were informed by data from the CONFIRM-HF study, a retrospective claims analysis of patients with HF, published literature, and model assumptions.

RESULTS: For a blended plan of commercial and Medicare patients, 11,828 adults were eligible for treatment per 1 million lives covered. In the scenario in which ferric carboxymaltose injection received approval for the expanded indication and experienced an increase in uptake, there was an incremental per-member per-month cost reduction of $0.11 in the first year and $0.23 in the second year compared with the market without approval and without an increase in uptake. This translates to approximately $1.3 million and $2.7 million in total cost reductions each year, respectively, per 1 million lives covered. Drug acquisition costs increased but were more than completely offset by cost reductions in drug administration, adverse events, and other HCRU. A majority (93.1%) of decreased costs was attributable to reduction in other HCRU. One-way sensitivity analyses showed that model results were robust to reasonable changes in model parameters.

CONCLUSIONS: The expansion of ferric carboxymaltose injection's indication to include the treatment of ID in adult patients with HF and NYHA class II/III to improve exercise capacity would result in cost reductions for a third-party US payer owing to reduced costs from hospitalizations and emergency department visits related to worsening HF.

SPONSORSHIP: DSI

D17 Evaluating the 10-year impact of an immunoglobulin utilization management and dose optimization program in the managed care setting

Ventura E, Liang A, MacDonald B, Almazan J, Makanji H; erin.ventura@primetherapeutics.com; alina.liang@primetherapeutics.com
Prime Therapeutics

BACKGROUND: There are various complexities associated with immunoglobulin (Ig) therapy (eg, lack of consensus guidelines, pharmacokinetic variances among patients). Rising costs and off-label use of Ig therapy pose challenges for patients, providers, and payers. To optimize Ig management, pharmacist outreach can lead to impactful dose optimization (DO) interventions and prevent adverse effects (AEs) due to overutilization. Taking this into account, Prime Therapeutics implemented an Ig utilization management (UM) and DO program in 2014 to evaluate opportunities for DO in eligible patients when medical necessity is established.

OBJECTIVE: To analyze and contextualize the 10-year savings and clinical interventions generated from an Ig UM and DO program at a health plan.

METHODS: A retrospective review of an Ig program for a health plan with approximately 800,000 commercial, Medicare, and Medicaid lives was conducted. Data were collected from prior authorization (PA) reviews completed from March 1, 2014, to December 31, 2023. Savings were derived from pharmacist-driven interventions: weight-based dose adjustments for adult patients with obesity, downward dose rounding to the nearest whole vial size, or dose titration. Patients with savings outside of these intervention types were excluded from analysis. Savings were calculated based on approved PA with accepted DO recommendations as such: Savings ($) = (Total requested units per PA period × [ASP × index rate]) – (Total approved units per PA period × [ASP × index rate]).

RESULTS: Out of 5,790 Ig PAs approved for 1,551 unique patients from March 2014 to December 2023, 49% (n = 758) of patients were eligible for DO. In total, 33% (n = 519) of patients were eligible for weight-based dosing, and 78% (n = 406) of these recommendations were accepted. Dose titration and vial rounding recommendations were accepted for 8% (n = 127) and 9% (n = 147) of patients, respectively. Over 10 years, program interventions resulted in 404,932 Ig units saved, equating to $15,134,728 in savings. Weight-based dosing interventions accounted for the majority of savings (67%). Average annual savings per patient was $4,256. From 2014 to 2023, the average number of Ig units saved and the savings per patient year over year increased by 8% and 9%, respectively.

CONCLUSIONS: Implementation of an Ig UM and DO program resulted in decreased spend and use of Ig therapy. Program results showed higher acceptance rates of DO recommendations compared with clinical benchmarks. Ultimately, the program's clinical interventions reduced overutilization of Ig, which likely reduced AEs and contributed to overall savings.

SPONSORSHIP: None

D18 Burden of hospitalizations and 30-day readmissions due to vaso-occlusive crisis in sickle cell disease: A growing crisis

Ambegaonkar A, Raturi M, Bhave S; ambi@apperturehealth.com; mayank@apperturehealth.com
APPERTURE LLC

BACKGROUND: Sickle cell disease (SCD) affects ~100,000 Americans primarily of Black and Hispanic origins. It is characterized by vaso-occlusive crisis and severe pain episodes and therefore hospitalizations. Frequent vaso-occlusive crises and hospitalizations add a tremendous disease burden on the patient and society.

OBJECTIVE: To compare and characterize hospitalizations of patients with SCD with and without 30-day readmissions.

METHODS: The 2019 Nationwide Readmissions Database from the Health Care and Utilization Project was used to study readmission rate and its determinants for SCD. SCD hospitalizations were identified using *International Classification of Diseases, Tenth Revision* code D57 and rehospitalizations were defined as those taking place within 30 days of a prior hospitalization. Patients' socioeconomic characteristics and hospitalization history were used to characterize and predict patients with readmission.

RESULTS: We identified 37,853 SCD-related hospitalizations in the Nationwide Readmissions Database for 13,456 unique patients aged 18 years and older (average 2.8 hospitalizations per patient). Their average comorbid conditions was 8.87 ± 4.89; average number of procedures was 1.67 ± 3.13; average cumulative length of stay was 14.8 ± 20.04 days; and mean total annual charges of $113,102 ± 175,127. There were 3,766 patients with SCD who had at least 1 less-than-or-equal-to-30-day readmission (RA) and 9,690 patients with no less-than-or-equal-to-30-day readmission events (nRA).

Those in the RA group were younger by 2 years (RA = 32.4 vs nRA = 34.7) and lived in zip codes with the lowest quartile income (RA = 49.49% vs nRA = 45.9%). There were no significant differences in their comorbidity burden (RA = 9.29 ± 4.35 and nRA = 8.7 ± 5.08). RA patients were prevalent in Medicaid (RA = 48.96% vs nRA = 41.52%) and Medicare (RA = 34.17% vs nRA = 31.02%) as compared with commercial insurance (RA = 16.86% vs nRA = 27.45%). Over the whole year, these 2 groups consumed care deferentially; the number of hospitalizations was 6.0 ± 3.4 for RA and 1.6 ± 1.0 for nRA, resulting in average cumulative length of stay of 32.5 days ± 27.7 for the RA group and 7.9 days ± 9.5 for the nRA group. The number of procedures performed also varied (RA = 3.22 ± 4.23 and nRA = 1.05 ± 1.91), resulting in total annual cost differences of almost 4-fold (RA = $237,258 ± 250,023 and nRA = $64,882 ± 99,978).

CONCLUSIONS: Patients with SCD with a less-than-30-day rehospitalization event were from lower-income neighborhoods, had Medicaid insurance, had a greater number of hospitalizations, and had increased length of stay, resulting in 4-fold annual costs. These patients need to be identified early and case managed to improve health outcomes and decrease societal burden.

SPONSORSHIP: None

D20 Health care resource utilization among patients initiating berotralstat for the long-term prophylaxis of hereditary angioedema in the United States

Christiansen S[1], *Lopez-Gonzalez L*[2], *MacKnight S*[3], *Laliberté F*[4], *Boudreau J*[5], *Nestler-Parr S*[6], *Johnston D*[7], *Gillard P*[7], *Zuraw B*[8]; *scchristiansen@ucsd.edu*; *llopez-gonzalez@biocryst.com*
[1]*Department of Medicine, University of California San Diego*; [2]*BioCryst Pharmaceuticals, Inc.*; [3]*Groupe d'analyse, Ltée*; [4]*Groupe d'analyse, Ltée*; [5]*Groupe d'Analyse, Ltée*; [6]*BioCryst Pharmaceuticals*; [7]*BioCryst*; [8]*University of California San Diego*

BACKGROUND: In the absence of effective treatment, hereditary angioedema (HAE) is associated with significant attack-related morbidity and the risk of mortality.

OBJECTIVE: To evaluate all-cause, angioedema-related, and HAE attack–related health care resource utilization (HCRU) before and after initiation of Orladeyo (berotralstat) for long-term prophylaxis (LTP) of HAE in the United States.

METHODS: This retrospective pre-post study used administrative claims data from Komodo's Healthcare Map to identify patients on berotralstat (December 2020 to December 2022; first dispensing = index). Patients had at least 6 months of continuous insurance enrollment pre-index, were aged 12 years or older at index, and had evidence of HAE anytime during continuous enrollment pre-index period. Evidence of HAE included diagnoses of HAE or angioedema, HAE-specific medication use (on-demand or LTP), or laboratory measurement of complement function. Follow-up spanned from index to end of enrollment or end of study period, whichever occurred first. Medical visits with evidence of HAE were considered angioedema related; HAE attack–related visits were defined by on-demand medication administration or primary diagnosis of HAE attack symptoms (swelling/edema or breathing difficulty). Rates per person-year in the follow-up period vs pre-index were compared using generalized estimating equations Poisson regression models with robust SEs.

RESULTS: The study population was 260 predominantly female (74.2%) patients with HAE with a mean age of 40 years. Significant reductions ($P < 0.05$) were observed in all-cause, angioedema-related, and HAE attack–related HCRU after berotralstat initiation. Rates of all-cause hospitalizations decreased by 34% and all-cause outpatient/emergency department (OP/ED) visits decreased by 14%. Rates of angioedema-related hospitalizations decreased by 52%, and angioedema-related OP/ED visits decreased by 44%. Rates of HAE attack–related visits decreased by 51%, driven by significant decreases in hospitalization (60%) and OP/ED (50%) visits. Attack-related HCRU stratified by body location revealed significant reductions associated with attacks in the head and upper airways (48%), gastrointestinal system (58%), and unspecified locations (52%). On-demand medications administered in a clinical setting decreased by 39% in those with previous on-demand treatment.

CONCLUSIONS: Our real-world study observed significant reductions in all-cause, angioedema-related, and HAE attack–related HCRU following initiation of berotralstat for LTP of HAE.

SPONSORSHIP: BioCryst Pharmaceuticals

D21 Health care resource utilization by patients with WHIM syndrome in the United States

Bradt P[1], *Nally J*[2], *Novak R*[2], *Church C*[2], *Zhang D*[2]; *pamela.bradt@x4pharma.com*
[1]*X4 Pharmaceuticals*; [2]*Veeva Systems*

BACKGROUND: WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome is an ultra-rare chronic primary immunodeficiency associated with highly variable multisystemic manifestations and increased risk of infections, malignancy, and end-organ damage. Historically, delayed diagnosis and lack of targeted therapies have contributed to suboptimal clinical outcomes. Long-term

complications with high morbidity and mortality lead to a high risk of hospitalization and elevated health care resource utilization (HCRU).

OBJECTIVE: To (1) describe HCRU in patients with WHIM syndrome in the United States and (2) compare their HCRU with matched controls.

METHODS: This study used anonymous patient longitudinal claims data from Veeva Compass Patient covering dispensed prescription, procedures, and diagnoses from 300+ million patients (January 2017 to December 2023). The study population (n = 605) had chronic neutropenia (≥2 neutropenia claims ≥90 days apart) and recurrent infections (>1 infection/year) plus 2 of the following WHIM syndrome features: chronic human papillomavirus, hypogammaglobulinemia, chronic lymphopenia. Risk ratios (RRs) for HCRU in patients with WHIM syndrome and control patients (1:10), matched on age, sex, geography, and zip-3 as a proxy for income, were calculated using Poisson regression models.

RESULTS: Across all HCRU metrics, patients with WHIM syndrome demonstrated significantly higher person-year utilization rates compared with controls. They experienced significantly more medical interaction days than controls (67.6 vs 17.8, $P < 0.001$) and more infection-related health care interactions (8.0 vs 1.3, $P < 0.001$). After controlling for all variables, risk of increased HCRU was significant across multiple types of care: overall medical interactions (RR = 3.8; 95% CI = 3.7-3.9), infection-related interactions (RR = 6.0; 95% CI = 5.8-6.2), hospitalizations (RR = 5.9; 95% CI = 5.6-6.3), and inpatient days (RR = 10.7; 95% CI = 10.5-11). Further, children (aged <18 years) with WHIM syndrome had an even greater risk of increased HCRU compared with adults for hospitalization (RR = 11.6 vs 5.6; $P < 0.001$) and inpatient days (RR = 17.4 vs 10.2; $P < 0.001$).

CONCLUSIONS: Overall, patients with WHIM syndrome had significantly higher HCRU than matched controls, with even greater risk among children. Decreasing HCRU is an important goal of a health care system, but reducing health care system interactions may also be an important goal for patients and caregivers. Improving patient outcomes has the potential to do both.

SPONSORSHIP: X4 Pharmaceuticals

D22 Phase 3 trial of an oral CXCR4 antagonist, mavorixafor, for treatment of patients with WHIM syndrome: Preliminary results from the ongoing open-label extension period of continuous mavorixafor treatment

Tarrant T[1], Badolato R[2], Donadieu J[3], Dubuc S[4], Hu Y[4], Li X[4], Li S[4], Steiner D[4], Yan T[4], Arbet-Engels C[4]; teresa.tarrant@duke.edu

[1]*Division of Rheumatology and Immunology, Department of Medicine, Duke University*; [2]*Department of Clinical and Experimental Sciences, University of Brescia & ASST Spedali Civili*; [3]*Hôpital d'Enfants Armand-Trousseau*; [4]*X4 Pharmaceuticals*

BACKGROUND: WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome is an ultra-rare chronic primary immunodeficiency disorder resulting from impaired leukocyte trafficking and characterized by neutropenia, lymphopenia, infections, and multisystem disease manifestations. It is predominantly caused by gain-of-function variants in CXCR4. In a 52-week randomized placebo-controlled phase 3 trial of participants aged 12 years and older with WHIM syndrome (NCT03995108), treatment with mavorixafor, an oral CXCR4 antagonist, led to sustained increases in neutrophils and lymphocytes, reduced infections, and was well tolerated.

OBJECTIVE: To present interim results from the open-label extension (OLE), which evaluated long-term safety and efficacy of mavorixafor in participants with WHIM syndrome.

METHODS: Patients were randomized 1:1 to mavorixafor or placebo during the 52-week randomized controlled period (RCP), after which patients could enter the optional OLE. All participants received mavorixafor during the OLE (up to 52 weeks). Assessments included safety, tolerability, absolute neutrophil count (ANC), absolute lymphocyte count (ALC), and infection rates.

RESULTS: Twenty-seven of 31 participants from the RCP entered the OLE. Median age was 18 years (range, 13-73 years). Mavorixafor was well tolerated and the safety profile remained consistent with mavorixafor continuation or initiation during OLE. Patients who received ongoing mavorixafor during the RCP and OLE had sustained ANC and ALC levels, which remained ~3-fold and 2-fold above pretreatment baseline over 104 weeks, respectively. Similarly, patients who received placebo in RCP and switched to mavorixafor during OLE demonstrated ~3-fold and 2-fold increases in trough ANC and ALC, respectively. One fewer annualized infection was observed in those who switched from placebo in the RCP to mavorixafor in OLE. Further analysis is ongoing.

CONCLUSIONS: This analysis of interim long-term data demonstrated a consistent safety profile with mavorixafor, sustained ANC and ALC improvements, and infection rate reduction in participants chronically treated with mavorixafor beyond 52 weeks. These data confirm the positive risk-benefit profile of mavorixafor therapy for the long-term treatment of patients with WHIM syndrome.

SPONSORSHIP: X4 Pharmaceuticals

E00-E90 Endocrine, Nutritional, and Metabolic Diseases

(eg, diabetes, growth hormone, lipids)

E5 Budget impact analysis of continuous glucose monitoring systems for Medicaid beneficiaries living with type 2 diabetes using insulin

Marinello A, Timmons J[1], Poon Y[1]; amarinello1@gmail.com; jack.timmons@abbott.com
[1]*Abbott Diabetes Care*

BACKGROUND: Diabetes-related health care costs, a large portion of total US health care spending, is driven by costs due to hospitalizations, emergency department (ED) visits, and outpatient visits. A recent real-world study examined the correlation of continuous glucose monitoring (CGM) use and health care resource utilization among Medicaid beneficiaries living with type 2 diabetes on insulin.

OBJECTIVE: To quantify the health economic implications of CGM, a budget impact analysis was developed leveraging results from this real-world study to estimate the net financial impact of reimbursement for CGM among all Medicaid beneficiaries living with type 2 diabetes on insulin.

METHODS: This analysis estimated annual budget impact of CGM reimbursement expansion to people with type 2 diabetes and on insulin, from a US Medicaid perspective over a 3-year time horizon. In the base-case analysis, budget impact was calculated as the difference in annual total cost between a reference scenario without CGM coverage and a scenario with CGM coverage for all insulin-using people with diabetes. Cost components included direct costs associated with glucose monitoring, all-cause (AC) ED visits, AC hospital inpatient visits, and AC outpatient office visits. Glucose monitoring costs were based on the wholesale acquisition costs of CGMs and blood glucose monitoring supplies. Sensor rebates were not included in cost calculations. Clinical inputs informing relative risk reductions in health care resource utilization due to CGM were based on the recently published real-world Medicaid analysis. Market share assumptions were based on published sources on CGM use in people living with diabetes. All cost inputs were inflation adjusted to 2024 USD.

RESULTS: From the Medicaid perspective, CGM reimbursement for all beneficiaries with type 2 diabetes on insulin was associated with a net per-member per-month budget impact of –$0.17 in year 1, –$0.19 in year 2, and –$0.22 in year 3. In total, reimbursement expansion for all people with type 2 diabetes on insulin may realize approximate net cost savings of $144 million in year 1, $165 million in year 2, and $190 million in year 3, with a cumulative cost savings of $500 million over 3 years. One-way sensitivity analysis identified baseline AC outpatient visits and AC inpatient visits as major cost drivers.

CONCLUSIONS: CGM coverage for beneficiaries with type 2 diabetes on insulin was estimated to result in significant cost savings to the Medicaid health system.

SPONSORSHIP: Abbott Diabetes Care, Inc.

E6 Budget impact analysis of implementing quantity limit on blood glucose monitoring supplies for people living with diabetes using continuous glucose monitors

Panjwani Q, Timmons J, Poon Y, Frazzitta M; qaashif.panjwani@abbott.com
Abbott Diabetes Care

BACKGROUND: The use of continuous glucose monitors (CGMs) has become the standard of care for those using insulin and at risk for hypoglycemia. There is an identified cost concern regarding the cost of covering both blood glucose monitoring supplies (BGMS) and CGM owing to the recommendation of a confirmatory BG level when indicated BGMS need to be available. A recent best practice identified was to cover a quantity limit of BGMS for those on CGM.

OBJECTIVE: To examine the budget impact of implementing a quantity limit (QL) on test strip use for patients using CGMs from a national US payer perspective.

METHODS: A budget impact analysis was developed using Microsoft Excel for all insured people with diabetes using CGMs, from a US payer perspective over a time horizon of 3 years. Budget impact was calculated as the difference in total glucose monitoring cost between a reference scenario without a test strips QL and a scenario with a QL for all people with diabetes using CGMs. In the scenario without a QL, test strips coverage for CGM users was assumed to be the same as self-monitoring blood glucose, at 6 times per day for people with type 1 diabetes mellitus (T1DM) or type 2 diabetes mellitus (T2DM) on multiple daily injections of insulin, 3 times per day for people with T2DM on

basal insulin only, and 1 time per day for people with T2DM not on insulin. In the scenario with a QL, test strips coverage for CGM users was assumed to drop significantly, to 100 test strips per 90 days for people with T1DM or T2DM on multiple daily injections of insulin, 50 test strips per 90 days for T2DM on basal insulin only, and 1 test strip per week for T2DM not on insulin. Analysis focused on glucose monitoring acquisition costs and assumed CGM market shares remained in both scenarios increased by 15% year over year. Glucose monitoring acquisition costs included the cost of CGMs and test strips based on wholesale acquisition costs.

RESULTS: Among all insured people with diabetes, the implementation of a QL for patients using CGMs resulted in an annual cost savings of $20 per patient per year (PPPY) in year 1, $23 PPPY in year 2, and $26 PPPY in year 3. From a national perspective, implementation of the QL is estimated to result in a $742 million in year 1, $855 million in year 2, and $985 million in year 3, with a cumulative cost savings of $2.6 billion over 3 years.

CONCLUSIONS: Implementation of a QL for people with diabetes using CGMs resulted in significant cost savings to the US health care system. Because the adoption of CGMs would allow for a QL for test strips, the totality of budget impact for diabetes management is further warranted.

SPONSORSHIP: Abbott Diabetes Care

E7 Trends in diabetes cost of illness

De Los Santos B[1], *Borroto D*[2], *Kim K*[3]; *bdelossantos94@gmail.com*
[1]*University of Illinois Chicago College of Pharmacy / Takeda*; [2]*University of Illinois Chicago, College of Pharmacy*; [3]*University of Illinois Chicago, College of Pharmacy, Department of Pharmacy Systems Outcomes & Policy*

BACKGROUND: Diabetes and its resulting complications have significant mortality and financial impact. This study aims to analyze trends in the cost of care for patients with type 2 diabetes (T2D).

OBJECTIVE: To examine the longitudinal trends in all-cause health care costs from the US payer's perspective and comorbidities among those with T2D who were covered by commercial health plans.

METHODS: We used the Merative MarketScan Commercial Insurance database from 2016 through 2021. Actively managed T2D prevalent cases were defined by T2D diagnosis codes on 2 different dates within a calendar year. Eligible subjects had to be enrolled each calendar year without a coverage gap and were free from a service claim for type 1 or gestational diabetes. Diabetes-related complications and comorbidity were assessed using the diabetes complication severity index (DCSI) score for each year. All-cause annual costs were calculated as the aggregated paid amount for inpatient, outpatient, and outpatient pharmacy services. Mean and median per-patient per-year (PPPY) costs were estimated along with annual costs by DCSI subgroups. All costs were adjusted to January 2024 US dollars.

RESULTS: The number of T2D prevalence cases included in this study was 613,430 in 2016 and 495,077 in 2021. The mean age for 2016 enrollees was 53 years (SD = 8.3), and 46.7% were female. In the 2016 cohort, 56.7%, 32.2%, 8.5%, and 2.6% had a DCSI score of 0, 1-2, 3-4, and 5+, respectively. Similarly, the mean age for 2021 enrollees was 53 years (SD = 8.4), and 45.7% were female. The 2021 enrollees had 54.7%, 33.3%, 9.1%, and 2.9% with a DCSI score of 0, 1-2, 3-4, and 5+, respectively. Annual costs continually increased over the 6-year period. The mean PPPY total cost increased from $15,479 to $17,018 PPPY from 2016 to 2021, respectively. Median total adjusted costs rose from $9,772 PPPY in 2016 to $11,423 PPPY in 2021. The cost escalation was primarily driven by a rise in outpatient drug costs, where drug spending went from 43.6% ($6,744) of total medical expenditures in 2016 to 47.2% ($8,026) in 2021. Focusing on 2021 enrollees, median total adjusted costs were $8,396 PPPY for those with a DCSI score of 0, $14,061 PPPY with a DCSI score of 1-2, $22,218 PPPY with a DCSI score of 3-4, and $32,790 PPPY with a DCSI score of 5+.

CONCLUSIONS: Cost of care for the management of patients with T2D increased over the study period. The cost of diabetes care increased along with DCSI scores. In general, medication cost escalation drives the care cost increase. The proportion of patients with complications remained high throughout the study period.

SPONSORSHIP: None

E8 Baseline demographics, clinical characteristics, and health care utilization in adult patients with type 2 diabetes mellitus and incident atherosclerotic cardiovascular disease enrolled in Medicare

Dunn T[1], *Rotroff D*[2], *Harton J*[3], *Cutler J*[3], *Kim S*[3], *Tan X*[4], *Noone J*[5], *Xie L*[5], *Gamble C*[4]; *tydz@novonordisk.com*
[1]*Novo Nordisk, Inc.*; [2]*Cleveland Clinic*; [3]*Genesis Research Group*; [4]*Novo Nordisk Inc*; [5]*Novo Nordisk*

BACKGROUND: Diabetes mellitus (DM) prevalence among US adults is approximately 11%, mostly attributable to type 2 diabetes mellitus (T2DM). Atherosclerotic cardiovascular disease (ASCVD) is the main macrovascular complication in T2DM, leading to myocardial infarction, cerebrovascular disease, and peripheral artery disease.

OBJECTIVE: To assess the preliminary baseline demographics, clinical characteristics, and health care resource utilization (HCRU) of 2 Medicare cohorts: patients with T2DM and patients with comorbid T2DM and incident ASCVD.

METHODS: Medicare Research Identifiable Files fee-for-service and Medicare Beneficiary Summary File administrative claims data from 2006 to 2021 were used to identify 2 patient cohorts (one a subcohort of the other): (1) patients diagnosed with T2DM (T2DM cohort) and (2) patients diagnosed with T2DM who subsequently develop ASCVD (T2DM/incident ASCVD cohort). The index date is defined as the earliest date within the identification period of a claim for a diagnosis code for T2DM (T2DM cohort) or the earliest claim for ASCVD (T2DM/incident ASCVD cohort). Descriptive statistics were used to characterize patients' demographic/clinical characteristics and HCRU during the 1-year baseline period.

RESULTS: There were 2,326,726 patients in the T2DM cohort and 640,666 patients in the T2DM/incident ASCVD cohort. Mean (SD) age at index date was 74.9 (7.3) and 76.5 (7.2) years, respectively. The majority of both cohorts were female (56.7% T2DM and 59.3% T2DM/incident ASCVD). The mean Charlson Comorbidity Index score excluding diabetes was 1.67 (1.96) and 1.51 (1.80) for the T2DM and T2DM/incident ASCVD cohorts, respectively. The most common comorbidities at baseline for both cohorts were hypertension (68.3%, 72.5%) and hyperlipidemia (55.6%, 56.7%). For the T2DM/incident ASCVD cohort, the mean time from T2DM diagnosis to ASCVD diagnosis was 39.7 (37.7) months; the most common type of index ASCVD was cardiovascular disease (48.4%, including myocardial infarction in 7.7% of patients), followed by cerebrovascular disease (28.4%) and peripheral artery disease (28.4%). At baseline, 22.3% of the T2DM cohort had inpatient visits and 31.8% had emergency department (ED) visits; utilization was similar for the T2DM/incident ASCVD cohort, with 21.1% and 35.1% experiencing inpatient or ED visits, respectively. Mean pharmacy claims were similar for both cohorts: 31.8 (26.2) T2DM and 32.1 (25.3) T2DM/incident ASCVD.

CONCLUSIONS: In our analysis of 2 large cohorts of Medicare patients spanning nearly 2 decades, we found a high disease burden and HCRU among patients with T2DM, with or without incident ASCVD.

SPONSORSHIP: Novo Nordisk Inc.

E9 How does the impact of injection frequency on quality of life affect the long-term cost-effectiveness of once-weekly insulin icodec in the United States?

Sullivan S[1], Sheth S[2], Nanaï A[2], Hunt B[3], Malkin S[3], Cattin J[3], Chubb B[2], Noone J[2], Jensen M[2]; sean.sullivan@curtahealth.com; zuns@novonordisk.com
[1]Curta Inc.; University of Washington, The CHOICE Institute; [2]Novo Nordisk; [3]Ossian Health Economics and Communications GmbH

BACKGROUND: Although many modern treatments are available for type 2 diabetes (T2D), insulin therapies are often eventually required to maintain blood glucose levels owing to the progressive nature of the disease. Traditional insulin injections are often required multiple times daily and can have a substantial impact on quality of life. Insulin icodec is a basal insulin analog developed for once-weekly administration.

OBJECTIVE: To assess the long-term outcomes associated with insulin icodec for treating insulin-naive people with T2D in the United States, including how patients' quality of life is impacted by injection frequency.

METHODS: Outcomes were projected over patients' lifetimes using the PRIME T2D Model. Clinical data were taken from ONWARDS 1 and 3, comparing icodec with glargine U100 and degludec. Modeled patients received icodec or a comparator basal insulin for 4 years, before intensifying with the addition of bolus insulin. Treatment effects applied in the first year of the analysis were maintained until intensification. Quality-of-life decrements (disutilities) relating to once-daily vs once-weekly injection (−0.0389 for the United Kingdom and −0.0569 for Canada) were taken from a published study and applied for 1 year or until intensification. Costs were expressed in 2022 US dollars from the health care payer perspective. The icodec wholesale acquisition cost (WAC) was specified relative to the degludec WAC.

RESULTS: Icodec was associated with improvements in quality-adjusted life expectancy of 0.05-0.22 quality-adjusted life-years vs glargine U100 and degludec. Benefits were greater when injection frequency had a larger impact on quality of life and when this was applied for longer durations. Compared with glargine U100, icodec improved clinical outcomes and was less costly when applying a WAC relative to degludec of up to 96%, and cost-effective in both the least (when applying the UK disutility for 1 year) and most favorable (when applying the Canadian disutility for 4 years) scenarios at 99% and 112% of the degludec WAC, respectively. Compared with degludec, icodec improved clinical outcomes and was less costly when applying a WAC relative

to degludec of up to 88%, and cost-effective at 92% and 104% of the degludec WAC in the least and most favorable scenarios, respectively.

CONCLUSIONS: Icodec improves patients' quality of life while being a cost-effective therapy at a range of prices vs glargine U100 and degludec in the United States, with a greater cost-effectiveness profile shown when higher injection frequency disutilities were applied over longer durations.

SPONSORSHIP: Funded by Novo Nordisk

E11 Are remote monitoring glucometers cost-effective in a pharmacist-run clinic? Evidence from a managed care setting

Pan X[1], *Brazeal T*[2], *Law A*[1]; *xinpan@buffalo.edu*
[1]*Western University of Health Sciences*;
[2]*Desert Oasis Healthcare*

BACKGROUND: Remote patient monitoring has the potential to improve health care by leveraging medical devices to continuously collect and transmit real-time patient health data to health care providers. A cellular-enabled glucometer facilitates remote monitoring of blood glucose levels, facilitating early detection of hypo/hyperglycemia. It allows for timely interventions, potentially preventing exacerbations and minimizing complications.

OBJECTIVE: To evaluate the cost-effectiveness of incorporating cellular-enabled glucometers vs standard care within a pharmacist-run clinic at Desert Oasis Healthcare from the health plan perspective.

METHODS: This institutional review board–approved retrospective cohort study included Desert Oasis Healthcare patients aged 18 years and older who were enrolled in the pharmacist-run cardiometabolic clinic. The intervention group consisted of patients who used cellular-enabled glucometers, whereas the comparison group received standard care (no cellular-enabled glucometers). Decision tree analysis compared costs and effectiveness outcomes between groups. Costs included device costs, pharmacist wage rates associated with patient management time, and health care utilization (acute care, skilled nursing, and emergency department [ED] visits). Effectiveness was assessed by the probability of diabetes-related health care utilization. Negative binomial regression models assessed the impact of both pharmacists and cellular-enabled glucometers on health care utilization.

RESULTS: In a cohort of 2,976 primarily male, White patients with a mean age of 64.3 (SD = 13.1), enrolled in the cardiometabolic clinic, the intervention group (n = 995) demonstrated a dominant option given its lower overall cost ($31,578.92 vs $61,725.89) and higher probability of zero utilization (93.7% vs 91.8%) compared with the comparison group (n = 1,981). Among patients with 1 or more utilizations, the intervention group remained dominant for acute care, skilled nursing, and ED visits. Our results indicated that enrolling in a pharmacist-run clinic significantly reduced the incidence rate of utilization by 29.7% (incidence rate ratio = 0.703; $P < 0.001$). Adding cellular-enabled glucometers further reduced the incidence rate of utilization by 27.6% (incidence rate ratio = 0.427; $P < 0.001$).

CONCLUSIONS: This study highlighted the cost-effectiveness and significant positive impact of pharmacist-run clinics on reducing health care utilization of patients with diabetes in a managed care setting, and the additive effect of remote patient monitoring integration. Future studies can explore patient perspectives and the inclusion of continuous glucose monitoring devices.

SPONSORSHIP: None

E12 Misdiagnosis of newly diagnosed type 1 diabetes in a US managed care population

Brixner D[1], *Beyene T*[2], *Venkataraman M*[3], *Teng J*[2], *Tan J*[2], *Li X*[4], *Wilson L*[4], *Cagle A*[4], *Malone D*[5]; *diana.brixner@utah.edu*; *laura.wilson@sanofi.com*
[1]*University of Utah College of Pharmacy*; [2]*Carelon*; [3]*Carelon Research*; [4]*Sanofi*; [5]*University of Utah's Skaggs College of Pharmacy*

BACKGROUND: People with type 1 diabetes (T1D) are often misdiagnosed with type 2 diabetes (T2D), which may lead to a delay in appropriate disease management. The importance of a correct diagnosis is highlighted by diabetes guidelines, but the degree of misdiagnosis of T1D has not been well characterized in the US population.

OBJECTIVE: To assess the rates of people with newly diagnosed T1D who had a prior T2D diagnosis in routine clinical practice in the United States.

METHODS: This observational cohort study used administrative claims (October 1, 2015, to December 31, 2023) from the Healthcare Integrated Research Database. People with at least 2 outpatient claims for T1D 30-183 days apart or at least 1 inpatient claim were identified. Date of first T1D diagnosis was the index date. People were required to have at least 12 months' continuous medical and pharmacy benefit before (baseline) and during and after index (follow-up). People were excluded if they had a prior T1D diagnosis during the 12-month baseline period; if they had at least 2 T2D diagnoses during the 12-month follow-up period; or if they reported secondary diabetes or pregnancy during baseline. People with an index T1D claim were stratified by whether they had

a previous T2D claim during baseline, and by whether they received autoantibody (AA) testing during the study period (baseline and follow-up). Results are presented descriptively.

RESULTS: From more than 38 million enrollees in the Healthcare Integrated Research Database, 36,418 had a qualifying index T1D claim; 26,372 (72%) were excluded as a result of having at least 2 subsequent T2D diagnoses. After excluding secondary diabetes or pregnancy during baseline, 8,577 people with index T1D diagnoses were included; 2,624 (31%) had at least 1 prior T2D diagnosis during baseline (41% female; mean [SD] age = 45.4 years; 6% aged younger than 18 years), and 5,953 (69%) had no prior T2D diagnosis (42% female; mean [SD] age = 27.2 years; 46% aged younger than 18 years). Fewer than a quarter (24%) of people with an index T1D claim received AA testing; this rate appeared higher among people aged younger than 18 years (n = 2,862; 32% with AA testing) than in those aged 18 years and older (n = 5,715; 19% with AA testing).

CONCLUSIONS: Almost a third of people with newly diagnosed T1D were initially misdiagnosed with T2D; these people were generally older than those with a T1D claim with no prior T2D diagnosis. The majority (more than three-quarters) of people with newly diagnosed T1D did not receive AA testing, with AA testing less likely to be performed in adults, emphasizing a need for more AA testing at screening and diagnosis.

SPONSORSHIP: Sanofi

E13 Clinical outcomes of an electronic health record–integrated proactive affordability program for glucagon-like peptide-1 receptor agonists prescribed to Medicare patients

Reynolds T, Chen L, Paulson D, King D;
tim.reynolds@bswhealth.org
Baylor Scott & White Health

BACKGROUND: For patients with a Medicare drug benefit (Part D), patient cost sharing can be unaffordable. For antidiabetic medications such as glucagon-like peptide-1 receptor agonists (GLP-1 RAs), a 25% patient coinsurance is typically $175-$275 per month. With the upcoming changes to the out-of-pocket spending cap in 2025, patients may still be responsible for average monthly costs up to $166. To address the potential risk of medication discontinuation because of financial barriers, a medication order queue was implemented in a large health system internal medicine clinic in Temple, Texas. This allowed outpatient GLP-1 RA orders to be intercepted by multidisciplinary clinical staff, where financial need could be assessed proactively before releasing to a dispensing pharmacy. A previous analysis of this pilot found that approximately one-third of Medicare-only patients with type 2 diabetes receiving GLP-1 RAs had financial need and accepted assistance. Most (87%) of those patients were enrolled into patient assistance programs.

OBJECTIVE: To evaluate clinic-level outcomes that will be used to inform expansion opportunities within the health system.

METHODS: A controlled pre-post analysis using combined electronic health record and claims data was performed. Multivariate logistic regression analyses were used to determine the association between the program and indicators of clinical efficacy at 6 months after ordering (1) a hemoglobin A1c of less than 7% and (2) body weight loss of greater than or equal to 5%. The convenience sample included medication orders for a GLP-1 RA written between March 8, 2023, and June 9, 2023 (the intervention period), or March 8, 2022, and June 9, 2022 (the historical control period), at the clinic of interest or 4 other internal medicine clinics (controls). Orders were excluded if the patient lacked Medicare or had secondary coverage. Additional variables for demographics and medical history were included in the adjusted regression model.

RESULTS: A total of 714 orders were evaluated. The affordability program was associated with higher odds of achieving A1c less than 7% at 6 months (adjusted odds ratio = 1.305, 95% CI=0.605-2.814). Similarly, the program was associated with higher odds of achieving greater than or equal to 5% weight loss (adjusted odds ratio = 1.279, 95% CI = 0.569-2.873). However, these results were not statistically significant.

CONCLUSIONS: Although not statistically significant, clinical findings did trend toward improved outcomes at the clinic level. Limitations include the short nature of the pilot (3 months) and the inability to use claims data to directly measure adherence owing to the use of patient assistance programs.

SPONSORSHIP: None

E14 Adherence in initiators of combination therapy among patients with drug-naive type 2 diabetes: A real-world study using a group-based trajectory model

Fatima B, Majd Z, Abughosh S; bfatima5@uh.edu
University of Houston

BACKGROUND: Adherence to combination therapy among patients with diabetes is recommended by data from clinical trials for more durable effects on glycated hemoglobin levels and reduced risk of macro- and microvascular complications. However, multidrug regimens and complexity associated with early intensified treatment make it more

challenging, and adherence to combination therapy is often inadequately captured.

OBJECTIVE: To assess adherence trajectories of initial combination therapy approach during the first 12 months of antidiabetic treatment initiation.

METHODS: Using Merative MarketScan research databases (2017-2019), drug-naive patients with type 2 diabetes (T2D) identified who received initial combination therapy and were continuously enrolled in Commercial or Medicare insurance plans during the 6-month pre-index and 12-month post-index period. The index date was defined as the first antidiabetic medication use date. Patients who received 2 distinct antidiabetic medications within 30 days or on the same date were identified as initial combination therapy. Adherence was measured within 12 months following the index date using proportion of days covered. A group-based trajectory model was used to identify distinct longitudinal patterns of adherence. Multinomial regression model was conducted to assess various predictors associated with each adherence trajectory.

RESULTS: Among 116,597 drug-naive patients with T2D identified, 14,118 (12.1%) initiated combination therapy. A group-based trajectory model with 4 distinct trajectories of adherence was selected using second-order polynomial function of time: adherent (30.5%), gradual decline (21.6%), gaps in adherence (21.2%), and rapid decline (26.7%). Regression analysis showed that patients with Health Maintenance Organization vs comprehensive plans were more likely to experience rapid decline (odds ratio [OR] =1.36, 95% CI =1.06-1.73), gradual decline (OR =1.41, 95% CI =1.12-1.80) and gaps in adherence (OR =1.29, 95% CI =1.01-1.64). Patients aged 55-64 vs 18-34 years were less likely to experience rapid decline in adherence (OR =0.39, 95% CI =0.31-0.49), gradual decline in adherence (OR =0.67, 95% CI =0.52-0.87), and gaps in adherence (OR =0.68, 95% CI =0.53-0.88).

CONCLUSIONS: Approximately 70% of patients with T2D who initiated combination therapy followed nonadherent trajectories during the first year of treatment. Patients with Health Maintenance Organization health plans and younger age groups were more likely to have lower adherence trajectories. Targeted interventions are needed to enhance adherence to combination therapy among patients likely to follow nonadherent trajectories.

SPONSORSHIP: None

E20 Modeling the impact of blister packing chronic medications on medication adherence, health care resource utilization, and health care costs for a Medicare Advantage health plan

Borrelli E, Saad P, Barnes N, Dumitru D, Lucaci J;
eric.borrelli@bd.com
Becton, Dickinson & Co

BACKGROUND: Medication nonadherence is a widespread issue in the United States, leading to increased health care resource utilization (HCRU), health care costs, and worsened outcomes. The Medicare Star Ratings (SR) is a program developed by the Centers for Medicare & Medicaid Services (CMS) to evaluate Medicare health plan quality and performance. Three of the SR quality measures assess medication adherence, showing the importance CMS places on medication adherence in older adults. Although a variety of medication adherence–enhancing interventions are available to help promote adherence, one intervention that has shown success historically is blister packing (BP).

OBJECTIVE: To model the potential impact of BP chronic medications on HCRU and health care costs in the Medicare population.

METHODS: An economic model was developed to assess the potential impact of BP the 3 Medicare SR adherence measure medication classes: renin-angiotensin system antagonists (RASAs), statins, and oral antidiabetics (OADs) using a 1-year time horizon. The model perspective was that of a hypothetical Medicare Advantage (MA) health plan with 100,000 members. Adherence was defined using dichotomous threshold of greater than or equal to 80% proportion of days covered. Literature-based references were used to inform the number of patients on each medication class, the impact of BP on the patients who become adherent (+12%), and the impact of medication adherence on HCRU and health care costs for each medication class. One-way sensitivity analyses and several scenario analyses were conducted to assess model uncertainty.

RESULTS: Owing to increased adherence from the BP intervention, the hypothetical health plan in the analysis saw 776 additional members adherent to RASAs, 1,651 additional members adherent to statins, and 414 additional members adherent to OADs. Although medication expenditure increased for all 3 medication classes (RASAs: +$274,963; statins: +$730,083; OADs: +$100,529), medical costs decreased across all classes (RASAs: –$4,098,848; statins: –$5,549,699; OADs: –$917,968). Total net health care costs decreased by $3,823,885 for RASAs (–$3.19 per member per month [PMPM]), $4,819,616 for statins (–$4.02 PMPM), and $817,438 for OADs (–$0.68 PMPM). The entire Medicare

population scenario analysis saw reduction in total health care costs of –$1,972,810,292 for RASAs, –$2,486,525,438 for statins, and –$421,730,836 for OADs.

CONCLUSIONS: Dispensing chronic medications with BP for MA patients was modeled to reduce HCRU and health care costs. Future studies are needed to assess whether the impact of BP medications is tied to reduced HCRU and costs in real-world settings.

SPONSORSHIP: None

E21 Barriers to growth hormone access in pediatric patients at an academic medical center

Baird D[1], *Parrish C*[1], *Redd T*[2], *Moore R*[1], *Choi L*[1], *Cruchelow K*[1]; *danielle.baird@vumc.org*; *charde.parrish@vumc.org*
[1]*Vanderbilt University Medical Center*; [2]*Lipscomb University College of Pharmacy*

BACKGROUND: Replacement of growth hormone therapy (hGH) at younger ages results in faster and greater growth. Evaluating barriers that may delay or limit hGH access is important to ensure timely and equitable treatment.

OBJECTIVE: To examine the time to hGH medication insurance approval, determine whether time to approval was associated with patient factors, and evaluate whether time to hGH initiation impacts patient growth velocity.

METHODS: Single center, retrospective review of electronic medical records of pediatric patients starting hGH treatment for a US Food and Drug Administration–approved indication at Vanderbilt Pediatric Endocrinology Clinic between January 1, 2018, and December 31, 2020. A Cox proportional hazards model assessed factors associated with time to approval: age, diagnosis, insurance, more testing required, and fulfilling pharmacy. A linear regression analysis assessed factors that impact patients' predicted height 1 year after the decision to treat: race, time to approval, interim medication usage, diagnosis, more testing required, and fulfilling pharmacy. Height z-scores are age and sex adjusted and were calculated using World Health Organization and Centers for Disease Control and Prevention growth standards

RESULTS: Inclusion criteria were met by 374 patients. Patients were a median age of 11 years (IQR = 8-13), 66% were male, and 80% identified as White, with a pretreatment height z-score of –2.5 (IQR = –3 to –2). Medication approval was received after 3 days (IQR = 1-6) with a prior authorization, 34 days (IQR = 22-69) with an appeal, 84 days (IQR = 59-126) with patient assistance program, and 118 days (IQR = 79-192) with cash pay. Patients with growth hormone deficiency ($P < 0.001$), Turner syndrome ($P = 0.016$), small for gestational age ($P = 0.05$), Prader-Willi syndrome ($P = 0.003$), panhypopituitarism ($P = 0.021$), and Noonan syndrome ($P < 0.002$) were more likely to have shorter times to medication approval relative to patients with idiopathic short stature. Patients whose insurance required additional testing were more likely to have longer times to approval ($P < 0.001$). Patients with Medicaid ($P = 0.02$), and patients who filled with an HSSP ($P < 0.001$) were more likely to have shorter time to approval. Baseline height was the most important variable predicting z-score height 1 year after the decision to treat ($P < 0.0001$), followed by diagnosis ($P = 0.002$) and race ($P = 0.02$).

CONCLUSIONS: Indication for hGH treatment, insurance, and fulfilling specialty pharmacy were shown to impact time to hGH access. However, time to hGh initiation did not impact patient growth velocity 1 year after the decision to treat.

SPONSORSHIP: None

E22 Influence of glucocorticoid dose reduction on US payer coverage decision-making

Kim H[1], *Mulrooney M*[2], *Leinwand B*[2], *Hadker N*[2], *Cicero S*[1], *Cheng H*[1]; *HKim@neurocrine.com*; *scicero@neurocrine.com*
[1]*Neurocrine Biosciences, Inc.*; [2]*Trinity Life Sciences*

BACKGROUND: Systemic glucocorticoids (GCs) are standard of care for many chronic conditions. However, long-term GC use carries significant risks of side effects (eg, skeletal, cardiometabolic, obesity) that lead to poor clinical, humanistic, and economic outcomes. Consequently, patients and providers seek to use the lowest efficacious dose of GCs to balance long-term efficacy with GC-related side effects. To our knowledge, no research has characterized US payers' coverage decisions on new medications that reduce patients' reliance on GCs.

OBJECTIVE: To understand US payer perceptions and coverage/access decisions for new therapies that reduce GC dose and the implications and significance of these decisions.

METHODS: Secondary research was conducted to identify and characterize 5 GC-reducing therapies to evaluate against payer coverage policies. Given the multifactorial nature of payer decision-making, qualitative interviews (n = 13) were also conducted across national/regional managed care organizations, pharmacy benefit managers, and managed Medicaid payer types to supplement the publicly available information.

RESULTS: GC dose reduction appeared to be a desirable goal/therapeutically beneficial for payers based on secondary review of national payer coverage policies; all therapies were covered in place of or in addition to GCs. Despite being priced at a substantial premium to low-cost alternatives, all therapies evaluated under the pharmacy benefit were

covered by some or all national payers with prior authorization to label or trial criteria. In qualitative interviews, payers understood the clinical and economic burden associated with long-term GC use. Payers noted that GC reduction is a secondary driver in coverage decision-making owing to focus on the trials' primary endpoints, none of which included GC reduction as a primary endpoint. A subset of payers highlighted the unique case of treatments for rare, chronic diseases without treatment alternatives, whereby GC reduction data are of particular relevance.

CONCLUSIONS: Despite carrying a premium price over GCs, each GC-reducing therapy was covered in place of or in addition to GCs. Additionally, payers acknowledged the clinical and economic value of reducing long-term GC use and may consider GC-reducing data in their decision-making. Payer recognition on the clinical, economic, and humanistic value of GC reduction may yield future actions that support coverage of and access to medications aiming to reduce clinical morbidities and patient burden associated with long-term use of GCs.

SPONSORSHIP: Neurocrine Biosciences, Inc.

E23 Disease- and glucocorticoid-related comorbidities in classic congenital adrenal hyperplasia: A claims-based retrospective cohort analysis

Cheng H[1], *Sen G*[1], *Kim H*[1], *Luo M*[1], *Cicero S*[1], *Leinwand B*[2], *Li M*[2], *Jeha G*[3]; *hkc80855@gmail.com*; *scicero@neurocrine.com*
[1]*Neurocrine Biosciences, Inc.*; [2]*Trinity Life Sciences*; [3]*Neurocrine Biosciences*

BACKGROUND: Classic congenital adrenal hyperplasia (CAH) due to 21-hydroxylase deficiency (21-OHD), a genetic disorder characterized by cortisol deficiency and excess adrenal androgens, typically requires management with supraphysiological glucocorticoid (GC) doses. Patients with classic CAH often face multiple complications related to excess androgen and/or supraphysiological GC doses

OBJECTIVE: To characterize complications among US patients with classic CAH.

METHODS: Merative's MarketScan retrospective insurance claims database from 2020 to 2022 was analyzed. The classic CAH cohort was defined as patients with (1) 2+ diagnoses of E25.0 at least 30 days apart anytime from 2020 to 2022; (2) a proprietary market definition specific to treatments and tests; and (3) 12 months of continuous 2022 enrollment. The frequency of comorbidities related to excess androgens and/or supraphysiological GC doses were captured using *International Classification of Diseases, Tenth Revision* codes and compared with a general population cohort matched by age, sex, payer type, region, and enrollment duration.

RESULTS: The classic CAH cohort consisted of 687 patients (57% female) with a mean age of 20 years. Compared with the general population, patients with classic CAH had significantly greater rates of multiple chronic conditions related to both excess androgens and supraphysiological GC doses, including cardiovascular disease (10.6% vs 6.6%), diabetes (5.1% vs 3.3%), and short stature (7.0% vs 0.9%). Rates of conditions often related to excess androgens, such as hirsutism and precocious puberty, were higher in patients with classic CAH (11.0% vs 1.0% and 10.0% vs 0.7%, respectively). Rates of comorbidities generally related to supraphysiological GC such as bone fracture (8.9% vs 6.5%), hypertension (13.2% vs 8.2%), and obesity (17.9% vs 8.7%) were also higher in patients with classic CAH. All *P* values were less than 0.05.

CONCLUSIONS: Patients with classic CAH have higher rates of comorbidities related to excess androgens and/or supraphysiological GC compared with the matched general population.

SPONSORSHIP: Neurocrine Biosciences, Inc.

E29 Assessing sociodemographic differences between adherent and nonadherent patients with obesity on glucagon-like peptide-1 medications

Wang J[1], *Irwin K*[2], *Lu K*[2], *Stolshek B*[2], *Saber J*[2], *Kennedy L*[2]; *jiayuw28@uci.edu*
[1]*University of California, Irvine, Innopiphany, LLC*; [2]*Innopiphany, LLC*

BACKGROUND: Glucagon-like peptide-1 (GLP-1) receptor agonists, a class of medication approved for patients with type 2 diabetes (T2D), have recently started seeing US Food and Drug Administration approval for obesity. Long-term adherence is a cornerstone to securing the best outcomes for patients with obesity. However, a recent study has found that 68% of individuals starting GLP-1s discontinue within a year.

OBJECTIVE: To assess the contribution of demographic and socioeconomic factors as well as payer mix on GLP-1 adherence among patients with obesity using data from the All of Us Research Program.

METHODS: Electronic health record and survey data from 2015 to 2022 were used to select adult patients with obesity (SNOMED: 414916001 or body mass index ≥ 30) who initiated GLP-1s within 2 years of diagnosis and had at least 12 months of complete GLP-1 prescription records. Pharmacy claims/pharmacy dispensing history and the number of days supply were used to calculate the percent days covered (PDC) over a 12-month period, with greater than 80% considered adherent. Univariable and multivariable logistic regression

(Bonferroni-corrected, $\alpha = 0.007$) were performed to evaluate the relationship between the patients' characteristics and GLP-1 adherence.

RESULTS: A total of 1,113 patients met the inclusion criteria. The mean total duration of GLP-1 coverage within the 12-month postinitiation period was 232 days, with 44% of patients considered adherent. GLP-1 users averaged age 56 years, and a majority were female (55%) and non-Hispanic White (60%), with commercial insurance (41%). Regression results found age at GLP-1 initiation, race and ethnicity, and insurance type to be statistically significant on adherence after controlling for sociodemographic and other covariates. Compared with White participants, Hispanic/Latinx individuals had a lower odds ratio (OR = 0.10, 95% CI = 0.04-0.22, $P < 0.001$) of adherence. Compared with commercially insured patients, those with Veterans Affairs/military/state-sponsored/other government/State Children's Health Insurance Program insurance had higher odds (OR = 2.06, 95% CI = 1.20-3.53, $P < 0.008$) of adherence. Additionally, age was positively associated with adherence (OR = 1.03, 95% CI = 1.01-1.04, $P < 0.001$), with each additional year increasing the odds by 3%.

CONCLUSIONS: GLP-1 adherence is critical to prevent relapse, and this study suggests that fewer than half of patients adhere to their medication. Further research is needed to understand the causes of nonadherence for certain subpopulations and insurance types.

SPONSORSHIP: Innopiphany, LLC

E30 Real-world trends in glucagon-like peptide-1 receptor agonist utilization for weight management among commercially insured individuals, 2021-2023

Coetzer H[1], *Pigoga J*[1], *Swami S*[1], *Anatale-Tardiff L*[1], *Kay J*[2], *Hagen S*[2], *Hashmi R*[2], *Darin R*[1]; *henriette.coetzer@bluehealthintelligence.com*; *sunil.swami@bluehealthintelligence.com*
[1]*Blue Health Intelligence*; [2]*Blue Cross Blue Shield Association*

BACKGROUND: In the United States, where 7 out of 10 adults experience overweight or obesity, use of glucagon-like peptide-1 receptor agonist (GLP-1) drugs for weight loss has significantly disrupted traditional weight management approaches. The rapid growth in GLP-1 prescriptions for this indication has led to intermittent shortages of the formulations, and access challenges may be affecting patients' abilities to use these products long-term. Insight into recent use of weight management GLP-1s, including duration of use and likelihood of achieving clinically meaningful weight loss, is necessary to inform effective care and spending related to these products.

OBJECTIVE: To describe trends in GLP-1 use for weight management.

METHODS: We examined national pharmacy and medical claims from January 1, 2021, to June 30, 2023, to identify first-time use of 2 weight management GLP-1s (semaglutide and liraglutide). Individuals were aged 18-64 years at index, with full commercial pharmacy and medical coverage for at least 6 months prior to and following initiation. Initiation was quantified by quarter, and medication persistence was measured, with nonpersistence defined as a gap following the most recent prescription of at least 2 times the expected duration of that prescription. A Kaplan-Meier analysis was used to identify changes in persistence over time.

RESULTS: From 2021 to 2023, 70,531 individuals initiated a weight management GLP-1, the majority of whom were female (79.0%) and aged 35-54 years (62.8%). GLP-1 initiation by quarter increased by more than 1,300%, from 1,479 new users in the first quarter of 2021 to 21,918 new users in the second quarter of 2023. As use increased, persistence to clinically meaningful weight loss decreased significantly: although 50.0% and 51.2% of individuals persisted to at least 12 weeks in 2021 and 2022, respectively, only 38.3% persisted to this point when initiating in 2023.

CONCLUSIONS: Use of weight management GLP-1s in commercially insured individuals has skyrocketed in recent years. Simultaneously, the likelihood of sustained use of these products decreased, and by 2023, nearly two-thirds of users were discontinuing medication prior to reaching meaningful weight loss thresholds. There are several potential drivers of lower persistence, including reduced access to medication, cost, and limited provider management of side effects and dosing. It is essential that payers and providers consider low persistence rates when evaluating the suitability of these medications for individual patients.

SPONSORSHIP: Blue Cross Blue Shield Association

E31 Association of prescription copayments and persistence on weight management glucagon-like peptide-1 receptor agonists: A real-world evidence study of commercially insured individuals, 2021-2023

Coetzer H[1], *Pigoga J*[1], *Swami S*[1], *Anatale-Tardiff L*[1], *Kay J*[2], *Hagen S*[2], *Hashmi R*[2], *Darin R*[1]; *henriette.coetzer@bluehealthintelligence.com*; *jennifer.pigoga@bluehealthintelligence.com*
[1]*Blue Health Intelligence*; [2]*Blue Cross Blue Shield Association*

BACKGROUND: The average monthly cost for weight management glucagon-like peptide-1 receptor agonists (GLP-1s) in the United States exceeds $1,000, and even when use of these products is approved by commercial insurance,

patients share a large burden of the cost through copayments. Many patients that start weight management GLP-1s do not continue them long-term, with a recent study finding that more than half of individuals that start GLP-1s do not continue beyond 12 weeks, when clinically meaningful weight loss is likely to occur. Poor persistence may be driven by a range of factors, including access and cost, and barriers to long-term use need to be described.

OBJECTIVE: To characterize the association between copayment amounts and persistence on GLP-1 products for weight management.

METHODS: We conducted a retrospective analysis of nationally representative commercial pharmacy and medical claims from January 1, 2021, to June 30, 2023, to identify users of 2 weight management GLP-1s (semaglutide and liraglutide). Included users were aged 18-64 years at the time of initiation, with a minimum of 6 months of pharmacy and medical coverage prior to and following initiation. Patient copayments were stratified by quartile, and medication persistence was measured, with nonpersistence defined as a gap following the most recent prescription of at least 2 times the duration of that prescription. Kaplan-Meier time-to-event analysis was used to evaluate differences in persistence across copayment groups.

RESULTS: From 2021 to 2023, 70,491 individuals initiated a GLP-1 for weight management and had copayment data available. Individuals paid a mean of $97.77 (SD of $148.08) per month in copayments. Approximately 20% (n = 17,302) of users paid less than $30.00 each month, whereas 29.7% (n = 20,936) paid $100.00 or more. Copayments had mixed effects on medication persistence: those with low (<$60.00) and high (>$99.99) copayments maintained GLP-1 use for significantly longer than those with a moderate payment ($60.00-$99.99).

CONCLUSIONS: Moderate monthly copayments may be associated with lower persistence on GLP-1s. Although high copayments were associated with better persistence than moderate, potentially because of financial buy-in, the most optimal persistence remains in the lowest quartile of costs to patients (<$30.00). Payers and providers alike should carefully consider patient cost sharing when considering the effective use of GLP-1s for weight management.

SPONSORSHIP: Blue Cross Blue Shield Association

E32 Prevalence and management and comorbid condition trends of obesity within US self-insured employers

Brook R[1], *Beren I*[2], *Rosenberg E*[2], *Peasah S*[3], *Good C*[4]; *rich@bh-ww.com*
[1]*Better Health Worldwide, Inc*; [2]*Workpartners*; [3]*UPMC*; [4]*UPMC Health Plan*

BACKGROUND: According to the National Institutes of Health 42.4% of US adults have obesity (men = 43%; women = 41.9%) and 9.2% have severe obesity (men = 6.9%; women = 11.5%). The approval of some glucagon-like peptide-1 (GLP1) agonists for obesity management has driven patients to seek a diagnosis (Dx) to access these medications.

OBJECTIVE: To examine obesity prevalence trends and, among those with an obesity Dx, examine trends in GLP1 use, costs, coexisting diabetes, and sleep apnea in employees and their covered spouses.

METHODS: Retrospective analysis of the US Workpartners Research Reference Database (2016-2022). Patients were identified by an obesity Dx (*International Classification of Diseases, Tenth Revision* = E66.0, E66.01, E66.02, E66.1, E66.2, E66.8, E66.9) and had at least 1 year of continuous data after Dx. Annual cohorts (employees and spouses) based on the Dx year and analysis focused on annual obesity: incidence, demographics, obesity and total medical costs, GLP1 and total drug costs, percentage receiving GLP1 and obesity drug therapy, percentage undergoing bariatric procedures, and percentage with type 2 diabetes mellitus (T2DM) or sleep apnea Dxs. Analyses also looked at annual trends and those with eligibility continuing GLP1 therapy in the second year. Costs are per patient per year and adjusted to December 2023 dollars.

RESULTS: Between 2016 and 2022, 94,526 patients were initially diagnosed with obesity (employees = 63,196; spouses = 31,330) with prevalence increasing from 4.8% to 6.4%. Employees with an obesity diagnosis (average = 45.9 years) were younger than spouses (average = 48 years), with more women employees (56.6%-60%, average = 58.4%) than spouses (61.8%-67%, average = 64.8%). GLP1 use increased over time and was higher in employees (3.9%-15.8%) than spouses (3%-10.9%). GLP1 costs increased for employees and spouses and were higher for employees ($230 to $1,147) than spouses ($152 to $796). Total drug costs increased (employees = $2,463 to $4,011; spouses = $2,668 to $3,934), and total medical costs increased (employees = $11,703 to $13,988; spouses = $15,207 to $17,894) The percentage with T2DM and sleep apnea decreased in both employees and spouses. The percentage of GLP1 users continuing in the second year increased from those initially diagnosed in

2016 from 73% to 84% in 2022. Employee absence costs and days decreased from 2016 to 2021 ($2,738 to $1,168, 9.8 to 5.3 days) and increased in 2022 ($1,212, 7.1 days), likely reflecting return to work-based utilization.

CONCLUSIONS: Among self-insured employers, the incidence of obesity increased during the study and is low compared with overall US reports. Higher use of GLPs is associated with reduced medical costs and, for employees, reduced absence costs and time. Further research is needed to determine whether those diagnosed are the most severely obese (highest body mass index) or randomly represent those seeking treatment.

SPONSORSHIP: None

E33 US commercial health plan coverage of semaglutide and tirzepatide for obesity management

Enright D, Beinfeld M, Chambers J;
denright1@tuftsmedicalcenter.org
Center for the Evaluation of Value and Risk in Health, Institute for Clinical Research and Health Policy Studies, Tufts Medical Center

BACKGROUND: Semaglutide and tirzepatide have proven extraordinarily effective for weight loss, garnering mass media attention and solidifying their blockbuster status. Beyond weight loss, recent studies suggest that semaglutide improves outcomes for patients with cardiovascular disease and has shown promise for other diseases, expanding the patient populations that could benefit from the drug. However, high demand and annual costs raise questions about how payers will facilitate access to these drugs while remaining budget conscious.

OBJECTIVE: To examine how large US commercial health plans cover semaglutide and tirzepatide for obesity management.

METHODS: We used the Tufts Medical Center Specialty Drug Evidence and Coverage Database to identify coverage policies for semaglutide and tirzepatide issued by 18 large US commercial health plans current as of April 2024. We examined the following coverage requirements: weight restrictions (eg, body mass index >30 kg/m^2), step therapy protocols, diet and exercise requirements, required enrollment in a lifestyle modification program, approval duration, and continuation criteria.

RESULTS: Thirteen of 18 health plans issued policies for semaglutide and 10 did so for tirzepatide. Five health plans had not issued publicly available coverage information for either drug. All plans included weight and diet and exercise requirements for both drugs. Two plans had a step therapy protocol for both drugs; one required patients to first try phentermine/topiramate or naltrexone/bupropion, whereas the other required a step through phentermine then Ozempic (the formulation of semaglutide US Food and Drug Administration (FDA) approved to treat type 2 diabetes in adults). For semaglutide, 8 plans required patients to enroll in a lifestyle modification program, whereas 6 plans required the same for tirzepatide. Approval duration for both drugs varied across plans (3-12 months). All plans required documented weight loss for continuation of coverage.

CONCLUSIONS: Health plans in our sample often implemented requirements for diet and exercise and enrollment in lifestyle modification programs for access to semaglutide or tirzepatide. Whereas some requirements were in line with the treatments' FDA label (eg, weight requirements), others were not (eg, step therapy protocols). Variation in coverage requirements across health plans may result in inconsistent access to semaglutide and tirzepatide for patients.

SPONSORSHIP: None

E34 Cardiovascular benefits of weight loss medications: An analysis of Mounjaro and Wegovy in the US Medicare population

Baser O[1], Samayoa G[2], Rodchenko K[2];
onur.baser@sph.cuny.edu; gabriela@cdanyc.com
[1]City University of New York (CUNY) School of Public Health; [2]Columbia Data Analytics

BACKGROUND: Clinical trials have shown that semaglutide use reduced risk to the heart. However, the effects of semaglutide and tirzepatide on cardiovascular risk have not been compared.

OBJECTIVE: To compare, using the Medicare dataset, risk of coronary artery disease, heart failure, atrial fibrillation, arrhythmia, ischemic heart disease, stroke, and peripheral vascular disease among patients with/without antiobesity medication (AOM) use; Mounjaro and Wegovy use were also compared.

METHODS: A retrospective study was performed using 2021-2024 Medicare claims data. Patients diagnosed with obesity were identified if they received Mounjaro or Wegovy from June 1, 2022, to May 31, 2023 (identification period), with 1-year follow-up to measure cardiovascular event risk. Patients with AOM use and/or any cardiovascular event prior to the identification period were excluded. Cox regression was used to examine the association between cardiovascular risk and medication use while adjusting for beneficiary demographics, comorbidity scores, socioeconomic status, and baseline cardiovascular-related comorbidities.

RESULTS: Among 4,384 beneficiaries who met the inclusion criteria, 2,794 used Mounjaro and 1,590 used Wegovy. The risk-adjusted likelihood of cardiovascular risk was 4% ($P<0.001$) lower among patients with vs without AOM use. Mounjaro users were more likely to be older (66.64 vs 65.54, $P<0.001$), be male (32.43% vs 20.0%, $P<0.001$), and have higher comorbidity scores (44.20% vs 17.30%, $p< 0.001$) and higher cardiovascular disease–related comorbidities (85.5% vs 72.33%, $P<0.001$) than Wegovy users. After adjusting for these factors, there were no significant differences for heart failure ($P=0.17$), atrial fibrillation ($P=0.14$), arrhythmia ($P=0.88$), ischemic heart disease ($P=0.38$), and stroke outcomes ($P=0.41$). However, for Mounjaro users, the likelihood of coronary artery disease (odds ratio=1.5, $P<0.001$) and peripheral vascular disease (odds ratio=1.8, $P<0.001$) was higher.

CONCLUSIONS: Economists have warned that the cost of expanding Medicare prescription drug coverage for new AOMs could be catastrophic. This exclusion is under scrutiny amid evidence that these medications offer health benefits extending well beyond shedding pounds. Although the effects differ, the results show that AOM use offers cardiovascular benefits.

SPONSORSHIP: None

E35 Semaglutide 2.4-mg treatment in patients with obesity or overweight: A real-world retrospective cohort study in the United States (SCOPE: long-term clinical outcomes)

Ruseva A[1], *Dabbous* F[2], *Ding* N[2], *Huse* S[2], *Nordstrom* B[2], *Fabricatore* A[1], *Michalak* W[1], *Ó Hartaigh* B[1], *Zhao* Z[1]; *uaru@novonordisk.com*
[1]*Novo Nordisk Inc.*; [2]*Evidera*

BACKGROUND: The glucagon-like peptide-1 receptor agonist semaglutide, at doses of 1.7 and 2.4 mg once weekly, is indicated as an adjunct to lifestyle changes in adults with obesity, or with overweight and at least 1 weight-related comorbidity, for chronic weight management.

OBJECTIVE: To assess the long-term effectiveness of semaglutide 2.4 mg for chronic weight management.

METHODS: Using claims data and electronic medical records from the US-based Komodo Health database, this retrospective, real-world cohort study included patients aged 18 years and older at the index date (date of first semaglutide treatment claim), with body mass index (BMI) greater than or equal to 30 kg/m^2, or BMI 27-29.9 kg/m^2 and at least 1 weight-related comorbid condition. Patients who had escalated to and maintained treatment with semaglutide 2.4 mg (initiation date: June 21, 2021, to June 30, 2022) were included. Eligible patients had available data for the 6 months prior to and at least 68 weeks after the index date. Study outcomes included change from baseline at week 68 in body weight, BMI, blood pressure (BP), hemoglobin A1c, and lipid profiles. The sample size assessed for each biomarker varied owing to availability of data from the electronic medical records. Outcomes were summarized descriptively.

RESULTS: Of patients who met the inclusion criteria (n=3,323), 77.4% were female and mean (SD) age was 47.1 (9.8) years. At baseline, mean (SD) weight (n=815) and BMI (n=2,048) were 106.9 (19.5) kg and 36.7 (3.6) kg/m^2, respectively. Overall, mean percentage change in body weight at week 68 was −14.8% (n=391) and mean change in BMI was −4.9 kg/m^2 (n=696). Body weight reduction of greater than or equal to 5% was achieved by 88.5% (n=346) of patients, greater than or equal to 10% by 74.4% (n=291), greater than or equal to 15% by 50.9% (n=199), and greater than or equal to 20% by 30.4% (n=119). Improvements in cardiometabolic risk factors from baseline to week 68 were also seen in mean changes of −6.1 mm Hg in systolic BP (n=446), −3.4 mm Hg in diastolic BP (n=449), −0.5% in A1c (n=150), −9.3 mg/dL in low-density lipoprotein cholesterol (n=62), 2.9 mg/dL in high-density lipoprotein cholesterol (n=75), and −36.2 mg/dL in triglycerides (n=73).

CONCLUSIONS: In this 68-week real-world study, patients with obesity/overweight who received continuous semaglutide 2.4-mg treatment for chronic weight management demonstrated clinically relevant reductions in body weight and associated clinical biomarkers. The 68-week clinical biomarker outcomes from this study are consistent with clinical trial findings.

SPONSORSHIP: Novo Nordisk Inc.

E36 Effect of weight loss medications on cardiovascular risk among active US military personnel

Baser O[1], *Kuclo* A[2], *Samayoa* G[2], *Baser* E[2], *Yapar* N[2]; *onur.baser@sph.cuny.edu*; *nehir@cdanyc.com*
[1]*City University of New York (CUNY) School of Public Health*; [2]*Columbia Data Analytics*

BACKGROUND: Weight-loss prescriptions for US service members remain low but have risen sharply since 2018, when the Department of Defense began authorizing coverage of medications for active-duty troops struggling with weight control. However, the beneficial effect of these medications on cardiovascular risk has not been assessed in this population.

OBJECTIVE: To analyze the impact of Mounjaro and Wegovy use on the likelihood of experiencing a cardiovascular event and to compare the risks associated with each medication among active US military personnel.

METHODS: A retrospective analysis was conducted using Department of Defense data spanning from January 1, 2021, to May 31, 2024. Patients diagnosed with obesity were identified based on their use of Mounjaro or Wegovy between January 1, 2022, and May 31, 2023 (identification period), with 1-year follow-up to assess cardiovascular events. Patients who previously used an obesity medication or experienced any cardiovascular events prior to identification were excluded. Cox regression analysis was used to examine the association between medication and cardiovascular risk while controlling for beneficiary demographics, comorbidity scores, socioeconomic status, and baseline cardiovascular disease–related comorbidities.

RESULTS: Of the 3,687 beneficiaries meeting the inclusion requirements, 1,604 used Mounjaro and 2,083 used Wegovy. Among those with medication use, 82.4% were female. Beneficiaries using medication were mostly aged 41–60 years (47.25%), and 51.34% had cardiovascular-related comorbidities in the baseline period. After controlling for demographics and socioeconomic and clinical factors, obesity medication use was associated with a 16% reduction in cardiovascular risk ($P < 0.05$). There were no significant differences in cardiovascular risk between Wegovy and Mounjaro users (odds ratio = 1.07; CI = 0.77–1.49).

CONCLUSIONS: The results showed clear evidence that the use of Mounjaro and Wegovy offers cardiovascular benefits. The type of medication did not significantly affect the risk of a cardiovascular event.

SPONSORSHIP: None

E37 Effect of antiobesity medications on cardiovascular events in the Veterans Affairs patient population

Baser O[1], *Kuclo A*[2], *Budhu A*[2], *Yapar N*[2];
onur.baser@sph.cuny.edu; *nehir@cdanyc.com*
[1]*City University of New York (CUNY) School of Public Health*; [2]*Columbia Data Analytics*

BACKGROUND: Obesity is a major public health challenge, and the US military veteran population is disproportionately affected. Antiobesity medications (AOMs) are associated with clinically significant weight loss among veterans with overweight or obesity, yet their beneficial effect on cardiovascular risk has not been studied.

OBJECTIVE: To examined the effect of AOMs on the risk of cardiovascular events and compare risk by medication in the US Veterans Affairs patient population.

METHODS: This retrospective analysis examined Veterans Affairs data from 2021 to 2024. Patients with an obesity diagnosis were identified if they had evidence of Mounjaro or Wegovy use between 2022 and June 2023 (identification period), with follow-up until June 2024 to examine any cardiovascular events. Patients were excluded if they had evidence of AOM use and/or any cardiovascular event prior to the identification period. Cox regression was used to investigate the relationship between cardiovascular event risk and medication use after accounting for beneficiary demographics, comorbidity scores, socioeconomic status, and baseline cardiovascular disease–related comorbidities.

RESULTS: Of the 2,347 beneficiaries who met the inclusion requirements, 1,083 used Mounjaro and 1,264 used Wegovy. The cardiovascular benefits of using these drugs were clear. Medication users had a 7.16% risk of any cardiovascular event vs nonmedication users (10.49%; $P < 0.001$). Mounjaro users were older (53.79 vs 49.47 years, $P < 0.001$) and more likely to be male (34.9% vs 24.11%, $P < 0.001$), had higher comorbidity scores (30.01% vs 10.05%, $P < 0.001$), and had more cardiovascular disease–related comorbidities (74.52% vs 57.67%, $P < 0.001$) vs Wegovy users. After accounting for these variables, there were no significant differences in heart failure ($P = 0.42$), atrial fibrillation ($P = 0.31$), arrhythmia ($P = 0.41$), ischemic heart disease ($P = 0.31$), or stroke ($P = 0.14$) between the cohorts. However, Mounjaro users had a higher risk of coronary artery disease (odds ratio = 1.79, $P < 0.001$).

CONCLUSIONS: Despite their differences, our findings provide evidence that use of Mounjaro and Wegovy can be beneficial to cardiovascular health.

SPONSORSHIP: None

E38 Unlocking value: The cost-effectiveness of glucagon-like peptide 1 agonists for obesity treatment in patients without diabetes—A comprehensive meta-analysis

Meraz M[1], *Dhippayom T*[2], *Lee H*[1], *Hur C*[3], *Inadomi J*[4], *Veettil S*[5], *Chaiyakunapruk N*[1];
Manuel.Meraz@pharm.utah.edu; *teerapond@nu.ac.th*
[1]*University of Utah College of Pharmacy*; [2]*Naresuan University*; [3]*Columbia University Irving Medical Center*; [4]*University of Utah School of Medicine*; [5]*University of Utah*

BACKGROUND: Glucagon-like peptide-1 receptor agonists (GLP-1RAs) are increasingly used for obesity treatment despite their relatively high cost. However, conclusive evidence of their cost-effectiveness is lacking.

OBJECTIVE: To conduct a systematic review and meta-analysis to quantitatively pool the INB of GLP-1RAs used in the treatment of obesity in patients without diabetes compared with other treatments and no treatment.

METHODS: We searched PubMed, Embase, EconLit, CEA Registry, and ProQuest Dissertations & Theses Global

from inception to April 2024. Cost-effectiveness studies were included if they reported economic outcomes of any GLP-1 RAs (semaglutide and liraglutide) in the treatment of obesity in patients without diabetes for a minimum time horizon of 5 years. The comparators included semaglutide, liraglutide, phentermine plus topiramate, lifestyle intervention, naltrexone plus bupropion, and no treatment. Monetary units were converted to 2023 US dollars. Heterogeneity was assessed using I^2 statistic. The INBs, with 95% CIs, were calculated and pooled across studies using a random-effects model, stratified by country income level and study perspective.

RESULTS: Of 634 studies identified, 8 studies with 23 comparisons were included in this meta-analysis. All comparisons were assessed from high-income countries and the health care/payer perspective. Pooled INB of GLP-1RAs compared with lifestyle interventions demonstrated that GLP-1RAs are not cost-effective (n = 6; pooled INB = –$57,762; 95% CI = –$65,971 to –$49,553; I^2 = 96.2%). When comparing with other pharmacotherapy interventions, GLP-1RAs are not cost-effective compared with phentermine plus topiramate (n = 5; pooled INB = –$25,992; 95% CI = –$40,552 to –$11,432; I^2 = 50.9%) and naltrexone plus topiramate (n = 2; pooled INB = –$7,496; 95% CI = –$30,034 to $15,041; I^2 = 28.9%). Semaglutide tends to be cost-effective compared with liraglutide (n = 4; pooled INB = $15,002; 95% CI = –$5,643 to $35,646; I^2 = 0%) without statistical significance.

CONCLUSIONS: GLP-1RAs are not cost-effective in the treatment of obesity compared with other interventions in patients who do not have diabetes in high-income countries from a health care/payer perspective. Clinicians and policymakers may consider this pooled economic evidence as part of their formulary decision-making process.

SPONSORSHIP: None

E41 Real-world effectiveness of bempedoic acid and bempedoic acid plus ezetimibe on low-density lipoprotein cholesterol using claims-linked electronic health record data

Nelson J[1], *Bloedon L*[2], *Lewandowski D*[1], *Bonafede M*[1], *Sarnes E*[2]; *esarnes@esperion.com*
[1]*Veradigm*; [2]*Esperion Therapeutics, Inc*

BACKGROUND: Bempedoic acid (BA) and the combination with ezetimibe (BA+EZE) have demonstrated low-density lipoprotein cholesterol (LDL-C) lowering in clinical trials of adults with primary hyperlipidemia at high risk for or with cardiovascular disease. The impact of BA+EZE on LDL-C outcomes in a real-world patient cohort has not been evaluated.

OBJECTIVE: To evaluate the effectiveness of BA and BA+EZE on LDL-C reduction and goal achievement using real-world data sources.

METHODS: This was a retrospective cohort study using deidentified electronic health record data linked with claims. Adult patients were included if they had a pharmacy claim for BA or for BA+EZE from March 1, 2020, to March 15, 2024 (date of first pharmacy = index date), at least 1 baseline LDL-C laboratory result on or within the 6 months prior to the index date, and at least 2 LDL-C laboratory results post-index, including 1 laboratory result 12 months post-index (±60 days). BA+EZE patients with documented use of ezetimibe on or within 6 weeks prior to index were excluded. LDL-C was determined at baseline (the value closest to but not after the index date), 3 months (±30 days), 6 months (±60 days), and 12 months (±60 days).

RESULTS: A total of 900 patients initiating BA therapy and 615 patients initiating BA+EZE therapy met all study criteria. The mean age was 64 ± 11 and 62 ± 11 years for BA and BA+EZE patients, respectively; 57% and 52% were women. Most patients had evidence of statin use in the prior 12 months (58% and 64%, respectively). At baseline, 25% of BA patients had LDL-C less than 100 mg/dL; at 3 months, the proportion with LDL-C less than 100 mg/dL increased to 42% and was sustained at 6 and 12 months. For BA+EZE patients, the proportion achieving LDL-C less than 100 mg/dL more than doubled from index (30%) to 3 months (67%) and was also sustained through 12 months (55%). In both cohorts, there were fewer patients with LDL-C greater than or equal to 130 mg/dL at 12 months vs index (BA: 33% vs 57%; BA+EZE: 24% vs 47%). For BA patients, the baseline LDL-C was 137 ± 46 mg/dL and decreased by 18% at 3 months post-index; this was sustained through 12 months post-index ($P < 0.0001$ for all vs index). The mean LDL-C reduction for the BA+EZE patients was 28% at 3 months (to 94 ± 47 mg/dL), 27% at 6 months (96 ± 44 mg/dL), and 22% at 12 months (102 ± 47 mg/dL) ($P < 0.0001$ for all vs index).

CONCLUSIONS: In an analysis of real-world data, patients who initiated BA or BA+EZE showed early and sustained LDL-C reduction and attainment of levels less than 100 mg/dL.

SPONSORSHIP: Esperion Therapeutics, Inc

E42 Real-world adherence and persistence to glucagon-like peptide-1 receptor agonists at 2 years among commercially insured adults with obesity without diabetes

Marshall L[1], *Qiu Y*[2], *Urick B*[1], *Friedlander N*[1], *Leslie S*[1], *Gleason P*[1]; *landon.marshall@primetherapeutics.com*
[1]*Prime Therapeutics*; [2]*Prime Therapeutics*

BACKGROUND: Real-world evidence has found most patients without diabetes initiating glucagon-like peptide-1 (GLP-1) drug therapy for weight loss discontinue within the first year. Little is known of the beyond-year-1 real-world GLP-1 obesity treatment adherence and persistency. This study is a 2-year follow-up extension to previously published work by Gleason and colleagues (doi:10.18553/jmcp.2024.23332) examining GLP-1 weight-loss treatment adherence and persistency at 1 year.

OBJECTIVE: To measure adherence and persistence to obesity GLP-1 therapy at the end of 2 years of follow-up in a real-world cohort of commercially insured members without diabetes.

METHODS: Prime Therapeutics' integrated pharmacy and medical claims data from an average monthly 16.5 million commercially insured membership were used to identify members without diabetes newly initiating GLP-1 weight loss treatment between January 1, 2021, and December 31, 2021 (index date period), with continuous enrollment 1 year before (pre-period) and 2 years after (post-period) the index date and no GLP-1 drug claim in the pre-period. During the pre-period, members were required to have a medical claim indicating obesity without a diabetes diagnosis or diabetes drug claim and to be aged 19 years or older. Adherence was measured as the proportion of days covered (PDC) in the post-period, and members with a PDC greater than or equal to 80% were considered adherent. Persistence was measured as no greater-than-or-equal-to-60-day gap between a claim days supply ending and a subsequent claim fill date in the post-period. GLP-1 product switching was allowed during the assessment period, and switch rates were descriptively assessed.

RESULTS: Among the 4,070 members in the initial 1-year follow-up, 3,364 (83%) were continuously enrolled in the 2-year post-period. The mean age was 46 years and 81% were female. Overall GLP-1 persistency was 47% at 180 days, 29% at 1 year, and 15% at 2 years. The highest and lowest persistency rates at 2 years were observed for weekly semaglutide (24%; Wegovy) and daily liraglutide (7%; Victoza), respectively. Average PDC over 2 years was 41%, with 17% of members adherent to therapy and 25.8% switching GLP-1 drugs.

CONCLUSIONS: Two-year GLP-1 weight-loss treatment adherence and persistency was poor, with 17% adherent and 1 in 7 members remaining on therapy. These findings highlight substantial GLP-1 therapy investment risk due to waste. Obesity care management programs and value-based contracts from pharmaceutical manufacturers may help mitigate financial risk.

SPONSORSHIP: Prime Therapeutics, LLC

E44 The cost of proprotein convertase subtilisin/kexin type 9 inhibitor monoclonal antibody (PCSK9i mAb) waste: Results from a patient-reported survey on PCSK9i mAb compliance and discontinuation

Ali Y[1], *LePetri B*[2], *Resaul R*[2]; *yousuf.ali@novartis.com*
[1]*Novartis Pharmaceuticals*; [2]*The Value Builders, a Bloc Agency*

BACKGROUND: Although several measurements are used to calculate adherence to self-administered (SA) proprotein convertase subtilisin/kexin type 9 inhibitor monoclonal antibody (PCSK9i mAb) therapy, wastage due to missed doses and unintentional autorefills is not typically accounted for in these calculations. We hypothesized that PCSK9i mAb wastage may have a financial impact on patients, health plans, and employers.

OBJECTIVE: To determine the degree of drug wastage and the resulting cost for SA PCSK9i mAbs.

METHODS: A patient-reported survey of adults in the United States was conducted from January 30 to February 27, 2024, in partnership between The Value Builders and Ipsos. Patients included may have hypercholesterolemia and/or were told by a physician that they were at risk of or have established cardiovascular disease and had been prescribed a PCSK9i mAb in the past 3 months. A survey link was emailed to existing patient panels. Patients remained anonymous to researchers. Potential dose wastage rate range was estimated by analyzing 2 definitions of wastage: wastage rate due to months of therapy missed in a given year (WMOT-M) and wastage rate due to months of therapy missed inclusive of refills despite stopping and never restarting treatments in a given year (WMOT-MR).

RESULTS: A sample of N = 500 was determined to be representative of the US population with a margin of error of ±4.8% at a 95% CI. To calculate WMOT-M, we observed 1,905 total filled doses with 1,701 total taken doses, resulting in 204 missed doses. Extrapolating across a year resulted in 816 annual doses missed, equating to 470 months of treatment missed. Total missed months of treatment was divided by the 4,298 total potential months of treatment, yielding a WMOT-M of 10.9%. To calculate WMOT-MR, we observed

an additional 51 individuals who, despite discontinuing treatment, continued receiving an autorefill. They refilled a total number of 82 times, which extrapolated to a total 176 months of therapy, yielding a WMOT-MR of 15.0%. WMOT-MR results in a US pharmacy spend of $240,011,885 based on wholesale acquisition costs and annual PCSK9i mAb prescriptions filled in 2023.

CONCLUSIONS: Estimated PCSK9i mAb wastage due to missed doses and unintentional autorefills has considerable cost implications for patients, health plans, and employers. To our knowledge, this is the first time wastage has been quantified for PCSK9i mAb therapies. These results show that wastage should be considered along with adherence measurements such as medical possession ratio to determine the overall value of SA PCSK9i mAb therapy.

SPONSORSHIP: Novartis Pharmaceuticals Corporation

F00-F99 Mental and Behavioral Disorders

(eg, antipsychotics, bipolar disorder, depression, schizophrenia)

F3 Understanding trends in the diagnosis of mild cognitive impairment and mild dementia from the Health and Retirement Study

Spargo A[1], *Gou R*[1], *Plamondon E*[2], *Klein E*[1], *Nelson D*[1]; *spargo_andrew@lilly.com*
[1]*Eli Lilly and Company*; [2]*Marion County Public Health Department*

BACKGROUND: Alzheimer disease (AD) is a common cause of mild cognitive impairment (MCI) and dementia, with AD pathology present in 50% and 60%-80% of cases, respectively. Timely and accurate AD diagnosis enables appropriate intervention earlier in the disease course, potentially improving outcomes. Understanding how often patients are diagnosed in early stages of impairment may help predict demand for new interventions and prioritize solutions to improve diagnosis.

OBJECTIVE: To characterize the prevalence and longitudinal trend in diagnosis of MCI and mild dementia in the United States.

METHODS: This retrospective analysis used longitudinal data from the Health and Retirement Study, a nationally representative, biennial panel survey on aging in adults aged 50 years and older and their partners. The analysis included respondents aged 60 years and older with classification of cognitive status during any survey wave from 2010 to 2018 and used sampling weights, clusters, and strata derived from the Health and Retirement Study complex survey design. Demographic and socioeconomic characteristics, cognitive status, and diagnosis of AD at each survey wave were described. Change in diagnosis over time (ie, in each subsequent survey wave), controlling for respondent characteristics (age, sex, and race and ethnicity), was evaluated using multivariable logistic regression.

RESULTS: The analysis included 13,842 respondents in 2010 (mean age 71.2 years, 55.8% female) decreasing to 11,856 in 2018 (mean age 70.9 years, 54.3% female). In 2018, this sample represents 73.8 million (M; SE = 2.3M) people aged 60 years and older, of whom 8.6M had MCI (13.5%; SE = 0.3M; mean age 74.9 years, 55.4% female) and 1.3M had mild dementia (2.1%; SE = 0.1M; mean age 76.1 years, 52.0% female). In 2018, only 1.8% of respondents with MCI and 6.9% of respondents with mild dementia reported an AD diagnosis. Holding all other predictor variables constant, the odds of AD diagnosis did not change over time among respondents with MCI (odds ratio [OR] =1.04 [95% CI = 0.90-1.21]) or mild dementia (OR = 1.12 [95% CI = 0.94-1.33]). Among those with MCI, respondents who were older (OR = 1.53 for every 10-year increase in age [95% CI = 1.22-1.91]) or male (OR = 1.75 [95% CI = 1.15-2.68]) were more likely to report an AD diagnosis.

CONCLUSIONS: Few respondents with MCI and mild dementia reported an AD diagnosis, and the likelihood of AD diagnosis did not change over time. Respondents with MCI who were older or male were more likely to report an AD diagnosis. Further solutions to improve timely assessment and diagnosis are needed.

SPONSORSHIP: Eli Lilly and Company

F11 Real-world TV-46000 prescribing behaviors in the United States since approval to treat schizophrenia: US claims database analysis of treatment patterns

Thompson S[1], *Nathan R*[2], *Suett M*[3], *Tripathi P*[2], *Franzenburg K*[4], *Hansen R*[4], *Russo P*[2]; *Stephen.Thompson01@tevapharm.com*; *parusso@eversana.com*
[1]*Teva Branded Pharmaceutical Products R&D, Inc., Global Health Economics and Outcomes Research*; [2]EVERSANA LLC; [3]*Teva UK Limited*; [4]*Teva Branded Pharmaceutical Products R&D, Inc.*

BACKGROUND: TV-46000, a subcutaneous long-acting injectable (LAI) risperidone formulation approved in the United States in April 2023 for adults with schizophrenia (SCZ), can be administered once monthly (q1m) or once every 2 months (q2m). Limited data are available on prescribing

patterns for TV-46000 since approval, particularly on both patient characteristics and dosing frequencies prescribed.

OBJECTIVE: To examine demographic, clinical, and treatment characteristics of eligible US patients who received a prescription for TV-46000.

METHODS: EVERSANA administrative claims data from August 2018 to February 2024 were examined. Patients prescribed TV-46000 who had either at least 2 months of data prior to index date or activity before May 1, 2023, were included. Index date was first TV-46000 claim date, unless there was a later first claim for an SCZ diagnosis (SCZ index date). Administration frequency was q1m with days supply values less than 35 days and q2m for greater than or equal to 35 days. Persistence was calculated using Kaplan-Meier methodology based on time to discontinuation, defined as exceeding the fixed 30-day permissible gap (time from end of one prescription to start of next) after the treatment's given days supply. Line of therapy (LOT) switching was studied considering only second-generation (SG) LAIs for adult patients diagnosed with SCZ and treated with TV-46000 with 1 year of data prior to SCZ index date.

RESULTS: Of the 1,773 patients treated with TV-46000 (1,421 *q*1m, 352 *q*2m; mean age 41.5 years; 38% aged 18-34 years; 42% female), 715 had an SCZ diagnosis (563 q1m, 152 q2m). Of the 764 without an SCZ diagnosis, 39% had anxiety disorders, 31% had bipolar disorder, and 28% had schizoaffective disorder. Most patients who received TV-46000 q1m or q2m did not change between the 2 dosing frequencies (96%, 1,697/1,773). Of the 4% (68/1,697) who switched, 80% changed from q1m to q2m and 20% from q2m to q1m when switching for the first time. LOT analysis found that 58% (218/378) of patients with an SCZ diagnosis prescribed TV-46000 had received TV-46000 as their first SG LAI (44% q1m and 12% q2m), and that TV-46000 overall was the most common second-line SG LAI, given to 61% (98/161) of patients who received a second-line SG LAI (47% q1m and 13% q2m). Most (≈74%) adult patients with SCZ prescribed TV-46000 were persistent with treatment 4 months after first prescription.

CONCLUSIONS: In the 10 months since approval, more than half of patients who received TV-46000 had started it as their first SG LAI. Persistence with TV-46000 was high, at 75% at 4 months.

SPONSORSHIP: Teva Branded Pharmaceutical Products R&D, Inc.

F12 Onset of metabolic disorders following schizophrenia diagnosis: Prevalence and associated health care costs

Cajigal A[1], *Sidovar* M[1], *Gillard* K[1], *Zulkarnain* Z[2], *Tkacz* J[2]; *alejandro.cajigal@karunatx.com*; *joseph.tkacz@inovalon.com*
[1]*Bristol Myers Squibb*; [2]*Inovalon*

BACKGROUND: Metabolic disorders are prevalent among the schizophrenia population owing to relatively poor access to health care, lifestyle factors, and the metabolic side effects associated with antipsychotic treatment.

OBJECTIVE: To evaluate the prevalence and associated health care costs of metabolic disorders among a national sample of patients with schizophrenia living in the United States.

METHODS: This study was a retrospective analysis of the MORE2 Registry of commercial, Medicare Advantage, and Managed Medicaid claims, in addition to the 100% Medicare Fee-for-Service claims database between January 1, 2016, and September 30, 2023. Inclusion criteria were as follows: (1) at least 1 inpatient or at least 2 nondiagnostic outpatient claims separated by 30-365 days with a schizophrenia diagnosis on or following January 1, 2017 (the earliest claim = index date); (2) at least 12 months of continuous enrollment prior to and following index; (3) an absence of any diagnoses of schizophrenia any time prior to the index date; and (4) an absence of any diagnoses of diabetes, obesity, hyperlipidemia, or metabolic syndrome any time preceding the index date. During the 12-month post-index period, the rate of onset of metabolic disorders was assessed by the appearance of at least 2 claims for a specific metabolic disorder. Total all-cause health care costs were assessed during the 12-month post-index period between patients with and without evidence of a metabolic disorder.

RESULTS: A total of 140,122 patients with schizophrenia without a history of diagnosis prior to the index date or evidence of a prior metabolic disorder qualified for the study. During the 12-month post-index period, 31.4% (n = 44,004) presented diagnostic evidence of a metabolic disorder. Compared with patients without a metabolic disorder, patients with a metabolic disorder were older (mean ± SD = 47.6 ± 16.1 vs 39.7 ± 16.9), were more likely to be female (44.6% vs 36.9%), and incurred significantly greater all-cause annual health care costs ($30,863 ± $44,376 vs $26,705 ± $38,623; all $P < 0.0001$) during the 12-month post-index period.

CONCLUSIONS: This descriptive analysis showed that nearly 1 in 3 patients with a schizophrenia diagnosis showed evidence of a new metabolic disorder following their first diagnosis within the analytic time frame, associated with an increase in health care costs. Schizophrenia treatment

options that do not contribute to the risk of metabolic disorders will be critical to maintain patients' overall health and reduce the economic burden of schizophrenia.

SPONSORSHIP: Karuna Therapeutics, a Bristol Myers Squibb company

F13 Cost-effectiveness analysis of KarXT for the treatment of schizophrenia in the United States

Mantaian T[1], *Gillard K*[2], *Sidovar M*[2], *Kowalski J*[1], *Meng Y*[1], *Kramer K*[2], *Cutler A*[3]; *Tyler.Mantaian@lumanity.com*; *matt.sidovar@karunatx.com*
[1]*Lumanity*; [2]*Bristol Myers Squibb*; [3]*SUNY Upstate Medical University*

BACKGROUND: Schizophrenia is a severe, lifelong mental disorder leading to significant disability. Current standard of care consists of antipsychotic agents, which block dopamine receptors in the brain and are linked to treatment-limiting adverse events (AEs) such as movement and metabolic disorders. KarXT, an investigational medication targeting muscarinic receptors, has demonstrated clinically meaningful efficacy and may offer a well-tolerated alternative to traditional dopaminergic antipsychotics.

OBJECTIVE: To assess the cost-effectiveness of KarXT in a first-line (1L) setting compared with generic aripiprazole in adult patients with schizophrenia in the United States.

METHODS: A Microsoft Excel model with lifetime time horizon, Markov-based approach, and US payer perspective compared KarXT with aripiprazole in 1L treatment. The model allowed 2 lines of subsequent treatment including risperidone or olanzapine, followed by clozapine. Patients entered the model based on initial treatment response rates; nonresponders entered on a subsequent treatment. Health states were defined as full compliant and noncompliant and included the following adverse events (AEs): metabolic syndrome, diabetes, cardiovascular disease, and tardive dyskinesia. State transitions were based on discontinuation (AE- and non–AE-specific) and relapse rates. Retreatment in the same line is allowed for some noncompliant patients who relapsed. Direct medical, pharmacy, and AE-related costs were included. Time spent with AEs, life-years, and quality-adjusted life-years (QALYs) were estimated. Because a price for KarXT was not available, threshold and sensitivity analyses were conducted to assess the price point at which KarXT would be cost-effective across a range of willingness-to-pay (WTP) thresholds.

RESULTS: Compared with aripiprazole, patients receiving KarXT had higher incremental costs, greater QALYs, greater time in 1L, less time with AEs, lower AE costs, and fewer relapses. Across a WTP range of $200K to $50K per QALY, estimated KarXT annual net price ranged from $36K to $18K, respectively. Sensitivity analyses indicated results were highly sensitive to tardive dyskinesia and relapse rates.

CONCLUSIONS: In this model, KarXT was associated with better clinical outcomes but greater incremental costs compared with aripiprazole. These findings support the potential of KarXT, an investigational therapy with a first-in-class mechanism of action, to be a cost-effective treatment option for schizophrenia, across a range of WTP thresholds.

SPONSORSHIP: Karuna, a Bristol Myers Squibb company

F14 Long-term metabolic change associated with KarXT (xanomeline and trospium) in patients with schizophrenia and preexisting metabolic conditions: Interim results from pooled, long-term safety studies EMERGENT-4 and EMERGENT-5

Claxton A, *Kaul I*, *Henderson C*, *Sidovar M*, *Marcus R*; *aclaxton@karunatx.com*; *matt.sidovar@karunatx.com*
Bristol Myers Squibb

BACKGROUND: Currently approved treatments for schizophrenia are associated with metabolic disturbances, including increased risk of diabetes, and block activity at the dopamine D2 receptor. KarXT, an investigational medication, combines the dual M1/M4 preferring muscarinic receptor agonist xanomeline and the peripherally restricted muscarinic receptor antagonist trospium chloride. The efficacy and safety of KarXT in schizophrenia was demonstrated in the 5-week, randomized, double-blind, placebo-controlled EMERGENT-1, EMERGENT-2, and EMERGENT-3 trials, and long-term safety was evaluated in 52-week open-label trials EMERGENT-4 and EMERGENT-5. In these trials, KarXT was not associated with adverse metabolic side effects.

OBJECTIVE: To examine the impact of KarXT in people with schizophrenia who have preexisting metabolic conditions.

METHODS: EMERGENT-4 and EMERGENT-5 are phase 3, 52-week, outpatient, open-label studies in people with schizophrenia. Participants either rolled over from the short-term, pivotal trials (EMERGENT-4) or had stable symptoms of schizophrenia appropriate for outpatient care (EMERGENT-5). Eligible participants received KarXT (xanomeline/trospium) starting at 50 mg/20 mg twice daily and were titrated up to a maximum of 125 mg/30 mg twice daily. Interim data from EMERGENT-4 and EMERGENT-5 were pooled, and at the time of the datacut, 275 (41%) subjects remained ongoing in the study. Participants discontinued previous antipsychotics prior to receiving KarXT.

RESULTS: At the datacut, 558 patients had received at least 1 dose of KarXT and had a baseline hemoglobin A1c value. Of these, 73 (13.1%) participants had A1c greater than 6.5%

at baseline, consistent with a diabetes diagnosis. Within this subgroup, A1c, weight, and triglycerides slightly improved over the course of treatment; cholesterol levels remained stable. A1c changed from baseline (CFB) −0.06% at week 24, −0.16% at week 52, and −0.13% at last on-treatment assessment. Weight CFB −2.3 kg at week 24, −2.8 kg at week 52, and −3.1 kg at last on-treatment assessment. Total cholesterol CFB +0.09 mmol/L at week 24, +0.31 mmol/L at week 52, but −0.03 mmol/L at last on-treatment assessment. Triglycerides CFB −0.27 mmol/L at week 24, −0.15 mmol/L at week 52, and −0.26 mmol/L at last on-treatment assessment. Concomitant medications for diabetes were rarely changed or added throughout the course of treatment.

CONCLUSIONS: The findings from pooled, long-term, open-label studies of KarXT provide evidence that treatment with KarXT is associated with slight improvement in metabolic parameters in people with preexisting metabolic conditions.

SPONSORSHIP: Karuna, a Bristol Myers Squibb company

F19 A descriptive analysis of comorbidities and health care resource use among patients with treatment-resistant depression with and without use of atypical antipsychotics

Wing V[1], *Lerner J*[1], *Teoh E*[1], *Orsini L*[1], *Gill J*[1], *O'Connor S*[1], *Taylor R*[1], *Young M*[2], *Levine S*[1]; *vicki.kwing@gmail.com*
[1]*Compass Pathways*; [2]*Compass Pathways, PLC*

BACKGROUND: Treatment-resistant depression (TRD) is defined as the failure to respond to 2 or more treatments of adequate dose and duration in an episode of major depressive disorder (MDD). To date, limited research has been conducted to understand the subpopulation of patients with TRD who receive atypical antipsychotics (AAPs).

OBJECTIVE: To explore differences in demographics, clinical characteristics, and health care resource utilization (HCRU) between patients who used AAPs and those who did not use AAPs after failing 2 pharmaceutical treatments.

METHODS: Adult patients with TRD were identified in the Merative MarketScan Commercial Database. Patients were labeled as having had an AAP if they received 1 or more AAP drugs after having failed 2 pharmaceutical treatments (TRD date). All patients who received AAPs prior to the TRD date were excluded. The AAP and no-AAP cohorts were compared during the 6 months prior to first antidepressant use (baseline period), between the first antidepressant date and TRD date (index to TRD period), and on or after TRD date (TRD period). Measures were compared between the AAP and no-AAP cohorts using chi-square, Student's t-tests, quasi-Poisson regressions, or negative binomial regressions, as appropriate.

RESULTS: Among the 8,957 patients with TRD who met selection criteria (mean age: 34 years), 1,771 (20%) patients were included in the AAP cohort. The AAP cohort had a lower proportion of female patients (60.2% vs 65.2%, $P<0.001$) and a higher proportion of patients with psychiatric comorbidities ($P<0.001$) compared with the no-AAP cohort. In all periods, the incidence of suicidal ideation was higher in the AAP cohort compared with the no-AAP cohort (baseline incidence rate ratio [IRR] =1.69, index to TRD IRR = 2.14, TRD IRR = 3.35; all $P<0.001$). Patients in the AAP cohort had a higher incidence of mental health–related visits compared with patients in the no-AAP cohort during the baseline period (IRRs = inpatient [IP] 1.84; outpatient [OP] 1.19; emergency department [ED] 1.62; all $P<0.001$), index to TRD period (IRRs = IP 2.20, OP 1.16, ED 2.03; all $P<0.001$), and TRD period (IRRs = IP 3.08, ED 2.27; all $P<0.001$).

CONCLUSIONS: The increased comorbidity burden and HCRU in patients with TRD receiving AAPs may reflect the greater severity and complexity of their psychiatric conditions, necessitating more intensive treatments. The higher burden of psychiatric comorbidities and risk of suicidal ideation identified at baseline also indicates that AAP users present with more severe psychiatric profiles from the onset, requiring more comprehensive management strategies.

SPONSORSHIP: Compass Pathways

F20 Change in symptoms, response, and remission among patients with treatment-resistant depression initiated on esketamine nasal spray across multisite practice in the United States

Marton T[1], *Joshi K*[2], *Zhdanava M*[3], *Shah A*[4], *Pilon D*[3], *Moore B*[3], *Jiang F*[3], *Guo J*[1]; *tmarton@mindfulhealthsolutions.com; kjoshi33@its.jnj.com*
[1]*Mindful Health Solutions*; [2]*Janssen Scientific Affairs, LLC, a Johnson & Johnson company*; [3]*Analysis Group, Inc.*; [4]*Analysis Group Inc.*

BACKGROUND: Although the efficacy of esketamine nasal spray in adults with treatment-resistant depression (TRD) is established in clinical trials, there are no large studies of the antidepressant's clinical performance in the real world.

OBJECTIVE: To evaluate meaningful change in depressive symptoms, response, and remission based on Patient Health Questionnaire (PHQ)-9 scores among patients who initiated esketamine in a large US multisite psychiatric practice.

METHODS: Adults with TRD treated with esketamine (first session = index date) at Mindful Health Solutions clinics from May 2018 to January 2024 were included. For this analysis, the baseline PHQ-9 score was measured before or on the index date and follow-up PHQ-9 scores were observed after the index

date until the end of patient clinical activity or data. Clinical outcomes included meaningful change in depressive symptoms (≥6-point decrease from baseline score), partial response (score<10), complete response (≥50% decrease from baseline score), and remission (score<5). Response was assessed among patients with baseline PHQ-9 score greater than or equal to 10 (moderate to severe depression), and analysis of meaningful change in symptoms and remission was replicated in this subgroup. Time to clinical outcomes was described with Kaplan-Meier survival analysis; patients without an outcome were censored at the last PHQ-9 score in the data.

RESULTS: Nine hundred eleven patients were identified (mean age: 43.7 years, 56.6% female, mean baseline PHQ-9 score: 16.3), of whom 773 (84.9%) had a baseline PHQ-9 score greater than or equal to 10. During the mean follow-up period of 13 months, patients had a mean of 24.9 esketamine treatment sessions, and 75.2% completed induction (ie, 8 sessions); the median time to complete induction was 30 days (per label: 28 days). At 12 months after the index date, the probability of achieving a meaningful change in depressive symptoms was 79.1%, probabilities of partial and complete response were 73.1% and 69.6%, and the probability of remission was 37.3%. Among patients with baseline PHQ-9 score greater than or equal to 10, the probability of achieving a meaningful change in depressive symptoms was 85.3% and the probability of remission was 33.8%.

CONCLUSIONS: Within a year, initiation of esketamine was associated with a meaningful change in depressive symptoms and achievement of treatment response in most patients with TRD, and achievement of remission in more than one-third of patients. The findings are consistent with the clinical trials data, supporting the real-world effectiveness of esketamine.

SPONSORSHIP: Janssen Scientific Affairs, LLC, a Johnson & Johnson company

F21 Medical, prescription, disability, and absence cost for employees with major depressive disorder: Developing real-world models using shorter pre-index time periods for Charlson Comorbidity Indexes and smaller sample sizes

Brook R[1], *Beren I*[2], *Kleinman N*[1], *Drnach A*[3], *Schramm S*[3], *Ghanjanasak T*[1], *Rosenberg E*[2]; *rich@bh-ww.com*
[1]*Better Health Worldwide, Inc*; [2]*Workpartners*; [3]*WorkPartners, LLC*

BACKGROUND: Models of disease outcomes often control for comorbidities using Charlson Comorbidity Index (CCI) scores and other measures. Predicting future outcomes based on prior period CCI scores are limited by samples with continuous eligibility (CE) for the entire pre-/post-periods.

OBJECTIVE: To assess precision of prescription (Rx), medical, absence, and disability cost models using various CCI pre-index durations to predict annual post-index metrics for employees with major depressive disorder (MDD).

METHODS: Retrospective analysis of US employees in Workpartners' Research and Reference Database (RRDb). Employees with MDD *International Classification of Diseases* codes were identified from medical claims (2010-2022). Employees had at least 1 year of CE after the index date (first claim for MDD cohort, average claim date for non-MDD controls). Ten cohorts with 3 controls were randomly selected per eligible employee. For each cohort, cost models were developed using 2-stage (logistic followed by generalized linear) model stepwise regression. All models controlled for differences in demographics (age, sex, self-reported race, marital status), job-related factors (salary, full-/part-time status, exempt/nonexempt status), US region, and comorbidities (using 6-, 9-, and 12-month pre-index baseline CCIs). Variables selected at least 8 of 10X were used in the final models. Costs inflation adjusted to December 2022 US dollars and mean modeled differences (MMDs) were presented for medical and Rx costs, short-term disability (STD) costs, and absence costs. All estimates were presented for 12-month, 9-month, and 6-month baseline CCIs [bCCIs], respectively. Employee STD and absence days were also modeled and reported.

RESULTS: All cost models included CCI at $P<0.05$ for at least 1 of the 2-part models, regardless of CCI time frame. For every metric tested, all 10 models run had differences (MDD vs controls, $P\leq0.05$), except the 12-month Rx cost bCCIs with 9/10 models ($P<0.05$). Medical costs MMDs were $6,370, $6,395, and $6,470 for 12-month, 9-month, and 6-month bCCIs, respectively. Rx costs were $1,043, $1,140, and $1,055 for 12-month, 9-month, and 6-month bCCIs, respectively. STD costs MMDs were $641, $656, and $621 for 12-month, 9-month, and 6-month bCCIs, respectively. MMD in 12-month post-index absence costs between cohorts were $945, $935, and $949 for 12-month, 9-month, and 6-month bCCIs, respectively. STD day MMDs were 3.80, 3.86, and 3.70 and absence day MMDs were 3.98, 3.97, and 3.91 for 12-month, 9-month, and 6-month bCCIs, respectively.

CONCLUSIONS: A 6-month CCI value is an effective covariate for predicting annual post-index outcomes for direct costs (Rx and medical), and indirect costs and times associated with absences and STD leaves. This strongly suggests the potential for pre-index CE periods less than 1 year, allowing increased sample sizes for populations with significant turnover.

SPONSORSHIP: None

F22 The consequences of formulary restrictions on health care resource utilization and atypical antipsychotic use among patients with Medicaid-prescribed adjunctive cariprazine for major depressive disorder

Nabulsi N[1], *Zanardo E*[2], *MacKnight S*[3], *Ma S*[3], *Laliberté F*[4], *Parikh M*[1]; *nadia.nabulsi@abbvie.com*
[1]*AbbVie*; [2]*Analysis Group, Inc.*; [3]*Groupe d'analyse, Ltée*; [4]*Groupe d'analyse, Ltée*

BACKGROUND: Cariprazine has demonstrated efficacy as an adjunct to antidepressant therapy (ADT) in adults with major depressive disorder (MDD). However, formulary-related restrictions may limit the access of patients with Medicaid insurance to cariprazine.

OBJECTIVE: To evaluate the impact of formulary-related cariprazine claim rejections on health care resource utilization (HCRU) and treatment patterns among Medicaid patients prescribed adjunctive cariprazine for MDD.

METHODS: Symphony Health Integrated Dataverse was used to identify adults with Medicaid, an MDD diagnosis during the 6-month baseline period, at least 1 cariprazine claim (first claim = index date), and at least 1 ADT dispensed in the 90 days pre-/post-index with at least 14 days of overlap with cariprazine during the 90 days post-index (to indicate adjunctive therapy). Patients with an initial cariprazine claim rejected for a formulary-related reason (ie, noncoverage, step therapy, or prior authorization) were required to have at least 1 approved claim for any atypical antipsychotic (AA) in follow-up (to increase comparability between cohorts) and were matched 1:1 to patients with an approved initial cariprazine claim using propensity scores. Post-index HCRU (per patient-year) was compared between approved and rejected cohorts and included all-cause and mental health–related hospitalizations, emergency department (ED) visits, and outpatient visits. Post-index AA use and time to receipt were assessed in the rejected cohort. 95% CIs and *P* values were calculated via nonparametric bootstrap procedures.

RESULTS: Rejected and matched approved cohorts each comprised 471 patients; the mean age was 42 years and 76%-77% were female. Mean follow-up duration was 0.9 years in both cohorts. Patients in the rejected cohort had significantly higher hospitalization rates per patient-year than those in the approved cohort (all-cause rate ratio [95% CI] = 1.49 [1.04-2.18], *P* = 0.032; mental health related = 1.84 [1.27-3.01], *P* = 0.008). Rates of ED and outpatient visits were similar between cohorts. Only 22.7% of patients with an initial rejection eventually received cariprazine. The mean delay to AA treatment (cariprazine or other AA) after initial rejection was 116 days.

CONCLUSIONS: Among Medicaid patients, cariprazine claim rejections for formulary-related reasons were associated with significantly more hospitalizations and an average AA treatment delay of nearly 4 months. These real-world findings suggest that cariprazine formulary restrictions may unintentionally lead to increased HCRU and treatment gaps among Medicaid patients with MDD.

SPONSORSHIP: AbbVie

F23 Creating a managed care framework for mental health in rare diseases using artificial intelligence, literature, and expert review

Richardson T[1], *Matthews N*[2], *Rice M*[3], *Lyon M*[4], *Kobernick M*[5], *Brackbill L*[6]; *nmatthews@amcp.org*
[1]*Impact Education, LLC*; [2]*AMCP*; [3]*Michelle Rice & Associates, LLC*; [4]*Center for Translational Research/Children's Research Institute Children's National Hospital*; [5]*BlueCross BlueShield Michigan*; [6]*Leukodystrophy Newborn Screening Action*

BACKGROUND: The Orphan Drug Act identifies a rare disease as any condition affecting fewer than 200,000 people in the United States. Although treatments for rare diseases can offer significant health improvements over those for more common ailments, more than 90% of rare diseases have no US Food and Drug Administration–approved treatments, leading to a substantial unmet need. This includes prolonged diagnostic processes, scarce treatment options, and considerable psychosocial stress because of the absence of coordinated, comprehensive care. Using artificial intelligence (AI) can significantly enhance the efficiency of developing solutions and resources for health care professionals. AI can analyze vast amounts of data rapidly, identifying patterns that might not be immediately obvious to humans.

OBJECTIVE: To develop a framework focused on the mental health of individuals with rare diseases to support the AMCP Market Insights panel discussion and assist managed care professionals in their efforts using a mix of traditional and AI methods.

METHODS: In the fall of 2023, AMCP Market Insights undertook a targeted literature review identifying key areas where managed care professionals can impact the mental health of persons living with rare diseases. AI prompts in chat.gpt4 were then used to identify supporting actions for each area of focus from the literature review and were synthesized into a conceptual framework. During a panel discussion in 2023, AMCP Market Insights expert panelists reviewed and further refined the framework's components.

RESULTS: The literature review identified 8 critical areas where managed care professionals can influence the

mental health of those with rare diseases. These areas are Identification and Assessment, Care Coordination, Mental Health Services, Patient and Caregiver Support, Behavioral Health Parity, Data and Outcomes Monitoring, Advocacy and Research, and Continuous Improvement. AI added additional tactics for each area and organized it into a framework. A panel of experts reviewed and refined the framework for accuracy.

CONCLUSIONS: The framework, designed to enhance mental health care for individuals with rare diseases, identifies key areas for managed care professional influence. The use of a targeted literature review and AI have streamlined the process of condensing extensive data into a framework for experts to review.

SPONSORSHIP: Takeda, Sanofi

F24 The economic burden of generalized anxiety disorder in the US general adult population: Insights from the patient and payer perspectives

Duong P[1], *Suponcic S*[2], *Maculaitis M*[3], *Karlin D*[1]; *pduong@mindmed.co*
[1]*Mind Medicine, Inc.*; [2]*Value & Access Advisors, LLC*; [3]*Oracle*

BACKGROUND: Current data on the economic burden of generalized anxiety disorder (GAD) are needed to inform health policy decisions.

OBJECTIVE: To investigate the burden of GAD from the perspectives of US patients and payers.

METHODS: Data from the 2022 National Health and Wellness Survey (N = 75,261), an online self-report survey of adults (aged ≥18 years) that is representative of US Census age, sex, and race and ethnicity distributions, were analyzed, including respondents defined as undiagnosed GAD or diagnosed GAD based on GAD-7 scores and GAD diagnosis and treatment. Undiagnosed and diagnosed GAD groups were compared on demographics, health characteristics, health care resource use (HCRU; past 6 months), and work productivity and activity impairment (Work Productivity and Activity Impairment scores; range: 0-100%; higher scores reflect greater impairment). Additionally, a double-blind, web-based survey was fielded in December 2023 to examine GAD perceptions among pharmacy and medical directors (N = 20: n = 7 national plans, n = 5 pharmacy benefit managers, and n = 8 regional payers/Blues Affiliates; 95% representing commercial plans).

RESULTS: Undiagnosed (n = 15,389 vs diagnosed, n = 5,526) adults were, on average, younger (36.6 vs 43.1 years) and were more often male (58% vs 21%) and employed (80% vs 51%). Undiagnosed (vs diagnosed) adults had a greater mean number of hospitalizations (1.2 vs 0.3) and emergency department visits (1.3 vs 0.5) but fewer health care provider visits (3.0 vs 8.7) (all $P < 0.001$). Undiagnosed (vs diagnosed) adults also had activity impairment, absenteeism, presenteeism, and overall work productivity loss on Work Productivity and Activity Impairment that were 1.6, 3.1, 1.9, and 1.9 times higher (all $P < 0.001$). A majority of payers (55%-80%) reported GAD is commonly diagnosed with delay, undiagnosed, or misdiagnosed. Few payers (15%) have implemented new GAD screening guidelines, 30% are considering it, and 50% are without a plan. Less than half (40%) perceived GAD as an economic burden on employers; 20% reported GAD is a direct budgetary concern for their plan.

CONCLUSIONS: Greater HCRU and work productivity loss were observed among undiagnosed, relative to diagnosed, US adults with GAD, suggesting substantial economic impact associated with undiagnosed GAD. Yet payers infrequently recognized GAD as costly for employers or insurance plans. Collectively, these results underscore the need for effectively implementing the US Preventive Services Task Force recommendations to screen adults for GAD to reduce the economic burden of undiagnosed cases.

SPONSORSHIP: Mind Medicine Inc.

F25 Cost-effectiveness of midomafetamine-assisted therapy for chronic post-traumatic stress disorder of moderate and higher severity in the United States: A health economic model

Zah V[1], *Stanicic F*[2], *Grbic D*[2], *DeAngelo D*[3]; *vladzah@outcomesresearch.ca*
[1]*ZRx Outcomes Research Inc.*; [2]*ZRx Outcomes Research Inc.*; [3]*Lykos Therapeutics*

BACKGROUND: Midomafetamine-assisted therapy (MDMA-AT) is under consideration for approval as treatment for adult patients with post-traumatic stress disorder (PTSD).

OBJECTIVE: To explore cost-effectiveness of MDMA-AT compared with placebo with therapy (PT) in US health care settings.

METHODS: A health state–transition model was used to analyze the cost-effectiveness of MDMA-AT for treating adults with chronic PTSD of moderate or higher severity. Both treatment arms consisted of 3 preparatory (90-minute), 3 interventional (8-hour), and 9 integration (90-minute) sessions, lasting ~4 months total. All sessions included psychotherapy, with interventional also including MDMA or placebo. After receiving treatment, patients were distributed across health states of no PTSD (not meeting PTSD diagnostic criteria), nonsevere PTSD (responders), severe PTSD (nonresponders), and death. Each state had unique health care costs and utilities sourced from real-world

data analysis and patient data from MDMA-AT clinical trials (including long-term follow-up). The base-case analysis considered payers' perspective with a 5-year horizon, 3.5% annual cost and effect discounts, and an assumed MDMA medication price of $12,000 per session. Trial-derived utilities and US life tables mortality data were used to calculate quality-adjusted life-years (QALYs). The main outcome was an incremental cost-effectiveness ratio (ICER) with a $150,000 willingness-to-pay (WTP) threshold. Univariate one-way sensitivity analysis was employed by varying inputs in the ±10% range. Probabilistic sensitivity analysis explored the impact of model input uncertainty with 5,000 iterations.

RESULTS: The base-case ICER was $83,845 per QALY. Total direct costs were $64,745 in the MDMA-AT and $33,132 in the PT arms ($31,613 increment). The costs of intervention were $48,376 for MDMA-AT and $12,376 for PT. The highest MDMA medication cost to fit under the WTP threshold was $20,314 per session. Costs related to PTSD health care visits and other PTSD treatments were lower with MDMA-AT than PT (–$2,511 and –$1,877 increments, respectively). Utility benefits were higher in MDMA-AT than PT, with 3.691 and 3.314 QALYs generated over 5 years, respectively (0.377 QALY increment). One-way sensitivity analysis identified utilities and MDMA medication price as inputs with the highest impact on ICER. Probabilistic sensitivity analysis reported an ICER of $84,240 per QALY, with $31,699 (±5,378) mean direct cost and 0.376 (±0.305) QALY increments. MDMA-AT acceptability rate under the WTP threshold was 72.12%.

CONCLUSIONS: These data suggest MDMA-AT may be a cost-effective treatment compared with PT for patients with chronic PTSD of moderate or higher severity.

SPONSORSHIP: Lykos Therapeutics

F26 Medical cost and health care resource utilization offset for zuranolone relative to selective serotonin reuptake inhibitors in the treatment of postpartum depression

Anderson S[1], *Bian B*[2], *Talbird S*[1], *Stratton M*[2], *Maxwell R*[2], *Ferries E*[3]; *saanderson@rti.org*; *erin.ferries@sagerx.com*
[1]*RTI Health Solutions*; [2]*Biogen Inc.*; [3]*Sage Therapeutics, Inc.*

BACKGROUND: The economic burden associated with postpartum depression (PPD) is substantial. Zuranolone was approved as an oral, once-daily, 14-day treatment indicated for PPD in adults by the US Food and Drug Administration (FDA). In the phase 3 SKYLARK placebo-controlled clinical trial of zuranolone (NCT04442503), patients treated with zuranolone demonstrated improvement in depressive symptoms at day 15 (primary endpoint) and as early as day 3. Although selective serotonin reuptake inhibitors (SSRIs) are commonly used in clinical practice for PPD treatment, they are not approved by the FDA for use in PPD and may require 4-8 weeks to demonstrate maximum efficacy.

OBJECTIVE: To estimate direct nonpharmacy medical costs and health care resource utilization (HCRU) associated with zuranolone use relative to SSRIs in the treatment of adults with PPD.

METHODS: A 1-year cost-offset analysis was developed for a cohort of US adults with incident PPD to estimate costs for treatment with zuranolone or a basket of SSRIs, as they are the most commonly prescribed class in PPD. Direct medical costs for adults with PPD were estimated, including costs for screening, diagnosis, treatment-related adverse events, and PPD-related HCRU (inpatient admissions and visits to health care providers, specialists for diagnosis and treatment, and emergency departments). Costs were adjusted to 2023 US dollars and not discounted. Data for model input costs, clinical effectiveness, HCRU, adverse events, and PPD-related mortality were obtained from trial data and other published US sources. Outcomes included total and per-person direct medical costs and HCRU.

RESULTS: For the 2020 US adult birth cohort (3,573,295 live births), an estimated 468,102 patients had incident PPD symptoms (13.1%). Of those, 130,600 (27.9%) were diagnosed with PPD, and of those, 73,789 (56.5%) were pharmacologically treated. Among the modeled treated cohorts (n = 73,789 treated with either zuranolone or SSRIs), zuranolone use was associated with $138.9 million in direct medical costs over 1 year compared with $147.1 million associated with SSRI use (–$8.3 million). Relative to SSRIs, zuranolone treatment was associated with reduced HCRU: –1,524 outpatient visits, –117 inpatient days, –1,145,437 adverse event–related events, and –32 emergency department visits over the 12-month period. Overall, nonpharmacy direct medical costs per treated adult were $1,882 with zuranolone and $1,994 with SSRIs (–$112) per year.

CONCLUSIONS: Treatment with zuranolone may reduce nonpharmacy direct medical costs and HCRU for adults with PPD relative to SSRIs.

SPONSORSHIP: Sage Therapeutics, Inc. (Cambridge, MA); Biogen, Inc. (Cambridge, MA)

F28 Impact of centanafadine, a novel nitrosamine drug substance–related impurity, on quality of life in adolescents with attention-deficit/hyperactivity disorder

Ward C[1], *Jin N*[2], *Skubiak T*[1], *Brunner E*[1], *Schein J*[1], *Oberdhan D*[1], *Childress A*[3]; *caroline.ward@otsuka-us.com*; *Elizabeth.Brunner@Otsuka-us.com*
[1]*Otsuka Pharmaceutical Development & Commercialization, Inc.*; [2]*Otsuka Pharmaceutical Development & Commercialization Inc.*; [3]*Center for Psychiatry and Behavioral Medicine, Inc.*

BACKGROUND: Attention-deficit/hyperactivity disorder (ADHD) is one of the most common pediatric neurodevelopmental disorders, characterized by symptoms of hyperactivity, impulsivity, and inattention—all of which can affect overall quality of life for patients and their families. Extended-release centanafadine (CTN)—a norepinephrine, dopamine, serotonin reuptake inhibitor (NDSRI)—was studied in a phase 3 trial (NCT05257265) for the treatment of ADHD in adolescents aged 13-17 years. Compared with placebo, CTN 328.8 mg demonstrated statistical significance at week 6 for the primary endpoint (change from baseline in ADHD Rating Scale–5) and was nominally significant for the key secondary endpoint (change from baseline in Clinical Global Impression–Severity–ADHD scale).

OBJECTIVE: To assess health-related quality of life (HRQL) in adolescents treated with CTN.

METHODS: Eligible patients with a primary ADHD diagnosis were randomized to CTN 328.8 mg, 164.4 mg, or placebo. The exploratory endpoint, change from baseline at week 6 in the Pediatric Quality of Life 2.0 Family Impact Module (PedsQL-FIM), analyzed via analysis of covariance (including treatment and trial site as fixed effect terms and baseline as covariate), included 3 parts: total score, the Parent HRQL summary score (derived from physical, emotional, social, and cognitive function domains), and the Family Functioning summary score (derived from Daily Activities and Family Relationship domains). The ADHD Treatment Satisfaction Questionnaire (ATSQ) consists of Comparison Rating and Daily Impact. All measures reported here were collected via caregiver proxy.

RESULTS: Overall, 371/459 (80 .8%) randomized patients completed the study (mean age 14.7 years; 59% male; 70% White). At week 6, the mean change from baseline in PedsQL-FIM total score, Family Functioning summary score, and Parent HRQL favored CTN 328.8 mg compared with placebo (mean difference = 6.33 [95% CI = 2.74-9.92], 8.14 [95% CI = 3.41-12.87], vs 5.32 [95% CI = 1.38-9.26], respectively). The ATSQ comparison rating and daily impact of prior ADHD treatments showed a preference for CTN (328.8 mg, mean [SD] score at week 6, 0.4 [0.6]; 164.4 mg, 0.2 [0.7]; placebo, 0.2 [0.6]; and 328.8 mg, 0.8 [0.7]; 164.4 mg, 0.5 [0.6]; placebo, 0.5 [0.6], respectively). Treatment-emergent adverse events were reported in 50.3% of patients treated with CTN 328.8 mg, 31.4% with CTN 164.4 mg, and 23.8% with placebo; most were mild to moderate.

CONCLUSIONS: PedsQL-FIM and ATSQ scores are in line with findings for the primary and key secondary endpoints. CTN, a potential first-in-class NDSRI, led to improved quality of life for families of adolescents with ADHD.

SPONSORSHIP: Otsuka Pharmaceutical Development & Commercialization, Inc.

F29 Impact of centanafadine, a novel norepinephrine, dopamine, and serotonin reuptake inhibitor, on quality of life in children with attention-deficit/hyperactivity disorder

Ward C[1], *Jin N*[2], *Skubiak T*[1], *Brunner E*[1], *Schein J*[1], *Oberdhan D*[1], *Childress A*[3]; *caroline.ward@otsuka-us.com*; *Elizabeth.Brunner@Otsuka-us.com*
[1]*Otsuka Pharmaceutical Development & Commercialization, Inc.*; [2]*Otsuka Pharmaceutical Development & Commercialization Inc.*; [3]*Center for Psychiatry and Behavioral Medicine, Inc.*

BACKGROUND: Attention-deficit/hyperactivity disorder (ADHD) is one of the most common pediatric neurodevelopmental disorders, which can affect overall quality of life for patients and families. Extended-release centanafadine (CTN)—a norepinephrine, dopamine, serotonin reuptake inhibitor (NDSRI)—was studied in a phase 3 trial (NCT05428033) for the treatment of ADHD in children aged 6-12 years. Compared with placebo, high-dose CTN demonstrated statistical significance at week 6 for the primary endpoint (change from baseline in ADHD Rating Scale–5) and both CTN groups were numerically favored for the key secondary endpoint (change from baseline in Clinical Global Impression–Severity–ADHD scale).

OBJECTIVE: To assess health-related quality of life (HRQL) in children treated with CTN.

METHODS: Eligible patients with a primary ADHD diagnosis were randomized to weight-based high- or low-dose CTN or placebo. The exploratory endpoint, change from baseline at week 6 in the Pediatric Quality of Life 2.0 Family Impact Module (PedsQL-FIM), analyzed via analysis of covariance (including treatment and trial site as fixed effect terms and baseline as covariate), included 3 parts: total score, the Parent HRQL summary score (derived from physical, emotional, social, and cognitive function domains), and the Family

Functioning summary score (derived from Daily Activities and Family Relationship domains). The ADHD Treatment Satisfaction Questionnaire (ATSQ) consists of Comparison Rating and Daily Impact. All measures were collected by caregiver proxy.

RESULTS: Overall, 367/480 (76.5%) randomized patients completed the study (mean age 9.2 years; 58% male; 65% White). At week 6, the mean change from baseline in PedsQL-FIM total score and Family Functioning summary score favored CTN high dose compared with placebo (mean difference = 4.31 [95% CI = 0.40–8.22] and 6.01 [95% CI = 1.25–10.77], respectively). A numeric benefit was observed over placebo for PedsQL-FIM total score (low-dose CTN) and HRQL summary score (low- and high-dose CTN). The ATSQ comparison rating and daily impact of prior ADHD treatments to high- or low-dose CTN showed a preference for CTN (high-dose mean [SD] score at week 6, 0.3 [0.6]; low-dose, 0.3 [0.6]; placebo, 0.1 [0.5]; and high-dose, 0.6 [0.7]; low-dose, 0.6 [0.6]; placebo, 0.4 [0.7], respectively). Treatment-emergent adverse events were reported in 38.9% of patients treated with high dose, 35.4% with low dose, and 25.5% with placebo; most were mild to moderate.

CONCLUSIONS: PedsQL-FIM and ATSQ scores are in line with findings for the primary and key secondary endpoints. CTN, a potential first-in-class NDSRI, led to improved quality of life for families of children with ADHD.

SPONSORSHIP: Otsuka Pharma Dev & Commercialization, Inc.

G00-G99 Diseases of the Nervous System

(eg, migraine, multiple sclerosis, restless leg, seizures, sleep apnea)

G1 Budget impact of subcutaneous efgartigimod PH20 for chronic inflammatory demyelinating polyneuropathy from a US payer perspective

Karkare S[1], *Arvin-Berod C*[2], *Mahajerin*, MD A[1], *Gelinas D*[3], *Kulkarni R*[4], *Jaffe J*[4], *Agathangelou G*[4], *Whangbo A*[4]; *skarkare@argenx.com*
[1]*argenx US*; [2]*argenx BVBA*; [3]*argenx*; [4]*ZS Associates*

BACKGROUND: In ADHERE (NCT04281472), the largest clinical trial among patients with chronic inflammatory demyelinating polyneuropathy (CIDP), subcutaneous efgartigimod PH20 (EFG) was well tolerated and demonstrated a significantly reduced risk of relapse among patients with CIDP vs placebo.

OBJECTIVE: To estimate the budget impact of introducing EFG as a treatment alternative for patients with CIDP from a third-party payer perspective in the United States.

METHODS: A budget impact model was developed to estimate the incremental budget impact and additional cost per member per month (PMPM) of introducing EFG into commercial, Medicare, and Medicaid plans over 3 years. The base case modeled a prevalent population of adults with CIDP treated with maintenance intravenous or subcutaneous immunoglobulins (IVIG/SCIG) and the entry of EFG. Inputs on epidemiology, costs, drug adherence, relapse rates, and market share were used to estimate a budget impact for each plan type. Payment mix of average sales price, wholesale drug acquisition costs, and average wholesale price, as well as administration costs by site of care, were included. Drug adherence was based on expert clinical opinion. Clinical relapse pathways were based on published literature for IVIG/SCIG and expert clinical opinion for EFG, which assumed that following relapse, patients would return to IVIG. Relapse rates were sourced from pivotal clinical trials. Other inputs were sourced from publicly available literature and datasets. All costs were reported in 2024 US dollars.

RESULTS: For a hypothetical health plan of 1 million members, the eligible population for EFG was 47 patients. Uptake predictions estimated 1 patient taking EFG in year 1 (year 3: 5 patients). For a commercial plan, the model estimated an annual incremental budget impact of $440,416 (PMPM $0.04) in year 1 and $1,631,082 (PMPM $0.14) in year 3. For a Medicare plan, the model estimated an annual incremental budget impact of $444,044 (PMPM $0.04) in year 1 and $1,644,521 (PMPM $0.14) in year 3. For a Medicaid plan, the model estimated an annual incremental budget impact of $359,645 (PMPM $0.03) in year 1 and $1,331,955 (PMPM $0.11) in year 3.

CONCLUSIONS: Introducing EFG as a treatment alternative for patients with CIDP is expected to have a minimal budget impact in year 1 from the perspective of a third-party US payer while providing a new innovative treatment option to help address unmet needs among patients with CIDP.

SPONSORSHIP: argenx US, Inc.

G2 Real-world case series study of pediatric patients with spinal muscular atrophy treated with onasemnogene abeparvovec (Zolgensma)

Leslie S, Gleason P, Marshall L; *scott.leslie@primetherapeutics.com*
Prime Therapeutics

BACKGROUND: Approved in May 2019, onasemnogene abeparvovec became the only one-time gene therapy for the treatment of spinal muscular atrophy (SMA) in patients

aged younger than 2 years. Reported real-world onasemnogene abeparvovec (SMA gene tx) outcomes data are needed to assess effectiveness and durability.

OBJECTIVE: To describe real-world SMA nongene drug treatment and SMA-related outcomes in a cohort of members administered onasemnogene abeparvovec.

METHODS: This study is a case series, descriptive analysis using integrated pharmacy and medical claims data from Prime Therapeutics' monthly membership averaging 1.5 million Medicaid and 16.5 million commercially insured members. Members with a paid SMA gene tx claim between June 1, 2019, and April 30, 2024, were monitored before (pretreatment) and after SMA gene tx infusion (posttreatment) for nongene disease-modifying treatment (DMT), nusinersen or risdiplam, as well as evidence of SMA gene tx failure defined as death or hospice as indicated by medical claim discharge status, or 2 or more medical claims for dependence on respirator or chronic respiratory failure occurring 30 or more days apart. The end of the posttreatment period was the earliest date of insurance disenrollment or April 30, 2024. Age at SMA gene tx, sex, previous nongene DMT, time in days from SMA gene tx date to posttreatment events and/or non-gene DMT, and members' posttreatment enrollment period length were summarized.

RESULTS: During the 4.9-year assessment period, 52 SMA gene tx members were identified (42 [81%] commercial and 10 [19%] Medicaid), 14 had prior nongene DMT, mean age at treatment was 7.2 months (SD = 8.0), 58% were female, and the median posttreatment follow-up time was 15 months (min-max = 1-56). There were no claims evidence of death or hospice. Seven members had medical claims indicating respirator dependence or chronic respiratory failure occurring 13 days to 42 months after treatment, with 4 of 7 (57%) having DMT prior to SMA gene tx. Two members initiated nongene DMT in the posttreatment period; neither member had SMA gene tx failure.

CONCLUSIONS: This real-world analysis of 52 members treated with onasemnogene abeparvovec and 15-month median follow-up found 1 in 7 members with claims indicating therapy failure and an additional 1 in 26 initiating nongene DMT. These findings can aid in the development of evidence-based coverage decisions, understanding risk for future non-DMT SMA therapy, and pharmaceutical manufacturer value-based contract negotiations.

SPONSORSHIP: Prime Therapeutics, LLC

G3 Clinical outcomes among risdiplam-treated patients with spinal muscular atrophy in the Cure SMA Clinical Data Registry

Shapouri S[1], *To T*[1], *Whitmire S*[2], *Belter L*[2], *Schroth M*[2], *Dickendesher T*[1], *Schuldt R*[1]; *shapouri.sheila@gene.com*
[1]*Genentech, Inc.*; [2]*Cure SMA*

BACKGROUND: The Cure SMA Clinical Data Registry (CDR) was launched in October 2018 and consists of data sourced from both electronic medical records and clinician-entered electronic case report forms on more than 1,100 patients with spinal muscular atrophy (SMA) from across 24 diverse care sites in the United States. The CDR includes data from patients treated with risdiplam (EVRYSDI), a survival of motor neuron 2 (SMN2) pre-mRNA splicing modifier approved by the US Food and Drug Administration (FDA) for the treatment of pediatric and adult individuals with SMA.

OBJECTIVE: To describe demographics, clinical characteristics, and motor function outcomes of risdiplam-treated individuals in the Cure SMA CDR.

METHODS: This analysis included patients with data captured in the CDR on or before June 2023 who had a completed electronic case report form, were treated with risdiplam monotherapy (ie, evidence of risdiplam treatment without any additional FDA-approved or investigational SMA treatment), and had both a pre- and posttreatment functional assessments. Data such as age at diagnosis, age at risdiplam start, sex, race and ethnicity, SMN2 copy number, and census region were described. Maximum functional status was assessed. Mean change in motor function score, as measured by the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND), Hammersmith Functional Motor Scale – Expanded (HFMSE), and Revised Upper Limb Module (RULM), was evaluated at or before risdiplam initiation (baseline) and after risdiplam initiation. All analyses were completed using descriptive statistics.

RESULTS: Fourteen patients met inclusion criteria. The mean (SD) age at SMA diagnosis was 2.3 (2.9) years, and the mean (SD) age at first risdiplam initiation was 14.9 (13.3) years. Four patients (28.6%) were aged older than 18 years. Three patients (21.4%) had 2 SMN2 copies, 8 (57.1%) had 3 copies, and 3 (21.4%) had 4 copies. Four patients (28.6%) achieved a maximum motor milestone of walking alone. Mean (SD) time from treatment initiation to post-risdiplam assessment using the CHOP INTEND (n = 7), HFMSE (n = 4), and RULM (n = 4) was 1.4 (0.5), 0.6 (0.2), and 1.2 (0.6) years, respectively. The mean (SD) change in score from baseline was 3 (5), −1 (1), and 0 (2) for the CHOP INTEND, HFMSE, and RULM, respectively.

CONCLUSIONS: Overall, data from this analysis suggest that patients improved or maintained function across multiple outcomes after receiving risdiplam treatment.

SPONSORSHIP: Genentech, Inc.

G4 Health care resource utilization of oral edaravone–treated patients with amyotrophic lateral sclerosis enrolled in an administrative claims database

Ciepielewska M[1], *Zhang J*[2], *Liu Y*[2], *Da Silva P*[1];
malgorzata_ciepielewska@mt-pharma-us.com
[1]*Mitsubishi Tanabe Pharma America, Inc.*;
[2]*Princeton Pharmatech, LLC*

BACKGROUND: Intravenous (IV) edaravone (Radicava) was approved by the US Food and Drug Administration (FDA) in 2017 for the treatment of amyotrophic lateral sclerosis (ALS) and was shown in clinical trials to slow the rate of physical functional decline. Oral edaravone (Radicava ORS) was FDA approved for use in patients with ALS in May 2022.

OBJECTIVE: To describe demographics, characteristics, and preliminary data on health care resource utilization (HCRU) of oral edaravone–treated patients with ALS in this real-world, observational, US-based administrative claims analysis.

METHODS: Patients with ALS who were continuously enrolled in Optum's deidentified Clinformatics Data Mart from June 15, 2022, through September 30, 2023, were included and divided into 2 groups: Group 1 initially received IV edaravone and switched to oral edaravone, and Group 2 received oral edaravone and was previously edaravone naive. The index date was the first dosing date of oral edaravone. HCRU was evaluated by group and by Medicare vs commercial insurance coverage.

RESULTS: Oral edaravone–treated patients with ALS (n = 436) comprised 71 patients in Group 1 and 365 patients in Group 2. Groups 1 and 2 were predominantly male (56.3% and 53.7%) and White (74.6% and 75.1%), with a mean ± SD age of 60.9 ± 11.7 and 65.6 ± 9.8 years, respectively. The mean ± SD treatment duration was 29.0 ± 17.0 months for Group 1 and 5.0 ± 4.3 months for Group 2. The percentage of patients in Groups 1 and 2, respectively, who reached the following pre-index progression milestones are listed: use of canes/walkers/wheelchairs (38.0% and 17.3%), artificial nutrition (32.4% and 16.4%), noninvasive ventilation (38.0% and 19.7%), invasive ventilation (2.8% and 1.4%), hospitalization (36.6% and 26.0%), and gastrostomy tube placement (21.1% and 11.2%). A higher percentage of patients were covered by Medicare in Group 1 (67.6%) and Group 2 (71.0%) than commercial insurance. For patients covered by Medicare or commercial insurance, respectively, the overall mean ± SD was 5.6 ± 20.8 and 2.5 ± 9.0 pre-index inpatient admissions, 36.9 ± 60.6 and 38.2 ± 62.5 pre-index outpatient visits, 4.5 ± 14.4 and 3.8 ± 10.8 post-index inpatient admissions, and 11.7 ± 21.0 and 15.1 ± 33.5 post-index outpatient visits.

CONCLUSIONS: Additional results are expected for these preliminary real-world data that may help clinicians and payers better understand the demographics, clinical characteristics, and HCRU of oral edaravone–treated patients with ALS.

SPONSORSHIP: Sponsored by Mitsubishi Tanabe Pharma America, Inc.

G5 Comparison of health care resource utilization and costs in patients with spinal muscular atrophy treated with risdiplam or nusinersen

Shapouri S[1], *Lebovitch D*[2], *Liu Y*[2], *Dickendesher T*[1], *To T*[1];
shapouri.sheila@gene.com
[1]*Genentech, Inc.*; [2]*Genesis Research*

BACKGROUND: Two disease-modifying therapies (DMTs) are currently US Food and Drug Administration (FDA) approved for the treatment of both pediatric and adult patients with spinal muscular atrophy (SMA). Nusinersen (SPINRAZA) is an intrathecally administered, antisense oligonucleotide, administered every 4 months after an initial series of loading doses. Risdiplam (EVRYSDI) is a once-daily, orally administered pre-mRNA splicing modifier. Prior claims analyses found substantial health care resource utilization (HCRU) and economic burden for patients with SMA before DMT approval; however, limited data exist for patients with SMA treated with DMTs.

OBJECTIVE: To compare real-world HCRU and total costs among US patients with SMA treated with risdiplam or nusinersen monotherapy.

METHODS: This retrospective cohort study used administrative claims data from IQVIA PharMetrics Plus, a closed database of adjudicated US medical and pharmacy claims. Newly treated individuals who initiated risdiplam or nusinersen from January 2018 through March 2023, received no other SMA DMT during continuous follow-up (ie, monotherapy), and had at least 1 inpatient claim or at least 2 outpatient claims for an SMA diagnosis prior to the index date (date of first SMA DMT claim) were included. All individuals had at least 12 months of pre-index and at least 1 month of post-index continuous enrollment in medical and pharmacy benefits; the pre-index requirement for individuals aged younger than 1 year was at least 1 month. All-cause HCRU and annualized costs during the follow-up period were compared between treatment groups after propensity

score matching on clinical and demographic characteristics. Costs were adjusted to 2023 USD.

RESULTS: We identified 17 matched pairs of individuals treated with risdiplam monotherapy vs nusinersen monotherapy. Mean (SD) annual total health care costs were more than 3 times higher for individuals treated with nusinersen than those treated with risdiplam ($1,490,932 [$3,328,919] vs $429,811 [$214,686]) and were primarily driven by outpatient visit costs ($1,483,906 [$3,331,211] vs $20,722 [$18,974]). Annual SMA treatment costs were higher for nusinersen-treated individuals than for those treated with risdiplam ($1,463,397 [$3,327,435] vs $350,256 [$46,111]. SMA treatment costs accounted for 98% of all costs for nusinersen-treated patients vs 81% of all costs for risdiplam-treated patients.

CONCLUSIONS: Patients with SMA treated with nusinersen had comparable HCRU but at higher costs than the risdiplam-treated individuals.

SPONSORSHIP: Genentech, Inc.

G8 Real-world impact of deutetrabenazine use on psychiatric stability and resource use in patients with tardive dyskinesia in the United States

Mychaskiw M[1], *Dotiwala Z*[2], *Finkbeiner S*[3], *Chaijale N*[4], *Thompson S*[1], *Casciano J*[2]; *Marko.Mychaskiw@tevapharm.com*; *Stephen.Thompson01@tevapharm.com*
[1]*Teva Branded Pharmaceutical Products R&D, Inc., Global Health Economics and Outcomes Research*; [2]*eMAX Health*; [3]*Teva Branded Pharmaceutical Products R&D, Inc., North America Medical Affairs*; [4]*Teva Branded Pharmaceutical Products R&D, Inc., Global Medical Affairs*

BACKGROUND: Tardive dyskinesia (TD) is a debilitating movement disorder often resulting from antipsychotic (AP) medication use. Deutetrabenazine (DTBZ) is a vesicular monoamine transporter type 2 inhibitor (VMAT2i) approved to treat TD.

OBJECTIVE: To assess the impact of DTBZ on psychiatric stability in patients with TD, as indicated by schizophrenia (SCZ)-related hospitalizations, emergency department (ED) visits for psychotic events, and missed outpatient (OP) visits.

METHODS: US-based claims data from Symphony Health Solutions Integrated Dataverse was used to identify US patients aged 18 years and older with newly diagnosed TD (July 2019 to June 2022). Patients were required to have at least 1 medical/pharmacy claim at least 6 months prior to and after their TD diagnosis, at least 1 AP prescription claim during the study period, and no VMAT2i claims prior to TD diagnosis. Patients with DTBZ claims were matched 1:2 with patients with no VMAT2i claims (non-VMAT2i). All patients had a minimum follow-up period of 12 months. The DTBZ group was limited to patients on the same dose for at least 60 days of the first 3 months of the follow-up (assessment) period. A composite psychiatric stability (CPS) score (primary outcome) was derived from individual factor scores for number of SCZ-related hospitalizations, number of related ED visits, and greater than 50% decrease in OP visits. A higher CPS score indicated worse outcomes. Health care resource utilization during follow-up period and associated costs were compared.

RESULTS: Analyses included 172 DTBZ patients and 731 non-VMAT2i patients. No statistical differences in baseline characteristics were observed; the average age was 57 years (SD = 12.8 and 14.0, respectively). Proportions of patients with hospitalizations, ED visits, and OP visits at baseline (pre-index period) were not significantly different ($P > 0.05$). Mean CPS scores were similar for DTBZ and non-VMAT2i groups at baseline (0.40 vs 0.39; $P = 0.891$) and during the assessment period (0.66 vs 0.66; $P = 0.981$). During follow-up, DTBZ patients had statistically significantly lower mean CPS scores vs non-VMAT2i patients (0.52 vs 0.78; $P = 0.007$). The DTBZ group had numerically lower average numbers of SCZ-related hospitalizations (0.01 vs 0.03), psychiatric-related ED visits (0.08 vs 0.15), and OP visits (3.46 vs 3.48) during follow-up, although differences were not statistically significant.

CONCLUSIONS: Patients with TD not receiving VMAT2is vs patients with TD on stable doses of DTBZ had a lower occurrence of poor mental health outcomes (as indicated by lower CPS score) that may indicate psychiatric stability (eg, SCZ-related hospitalizations and ED visits for psychotic events).

SPONSORSHIP: Teva Branded Pharmaceutical Products R&D, Inc.

G9 Impact of valbenazine on health-related quality of life in patients with tardive dyskinesia: Results from a phase 4, double-blind, placebo-controlled, randomized withdrawal study

Serbin M[1], *Nedzesky J*[2], *Bron M*[1], *Jimenez R*[3], *Matson E*[2]; *mserbin@neurocrine.com*
[1]*Neurocrine Biosciences*; [2]*Neurocrine Biosciences Inc.*; [3]*Neurocrine Biosciences, Inc*

BACKGROUND: Tardive dyskinesia (TD) is a movement disorder associated with prolonged exposure to dopamine receptor–blocking agents, most commonly antipsychotics, that can significantly impact health-related quality of life (HRQOL). Valbenazine (VBZ) is a selective, potent vesicular monoamine transporter 2 (VMAT2) inhibitor approved for treatment of TD in adults. A phase 4 study (NCT03891862) was conducted to assess persistence of VBZ effect in patients with TD.

OBJECTIVE: To report post hoc analyses of effects of VBZ on HRQOL and self-reported disability from patients receiving VBZ for the entire phase 4 study.

METHODS: Details of the phase 4 study have been published previously. In brief, patients received VBZ 40 mg or 80 mg for an 8-week open-label period and then were randomized to VBZ 40 mg or 80 mg or placebo for an 8-week double-blind period followed by a 4-week drug-free washout. Descriptive analyses were conducted for participants receiving VBZ who had available assessments from baseline (BL) to week 16 (end of treatment) with EuroQoL's 5-Dimension 5-Level questionnaire (EQ-5D-5L) and the Sheehan Disability Score (SDS). EQ-5D-5L included a utility index score from 0 (health state equivalent to death) to 1 (perfect health) and health state visual analog scale (VAS) score from 0 (worst health state imaginable) to 100 (best health state imaginable). SDS measured disruption in 3 domains (work/school, social life, family/home life); total score ranged from 0 (no disruption/impairment) to 30 (extreme disruption/impairment).

RESULTS: At BL, mean (SEM) EQ-5D-5L utility index, VAS, and SDS total scores were 0.64 (0.04; n = 59), 72.9 (2.6; n = 59), and 13.6 (1.6; n = 27). At randomization BL (week 8), the mean change from study BL was 0.07 (0.03; n = 59), 4.8 (3.0; n = 59), and −4.8 (1.8; n = 20), respectively, indicating improvement in all 3 scales. At the end of treatment, EQ-5D-5L utility (n = 56), VAS (n = 56), and SDS total scores (n = 17) showed treatment with VBZ improved HRQOL utility by 0.17 (0.04), 6.4 (3.0), and −9.1 (1.5) over 16 weeks.

CONCLUSIONS: Treatment with VBZ for TD greatly improved patients' HRQOL, as shown in EQ-5D-5L utility index scores, EQ-5D-5L health state VAS scores, and SDS scores. HRQOL improvement exceeded the estimated deficit of TD in available literature. The magnitude of HRQOL improvement observed in this study was similar to the total deficit of many debilitating diseases, displaying the substantial positive impact of VBZ treatment of patients with TD.

SPONSORSHIP: Neurocrine Biosciences, Inc.

G10 Results from a vesicular monoamine transporter 2 inhibitors chart extraction and clinician survey: A subgroup analysis of the impact on patients with tardive dyskinesia treated with valbenazine

Bera R[1], *Nedzesky J*[2], *Bron M*[3], *Luo M*[4], *Vanderhoef D*[5]; *rbera@hs.uci.edu*; *jnedzesky@neurocrine.com*; *MLuo@neurocrine.com*
[1]*University of California, Irvine*; [2]*Neurocrine Biosciences Inc.*; [3]*Neurocrine Biosciences*; [4]*Neurocrine Biosciences, Inc.*; [5]*Neurocrine Biosciences, Inc*

BACKGROUND: Tardive dyskinesia (TD) is a movement disorder that can significantly impact quality of life (QoL), including physical functioning, social activities, and mental well-being. A recent web-based survey was conducted among US physicians, nurse practitioners, and physician assistants characterizing TD burden and symptom improvement following vesicular monoamine transporter 2 (VMAT2) inhibitor treatment. Clinicians reported improvement in TD and associated symptoms was often accompanied by improvements in social, physical, and mental well-being. However, the impact of specific VMAT2 inhibitors was not reported.

OBJECTIVE: To assess the impact of treatment with VBZ for patients with TD on clinician-reported improvement in TD, social and physical well-being, and psychiatric condition.

METHODS: Details of the chart extraction and clinical survey have been published previously. This analysis reported on a subgroup of patients with TD who received VBZ.

RESULTS: Respondents included 145 clinicians providing care for 413 patients taking VBZ. The mean (SD) patient age was 51 (14) years and 51.3% were female. All patients had at least 1 psychiatric condition; the most common primary psychiatric conditions were schizophrenia (31.2%), bipolar disorder (26.6%), schizoaffective disorder (19.1%), and major depressive disorder (9.2%). Most patients had Medicare (28.1%) or Medicaid (30.5%) or were dual eligible (15.7%); 49.4% had TD in at least 2 body regions, most commonly the head/face and upper extremities, and 61.3% had TD present for 1-5 years. Clinicians indicated nearly all patients were impacted by TD in social/emotional (n = 383, 92.7%) and/or physical function (n = 362, 87.7%) domains. Following VBZ initiation, almost all (n = 378/413, 91.5%) patients experienced TD improvement (clinician measured as none, somewhat improved, or significantly improved). Among patients impacted by TD, 88.0% (n = 337/383) showed improvement in at least 1 social/emotional component and 88.1% (n = 319/362) showed improvement in at least 1 physical function component. The most commonly improved components were mobility,

socializing, and eating. For those with available information on psychiatric status improvements, most (n = 271/382, 70.9%) showed improvement in their psychiatric condition (clinician measured as none, somewhat improved, or significantly improved).

CONCLUSIONS: Overall, patients impacted by TD and treated with VBZ experienced improvement in social or physical well-being or psychiatric condition. These results align with our prior analysis, which reported improvements in functional or QoL domains beyond movement symptoms following treatment with a VMAT2 inhibitor.

SPONSORSHIP: Neurocrine Biosciences, Inc.

G11 Treatment patterns of patients with essential tremor newly initiating treatment: A retrospective claims database analysis in the United States

Williams J[1], *Pahwa R*[2], *Barbato L*[3], *Lin J*[3], *Tefos F*[3], *Sillah A*[3], *Thomas S*[3], *Geng F*[4], *Pan Y*[4], *Friderici J*[4], *Shah S*[3]; *drjyw@jywneuro.com*; *Junji.Lin@jazzpharma.com*
[1]*Movement Disorders Neurology*; [2]*University of Kansas Medical Center*; [3]*Jazz Pharmaceuticals*; [4]*KMK Consulting Inc.*

BACKGROUND: Essential tremor (ET) is a prevalent, chronic movement disorder estimated to affect more than 1 million people in the United States. ET is often under-/untreated with limited treatment options; propranolol is the only medication approved in the United States to treat ET. Per ET treatment guidelines, currently available medications can have efficacy and/or safety limitations, and medications used off label should be prescribed with caution.

OBJECTIVE: To understand real-world treatment patterns of patients with ET initiating pharmacological treatment(s) in the United States.

METHODS: Merative MarketScan administrative claims were analyzed from January 1, 2016, to June 30, 2022. Eligible patients were aged 18 years or older, had at least 2 diagnoses of ET at least 1 and less than 365 day(s) apart, entered the cohort on first date of ET diagnosis (index), had continuous medical and pharmacy coverage at least 365 days prior (baseline) and at least 364 days after index (follow-up), had no ET or other tremor disorder diagnosis claims in the baseline period, and had no Parkinson disease and/or use of drugs that might induce tremor after index. Descriptive analyses were performed for treatment patterns and derived lines of therapy.

RESULTS: Among the 7,876 treated patients included, the median (IQR) treatment evaluation period lasted 793 (537-1,190) days, during which 44% of patients received 1, 14% received 2, and 41% received at least 3 lines of therapy. Of the 7,830 patients prescribed first-line (1L) therapy (46 patients received therapy <14 days and were excluded), propranolol was most frequently used (34%), with barbiturates (15%) and antiepileptics (12%) being the most frequently used off-label medications. Median (IQR) time from diagnosis to 1L initiation was 7 (0-35) days. Approximately 14% of patients taking 1L began with combination therapy, which included propranolol with another therapy (5%) and barbiturates with another therapy (4%). Of patients receiving 1L, more than 55% proceeded to 2L, with a median (IQR) 1L duration of 206 (42-592) days. Of patients who initiated 2L, 61% used combination therapy such as propranolol (21%) or barbiturates (14%) with another treatment. Of patients receiving 2L, more than 70% proceeded to 3L, with a median (IQR) 2L duration of 81 (30-251) days.

CONCLUSIONS: This study found that more than 50% of patients with ET receiving treatment may require more than 1 line of therapy and may progress to additional lines of therapy, with a progressively shorter median time spent on each line after 1L. These results emphasize the challenges with currently available medication options in ET.

SPONSORSHIP: Jazz Pharmaceuticals

G13 Current state of timely and accurate diagnosis of mild cognitive impairment and dementia: A systematic literature review

Spargo A[1], *Vasanthaprasad V*[2], *Bashir A*[2], *Fanara D*[3], *Hendrix J*[1]; *spargo_andrew@lilly.com*
[1]*Eli Lilly and Company*; [2]*Eli Lilly Services India Private Limited*; [3]*Eli Lilly*

BACKGROUND: Globally, 126 million people are living with mild cognitive impairment (MCI) and 55 million with dementia; Alzheimer disease (AD) is their most common cause. In AD, pathological changes occur decades before onset of symptoms, which then progress over ~10 years from MCI through severe dementia. Timely and accurate diagnosis of AD help patients and caregivers access support services, consider treatments, and plan for the future.

OBJECTIVE: To identify and characterize global literature reporting on timely diagnosis of MCI or dementia and to understand what factors put patients at risk of delayed diagnosis of MCI.

METHODS: A systematic search of the Embase and Medline databases and select conference proceedings was performed for studies published in English (2015-2023) that evaluated the percentage of patients with timely diagnosis or measured time from symptom onset to diagnosis as outcomes in adult patients with AD, MCI, or dementia. Studies

of patients with Down syndrome were also included owing to the high prevalence of AD in this population.

RESULTS: The search identified 2,769 records, of which 18 records, representing 17 unique studies, met inclusion criteria. Most studies were prospective (n = 8) or retrospective (n = 4) cohort studies, published from 2021 to 2022 (n = 9), and were limited to patients with dementia (n = 8). Included studies were heterogeneous in definitions and measurement of timely diagnosis: 5 evaluated the percentage of patients with timely diagnosis (or, conversely, delayed diagnosis), 11 time from symptom onset to diagnosis, and 6 factors associated with longer time from symptom onset to diagnosis. Timely diagnosis was defined as the presence of a diagnosis within a period of time after screening positive for symptoms of MCI or dementia in 2 studies, whereas other studies considered caregivers' perspectives (n = 1), the presence of MCI diagnosis before dementia diagnosis (n = 1), or measures of cognition and function at diagnosis (n = 1). Timely diagnosis was less frequent when measured in patients with MCI (vs dementia). Older age, racial and ethnic minority status, residential (vs home) care, lower educational attainment, living alone, family history of dementia, and certain medical comorbidities were associated with a longer time from symptom onset to diagnosis.

CONCLUSIONS: To ensure people with Alzheimer disease have a chance to access current and future interventions, coordinated efforts are imperative to improve the timeliness and accuracy of MCI and dementia diagnosis across the globe.

SPONSORSHIP: Eli Lilly and Company

G14 Impact of maximum out-of-pocket spending on affordability of high-cost treatments for Alzheimer disease

Hames A[1], *Ibrahim* K[1], *Rosen* J[2], *Lee* K[2], *Poonawalla* I[1]; *ahames1@humana.com*
[1]*Humana Healthcare Research;* [2]*Humana*

BACKGROUND: Patients with Alzheimer disease (AD) face cost-related barriers to initiating high-cost treatments such as lecanemab, with estimated annual out-of-pocket costs of more than $6,000 under Medicare Part B. New subcutaneous formulations expected to come to market likely qualify for Medicare Part D coverage, which may alleviate patient cost burden under the new Part D standard benefit design. Prior to 2024, Part D plans were not required to cap beneficiary maximum out-of-pocket spending (MOOP). Beginning in 2025, Part D plans will be required to cap MOOP at $2,000. Patients with AD who reach this $2,000 out-of-pocket threshold may be more likely to initiate high-cost pipeline treatments.

OBJECTIVE: To understand the expected impact of the $2,000 Part D MOOP on patients with AD, we described the month/quarter in which patients reached $2,000 in Part D out-of-pocket spend in 2023.

METHODS: We performed a cross-sectional analysis of patients with prevalent AD enrolled in a Medicare Advantage Prescription Drug plan in 2023. Part D pharmacy claims were used to estimate the proportion of patients who reached $2,000 in out-of-pocket spend. Out-of-pocket costs were measured using true out-of-pocket spend as defined for 2025 (excluding drug manufacturer discounts). Month/quarter was identified using the service date of the first claim in which cumulative true out-of-pocket spend of $2,000 was reached.

RESULTS: Among patients with AD (n = 114,396), 11.7% (n = 13,404) reached $2,000 in Part D out-of-pocket spend by year end (1.8% in Q1, 3.0% in Q2, 3.7% in Q3, and 3.2% in Q4). Among those who reached $2,000 in out-of-pocket spend, half reached $2,000 between May and September of the calendar year and 27% did not reach $2,000 until Q4. Spending was primarily on drugs for comorbid conditions (<15% of $2,000 spend was on AD drugs). Patients who reached $2,000 in out-of-pocket spend had greater comorbidity burden (RX-Risk-V score: 9.3 vs 6.2) than patients who did not reach $2,000. Comorbidity scores were highest among patients who met the $2,000 threshold in Q1.

CONCLUSIONS: Most patients with AD are not expected to reach the new Part D MOOP of $2,000, and patients who do reach MOOP are expected do so mid- to late year. Thus, affordability will continue to be a barrier to uptake of pipeline AD therapies that may be covered under Medicare Part D. These findings help inform the expected impact of MOOP policy changes for health plans and a better understanding of patient burden.

SPONSORSHIP: None

G24 Real-world delayed infusion patterns in patients with multiple sclerosis treated with ocrelizumab and natalizumab

Tai M[1], *Gadkari* A[1], *Brown* B[1], *Ionescu-Ittu* R[2], *Taiji* R[2], *Vekeman* F[2]; *mindy.tai@novartis.com*; *abhijit.gadkari@novartis.com*
[1]*Novartis Pharmaceuticals Corporation;* [2]*STATLOG Inc*

BACKGROUND: Infusible disease-modifying therapies (DMTs), such as ocrelizumab (OCR) and natalizumab (NTZ), are commonly used for managing multiple sclerosis (MS). The real-world (RW) adherence to labeled infusion schedules for these therapies, which may impact MS outcomes, is not well described.

OBJECTIVE: To investigate infusion delays among patients with MS treated with infusible DMTs in RW settings.

METHODS: A retrospective cohort study was conducted using Optum Clinformatics claims data (March 2016 to December 2023; study period). The study sample included adults diagnosed with MS with a first OCR or NTZ infusion (index date) on or after March 2017 and who had continuous enrollment (CE) 12 months prior to (baseline period) and at least 6 months after the first infusion. Included patients were also required to have at least 3 infusions of the DMT initiated on the index date prior to DMT switch, end of CE, or end of the study period (follow-up period). The follow-up period ended once a patient switched to a different DMT. Alemtuzumab, ublituximab, and mitoxantrone were not assessed owing to insufficient sample size. Delayed infusion was defined as dose interval at least 8 days longer than the labeled schedule (OCR, 182 days; NTZ, 28 days). The proportion of patients with, and average number and length of, delayed infusions was described from the second infusion onward (ie, after the split-loading dose in OCR) by index DMT.

RESULTS: In 4,902 included patients (mean [range] age, 49 [18-85] years; female, 70%; commercially insured, 63%), 4,033 and 869 patients were treated with OCR and NTZ, with 6 (3) and 22 (17) as the mean (SD) number of infusions in the follow-up period, respectively. Among OCR-treated patients, 55% had at least 1 delayed infusion. Proportions of OCR patients categorized by the longest delay between 2 consecutive infusions were as follows: (1) 8-30 days, 24%; (2) 1-2 months, 10%; (3) 2-6 months, 11%; and (4) greater than or equal to 6 months, 10%. Mean (SD) length of delay in OCR infusions was 72 (125) days. Among NTZ-treated patients, 70% had at least 1 delayed infusion. Proportions of NTZ patients categorized by the longest delay between 2 consecutive infusions were as follows: (1) 8-30 days, 39%; (2) 1-2 months, 15%; (3) 2-6 months, 10%; and (4) greater than or equal to 6 months, 5%. Mean (SD) length of delay in NTZ infusions was 27 (52) days.

CONCLUSIONS: In a RW sample of patients with MS treated with infusible DMTs, the majority experienced a delayed infusion of at least 8 days compared with the label, with 20% experiencing a delay of at least 2 months. These findings indicate that infusion delays compared with the label are common in clinical practice. This may potentially impact MS outcomes.

SPONSORSHIP: Novartis Pharmaceuticals Corporation

G25 Patient-reported outcomes following treatment with cladribine tablets: A cross-sectional survey of patients with multiple sclerosis in the United States enrolled in the MS LifeLines patient support program

M. Kozma C[1], *Evans E*[2], *Phillips A*[2], *Chen X*[2]; *xiaoxue.chen@emdserono.com*
[1]*CK Consulting Associates, LLC.*; [2]*EMD Serono*

BACKGROUND: Cladribine tablets (CladT) are approved for treatment of relapsing forms of multiple sclerosis (MS) in adults, with a maximum of 20 days of oral treatment over 2 years. As real-world evidence for CladT in patients with MS in the United States continues to emerge, there is a need to further understand the impact of CladT on patients' quality of life and perspectives.

OBJECTIVE: To evaluate patient-reported outcomes (PROs) among participants in the MS LifeLines patient survey taking CladT, stratified by treatment year.

METHODS: This cross-sectional study invited enrollees from the US MS LifeLines patient support program to participate in an internet-based survey from July 2022 to August 2022. Eligibility criteria included a self-reported physician diagnosis of relapsing MS, treatment with CladT, and age 18 years and older. Patients were categorized into 4 cohorts based on their time since the initiation of CladT treatment (Year 1 to Year 4). Treatment switch to other disease-modifying therapies (DMTs) were reported. Evaluated PROs included quality of life (QoL; Short Form [SF]-12 mental and physical component scores and SF-6 health utilities domain scores), fatigue (Modified Fatigue Impact Scale - 5 Item), depression (Patient Health Questionnaire - 9 Item), cognitive function (PROMIS Cognitive Function Short Form 4a), and pain interference (PROMIS Pain Interference Short Form 6b) were assessed by one-way analysis of variance.

RESULTS: A total of 602 participants were included in the analysis (mean [SD] age 47.8 [11.9] years; 81.6% female; 76.9% White, 8.1% Black/African American, 7.0% Hispanic), with 197 in Year 1, 219 in Year 2, 157 in Year 3, and 29 in Year 4. In the survey, 1.5% of participants in the Year 1 cohort switched to another DMT, followed by 2.7% in the Year 2 cohort, 8.9% in the Year 3 cohort, and 17.2% in the Year 4 cohort. Mean QoL scores were similar across treatment year cohorts (mental component ranged from 44.92 to 47.08, $P = 0.78$; physical component ranged from 42.06 to 44.11, $P = 0.37$; health utility ranged from 0.639 to 0.671, $P = 0.70$). Similar scores for fatigue (9.61-10.45, $P = 0.48$), depression (7.68-8.69, $P = 0.84$), cognitive function (46.32-47.22, $P = 0.86$), and pain interference (53.98-55.86, $P = 0.42$) were observed across cohorts.

CONCLUSIONS: Average PRO scores, including pain, fatigue, depression, and cognitive function, were similar among groups of patients during the active dosing period of CladT and the subsequent 2 follow-up years. These findings are noteworthy considering that CladT are administered only in years 1 and 2.

SPONSORSHIP: EMD Serono (CrossRef Funder ID: 10.13039/100004755)

G26 Treatment patterns, health care resource utilization, and direct costs associated with multiple sclerosis therapies in US retrospective claims

Scharf L, Hoyt M, Gandhy C; lucas.scharfdacosta@lilly.com; hoyt_margaret_l@lilly.com
Eli Lilly and Co.

BACKGROUND: Multiple sclerosis (MS) is a neurodegenerative autoimmune disease of the central nervous system, characterized by inflammatory demyelination in the brain and spinal cord leading to physical disability. To date, MS has no cure even though there are several approved disease-modifying therapies (DMTs) available.

OBJECTIVE: To visualize DMT treatment patterns, health care resource utilization (HCRU), and associated costs in a population with MS.

METHODS: This claims-based retrospective observational study used Optum's deidentified Clinformatics Data Mart Database, which is derived from a database of administrative health claims for members of large commercial and Medicare Advantage health plans. Adult patients with at least 2 MS diagnoses from January 1, 2017, to December 31, 2020, who newly initiated a DMT after the initial MS diagnosis were selected for the study. The first DMT administration served as the index event, and a follow-up of 24-month post-index was used to measure treatment patterns, HCRU, and costs.

RESULTS: A cohort of 3,720 patients were included in this analysis. In the 2 years after DMT initiation, 38% of patients had continuous treatment with their index DMT, 44% discontinued DMT usage, 17% switched to another DMT, and less than 1% added a new DMT to their initial treatment. The most commonly initiated DMTs were Ocrevus (ocrelizumab; 26%), Tecfidera (dimethyl fumarate; 19%), and Copaxone (glatiramer acetate injection; 14%). In the 12 months pre-index, 33% of patients had at least 1 emergency department (ED) visit, decreasing to 26% in the first year post-index and 25% in the second year post-index. Similarly, inpatient hospitalizations decreased from 23% of patients in the year pre-index to 17% in both the first and second year of follow-up. Mean (SD) total health care costs increased from $53,184 ($123,442) in the year prior to DMT initiation to $152,112 ($133,991) in the first year post-index and $119,116 ($119,574) in the second year, mostly because of increased medication costs.

CONCLUSIONS: Within 2 years of initiating therapy, more than one-third of our MS cohort discontinued DMT, indicating a continued need for further treatment options. Although lower ED use and inpatient hospitalizations could be due to effective disease management for some patients using DMTs, there is a high cost associated with these therapies. Our study provides increased understanding of HCRU and treatment patterns in a population with MS, which could provide valuable insights to health care providers treating this disease.

SPONSORSHIP: Eli Lilly and Co.

G27 Comparing first-line natalizumab and ocrelizumab use for the treatment of multiple sclerosis: A Komodo Health claims database study

Ni D[1], Beenstock D[2], Belkin M[3], Crayton H[4], Eckstein C[5], Fredericks R[6], Kaplan J[7], Bian B[1], Belviso N[1], Rutledge D[1]; daphne.ni@biogen.com
[1]Biogen; [2]Linda E. Cardinale Multiple Sclerosis Center; [3]Michigan Institute for Neurological Disorders; [4]MS Center of Greater Washington; [5]Duke University, Department of Neuroimmunology and MS; [6]St Dominic Neuroscience Center; [7]Kansas City MS Center

BACKGROUND: Multiple sclerosis (MS), a chronic inflammatory demyelinating disease of the central nervous system, is the most common neurological cause of disability for young adults. Relapses contribute to the accumulation of disability, which impacts health care resource utilization (HCRU) and health care costs (HCC). Early use of high-efficacy disease-modifying therapies (DMTs) such as natalizumab (NTZ, TYSABRI) and ocrelizumab (OCR) in patients with newly diagnosed MS has been shown to improve clinical outcomes. More comparative studies of the clinical and economic outcomes for patients who initiate first-line treatment with NTZ or OCR are needed.

OBJECTIVE: To compare the difference of post-treatment initiation all-cause and MS-related non-DMT HCRU and HCC, as well as relapse outcomes in patients with MS who were treated with either NTZ or OCR as first-line treatment.

METHODS: This retrospective observational study used data from a comprehensive US health care claims database. The study focused on patients (aged 18-64 years) newly diagnosed with MS between January 1, 2017, and March 31, 2022, who began first-line treatment with either NTZ or OCR less than or equal to 2 years of the initial MS diagnosis claim. Propensity score (PS) matching of NTZ and OCR cohorts was performed (1:2 ratio). Study outcomes included all-cause and

MS-related non-DMT HCRU and HCC (inpatient [IP] admissions, emergency department [ED] and outpatient visits), as well as annualized relapse rate (ARR) estimated by generalized linear models.

RESULTS: A total of 1,261 patients who initiated NTZ and 2,522 patients who initiated OCR were included in the study following PS matching. Patients who began first-line therapy with NTZ experienced significantly lower (1) rates for all-cause and MS-related non-DMT IP admissions (mean 0.06 vs 0.09 admissions per person per year [PPPY], $P = 0.003$; 0.05 vs 0.08 PPPY, $P = 0.002$) and ED visits (mean 0.39 vs 0.48 PPPY, $P = 0.015$; 0.17 vs 0.22 PPPY, $P = 0.013$); (2) all-cause and MS-related non-DMT IP associated costs (mean $15,924 vs $23,124 PPPY, $P = 0.011$; mean $15,623 vs $22,317 PPPY, $P = 0.023$); and (3) ARR (mean 0.2 vs 0.28, $P < 0.001$) compared with those who initiated first-line therapy with OCR.

CONCLUSIONS: First-line initiation of NTZ was associated with significantly lower inpatient and ED HCRU, IP HCC, and ARR compared with first-line treatment of OCR. With the increasing use of high-efficacy therapies early in the disease course, this study adds to the evidence that NTZ is a clinical and cost-effective first-line therapy for patients with newly diagnosed MS.

SPONSORSHIP: Biogen

G28 Impact of step-therapy policies on clinical outcomes, health care resource utilization, and costs among Medicaid beneficiaries living with multiple sclerosis

Pineda E[1], *Geiger C*[1], *Swallow E*[2], *Gao E*[2], *Greatsinger A*[2], *Mohan M*[2], *Sarathy K*[2], *Ontaneda D*[3]; *pineda.elmor@gene.com*
[1]*Genentech, Inc.*; [2]*Analysis Group*; [3]*Cleveland Clinic*

BACKGROUND: Early use of high-efficacy disease-modifying therapies (heDMTs) provides more benefit in delaying MS disease progression vs escalation approaches. However, some payers still implement formulary restrictions (ie, step-therapy [ST] policies requiring initial trials of lower-efficacy DMTs) to reduce costs despite potential consequences on clinical outcomes and future health care resource utilization (HCRU), particularly in vulnerable Medicaid populations.

OBJECTIVE: To identify characteristics of Medicaid beneficiaries with and without state/plan-level access restrictions to heDMTs under ST policies and uncover ST policy impact on MS clinical outcomes, HCRU, and costs.

METHODS: Medicaid eligibility data and drug and medical claims for 3 US states were used. The index event was the start of the first DMT. Medicaid beneficiaries (aged ≥18 years) were included if they had 6 months' continuous enrollment pre-index and at least 12 months post-index, MS diagnosis, and at least 1 DMT claim between July 1, 2016, and December 31, 2020. Individuals were assigned to ST or non-ST cohorts based on the presence of ST policies at index using formulary data. All-cause, 12-month HCRU and costs based on billed claims were assessed post-index.

RESULTS: In a preliminary analysis of Medicaid beneficiaries from Missouri, Mississippi, and Kansas (n = 703), 576 people living with MS (plwMS) were included in the ST cohort and 127 were included in the non-ST cohort. Black plwMS and those qualified for Medicaid by disability were overrepresented in the ST cohort ($P < 0.01$ for all). White plwMS, those in cities, and those qualified for Medicaid by low income were overrepresented in the non-ST cohort ($P < 0.01$ for all). The ST cohort had a higher adjusted annualized relapse rate than the non-ST cohort (0.28 [95% CI = 0.24-0.32] vs 0.12 [0.09-0.18]; $P = 0.008$). Over 12 months, the ST cohort had higher rates of all-cause inpatient admissions; the non-ST cohort had higher rates of all-cause outpatient, emergency, and other medical visits. The ST cohort had higher inpatient and other medical costs but lower outpatient costs vs the non-ST cohort (mean difference, $10,829, $18,399, and –$29,024; $P < 0.001$ for both). Results for the full cohort (N = 13,169) using 100% Medicaid data are underway.

CONCLUSIONS: Preliminary data suggest that limited economic benefits of ST policies may be offset by future medical costs and may lead to accumulated disease progression and poorer long-term clinical outcomes for plwMS.

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G35 Treatment journey and health care resource utilization associated with patients reporting focal onset seizures

Ong A[1], *Oak B*[2], *Smith B*[2], *Athavale A*[2], *Skaar J*[2], *Wagner J*[1], *Harden C*[1]; *aong@xenon-pharma.com*
[1]*Xenon Pharmaceuticals*; [2]*Trinity Life Sciences*

BACKGROUND: Focal onset seizures (FOS) are primarily treated with antiseizure medications (ASMs), but there remains a lack of understanding of patient perceptions of their treatment journey and its economic impact.

OBJECTIVE: To assess the treatment journey, health care resource utilization (HCRU), economic impact, and quality of life (QoL) in patients reporting FOS.

METHODS: A cross-sectional survey of patients reporting FOS was conducted from July to September 2023 to examine their comprehensive burden of illness. Participants were recruited via patient panels or point-of-care physician

referrals. Eligible participants were US residents and aged 18 years and older, with a physician-confirmed diagnosis of FOS for at least 1 year, at least 1 seizure per month, past or present use of more than 2 ASMs, and currently receiving an ASM for more than 1 month. Data were collected via a web-enabled instrument including validated measures, such as the Work Productivity and Activity Impairment questionnaire to assess work productivity impairment and the Quality of Life in Epilepsy Inventory-10 to assess disease-specific quality of life.

RESULTS: A total of 170 participants reporting FOS completed the survey; 84% (143/170) reported more than 3 prior ASM treatment regimens, and 71% (120/170) reported currently taking more than 1 ASM. Despite being on various ASM treatment regimens, 67% (113/170) of participants reported having seizures more than once a month, and 75% (128/170) indicated their seizures were moderate to extremely severe on a 5-point Likert scale. Participants had an overall mean of 9.4 (±6.0) outpatient visits in the last 12 months related to their epilepsy. At least 1 emergency visit or inpatient hospitalization was reported by 66% (113/170) and 49% (83/170) of participants, respectively. The mean Quality of Life in Epilepsy Inventory-10 score (0-100 scale; higher scores indicate fewer problems) was 44.5 (±17). Of the 170 participants who completed the survey, 74 responded they are currently employed. These participants indicated a total work productivity loss of 61%, primarily driven by presenteeism.

CONCLUSIONS: Despite current ASM treatment regimens, patients reporting FOS continue to experience considerable disease burden that extends beyond clinical manifestations to impacting quality of life and work productivity, with an accompanying increase in HCRU.

SPONSORSHIP: Xenon Pharmaceuticals Inc.

G36 Characterization, health care resource utilization, and costs of health equity clusters of Medicaid-insured patients with epilepsy: An exploratory machine learning approach

Peeples H[1], *Maughn K*[2], *Dieyi C*[2], *Achter E*[2]; *herbert.peeples@ucb.com*
[1]*UCB Pharma*; [2]*STATinMED, LLC*

BACKGROUND: Claims data from adults with newly diagnosed and treated epilepsy were previously used to describe the effects of formulary restrictions on antiseizure medication (ASM) use and health care resource utilization (HCRU)/costs across payer channels.

OBJECTIVE: To describe health equity segments among Medicaid enrollees by identifying clusters and describing their characteristics, formulary restrictions, and economic burden.

METHODS: Deidentified data of Medicaid-insured adults with epilepsy prescribed at least 1 ASM on/after initial diagnosis (first ASM = index date) with continuous medical/pharmacy benefits for at least 12 months pre-/post-index from an all-payer claims database (January 1, 2014, to June 30, 2021) were used. A statistical model machine learning approach (K-prototype) was used to assign patients to health equity clusters based on number of third-generation ASMs a patient had access to and number of steps before brivaracetam (BRV) could be prescribed. Demographics, clinical characteristics, HCRU, and costs (per patient per year; 2021 US dollars) over 12 months post-index (follow-up) were examined.

RESULTS: Of 24,722 Medicaid enrollees, 5 clusters were identified: (1) mostly White, easy access to BRV and third-generation ASMs, low HCRU (44.1%); (2) mostly Black, easy access to BRV and third-generation ASMs, low HCRU (12.7%); (3) high BRV and some third-generation ASM access barriers, high HCRU (19.1%); (4) high access barriers to third-generation ASMs (6.7%); and (5) intermediate access barriers to third-generation ASMs, low HCRU (17.3%). Clusters 1 and 2 were considered "average"; clusters 3 and 4 were "higher access barriers and burden" patients. Clusters were generally similar in age, sex, ethnicity, and ASM use, with the majority aged 45-64 years, female, and non-Hispanic, and 82.7%-87.4%, 22.9%-26.1%, and 5.8%-9.2% on second-generation, first-generation, and third-generation ASMs, respectively. Over follow-up, cluster 3 had the highest inpatient and outpatient HCRU and total costs ($177,524 vs $28,734-$64,470 for clusters 1/2/4/5). Mean number/cost of prescriptions were highest in cluster 4, followed by cluster 3 (30, $18,221 and 28, $15,840 vs 21-25, $6,947-$11,458 for clusters 1/2/5). In general, higher access restrictions had higher prescriptions, emergency department visits, other visits, long-term care use, and costs.

CONCLUSIONS: Results suggest a direct relationship between formulary restrictions and economic burden, which was not explained by patient characteristics or ASM use. Because managing epilepsy may require frequent ASM changes, patients with access restrictions may experience poorer economic outcomes than those with easier access to newer, more effective ASMs.

SPONSORSHIP: UCB Pharma

G37 Characterizing use and productivity losses among US employees with migraines using calcitonin gene–related peptide inhibitors: 2017-2022

Brook R[1], *Drnach A*[2], *Good C*[3], *Peasah S*[4], *Rosenberg E*[5]; *rich@bh-ww.com*
[1]*Better Health Worldwide, Inc*; [2]*WorkPartners, LLC*; [3]*UPMC Health Plan*; [4]*UPMC*; [5]*Workpartners*

BACKGROUND: Migraine is associated with high disability rates, especially among those needing preventive medications. Calcitonin gene–related peptide inhibitors (CGRPis) are newer preventive medications often requiring prior authorization with documented frequency of migraines and a diagnosis (Dx).

OBJECTIVE: To characterize migraine diagnoses, prescriptions, and short-term disability claims of employees with migraines and those initiating a CGRPi from 2017 to 2022.

METHODS: Retrospective analysis of WorkPartners' Research Reference Database for of employees with an initial migraine Dx (*International Classification of Diseases, Tenth Revision* G43.x) between 2017 and 2022. All employees had at least 1 year of eligibility after Dx with continuous data into 2023. People with migraine (migraineurs) were reported based on their initial Dx year. Outcomes included direct (plan plus employee medical and prescription) costs, indirect costs, and lost days due to short-term disability (STD) claims (with a migraine Dx) anytime following their index date, initial and use of CGRPis in 2023. Costs were inflation adjusted to December 2023.

RESULTS: A total of 16,588 migraineurs were identified, with greater than or equal to 77.9% female individuals in each year, and an average of 13.8% had CGRPi claims. Overall migraine STD claims anytime post-index Dx annually decreased from 2.4% to 0.7%, mean STD claim durations ranged from 37 to 58.2 lost days/claim, and mean STD costs/claim ranged from $6,368 to $12,370 with lost STD days per employee decreasing from 1.5 to 0.3 days. Migraineurs initiating a CGRPi within12 months post-Dx increased annually from 0.9% (2017) to 88.4% (2022), and those still using a CGRPi in 2023 increased annually from 0.4% to 75% with mean time until the first CGRPi claim annually decreasing from 3.3 to 0.4 years. In the 12 months after Dx, mean CGRPi claims ranged from 1.3 to 6.3 days with mean direct cost/CGRPi claim decreasing from $539 in 2017 before increasing from $470 in 2018 to $758 in 2022. CGRPi migraineurs' mean STD claims any time after CGRPi ranged from 1.4% to 3.7%, with mean claim durations ranging from 44.9 to 66.6 lost days/claim, and mean costs/claim ranging from $8,685 to $16,679.

CONCLUSIONS: Use of CGRPis has increased significantly among employees since their introduction in 2018. Employees who initiated a CGRPi had a higher proportion of STD claims and costs/claim than a typical employee with migraine.

SPONSORSHIP: None

G38 Benefits of an educational migraine program on those living with migraine in the workplace: The ENLIGHTEN study

Atkinson C[1], *Wallis H*[2], *Amet M*[3], *Quinones E*[3], *Park T*[4], *Shah D*[4]; *tae.park@abbvie.com*
[1]*Observational Research, Adelphi Real World - Adelphi Group*; [2]*Adelphi Real World*; [3]*Adelphi Real World - Adelphi Group*; [4]*AbbVie*

BACKGROUND: Migraine is a primary neurological disorder that often affects patients during their prime working years. It is estimated that 26% of the US workforce is affected by migraine, resulting in significant direct and indirect costs to employers. The Migraine at Work program was a 12-week educational program provided to US employees at AbbVie Inc and was designed to raise awareness on migraine symptoms, triggers, and treatment options.

OBJECTIVE: To determine the impact of Migraine at Work on migraine management and workplace productivity among employees with migraine.

METHODS: Employees taking part in the Migraine at Work program voluntarily enrolled in a survey study alongside the program. The study consisted of 3 surveys administered at program start (week 0), program end (week 12), and after the program (week 24). At each survey timepoint, participants reported their migraine management characteristics (seeing a health care provider, medication use, and migraine frequency/severity) and workplace productivity (absenteeism and presenteeism) as measured by the Migraine Disability Assessment. Only employees with a self-reported confirmed or probable migraine diagnosis at program start and who completed at least the week 0 and one additional survey were included in this analysis. All analyses were descriptive in nature.

RESULTS: A total of 58 participants met the study inclusion criteria, of whom 31 participants also completed the week 24 survey. Participants were primarily between the ages of 35 and 54 years (72%), female (93%), and affected by migraine for 11+ years (62%). The proportion of participants who were actively being seen by a health care provider for their migraine increased from 36/58 (62%) at week 0 to 41/58 (71%) and 23/31 (74%) at week 12 and week 24, respectively. The proportion of participants taking migraine preventive medications increased from 16/58 (28%) at week 0 to 18/58 (31%) and 10/31 (32%) at week 12 and week 24, respectively.

The mean number of absenteeism days due to migraine over the previous 3 months was 1.21 days at week 0 and 0.84 days and 0.90 days at week 12 and week 24, respectively. The mean number of presenteeism days due to migraine over the past 3 months was 4.74 days at week 0 and 3.90 days and 3.42 days at week 12 and week 24, respectively.

CONCLUSIONS: Employees with migraine who participated in a migraine educational program demonstrated consistent improvements in migraine management characteristics and workplace productivity.

SPONSORSHIP: AbbVie

G39 Health care resource utilization and workplace productivity outcomes in a large employer patient population: The ILLUMINATE study

Shanahan K[1], *Brady B*[1], *Ross R*[1], *Park T*[2], *Shah D*[2], *Piarowski Y*[2], *Goldfarb N*[3]; *tae.park@abbvie.com*
[1]*Merative*; [2]*AbbVie*; [3]*Greater Philadelphia Business Coalition on Health*

BACKGROUND: Migraine is a debilitating neurological disorder that often affects patients during their working years. It is estimated that 26% of the US workforce is affected by migraine, resulting in significant health care costs.

OBJECTIVE: To evaluate the direct health care costs associated with migraine and describe the impact of migraine preventive therapy on workplace productivity among AbbVie's employee population with health benefits.

METHODS: A retrospective cohort analysis was conducted using deidentified health care claims from the 2020-2022 calendar years. Patients with a confirmed migraine diagnosis were compared with nonmigraine controls on direct health care costs. Propensity score matching was used to adjust for age, sex, and select comorbidities. These analyses were replicated on those with potential migraine (headache diagnosis and/or a claim for a migraine-specific medication) and nonmigraine controls. A descriptive analysis on all-cause disability leave days was conducted on those who initiated a branded migraine preventive therapy between 2020 and 2022.

RESULTS: The prevalence of confirmed and potential migraine was 4.4% and 3.0%, respectively. A total of 1,544 confirmed patients with migraine (mean age 42.6 years and 80.8% female) were propensity score matched to 1,544 controls without migraine (mean age 42.7 years and 82.2% female). Mean annual total direct health care costs were $19,150 vs $11,084 for confirmed patients with migraine and those without migraine, respectively ($P<0.001$), with the outpatient pharmacy costs making up the largest difference ($9,585 vs $3,738, $P<0.001$). Mean annual total direct health care costs were $20,708 vs $10,932 for potential patients with migraine and those without migraine, respectively ($P<0.001$), with inpatient/outpatient medical costs making up the largest difference ($14,109 vs $7,299, $P<0.01$). A total of 142 patients initiated a migraine preventive therapy between 2020 and 2022. These patients experienced 1,308 disability leave days in the 6 months prior to initiating a branded migraine preventive, and only 361 disability days in the next 6 months.

CONCLUSIONS: Both confirmed and potential migraine were associated with significantly higher direct health care costs in this population with employer-sponsored health benefits. Those with confirmed migraine had markedly higher outpatient pharmacy costs, whereas those with potential migraine had markedly higher medical costs than their respective controls without migraine. Descriptive analyses suggest that initiation of a branded preventive therapy may be associated with less disability leave.

SPONSORSHIP: AbbVie

G40 Long-term persistence of patients with chronic migraine switching to onabotulinumtoxinA or a different calcitonin gene–related peptide monoclonal antibody (CGRP mAb) after initial CGRP mAb treatment

Ailani J[1], *Blumenfeld A*[2], *Ahmadyar G*[3], *Dong Y*[3], *Shakhmantsir I*[3], *Gandhi P*[3], *Nahas S*[4]; *Jessica.Ailani@medstar.net*
[1]*MedStar Georgetown University Hospital*; [2]*The Los Angeles Headache Center*; [3]*AbbVie*; [4]*Department of Neurology, Thomas Jefferson University, Jefferson Headache Center*

BACKGROUND: Chronic migraine (CM) is associated with high utilization of health care resources and significant costs, which is further increased among individuals with medication discontinuations and switches vs those who remain persistent. OnabotulinumtoxinA (onabotA) is an established preventive treatment for CM. OnabotA has demonstrated greater persistence over calcitonin gene–related peptide monoclonal antibodies (CGRP mAbs) in real-world studies among patients naive to branded preventive treatments. However, no study has assessed persistence to onabotA among patients initially treated with a CGRP mAb.

OBJECTIVE: To compare real-world long-term persistence (12-24 months) in adult patients with CM who switched CGRP mAb brands vs patients who switched to onabotA after initial CGRP mAb treatment.

METHODS: This was a retrospective observational study of adult patients with CM using Merative Marketscan Commercial and Medicare database. This study included patients who had at least 1 claim of a CGRP mAb, then

initiated another CGRP mAb (erenumab, fremanezumab, galcanezumab, eptinezumab) or onabotA for 12-24 months post-index. Exclusion criteria included gepant or onabotA treatment during the greater-than-or-equal-to-6-month pre-index period, diagnosis of onabotA nonmigraine indications during the study period, or combination use of onabotA and CGRP mAb or more than 1 CGRP mAb on the index date. Persistence was measured as the proportion of patients without treatment gaps (>60 days). Adjusted persistence was calculated using logistic regression adjusting for age, sex, baseline health plan, region, Charlson Comorbidity Index, number of baseline acute and preventive medication classes, index year, number of migraine-related comorbidities, and index treatment provider.

RESULTS: Patients were treated with CGRP mAbs (N = 1,734, mean age 45 years, 86% female) or onabotA (N = 805, mean age 43 years, 88% female) for at least 12 months. After adjusting for confounding variables, a significantly higher proportion of onabotA-treated patients were persistent at 12 months (55.5%), compared with CGRP mAb–treated patients (32.9%; odds ratio [OR] = 0.39 [95% CI = 0.33-0.47]; $P < 0.001$). A significantly higher persistence with onabotA continued through 18 months (46.2% vs 25.9%; OR = 0.41 [95% CI = 0.33-0.51]; $P < 0.001$), and 24 months (35.1% vs 17.8%; OR = 0.40 [95% CI = 0.30-0.54]; $P < 0.001$).

CONCLUSIONS: This retrospective, real-world study demonstrated that patients with CM who switched to onabotA treatment after an initial CGRP mAb were more likely to remain persistent on therapy for 12-24 months compared with patients with CM who switched to another CGRP mAb.

SPONSORSHIP: AbbVie

G41 Long-term persistence of onabotulinumtoxinA vs calcitonin gene–related peptide monoclonal antibodies in new initiators with chronic migraine

Blumenfeld A[1], *Ailani J*[2], *Ahmadyar G*[3], *Dong Y*[3], *Kim J*[3], *Shakhmantsir I*[3], *Gandhi P*[3], *Nahas S*[4]; *ablumenfeldmd@gmail.com*
[1]*The Los Angeles Headache Center*; [2]*MedStar Georgetown University Hospital*; [3]*AbbVie*; [4]*Department of Neurology, Thomas Jefferson University, Jefferson Headache Center*

BACKGROUND: Chronic migraine (CM) is a disabling disease associated with significant burden. Traditional oral generic migraine preventive medications have low rates of persistence, which is linked to poor clinical and economic outcomes. Initial studies of onabotulinumtoxinA (onabotA) treatment for the prevention of CM suggest significantly better persistence than calcitonin gene–related peptide (CGRP) monoclonal antibodies (mAbs) in new initiators. These studies were limited in that they did not compare long-term persistence of patients (>12 months).

OBJECTIVE: To compare real-world long-term persistence (12-24 months) among adult patients with CM newly initiating onabotA relative to those newly initiating branded CGRP mAbs.

METHODS: This was a retrospective observational study of adult patients with CM using the Merative Marketscan Commercial and Medicare database. This study included new initiators of onabotA or a CGRP mAb (erenumab, fremanezumab, galcanezumab, eptinezumab) who stayed on index treatment from 12 months to 24 months. Exclusion criteria included preventive gepant, onabotA, or CGRP mAb treatment during the greater-than-or-equal-to-6-month pre-index period, diagnosis of onabotA nonmigraine indications during the study period, or patients who initiated more than 1 CGRP mAb or onabotA on the index date. Persistence was measured as the proportion of patients without treatment gaps (>60 days for fills or administration). Adjusted persistence was calculated using logistic regression adjusting for age, sex, baseline health plan, region, Charlson Comorbidity Index, number of baseline acute and preventive medication classes, index year, number of migraine-related comorbidities, and index treatment provider.

RESULTS: Of the patients who met inclusion criteria, 5,079 (mean age 43 years, 84% female) initiated with CGRP mAbs, and 2,223 (mean age 43 years, 87% female) were treated with onabotA. After adjusting for confounding variables, a significantly higher proportion of onabotA-treated patients were persistent at 12 months (50.3%), compared with CGRP mAb-treated patients (40.4%; odds ratio [OR] = 0.67 [95% CI = 0.61-0.74]; $P < 0.001$). A significantly higher persistence with onabotA continued through 18 months (38.1% vs 29.8%; OR = 0.69 [95% CI = 0.61-0.78]; $P < 0.001$), and 24 months (32.1% vs 23.0%; OR = 0.63 [95% CI = 0.54-0.74]; $P < 0.001$).

CONCLUSIONS: This retrospective claims analysis demonstrated that patients with CM who initiate onabotA are more likely to remain persistent on therapy at all long-term time points for up to 24 months compared with patients with CM who initiate a CGRP mAb.

SPONSORSHIP: AbbVie

G42 Comparative analysis of fremanezumab vs preventive gepants for the prevention of episodic migraine on migraine-related health care resource utilization and direct costs

Yang A[1], *Xie H*[1], *Zhang Y*[1], *Chatterjee S*[2], *Ortega M*[3], *Driessen M*[4]; *amy.yang@tevapharm.com*
[1]*Teva Branded Pharmaceutical Products R&D, Inc.*; [2]*KMK Consulting, Inc.*; [3]*Teva Pharmaceutical Industries Ltd.*; [4]*Teva Netherlands B.V.*

BACKGROUND: Fremanezumab, an injectable monoclonal antibody targeting calcitonin gene–related peptide (CGRP), and gepants, oral small-molecule CGRP receptor antagonists, are approved for preventive migraine treatment.

OBJECTIVE: To assess adherence, persistence, and migraine-related direct costs and health care resource utilization (HCRU) associated with treating patients with fremanezumab vs gepants (rimegepant/atogepant) for episodic migraine (EM) prevention.

METHODS: This retrospective analysis used data from the Merative MarketScan Commercial and Medicare supplemental databases and included adult patients diagnosed with EM who had 1 fremanezumab or 1 rimegepant/atogepant claim. Inverse probability of treatment weighting was used to balance baseline demographic and clinical characteristics between cohorts. Adherence (proportion of days covered [PDC]), persistence, and migraine-related direct costs and HCRU were assessed during a 12-month post-index period, where the index date was the first drug dispense date on or after migraine diagnosis. Migraine-related direct costs and HCRU per person per month (PPPM) were also analyzed during patients' treatment persistence period.

RESULTS: Before weighting, there were 256 patients in the fremanezumab cohort (monthly dosing, 87.9%; quarterly dosing, 12.1%) and 357 patients in the gepants cohort (rimegepant, 93%; atogepant, 7.0%). After weighting, there were 256 and 356 patients in each cohort and all baseline covariates were balanced. During the 12-month follow-up period, the proportion of patients with PDC greater than or equal to 80% was higher in the fremanezumab vs the gepants cohort (35.2% [90/256] vs 11.6% [41/356]; $P<0.0001$). Similarly, mean persistence was higher in the fremanezumab vs the gepants cohort (190.7 days vs 103.9 days; $P<0.0001$). Mean total direct costs during the follow-up period were significantly lower in the fremanezumab vs the gepants cohort ($7,607 vs $10,315; $P<0.0001$). These totals included the mean drug acquisition cost of fremanezumab ($4,548) and gepants ($7,886). Similar trends were seen for mean total direct costs PPPM in the fremanezumab vs the gepants cohort ($873 vs $1,747; $P<0.0001$). HCRU over 12 months and PPPM were similar between cohorts.

CONCLUSIONS: Among patients with EM using preventive migraine treatment, adherence and persistence were higher in the fremanezumab versus the gepants cohort. Despite longer exposure to treatment, mean total direct costs, which were largely driven by drug acquisition costs, were lower in the fremanezumab cohort than in the gepants cohort.

SPONSORSHIP: Teva Pharmaceuticals

G43 Cost-effectiveness analysis of ubrogepant, rimegepant, and zavegepant compared with each other and usual care for the acute treatment of migraine

Gokhale P, Villa-Zapata L; poojaGokhale@uga.edu
University of Georgia College of Pharmacy

BACKGROUND: Migraine is a chronic disorder characterized by episodic moderate to severe headaches, requiring a multifaceted treatment approach that includes acute and preventive therapies and nonpharmacological interventions. For those refractory to standard first- and second-line treatments, gepants offer a new class of third-line treatment options as calcitonin gene–related peptide (CGRP) antagonists.

OBJECTIVE: To evaluate and compare the cost-effectiveness of 3 CGRP antagonists—ubrogepant, rimegepant, and zavegepant—approved for the acute treatment of migraine, against both each other and standard care.

METHODS: A Markov model was constructed to assess the cost-effectiveness from a payer's perspective, using 5 health states: mild, moderate, and severe pain while on treatment, no pain while on treatment, and off treatment. The analysis was conducted over a 5-year horizon with a 48-hour cycle length, discounting costs, and quality-adjusted life-years (QALYs) annually at 3%. Sensitivity analyses were used to determine the robustness of the results.

RESULTS: All gepants demonstrated cost-effectiveness below the $100,000 per QALY threshold, with incremental cost-effectiveness ratios of $55,826 for ubrogepant, $48,286 for rimegepant, and $83,635 for zavegepant. Rimegepant was the most cost-effective, offering savings and improved outcomes compared with ubrogepant ($32,142/QALY saved) and dominating zavegepant by being both less costly and more effective.

CONCLUSIONS: Ubrogepant, rimegepant, and zavegepant are cost-effective alternatives to usual care for acute migraine treatment, particularly suitable for patients who do not respond to traditional treatments like nonsteroidal

anti-inflammatory drugs and triptans. These findings support the inclusion of gepants in clinical considerations for such patients.

SPONSORSHIP: None

G44 Relative benefits and economic value of immunomodulatory therapies for patients with generalized myasthenia gravis

Smith A[1], *Wolfe G*[2], *Habib A*[3], *Qi C*[4], *Yang H*[5], *Du M*[5], *Chen X*[5], *Gelinas D*[4], *Brauer E*[4], *Phillips G*[4], *Saccà F*[6]; *cqi@argenx.com*; *Hongbo.Yang@analysisgroup.com*
[1]*Department of Neurology, Virginia Commonwealth University*; [2]*Department of Neurology, Jacobs School of Medicine and Biomedical Sciences*; [3]*Department of Neurology, University of California- Irvine Medical Center*; [4]*argenx*; [5]*Analysis Group, Inc.*; [6]*University of Naples Federico II*

BACKGROUND: Generalized myasthenia gravis (gMG), a rare autoimmune disease of the neuromuscular junction, causes potentially life-threatening muscle weakness. Several new therapies have been recently approved in the United States for gMG following clinical trials demonstrating improved outcomes compared with placebo (PBO). Understanding the comparative efficacy and economic value of these therapies is important to inform treatment decision-making, although no head-to-head studies exist.

OBJECTIVE: To evaluate the relative benefits and costs per improved outcome (CPIO) of efgartigimod, ravulizumab, rozanolixizumab, and zilucoplan for anti–acetylcholine receptor antibody–positive (AChR Ab+) gMG.

METHODS: Clinical trials of PBO vs efgartigimod intravenous (IV) (ADAPT trial), rozanolixizumab (MycarinG), ravulizumab (CHAMPION-MG), and zilucoplan (RAISE) in AChR Ab+ gMG, where feasible, were included in network meta-analyses comparing efficacy outcomes. Outcomes included greater-than-or-equal-to-3-point and greater-than-or-equal-to-5-point reductions from baseline in Myasthenia Gravis-Activities of Daily Living (MG-ADL) and Quantitative Myasthenia Gravis (QMG) scores. Network meta-analysis outputs were used to estimate the number needed to treat (NNT) for each treatment vs PBO and for the treatment with lowest NNT vs other treatments. The CPIO (2024 USD) vs PBO was estimated using the efficacy outcomes. Drug acquisition and administration costs were estimated using the weight distribution observed in a post-approval real-world study.

RESULTS: Efgartigimod IV had the lowest NNT vs PBO for greater-than-or-equal-to-5-point reduction in MG-ADL (1.98; significantly lower vs ravulizumab [5.06] or zilucoplan [5.12]), greater-than-or-equal-to-3-point reduction in QMG (2.21; significantly lower vs ravulizumab [4.37] or zilucoplan [4.03]), and greater-than-or-equal-to-5-point reduction in QMG (1.88; significantly lower vs zilucoplan [4.91]) (all $P < 0.05$). There were no significant differences in NNT for a greater-than-or-equal-to-3-point reduction in MG-ADL across treatments. Efgartigimod IV had the lowest CPIO vs PBO across all efficacy outcomes. CPIO for greater-than-or-equal-to-3-point or greater-than-or-equal-to-5-point reductions in MG-ADL were $796,260 and $578,820, respectively, which were significantly lower than ravulizumab and zilucoplan. CPIO for greater-than-or-equal-to-3-point and greater-than-or-equal-to-5-point reduction in QMG were $645,406 and 548,717, respectively, which were significantly lower than other treatments.

CONCLUSIONS: Although all treatments were effective, efgartigimod IV had the lowest NNT values. It was associated with a favorable CPIO compared with ravulizumab, rozanolixizumab, and zilucoplan for treatments of AChR Ab+ gMG.

SPONSORSHIP: argenx

G46 Productivity loss and indirect burden among patients with migraine in the United States

Umashankar K[1], *Dong Y*[2], *Parikh K*[1], *Gandhi P*[2], *Duan M*[1]; *kandavadivu.umashankar@abbvie.com*
[1]*AbbVie*; [2]*AbbVie*

BACKGROUND: Previous studies, using data up to 2013, found substantial economic burden of migraine for patients, payers, and employers in the United States. Recent assessments on indirect and productivity burden of migraine are sparse.

OBJECTIVE: To determine the productivity loss and indirect burden among patients with migraine stratified by episodic (EM) or chronic migraine (CM).

METHODS: This retrospective cohort study identified adult patients with migraine (first migraine diagnosis as index) or without migraine (nonmigraine cohort, random service date as index) from Merative MarketScan Health Productive Management databases (January 1, 2018, to December 31, 2020). Participants had at least 12 months of post-index enrollment in work absence (WA), short-term (ST), or long-term (LT) disability benefits. Patients with migraine were matched 1:1 to patients without migraine on age in years, sex, region, health plan type, and Quan-Charlson Comorbidity Index for each benefit type. To estimate indirect burden, the proportion of patients using each benefit (WA, ST, LT), along with days lost per benefit type, was calculated. Costs were determined by multiplying employer-recorded work absence days and mean hourly

wages from the Bureau of Labor Statistics 2024 data for non–farm workers, and a 70% wage replacement rate for ST and LT, assuming an 8-hour workday. Differences in outcomes between patients with migraine and those without migraine were compared using a Student's t-test or chi-square test.

RESULTS: A total of 61,181 patients with migraine were included, among whom 13% had a CM diagnosis. A total of 6,806 individuals with migraine were matched for WA, 50,801 for ST, and 52,001 for LT database eligibility. A significantly higher proportion of individuals with migraine reported work loss due to any absence (WA, ST, or LT) compared with those without migraine (23% vs. 10%). Annual workday loss due to any absence was significantly higher among patients with migraine (35 days vs 26 days, $P<0.001$). Total annual indirect costs were 29% higher among patients with migraine vs patients without migraine ($12,717 vs $9,882). The costs due to absence ($9,886 vs $8,499), ST ($2,602 vs $1,291), and LT ($230 vs $92) were significantly higher ($P<0.001$) for patients with migraine vs non-individuals without migraine. Similar results were observed for EM and CM.

CONCLUSIONS: The results show a significant indirect burden among employed, commercially insured patients with migraine in the United States. Future research should investigate how the evolving migraine-specific treatment landscape can contribute to its reduction.

SPONSORSHIP: AbbVie

G47 Clinical, economic, and humanistic burden associated with narcolepsy: Results from a systematic literature review

Doane M[1], *Lauher-Charest M*[2], *Huynh G*[2], *McCausland K*[2]; *michael.doane@alkermes.com*
[1]*Alkermes, Inc.*; [2]*QualityMetric, an IQVIA business*

BACKGROUND: Narcolepsy, a rare, chronic neurological condition characterized by excessive daytime sleepiness, may be associated with negative impacts to patients' lives, including clinical (eg, comorbidities), economic (eg, increased medical costs), and humanistic burden (eg, impaired quality of life [QoL]).

OBJECTIVE: To systematically review studies examining clinical, economic, and humanistic burden associated with narcolepsy.

METHODS: Systematic literature searches in PubMed (2012-2022) and select conference databases (2020-2022) identified English-language studies related to burden of narcolepsy. Outcomes included clinical, economic, and humanistic burden.

RESULTS: After applying inclusion/exclusion criteria, 64 studies were included for review; 53 (83%) summarized data for clinical, 8 (13%) for economic, and 27 (42%) for humanistic burden. Mean time from symptom onset to diagnosis ranged from 8.7 to 14.6 years, reflecting significantly delayed time to diagnosis. Early age of onset generally predicted greater diagnostic delays. Compared with healthy controls (HCs), patients with narcolepsy presented with high rates of comorbidities (eg, neuropsychiatric disorders and cardiovascular diseases). Narcolepsy was also associated with higher rates of obesity (2–5 times greater), smoking (1.7–1.9 times greater), and mortality (1.5 times greater) relative to HCs. Patients with narcolepsy faced greater medical costs attributable to hospitalizations, emergency department visits, outpatient visits, and medications vs HCs. Between 2011 and 2015, the mean total annual health care costs in the United States for pediatric patients with narcolepsy were more than 6 times greater than those in the general population ($20,932 vs $3,245; 2022 USD). Regarding employment, narcolepsy was associated with short-term disability incidents, presenteeism, absenteeism, and loss of employment. Patients with narcolepsy exhibited lower QoL compared with HCs across studies. There were also functional limitations associated with narcolepsy, including the inability to care for children. Education was significantly impacted by narcolepsy due, in part, to attention deficits.

CONCLUSIONS: The diagnostic journey in narcolepsy is long and challenging. Narcolepsy is associated with physical/mental health comorbidities, impaired QoL, reduced work productivity, and increased use of health care resources. Given these burdens, direct medical and indirect costs are also relatively high among those living with narcolepsy. Future research is needed to better understand clinical, economic, and humanistic outcomes in subgroups (eg, narcolepsy subtype).

SPONSORSHIP: Alkermes, Inc.

G48 Payers should pay close attention to commonly elucidated concepts associated with dementia caregivers: Targeted literature review

Phatak A[1], *Ambegaonkar A*[2]; *projectreminiscence2024@gmail.com*; *ambi@apperturehealth.com*
[1]*Project Reminiscence*; [2]*APPERTURE LLC*

BACKGROUND: Caregivers for patients with dementia (DCG) have been known to experience more caregiving and health burden than the general population or nondementia caregivers. This can be a significant burden to the health care system as dementia may affect multiple beneficiaries for a prolonged period of time.

OBJECTIVE: To explain the concepts associated with caregiver burden and identify potential areas of interventions to reduce the burden.

METHODS: Targeted literature review of published studies over a period from 2000 to 2024 with a specific focus on dementia with or without other conditions.

RESULTS: Overall, 14 unique studies were identified with specific emphasis on DCGs. Most of these studies were conducted in North America (85%), with 1 study conducted in Japan and another in Europe. Various caregiver burden–related concepts elucidated were mental health of caregivers (n = 8), caregiver stress (n = 6), caregiver depression (n = 4), emotional disturbances (n = 3), sleep issues in DCGs (n = 2, one study also looked at sleep issues in patients with dementia as a root cause), cognitive impairment in DCGs (n = 2), family support/use of home residence (n = 1), and work-related problems/stress (n = 1). Caregiver age (most were older), age of person receiving care, total disease burden, and number of years of caregiving were deemed as important factors related to caregiver stress or burden.

CONCLUSIONS: DCGs bear significant stress or burden related to caring for patients with dementia. Because this is a multifaceted issue, it would be important to systematically assess caregiver burden using Zarit burden interviews. Further research into the impacts of caregiving on younger caregivers should also be addressed in the future, as they become active caregivers, primarily driven by proliferation of digital technology including social media and web-based resources. Further, any caregiver-oriented intervention should include pharmacological therapies including the use of antidepressants, antianxiety medications, sleep aids, and nonpharmacological therapy options such as stress management. As it is highly likely that those with dementia and DCG use the same health care system, payers may be benefited by targeting disease management interventions for DCGs, in addition to existing interventions for patients.

SPONSORSHIP: None

H00-H95 Diseases of the Eye and Adnexa

(eg, macular degeneration)

H1 Cost-utility analysis of the TearCare System vs Restasis for the treatment of meibomian gland dysfunction–associated dry eye disease in the United States

Lighthizer N[1], *Schwertz B*[2], *Chester T*[3], *Longo R*[4], *Riley P*[4], *Masseria C*[4], *Patel C*[4], *Mody L*[4]; *lighthiz@nsuok.edu*; *chad.patel@aesara.com*

[1]*Northeastern State University Oklahoma College of Optometry* (NSUOCO); [2]*Dry Eye Center of Alabama*; [3]*Cleveland Eye Clinic*; [4]AESARA

BACKGROUND: Current treatment in adult patients with meibomian gland dysfunction (MGD)–associated dry eye disease (DED) involves prescription eye drops. Although prescription eye drops offer symptomatic relief, they have limitations, including delayed onset of action, tolerability issues, and an inability to address the root cause of MGD. The TearCare System, a US Food and Drug Administration–cleared device, is indicated for adult patients with moderate to severe MGD-associated DED. Clinical trials have shown that TearCare reduces dry eye symptoms and Ocular Surface Disease Index scores in patients with moderate to severe MGD-associated DED. Although clinical data on the efficacy and safety of TearCare are available, the cost-effectiveness of TearCare has not been studied.

OBJECTIVE: To provide US payers with the functionality, using a CUA, to demonstrate the cost-effectiveness of using TearCare compared with Restasis for moderate to severe MGD-associated DED.

METHODS: The CUA was developed in Microsoft Excel using a US health care plan perspective and a 1-year time horizon with 3-month Markov cycles based on typical treatment monitoring. In this analysis, the population included patients with moderate to severe MGD-associated DED. Health states were defined by disease severity and categorized by Ocular Surface Disease Index scores (normal, mild, moderate, severe). The cycles incorporated persistence rates from literature on the use of Restasis. At the end of each cycle, patients could either remain in their original health state or transition to others based on calculated transition probabilities using data from the randomized, controlled SAHARA trial. Model outcomes included quality-adjusted life-years (QALYs), total direct health care costs (treatment cost and eye clinic visits), and the incremental cost-effectiveness ratio.

RESULTS: Total per-patient costs and QALYs were $2,205 and 0.76, respectively, for TearCare, compared with $6,066 and 0.70 for Restasis. TearCare reduced health care costs by $3,861 and provided an incremental benefit of 0.06 QALYs over Restasis across a 1-year time horizon. The main cost driver was the recurring expense of Restasis at each cycle, whereas TearCare incurs costs only twice a year. These results indicate that TearCare is the more cost-effective strategy, being both less costly and more effective than Restasis.

CONCLUSIONS: The CUA evaluating TearCare for the treatment of moderate to severe MGD-associated DED demonstrated higher QALY gains at a lower cost compared with Restasis.

SPONSORSHIP: Sight Sciences

H2 Real-world extended-dosing assessment for eye(s) new to faricimab-svoa therapy

Eckwright D, Gleason P, Thunselle S;
david.eckwright@primetherapeutics.com
Prime Therapeutics

BACKGROUND: Ophthalmic vascular endothelial growth factor inhibitors (OVEGFIs) are used to treat age-related macular degeneration (AMD) and diabetic macular edema (DME) and are a top 10 medical drug spend category for both commercial and Medicare. Annual OVEGFI single-eye treatment costs range widely from $1,000 to more than $20,000, depending on the drug and dosing frequency (every 4 to 8 weeks). In January 2022, faricimab-svoa (FAR) was approved, enabling extended dosing (ED) up to every 16 weeks after 4 monthly loading doses. FAR is more costly than competitor products when not used at an extended dose. Discerning real-world dosing of OVEGFIs is a challenge owing to claim variation in eye(s) treated (ie, left, right, or both eyes). Understanding FAR dosing at the eye level can more accurately assess dosing to inform management strategies.

OBJECTIVE: To determine the proportion of eyes having received ED for members new to FAR therapy.

METHODS: Medical FAR claims between January and December 2022 were queried from 13.9 million and 670,000 commercial and Medicare members, respectively. New start members were defined as having continuous enrollment 1 year prior to and 1 year after their first (index) claim. Members were considered treatment naive or experienced based on the presence of an OVEGFI claim within 1 year prior to their index claim. Members were assigned AMD or DME diagnosis using *International Classification of Diseases, Tenth Revision* (ICD-10) codes. Members' claims were assigned sidedness using units, procedure modifier, and ICD-10 codes. Members having 1 or more claim(s) not assigned were excluded. Eye(s) having more than 4 FAR claims were defined as achieving maintenance phase (MP). ED was defined as MP eye(s) having greater than 63 days between their most recent 2 claims.

RESULTS: Of the 371 members meeting continuous enrollment criteria, 200 had sidedness assigned, resulting in 247 analyzable eyes. The majority of eyes were MED (62%), AMD (68%), and treatment experienced (92%). There were 62% (153 of 247) of eyes that reached the MP with an average of 7.3 annual claims per eye. ED occurred in 36% of MP eyes (55 of 153), averaging 89 days between claims. In contrast, 64% (98 of 153) did not have ED, averaging 45 days between claims.

CONCLUSIONS: Only 1 in 5 eyes newly initiating FAR received ED during their first year of treatment. This low proportion of ED provides a key insight into FAR's competitiveness in the OVEGFI category, which may influence its position and importance in management strategies such as step therapy. However, the use of value-based contracts providing remuneration when ED is not achieved would improve FAR's proposition in this competitive category while meeting the varying treatment needs of patients.

SPONSORSHIP: Prime Therapeutics

H3 Economic benefit of aflibercept 8 mg vs faricimab in the treatment of patients with neovascular age-related macular degeneration or diabetic macular edema in the United States

Kuznik A[1], Sherman S[2], Patel N[3];
andreas.kuznik@regeneron.com;
steven.sherman@regeneron.com
[1]Regeneron Pharmaceuticals, Inc.; [2]Regeneron Pharmaceuticals, Inc; [3]Massachusetts Eye and Ear and Massachusetts General Hospital

BACKGROUND: Patients treated with intravitreal aflibercept 8 mg (AFL 8 mg) achieved treatment intervals less than or equal to 24 weeks through week 96 of the PULSAR (neovascular age-related macular degeneration [nAMD]) and PHOTON (diabetic macular edema [DME]) trials after 3 (PULSAR) and 5 (PHOTON) initial monthly doses. Treatment intervals for AFL 8 mg in these trials are longer than those reported for other therapeutic agents such as faricimab (FAR), which were less than or equal to 16 weeks. Over 2 years, a numerically lower mean number of injections was observed with AFL 8 mg compared with FAR in pivotal trials.

OBJECTIVE: To quantify the impact of less frequent dosing with AFL 8 mg vs FAR on direct and indirect economic cost burden over 3 years.

METHODS: An economic model was developed to estimate direct and indirect costs for AFL 8 mg and FAR over 3 years. The mean number of AFL 8-mg injections over 2 years in patients randomized to 12- and 16-week treatment intervals was 9.7 and 8.2 (PULSAR) and 9.5 and 7.8 (PHOTON), respectively. The mean number of FAR injections over 2 years in pivotal trials comparing FAR with AFL 2 mg, TENAYA/LUCERNE (nAMD) and YOSEMITE/RHINE (DME), was 10.5 and 10.7 and 11.5 and 12.1, respectively. All trials reported the mean number of injections in years 1 and 2. To exclude the effect of initial monthly doses, injection frequency in year 3 was imputed from year 2 data. Direct costs were calculated as the product of the mean number of injections over 3 years and wholesale acquisition costs (AFL 8 mg: $2,625; FAR: $2,190). Indirect costs were based on published transportation and time travel cost data and were reported separately for patients in urban, suburban, and rural settings.

RESULTS: Estimated mean direct cost over 3 years for AFL 8 mg vs FAR in nAMD was $32,156 vs $32,412, a savings of $256 in favor of AFL 8 mg. Adding indirect costs for representative urban patients with nAMD increased total costs to $32,434 vs $32,747 for AFL 8 mg vs FAR (savings: $313). For suburban and rural patients with nAMD, corresponding estimates were $32,494 vs $32,820 (savings: $326) and $32,730 vs $33,105 (savings: $375) for AFL 8 mg vs FAR, respectively. In DME, the estimated mean direct cost over 3 years for AFL 8 mg vs FAR was $30,975 vs $34,274 (savings: $3299). Adding indirect costs for representative urban, suburban, and rural patients with DME increased estimated costs to $31,242 vs $34,628 (savings: $3,386), $31,300 vs $34,705 (savings: $3,405), and $31,528 vs $35,007 (savings: $3,479) for AFL 8 mg vs FAR, respectively.

CONCLUSIONS: Less frequent dosing of AFL 8 mg may result in lower direct and indirect economic cost burden to payers and patients compared with FAR, with the greatest savings in DME and rural settings.

SPONSORSHIP: Regeneron Pharmaceuticals, Inc.

H4 Examining trends in coverage and access for anti–vascular endothelial growth factor therapies in the treatment of retinal diseases among various payers from the Tufts University Specialty Drug Evidence and Coverage Database

Chambers J[1], *Richardson T*[2], *Casebeer S*[2], *Pangrace M*[2]; *terry.richardson@impactedu.net*
[1]*Center for the Evaluation of Value and Risk in Health, Institute for Clinical Research and Health Policy Studies, Tufts Medical Center*; [2]*Impact Education, LLC*

BACKGROUND: Retinal diseases require prompt treatment with anti–vascular endothelial growth factor (anti-VEGF) agents to improve visual outcomes and prevent blindness. Coverage and access to anti-VEGF therapies for the management of retinal diseases is unique in that off-label repackaged/compounded bevacizumab is often given preferred formulary positioning owing to its well-established clinical experience and relative affordability. Ophthalmologists are responsible for navigating different distribution channels and coverage policies from numerous insurers.

OBJECTIVE: To assess the recent status of coverage for anti-VEGF agents in the treatment of retinal diseases, including age-related macular degeneration (AMD), diabetic retinopathy/diabetic macular edema (DR/DME), retinal vein occlusion (RVO), and retinopathy of prematurity (ROP).

METHODS: The Tufts University SPEC database was analyzed to assess the recent status of coverage for anti-VEGF agents in the treatment of retinal diseases (AMD, DR/DME, RVO, and ROP). A total of 353 commercial payer coverage policies were included in the analysis.

RESULTS: Overall, 44% of commercial plans had coverage for anti-VEGF therapies consistent with the US Food and Drug Administration (FDA) label, 56% of plans had coverage with restrictions beyond the FDA label, and 1% had no coverage. In addition, 54% of plans featured step therapy protocols, whereas 46% had no step therapy. Among those plans with step therapy protocols, 88% featured a step through off-label bevacizumab, 10% featured a step through bevacizumab and another agent, and 2% featured a step through only other agent other than bevacizumab. Across all therapies, FDA-approved anti-VEGFs were more likely to have coverage with restrictions beyond the label, whereas biosimilars of reference products were more likely to have coverage consistent with the FDA label. Across specific retinal diseases, RVO, AMD, and DR were more likely to have coverage with restrictions beyond the FDA label, whereas ROP and DME were more likely to have coverage consistent with the FDA label.

CONCLUSIONS: Substantial variation exists in commercial coverage of anti-VEGF therapies for the treatment of retinal diseases across the United States. Off-label bevacizumab and biosimilars of FDA-approved anti-VEGFs are generally covered with fewer restrictions than the reference products. Retinal disease states with marked morbidity and more severe consequences of nontreatment have coverage that is more consistent with the FDA label.

SPONSORSHIP: Regeneron Pharmaceuticals, Inc.

H5 The association of visual impairment with cost of medical care and risk of falls and fractures in real-world patients with geographic atrophy in the IRIS Registry

Borkar D[1], *Arkin-Leydig K*[2], *Harris J*[2], *Patel N*[3], *Abulon D*[3], *Shiozawa A*[4]; *durgaborkarmd@gmail.com*; *aki.shiozawa@astellas.com*
[1]*Duke University Eye Center*; [2]*Verana Health*; [3]*Astellas Pharma US*; [4]*Astellas Pharma, US*

BACKGROUND: Geographic atrophy (GA) is an advanced form of age-related macular degeneration (AMD) characterized by atrophic lesions in the macula that lead to progressive vision loss.

OBJECTIVE: To examine the progression of vision loss and the association of vision loss with cost of medical care and falls and fractures in patients with GA.

METHODS: This was a retrospective cohort study using the American Academy of Ophthalmology IRIS Registry (Intelligent Research in Sight). Patients had an *International Classification of Diseases, Tenth Revision, Clinical Modification* diagnosis of GA between April 1, 2016, and December 31, 2021; age 50 years or older at diagnosis; at least 1 visual acuity (VA) measurement at diagnosis (±90 days) and 1 year after diagnosis (±90 days); VA better than 20/400 and no neovascular AMD diagnosis, anti-VEGF treatment, or intravitreal treatment during the preceding 90-day baseline period; and no history of ophthalmic procedures or diagnoses of other causes of GA. Time to VA of 20/40 or worse in at least 1 eye was examined in patients with VA better than 20/40 at diagnosis. For a subgroup with closed-claims availability, health care resource utilization and associated costs were analyzed using the Komodo Health Research Dataset. The relationship of VA with total cost of medical care and with risk of falls and fractures was analyzed using generalized linear models following a γ and binomial distribution, respectively.

RESULTS: A total of 63,616 patients were included, with 25,359 patients (39.9%) followed for 2 years. VA was worse than 20/100 for 12.8% of patients at diagnosis, which increased to 24.3% by year 2. The median time to VA worse than 20/40 was 3.0 years. Patients with subfoveal involvement had a shorter median time to VA worse than 20/40 than patients without subfoveal involvement (2.2 vs 3.8 years). A total of 5,827 patients were included in the closed-claims subcohort, with 2,428 (41.7%) followed for 2 years. In models through year 2, mild (20/25 to 20/40) and moderate (20/50 to 20/100) VA impairment were associated with a greater than 30% increase in total cost of care (mild: adjusted cost ratio = 1.31, $P = 0.061$; moderate: adjusted cost ratio = 1.37, $P = 0.045$). Mild (20/25 to 20/40), moderate (20/50 to 20/100), and severe (<20/200) VA impairment were each associated with increased risk of falls and fractures in unadjusted models through year 2 (mild: odds ratio [OR] = 2.1, $P = 0.019$; moderate: OR = 2.4, $P = 0.007$; severe: OR = 2.4, $P = 0.014$).

CONCLUSIONS: Patients with subfoveal involvement had a shorter time to vision loss than patients without subfoveal involvement. Vision impairment was associated with increased cost of care and risk of falls and fractures.

SPONSORSHIP: Astellas Pharma US

H6 Impact of prescription access on latanoprostene bunod 0.024% ophthalmic solution adherence trajectories

Munnangi S[1], *Langford E*[1], *Zhou X*[2], *Divino V*[1], *Nair A*[3]; *swapna.munnangi@iqvia.com*; *abhisheknairxi@gmail.com*
[1]IQVIA; [2]IQVIA *Inc.*; [3]*Bausch + Lomb*

BACKGROUND: Latanoprostene bunod (LBN) 0.024% ophthalmic solution is a prostaglandin analog indicated for the reduction of intraocular pressure in patients with open-angle glaucoma (OAG) or ocular hypertension (OHT). Patients with OHT are at increased risk for developing glaucoma. Patient adherence to medications is pivotal in preventing worsening outcomes, and prescription access can impact long-term patient adherence.

OBJECTIVE: To identify prescription access factors associated with adherence trajectories for patients treated with LBN using group-based trajectory modeling (GBTM).

METHODS: Adults with diagnostic claims of OAG or OHT initiating LBN from January 1, 2019, to December 31, 2021 (date of initiation = index date) were identified in the IQVIA Longitudinal Prescription Claims, Professional Fee Claims, and Formulary Impact Analyzer databases. Medication adherence patterns using group-based trajectory modeling and patient access factors were identified over a 2-year follow-up period. Proportion of days covered (PDC) for each group was measured.

RESULTS: The final study sample comprised 10,182 LBN patients. Four distinct trajectory patterns were identified, with 36.6% of patients nearly adherent (mean PDC = 0.79)

and 19.9% with low adherence (mean PDC = 0.38). A steady declining adherence was observed among 16.8% (mean PDC = 0.29), whereas the remaining 26.8% of patients became nonadherent within 6 months (mean PDC = 0.08). Patients in the declining and nearly adherent group had a higher days supply of index LBN prescription compared with those in the low-adherence and nonadherent group. Of the 10,182 LBN patients, 8,583 (84.3%) linked to the Formulary Impact Analyzer dataset with at least 1 LBN claim. In this subset of patients, on average, 10 LBN prescriptions (Rxs) for patients in the nearly adherent group, 5 Rxs in the low-adherence group, 4 Rxs in the declining-adherence group, and 1 Rx in the nonadherent group were paid ($P < 0.0001$). The average out-of-pocket cost for these patients was significantly lower for those in the nearly adherent group ($78.71) and gradually declining group ($82.67), compared with those in the low-adherence group ($100.91) and nonadherent group ($91.87) ($P < 0.0001$).

CONCLUSIONS: Access to prescription medications in terms of paid prescription claims and out-of-pocket costs has a significant impact on patient adherence trajectories. Lower out-of-pocket costs for LBN patients is associated with significantly higher patient adherence, which is essential for preventing or delaying progressive damage and irreversible vision loss.

SPONSORSHIP: The study was funded by Bausch & Lomb Americas

H7 Real-world vision and health equity outcomes with a dual-mechanism digital treatment for amblyopia: Results from the PUPiL Registry

Hancock S[1], *Warner N*[2], *Repka M*[3]; *nwarner@nepnv.com*
[1]*Luminopia*; [2]*Nevada Eye Physician*; [3]*Wilmer Eye Institute, Johns Hopkins Hospital*

BACKGROUND: Amblyopia is a common neuro-visual condition in which visual acuity is reduced in one eye because of neurologic suppression of that eye. Luminopia is a binocular, digital treatment, shown in phase 1, 2, and 3 trials to improve amblyopic eye best-corrected visual acuity (BCVA). A real-world study of Luminopia is being conducted with an observational, institutional review board–approved registry, the PUPiL Registry. Prior studies, conducted before US Food and Drug Administration de novo approval of Luminopia, have shown disparities in amblyopia treatment outcomes for those with Medicaid insurance.

OBJECTIVE: To describe outcomes of Luminopia treatment in clinical practice for amblyopia and identify differences in outcomes in patients covered by government (Medicaid) vs commercial insurance.

METHODS: PUPiL Registry (NCT06429280) includes patients with a diagnosis of amblyopia and 12+ weeks' prescription of Luminopia from participating sites. Demographic and vision data are collected retrospectively per visit per patient. Treatment and follow-up are at prescriber discretion. Treatment adherence is objective collected via software. For this analysis, patients were classified by health care insurance type into government or commercial. Data evaluated include demographics, change in lines BCVA, and adherence to prescribed treatment.

RESULTS: As of February 21, 2024, 120 patients from 10 sites in the registry with insurance type reported and at least 1 follow-up visit, 12 ± 6 weeks after starting treatment, included 53 (44%) Medicaid and 67 (56%) commercial patients. Mild (better than 20/40) and moderate [20/40 to 20/100) amblyopia at baseline was noted in 11% and 74% of Medicaid patients, respectively, vs 34% and 48% of commercial patients. Mean improvement BCVA was not statistically different between the groups (0.8 lines vs 1.3 lines in Medicaid vs commercial, $P = 0.24$) or among patients with moderate amblyopia (1.1 lines vs 0.9 lines, $P = 0.49$). Median treatment adherence was 71% (IQR = 50-86) for patients with Medicaid and 72% (IQR = 42-95) with commercial insurance. No safety events were reported within the registry data collected.

CONCLUSIONS: Using outcomes from actual clinical practice, improvement in visual acuity with this digital therapeutic was seen in both insurance groups. There was no significant difference between children with government or commercial insurance. Adherence to prescribed treatment was good in both groups. As registry enrollment and duration of treatment both increase, characterization of treatment effect on these patient populations will continue to be refined.

SPONSORSHIP: Luminopia, Inc.

I00-I99 Diseases of the Circulatory System

(eg, atrial fibrillation, pulmonary hypertension)

I1 Racial disparities in health-related quality of life among patients with cardiovascular disease

Oliorah P[1], *Zakeri M*[2], *Sujit S*[1], *Alvarez S*[1]; *pioliora@cougarnet.uh.edu*; *mzakeri@central.uh.edu*
[1]*University of Houston*; [2]*Pharmaceutical Health Outcomes and Policy, College of Pharmacy, University of Houston*

BACKGROUND: Understanding health outcomes among patients with cardiovascular disease (CVD) is essential for enhancing treatment strategies and improving patient quality of life.

OBJECTIVE: To examine racial and ethnic disparities in health-related quality of life among patients with CVD.

METHODS: This retrospective cross-sectional study used data from the Medical Expenditure Panel Survey over 8 years (2014-2021). The study population included individuals diagnosed with various CVDs. Linear regression models adjusted for demographic, socioeconomic factors, CVD severity, comorbidities, and health care expenditures to assess racial and ethnic disparities with patients' health-related quality of life. The study examined Physical and Mental Component Scores (PCS and MCS) across non-Hispanic Black (Black), Hispanic, and non-Hispanic White (White) populations.

RESULTS: There were 11,446 (weighted frequency: 15,071,781) individuals with CVD identified. Notable differences were observed in age distribution, with a higher proportion of individuals aged 65 and older in the White cohort (68.82%) compared with the Black (53.68%) and Hispanic (51.99%) cohorts ($P < 0.0001$). Unadjusted linear regression indicated significantly lower MCS and PCS, respectively, for Hispanic (MCS: estimate = –4.11, $P < 0.0001$; PCS: estimate = –1.52, $P < 0.001$) and Black cohorts (MCS: estimate = –3.00 $P < 0.0001$; PCS: estimate = –2.83 $P < 0.0001$) compared with White. The White and Hispanic women had lower MCS (White: adjusted estimate (AE) = –0.84, $P < 0.001$; Hispanic: AE = –1.58, $P < 0.01$) compared with men. Age 65 years and older was positively associated with MCS for all cohorts (Black: AE = 2.95, $P < 0.05$; White: AE = 3.90, $P < 0.001$; Hispanic: AE = 2.76, $P < 0.05$). Physical limitations significantly lowered MCS (Black: AE = –1.72; White: AE = –2.10; Hispanic: AE = –2.63, all $P < 0.001$) and PCS (Black: AE = –8.15, $P < 0.001$; White: AE = –10.40, $P < 0.001$; Hispanic: AE = –9.16, $P < 0.01$). Reliance only on public insurance or being uninsured, lower income, and higher comorbidities were all significantly associated with lower PCS and MCS.

CONCLUSIONS: The poorer physical and mental health status among the Black and Hispanic cohorts, despite consistent predictive factors across all races, underscores a disproportionate distribution of predictive factors within racial and ethnic groups. Targeted clinical interventions and health policies are required to address these disparities, focusing on improving access to care, education, and support for managing physical limitations and comorbidities.

SPONSORSHIP: None

12 Impact of RBT-1 on postoperative complication rates and costs for cardiac surgery

van Wagenberg F[1], Cherry L[2], Singh B[3], Ruiz S[3], Magar R[2]; fvanwag@gmail.com; rmagar@ahrminc.com
[1]Huntsville Hospital Heart Center; [2]AHRM Inc.; [3]Renibus Therapeutics

BACKGROUND: Postoperative complications of cardiac surgery (CABG, Valve, or Combined CABG/Valve) occur in up to 67% of patients. There are no approved pharmacological therapies that reduce the risk of postoperative complications. Short- and long-term complications may lead to worse outcomes and a substantial increase in health care utilization costs. A novel drug, RBT-1, has been evaluated in a phase 2 clinical trial and demonstrated a substantial reduction in postoperative complications when administered prior to cardiac surgery. The most common postoperative complications reported include prolonged intensive care unit stay, new-onset postoperative atrial fibrillation, and blood transfusion. We report on the magnitude of incremental cost savings based on complication rates between groups.

OBJECTIVE: To evaluate the incremental cost savings for RBT-1 vs placebo (PBO) based on clinical trial results.

METHODS: Complication rates from a clinical trial for RBT-1 (NCT04564833) were used in a decision tree model to estimate the average expected cost of patients who were dosed with RBT-1 vs PBO. Complication rates were categorized from 0, 1, 2, and 3 or more complications for each treatment group. Costs for each category were estimated based on the literature.

RESULTS: A total of 121 patients (80 RBT-1/41 PBO) were evaluated for clinical outcomes in a phase 2 trial. Complication rates for each (category) by treatment group were as follows: 31.3% (0), 46.3% (1), 11.3% (2), and 11.3% (3 or more) for RBT-1 and 14.6% (0), 31.7% (1), 26.8% (2), and 26.8% (3 or more) for PBO. The cost of 0% complications was estimated at $50K, which is the average cost of procedures. The expected costs when 1, 2, and 3 or more complications occurred were $84K, $121K, and $254K, respectively. Based on the phase 2 trial, the average expected cost of the RBT-1 treatment group was $99.7K vs $142K for PBO, leading to a 30% or $42K incremental cost savings in favor of RBT-1.

CONCLUSIONS: Cardiac surgery complications are common and costly to the health care system. For patients who have 1 or more complications, costs are not additive but increase exponentially. Results from the phase 2 trial suggest a protective effect of RBT-1 leading to a lower complication rate, which also reduces the overall average expected cost. Additional data from an ongoing phase 3 trial, which includes a 1-year follow-up after discharge, will contribute

to evaluating the impact of RBT-1 on clinical, economic, and qualitative outcomes compared with standard of care.

SPONSORSHIP: Renibus Therapeutics

13 Impact of RBT-1, a novel treatment to reduce postoperative complication rates and costs for coronary artery bypass graft surgery

van Wagenberg F[1], *Cherry L*[2], *Singh B*[3], *Ruiz S*[3], *Magar R*[2]; *fvanwag@gmail.com*; *lcherry@ahrminc.com*
[1]*Huntsville Hospital Heart Center*; [2]*AHRM Inc.*; [3]*Renibus Therapeutics*

BACKGROUND: Postoperative complications of coronary artery bypass graft (CABG) surgery occur in up to 66% of patients. There are no approved pharmacological therapies that reduce the risk of postoperative complications. A novel drug, RBT-1, has been evaluated in a phase 2 clinical trial and demonstrated a substantial reduction in postoperative complications when administered prior to CABG surgery. The most common postoperative complications reported include prolonged intensive care unit stay, new-onset postoperative atrial fibrillation, and blood transfusion. We report on the magnitude of incremental cost savings based on complication rates between groups for CABG surgery.

OBJECTIVE: To evaluate the incremental cost savings for RBT-1 when compared with placebo (PBO) based on clinical trial results for CABG surgery.

METHODS: Complication rates from a phase 2 trial of RBT-1 (NCT04564833) were used in a decision tree model to estimate the average expected cost of patients who were dosed with RBT-1 vs PBO for CABG. Complication rates were categorized from 0, 1, 2, and 3 or more complications for each treatment group. Costs for each category were estimated based on the literature.

RESULTS: A total of 121 patients were assessed for clinical outcomes in the phase 2 trial, of whom 64 patients had a CABG-alone surgery (44 RBT-1/20PBO). Rates of complications for each (category) by treatment group were as follows: 36.4% (0), 50% (1), 9.1% (2), and 4.6% (3 or more) for RBT-1 and 25% (0), 40% (1), 25% (2), and 10% (3 or more) for PBO. The cost of 0% complications was $50K, the average cost of a CABG procedure. The expected cost when 1, 2, and 3 or more complications occurred were $84K, $121K, and $254K, respectively. Based on the phase 2 trial, the average expected cost of RBT-1 was $85K vs $108K for PBO, leading to a 19% or $23K incremental cost savings in favor of RBT-1 in the CABG group.

CONCLUSIONS: CABG surgery complications are common and costly to the health care system. For patients who have 1 or more complications, costs are not additive but increase exponentially. Results from the phase 2 study suggest a protective effect of RBT-1 leading to a lower complication rate, which also reduces the overall average expected cost for CABG surgery. Additional data from an ongoing phase 3 trial, which includes a 1-year follow-up after discharge, will contribute to evaluating the impact of RBT-1 on clinical, economic, and qualitative outcomes compared with standard of care.

SPONSORSHIP: Renibus Therapeutics

14 Pharmacist-led statin improvement project in a multipayer care organization

Huynh T[1], *Pezzella S*[2], *Smith M*[2], *Rodriguez de Bittner M*[1], *Rochester-Eyeguokan C*[1], *Hurley J*[3], *Robin M*[3]; *thuyn001@rx.umaryland.edu*; *spezzella@rx.umaryland.edu*; *melissa.smith2@rx.umaryland.edu*
[1]*University of Maryland School of Pharmacy*; [2]*University of Maryland School of Pharmacy Center for Innovative Pharmacy Solutions*; [3]*University of Maryland Medical System*

BACKGROUND: As the health care industry transitions from a fee-for-service model to value-based care, there is an increasing focus on performance measures that incentivize improved patient outcomes. One such measure is the Merit-based Incentive Payment System (MIPS) quality measure. In this context, clinical pharmacists' expertise can be leveraged to enhance statin-related quality metrics in a multipayer accountable care organization.

OBJECTIVE: To investigate the potential benefits of this approach.

METHODS: The study included patients who were not on statin therapy and thus did not meet the MIPS quality measures. Their charts and claims data were carefully screened for preexisting statin prescriptions. Patients who were pregnant, breastfeeding, diagnosed with rhabdomyolysis, or receiving palliative/hospice care or who had active hepatic disease, end-stage renal disease, statin-associated muscle symptoms, or a statin allergy were excluded from the study. Clinical pharmacists sent recommendations to initiate statin therapy via electronic health record messaging to eligible patients' primary care providers before their upcoming visits.

RESULTS: Between July 1 and November 24, 2023, clinical pharmacists reviewed 462 patient records. Of these, 17% of them had diabetes, 40% showed established cardiovascular disease, and 43% demonstrated hypercholesterolemia (familial hypocholesteremia and/or low-density lipoprotein levels>190 mg/dL). After eliminating exclusions and determining the urgency of statin therapy, 126 recommendations for statin initiation were sent to providers. Of these recommendations, statin prescriptions were initiated in

25% of patients. Reasons for not initiating statin therapy in the remaining patients were not addressed (64%), patient missed scheduled appointment (5%), patient refused (2%), provider discussed therapy with patient without prescribing (2%), provider determined statin therapy was unnecessary (1%), and other category (1%).

CONCLUSIONS: The implementation of a collaborative approach involving clinical pharmacists and quality teams has proved to be an effective means of improving statin therapy initiation rates within health care organizations. Pharmacists play a pivotal role in identifying eligible patients and facilitating targeted recommendations to providers, highlighting the value of interdisciplinary teamwork in achieving quality care metrics. Results show that such an approach may be beneficial in improving patient outcomes and promoting evidence-based practice in health care settings.

SPONSORSHIP: None

15 Real-world clinical and economic burden among commercially insured ST-segment elevation myocardial infarction survivors in the United States

Veloz A[1], *Pizzicato L*[2], *Chen J*[2], *Gu* Y[2], *Schultz K*[1], *Divino* V[2]; *aveloz@zoll.com*; *lia.pizzicato@iqvia.com*; *justin.chen@iqvia.com*; *yifan.gu@iqvia.com*; *kschultz@zoll.com*; *victoria.divino@iqvia.com*
[1]*ZOLL Cardiac Management Solutions*; [2]IQVIA

BACKGROUND: ST-segment elevation myocardial infarction (STEMI) is a severe and acute manifestation of coronary artery disease. Although percutaneous coronary intervention is the preferred reperfusion strategy, heart failure (HF) continues to be a frequent and costly complication of STEMI. Previous studies that have examined clinical and economic outcomes of STEMI in the United States have primarily used the National Inpatient Sample or The Nationwide Readmissions Database and have only focused on in-hospital outcomes and costs. There is limited research available that has evaluated both clinical and economic outcomes of STEMI survivors, and these analyses have been conducted among Medicare beneficiaries in the United States or non-US populations. No such real-world data are available specific to commercially insured STEMI survivors in the United States.

OBJECTIVE: To quantify the clinical and economic burden of STEMI survivors among a commercially insured population in the United States leveraging real-world data.

METHODS: This was a retrospective, observational cohort study using administrative claims data from the IQVIA PharMetrics Plus for MedTech database. Adult patients with a primary discharge diagnosis of STEMI during an inpatient hospitalization from January 2018 to June 2021 were identified (admission date = index date). Eligible patients had continuous enrollment in the 6-month pre-index (without prior STEMI diagnosis) and 1-year post-index periods. Clinical outcomes and all-cause health care resource utilization and costs were evaluated over the 1-year post-index. Subgroup included patients that developed incident HF over the post-index.

RESULTS: The final sample comprised 3,236 patients (mean age [SD] = 62.6 [11.7] years; 72.1% male). During the index hospitalization, the mean (SD) length of stay was 5.1 (6.6) days, and the vast majority (88.9%) were treated with percutaneous coronary intervention. Mean (SD) all-cause total cost was $76,161 ($111,789). Inpatient cost represented 73.8% of mean all-cause total cost. Overall, approximately one-third (34.4%) of patients experienced incident HF in a mean (SD) of 61.4 (141.9) days post-index. Mean (SD) all-cause total cost was notably higher among this subgroup ($99,589 [$173,217]).

CONCLUSIONS: The burden of STEMI survivors is substantial. This first-ever analysis among a commercial population in the United States reveals significant health care resource utilization and costs associated with STEMI management, highlighting an urgent need for enhanced therapies, treatment protocols, and comprehensive care strategies to enhance STEMI survivors' quality of life and reduce the long-term burden to the health care system.

SPONSORSHIP: ZOLL Medical

16 Use of cardiovascular disease risk–mitigating interventions among patients with overweight or obesity and established atherosclerotic cardiovascular disease in a US real-world setting

Michalak W[1], *Boame* N[2], *Zhao* X[2], *Lamarre* N[2], *Rava* A[2], *Faurby* M[1], *Ó Hartaigh* B[1], *Fabricatore* A[1], *Zhao* Z[1], *Lopez-Jimenez* F[3]; *wqmi@novonordisk.com*
[1]*Novo Nordisk Inc.*; [2]*Genesis Research Group*; [3]*Mayo Clinic*

BACKGROUND: Patients with established atherosclerotic cardiovascular disease (eASCVD; including history of myocardial infarction, hemorrhagic or ischemic stroke, or peripheral artery disease) are at high risk for adverse cardiovascular outcomes. Cardiology practice guidelines recommend the use of lipid-lowering (LL), antithrombotic (AT), antihypertensive (AHTN), and cardioprotective antihyperglycemic (CAHG; glucagon-like peptide-1 receptor agonists and sodium-glucose cotransporter-2 inhibitors) medications, as well as weight loss through lifestyle therapy (LT), antiobesity medication (AOM), and bariatric surgery (BaS), as cardiovascular disease risk–mitigating interventions (CVD-RMIs). Data on the use of these guideline-recommended CVD-RMIs among those with eASCVD, with and without overweight or obesity (Ov/Ob), are limited.

OBJECTIVE: To compare current use of CVD-RMIs among patients with eASCVD and either Ov/Ob (body mass index [BMI] ≥ 25.0 kg/m^2) or normal weight (NW; BMI = 18.5-24.9 kg/m^2).

METHODS: IQVIA Ambulatory Electronic Medical Records and IQVIA PharMetrics Plus claims databases were used to identify adults with eASCVD (index date 2017-2022). Use of CVD-RMIs was compared in patients with Ov/Ob vs NW during the 12 months preceding the index date, which included the use of LL, AT, AHTNs for those with a diagnosis of hypertension or high blood pressure (BP; systolic BP ≥ 130 mm Hg/diastolic BP ≥ 80 mm Hg), CAHGs for those with type 2 diabetes or hemoglobin A1c ≥ 6.5%, and AOM or LT for those with a BMI ≥ 27 kg/m^2, along with BaS among patients with BMI ≥ 35 kg/m^2. Demographic characteristics were assessed at the index date.

RESULTS: Among those with eASCVD, 6,055 patients with NW and 26,238 with Ov/Ob were identified. Baseline demographics were similar across both cohorts; however, those with Ov/Ob were more frequently male (59.4% vs 46.6%, $P < 0.001$), and a smaller proportion were aged 75 years or older compared with those with NW (9.9% vs 15.7%, $P < 0.001$). Use of all CVD-RMIs was greater among eligible patients with eASCVD in the Ov/Ob vs NW cohort (all $P < 0.001$), respectively: LL (60.8% vs 49.5%); AT (35.7% vs 32.3%); AHTN (81.1% vs 71.8%); and CAHG (20.1% vs 9.9%) medications. AOM, LT, and BaS were used by 0.1%, 5.1%, and 2.4% of eligible patients, respectively.

CONCLUSIONS: A considerable proportion of individuals with eASCVD are not receiving guideline-recommended therapy; those with Ov/Ob have higher utilization of CVD-RMIs than those with NW. LT, AOM, and BaS are substantially underutilized to mitigate CVD risk, despite their high potential clinical benefit in those with eASCVD and Ov/Ob.

SPONSORSHIP: Novo Nordisk Inc.

17 Real-world health care resource utilization before and after initiation of inhaled treprostinil dry powder inhaler in patients with pulmonary hypertension due to interstitial lung disease

Parikh R[1], *Classi P*[2], *Morland K*[2], *Wu B*[2], *McEvoy C*[3]; *raj.parikh@hhchealth.org*
[1]*Hartford Hospital*; [2]*United Therapeutics Corporation*; [3]*Washington University School of Medicine*

BACKGROUND: Pulmonary hypertension due to interstitial lung disease (PH-ILD) is a rapidly progressing disease associated with poor functional status and outcomes. Inhaled treprostinil is currently the only US Food and Drug Administration–approved therapy for PH-ILD and is available as a nebulizer and a dry powder inhaler (DPI). Real-world claims data evaluating DPI use and impact on outcomes have not been established.

OBJECTIVE: To understand differences in real-world health care resource utilization before and after initiation of inhaled treprostinil DPI in patients with PH-ILD.

METHODS: This is a retrospective cohort study using the IQVIA PharMetrics Plus database. DPI initiators were indexed on date of DPI initiation between May 25, 2022, and September 30, 2024. Initiators were required to be aged 18 years or older on index, have at least 1 inpatient or at least 2 outpatient medical claims separated by at least 30 days for pulmonary hypertension and interstitial lung disease in the 6-month pre-index, be continuously enrolled for 6 months pre-index, and have at least 1 month of follow-up time. Patients with inhaled treprostinil nebulizer claims 60 days prior to index were classified as transition DPI initiators. Patients without any prostacyclin in the 60 days prior to index were classified as de novo DPI initiators. Outcomes of interest included all-cause per-person per-month (PPPM) hospitalization utilization in the pre- and post-index periods. A paired t-test was used to compare change in PPPM hospitalizations in the pre-index vs post-index.

RESULTS: After applying selection criteria, a total of 108 DPI initiators were identified. Seventy-five patients initiated DPI de novo and 31 transitioned from inhaled treprostinil nebulizer. Mean (SD) follow-up time was 6.0 (3.4) months for de novo initiators and 9.5 (2.9) months for transition initiators. Mean (SD) age at index was 59.9 (12.2) and 62 (10.5), 67% and 65% were female, 48% and 35% were from the South and 35% and 26% were from the East for the de novo and transition DPI cohorts, respectively. In the de novo DPI cohort, mean (SD) all-cause PPPM hospitalizations decreased from 0.06 (0.17) in the pre-index to 0.02 (0.07) in the post-index, $P = 0.04$. In the DPI transition cohort, mean (SD) all-cause PPPM hospitalizations decreased from 0.03 (0.11) to 0.01 (0.03), $P = 0.29$, from the pre- to post-index, respectively.

CONCLUSIONS: In patients with pulmonary hypertension due to interstitial lung disease, initiation of inhaled treprostinil DPI resulted in fewer all-cause hospitalizations. A decrease was observed for patients that initiated as de novo or transitioned from prior inhaled treprostinil nebulizer therapy.

SPONSORSHIP: United Therapeutics Corporation

I10 Payer differences on costs of care in obstructive hypertrophic cardiomyopathy

Reza N[1], *Butzner M*[2], *Batra K*[3], *Amos Q*[3], *Buikema A*[3], *Anderson A*[3], *Shreay S*[2], *Owens A*[1]; *Nosheen.Reza@pennmedicine.upenn.edu*; *mbutzner@cytokinetics.com*
[1]*Perelman School of Medicine at the University of Pennsylvania*; [2]*Cytokinetics, Inc.*; [3]*Optum*

BACKGROUND: There is no economic evidence on the impact of insurance coverage on costs of care for patients with obstructive hypertrophic cardiomyopathy (oHCM).

OBJECTIVE: To evaluate 5-year cumulative costs by insurance coverage using Optum claims and electronic medical record data.

METHODS: Retrospective cohort study of adults diagnosed with oHCM from January 2013 to December 2021. Eligible patients had at least 2 claims for HCM (*International Classification of Diseases, Ninth Revision* [ICD-9] and ICD-10) at least 30 days apart (index date = earliest HCM claim) and post-index HCM treatment or evidence of septal reduction therapy and 6-month pre-index and 5-year post-index continuous enrollment. HCM-related cumulative costs (2022 Consumer Price Index adjusted) were reported for medical (ambulatory: office visits, outpatient [OP] visits; emergency department [ED] visits; inpatient admissions [IP]) and pharmacy and stratified by insurance coverage (commercial, Medicare, Medicaid, unknown).

RESULTS: Among 5,129 patients with oHCM, 52% were female with a mean age 63.9±14.3 years, 77.6% were non-Hispanic White, and 40.6% and 29.7% lived in the Midwest and Northeast, respectively. Insurance coverage included 40% Medicare, 36.8% commercial, 16.2% unknown, and 6.4% Medicaid. There were significant differences across HCM-related cost categories by insurance, with costs being driven by IP and OP costs ($P<0.001$). OP costs by insurance coverage were commercial: $15,739, unknown: $9,533, Medicare: $9,002, and Medicaid: $8,502 ($P<0.001$). Patients with Medicaid experienced greater total (Medicaid: $85,815, Medicare: $66,092, unknown: $66,019, commercial: $65,812; $P<0.01$), medical (Medicaid: $84,457, Medicare: $65,088, unknown: $64,860, commercial: $64,349; $P<0.01$), and IP costs (Medicaid: $58,332, Medicare: $48,233, unknown: $45,683, commercial: $37,782; $P<0.001$). ED costs were greatest among patients with Medicaid (Medicaid: $2,750, Medicare: $840, unknown: $818, commercial: $789; $P<0.001$). Pharmacy costs were greatest among commercially insured patients (commercial: $1,464, Medicaid: $1,359, unknown: $1,159, Medicare: $1,004; $P<0.001$).

CONCLUSIONS: There are significant differences for cost of care among patients with oHCM across insurance coverage, with patients covered by Medicaid insurance experiencing greater total, medical, and IP costs. Future research is needed to understand the root cause of these economic differences.

SPONSORSHIP: Cytokinetics, Incorporated

I13 Anticipated reduction in oral anticoagulant drug spending for an average Medicare Part D plan

Zenker G, Shetty A, Biggs E, McCoy M; *mark.mccoy@iconplc.com*
ICON plc

BACKGROUND: Two of the first 10 drugs selected for Medicare Part D price negotiation are the oral anticoagulants Eliquis (apixaban) and Xarelto (rivaroxaban). These 2 drugs are the top-selling oral anticoagulants. The Medicare price cuts will take effect January 1, 2026.

OBJECTIVE: To assess how oral anticoagulant drug spend for an average 1-million-member Medicare Part D plan could be impacted by Centers for Medicare & Medicaid Services (CMS) price negotiations for Eliquis and Xarelto.

METHODS: We analyzed information available from CMS, Department of Health and Human Services, Kaiser Family Foundation, the Commonwealth Fund, and the Biden-Harris Administration.

RESULTS: Gross spending by Medicare Part D on Eliquis and Xarelto in the 12 months ending May 2023 was $22.5 billion. Prior to CMS price negotiations, both products were heavily discounted by the manufacturers, with rebates estimated at 49%. Net spending by Medicare Part D plans for Eliquis and Xarelto is estimated to be $11.5 billion. Assuming that the Medicare Part D population is 48 million, an average 1-million-member Part D plan spends approximately $240 million per year on Eliquis and Xarelto. Price reduction percentage and average savings per 1-million-member plan would be 25%/$60 million, 30%/$72 million, 35%/$84 million, 40%/$96 million, 45%/$108 million, and 50%/$120 million. According to CMS, the minimum discount for short monopoly drugs such as Eliquis and Xarelto is 25%. Based on negotiations between CMS and the manufacturers, a discount will be determined. CMS has indicated that the precise discount will be announced in September 2024. At a national level, the minimum 25% price reduction would decrease Eliquis/Xarelto spend by the following amounts at the top Medicare Part D firms: United Health: $661 million, Humana: $489 million, and CVS Health: $489 million.

CONCLUSIONS: An average Medicare Part D plan with 1 million members will see spending on Eliquis and Xarelto decrease by at least $60 million based on sales in the year ending May 2023. Depending on the results of negotiations,

the same plan could save up to $120 million per year if the discount reached 50% for both products.

SPONSORSHIP: None

I15 Association of out-of-pocket costs of direct oral anticoagulants and prescription abandonment among patients with nonvalvular atrial fibrillation or venous thromboembolism

Zhdanava M[1], *Ashton V*[2], *Korsiak J*[1], *Jiang F*[1], *Pilon D*[1], *Alberts M*[3]; *masha.zhdanava@analysisgroup.com*; *vashton1@its.jnj.com*
[1]*Analysis Group, Inc.*; [2]*Janssen Scientific Affairs, LLC, a Johnson & Johnson company*; [3]*The Ayer Neuroscience Institute at Hartford Hospital*

BACKGROUND: Direct oral anticoagulants (DOACs) are used to prevent thrombosis in patients with nonvalvular atrial fibrillation (NVAF) and venous thromboembolism (VTE). Despite clinical benefits, some patients abandon their DOAC prescription.

OBJECTIVE: To evaluate the association between out-of-pocket (OOP) costs and abandonment of the first prescribed DOAC among US patients with NVAF or VTE.

METHODS: Symphony Health, an ICON plc Company, Patient Source (April 1, 2017, to October 31, 2020) was used to select patients with NVAF or VTE starting a DOAC (apixaban, dabigatran, rivaroxaban; index date). Patients were classified into approved or abandoned cohorts based on their index DOAC claim status. OOP costs (2021 US dollars) of the index claim were described by abandonment status, and multivariable logistic regression models were used to evaluate the association between OOP costs and abandonment of the index DOAC claim. Analyses were performed in patients with NVAF and VTE separately.

RESULTS: Among patients with NVAF, 667,417 (mean age: 70 years; 45% female) had an approved and 86,338 (mean age: 71 years; 45% female) had an abandoned index DOAC claim. Among patients with VTE, 282,066 (mean age: 60 years; 53% female) had an approved and 26,363 (mean age: 63 years; 53% female) had an abandoned index DOAC claim. Mean OOP costs of the index DOAC claim were higher in those with an abandoned claim (NVAF abandoned vs approved: $175 vs $79; VTE abandoned vs approved: $133 vs $65). Among patients with NVAF, 9% in the abandoned and 21% in the approved cohort had no OOP costs, 49% (abandoned) and 59% (approved) had OOP costs greater than $0 to less than $100, and 42% (abandoned) and 20% (approved) had OOP costs greater than or equal to $100; among patients with VTE, 16% (abandoned) and 28% (approved) had no OOP costs, 55% (abandoned) and 58% (approved) had OOP costs greater than $0 to less than $100, and 30% (abandoned) and 14% (approved) had OOP costs greater than or equal to $100. In multivariable models, the risk of abandonment increased by 21% (NVAF) and 17% (VTE) for each $100 in OOP costs (both $P<0.001$). Relative to patients with no OOP costs, patients with OOP costs greater than $0 to less than $100 were 85% (NVAF) and 61% (VTE) more likely to abandon their index DOAC, and patients with OOP costs greater than or equal to $100 were 4.32 (NVAF) and 3.43 (VTE) times more likely to abandon their index DOAC (all $P<0.001$).

CONCLUSIONS: Among patients with NVAF (11% with abandoned index DOAC claim) or VTE (8% with abandoned index DOAC claim), increasing OOP costs above $100 quadruples for NVAF and triples for VTE the risk of abandonment.

SPONSORSHIP: Janssen Scientific Affairs

I16 Health care resource utilization among patients with paroxysmal supraventricular tachycardia

Desai N[1], *Sacks N*[2], *Sherwood R*[3], *Bharucha D*[3], *Pokorney S*[4]; *nihar.desai@yale.edu*
[1]*Section of Cardiovascular Medicine, Department of Internal Medicine, Yale School of Medicine*; [2]*HEORStrategies, A Division of ToxStrategies LLC*; [3]*Milestone Pharmaceuticals*; [4]*Duke University Medical Center and Duke Clinical Research Institute*

BACKGROUND: Paroxysmal supraventricular tachycardia (PSVT), a common arrhythmia characterized by episodic tachycardia, is associated with substantial health care resource utilization (HCRU), including emergency department (ED) visits, inpatient admissions (IP), physician office visits (OV), and outpatient hospital (OH) visits. Data on HCRU among prevalent patients with PSVT is limited.

OBJECTIVE: Evaluate HRU and expenditures among prevalent patients with PSVT relative to a matched non-PSVT comparator population.

METHODS: This retrospective, observational cohort study used IQVIA PharMetrics (2013-2019) data, with adjudicated claims for more than 210 million unique individuals with commercial or Medicare insurance across the United States. Patients with PSVT had at least 1 IP admission or ED visit or at least 2 outpatient visits with a PSVT diagnosis (*International Classification of Diseases, Tenth Revision*: I47.1) prior to 2019, were continuously enrolled in their health plan in 2019, and had at least 1 visit with a cardiologist or at least 1 visit for PSVT with a primary care provider in the past 3 years (2016-2019). Patients with PSVT were matched to individuals with no evidence of PSVT on demographics (age, sex), payer (commercial, Medicare), and Charlson Comorbidity Score. The percentages of patients with PSVT and

comparator patients with at least 1 ED visit, IP admission, or OV or OH visit in 2019 were calculated, as were the mean number of these encounters among those with at least 1. Expenditures by payers and patients were calculated, and IP length of stay was estimated.

RESULTS: Among 105,000 patients with PSVT and 715,000 matched patients without PSVT, 20% were aged younger than 45 years, 53% were aged 45-64 years, and 27% were aged 65 years and older; 61% were female; 82% had commercial and 18% Medicare coverage. In 2019, 29% of the prevalent PSVT cohort had at least 1 ED visit (vs 11% comparator), 20% had at least 1 IP admission (vs 4%), 95% had at least 1 OV (vs 90%), and 78% had at least 1 OH visit (vs 50%; all $P<0.0001$). Mean annual per-patient visit rates among those with at least 1 visit were higher for PSVT than non-PSVT (ED: 2.2 vs 1.6; IP: 1.8 vs 1.3; OV: 14.7 vs 7.9; OH: 7.1 vs 3.6;). Mean annual per-patient payer expenditures were higher for all patients with PSVT vs comparator patients ($21,282 vs $4,889), as were expenditures for patients with PSVT ($1,782 vs. $785). Mean length of stay was also greater (7.8 vs 5.7 days) among patients with PSVT.

CONCLUSIONS: Patients with PSVT had higher HCRU and expenditures for all settings of care compared with those without PSVT, although the greatest differences were in settings other than OVs. Treatments and initiatives to manage PSVT in the outpatient setting are needed to minimize the impact of PSVT on HCRU and expenditures.

SPONSORSHIP: Milestone Pharmaceuticals

J00-J99 Diseases of the Respiratory System

(eg, asthma, COPD, rhinitis)

J13 A retrospective review of the Healthcare Effectiveness Data and Information Set Asthma Medication Ratio quality measure and Area Deprivation Index, health care utilization, and health care costs in a regional health plan population

Modany A[1], *Marr D*[1], *Bryk A*[1], *McGraw M*[1], *Joseph L*[1], *Christian N*[2], *Good C*[1]; *modanyad4@upmc.edu*; *marrd@upmc.edu*
[1]*UPMC Health Plan*; [2]*UPMC*

BACKGROUND: The Health Effectiveness Data and Information Set (HEDIS) Asthma Medication Ratio (AMR) quality measure acts as a surrogate marker for identifying possible reliever medication overuse and controller medication underuse in people aged 5-64 years with persistent asthma. Studies have evaluated the relationship between AMR and health care utilization and costs but have not explored the correlation between AMR and area deprivation index (ADI).

OBJECTIVE: To examine the relationship of HEDIS AMR attainment with ADI, clinical outcomes, and economic outcomes.

METHODS: Retrospective claims review of Medicaid members aged 5-64 with a HEDIS definition of persistent asthma in calendar year 2023. Members were continuously enrolled during the study period and classified as AMR attainers (AMR ≥ 0.5) or nonattainers (AMR < 0.5) based on HEDIS definition. The effect of ADI on AMR attainment was measured using logistic regression. To appropriately reflect the structure of utilization data, effects of AMR attainment were measured using a combination of tobit, loglinear, and zero-inflated Poisson regression.

RESULTS: Members were identified as AMR attainers (3,187) and nonattainers (1,059). Increases in ADI predicted lower AMR attainment (odds ratio = 0.96; $P = 0.02$). AMR attainers had fewer asthma-related facility visits ($P = 0.03$) and more asthma-related outpatient visits ($P < 0.01$). AMR attainers had fewer reliever fills ($P < 0.01$) but were more likely to have at least 1 oral corticosteroid burst ($P < 0.01$). Overall per-member per-month (PMPM) total cost of care, medical cost, and pharmacy costs were all higher for AMR attainers (37%, 14%, and 52% respectively; $P < 0.01$). Asthma-associated costs are also higher for AMR attainers (90%, 82%, and 99%, respectively; $P \leq 0.01$).

CONCLUSIONS: Higher ADI predicted a significant reduction in the HEDIS AMR attainment. This is the first study to our knowledge to evaluate this association. AMR failure was associated with higher facility visits and less outpatient professional engagement, which is consistent with findings in previous studies. Finally, AMR attainment was associated with higher PMPM costs, primarily driven by medication costs.

SPONSORSHIP: None

J14 Cost comparisons of next-generation sequencing vs sequential single-gene testing in advanced nonsquamous non–small cell lung cancer in the United States

Grossman J[1], *Hocum B*[2], *Paracha N*[2], *Monegato D*[3], *Kaitz M*[3], *Lopez-Chavez A*[4], *Laframboise W*[5], *Vail E*[6]; *jamie.grossman@bayer.com*
[1]*Bayer US LLC*; [2]*Bayer*; [3]*Cencora*; [4]*Allegheny Health Network Cancer Institute*; [5]*Allegheny Health Network - Genomics Facility*; [6]*Cedars-Sinai*

BACKGROUND: Next-generation sequencing (NGS) testing has become increasingly important as it enables identification of multiple biomarkers but may be underutilized in the United States because of cost concerns.

OBJECTIVE: Using a previously developed cost calculator with testing costs in Euros (Stenzinger et al, 2023), we estimated cost per patient (CPP) and cost per correctly identified patient (CCIP) to compare sequential single-gene testing (SGT) vs multiplex NGS in advanced non–small cell lung cancer (NSCLC) for US patients.

METHODS: We modified a previously developed genomic testing cost calculator based on clinically actionable genomic alterations identified in the European Society for Medical Oncology (ESMO) Scale for clinical actionability of molecular targets (ESCAT) to include testing costs in USD. We used ESCAT data in our analysis as there is general concordance between US guidelines and international organizations such as ESMO. Using sensitivity/specificity data for SGTs and NGS and marker prevalence, the number needed to predict metric was monetarized using Centers for Medicare & Medicaid Services data (Clinical Lab Fee Schedule and Outpatient Prospective Payment System Addendum B) based on the appropriate Current Procedural Terminology codes to estimate CCIP for NSCLC. We used reimbursement rates for biomarkers specific to NCSLC and averaged for each commonly used methodology, eg, fluorescence in situ hybridization, immunohistochemistry, polymerase chain reaction, and NGS.

RESULTS: CCIP was lower with NGS than sequential SGT for advanced/metastatic NSCLC. Using ESCAT I and II, NGS CPP was $1,759 and CCIP was $1,998 (95% CI = $1,991-$2,117) whereas sequential SGT CPP was $1,611 and CCIP was $2,249 (95% CI = $1,596-$3,721). Results are consistent with previously published data using European testing costs.

CONCLUSIONS: The cost to correctly identify clinically actionable genomic alterations was lower for NGS than sequential SGT in advanced nonsquamous NSCLC for US patients. Sequential SGT is often used despite its limitations of longer turnaround time and tissue wastage to reduce costs. However, our results found that sequential SGT is not cost saving. The benefits of NGS testing continue to expand with implementation of combined DNA and RNA panels, and recently, NGS assays have moved into the plasma domain where they can be run when tissues are inaccessible or unavailable, which occurs frequently in NSCLC.

SPONSORSHIP: Bayer

J16 Demographics and health care resource utilization in patients in the United States with α-1 antitrypsin deficiency who are treated with Glassia (α-1 proteinase inhibitor)

Khandelwal N[1], Higgins S[2], Hinson J[2], Amin S[3], Bello T[3], Stoller J[4]; takedauspubs@pharmagenesis.com
[1]Takeda Pharmaceuticals USA, Inc.; [2]Takeda Pharmaceuticals USA, Inc.; [3]Global Consulting, Cencora; [4]Cleveland Clinic

BACKGROUND: α-1 Antitrypsin deficiency (AATD) is an inherited disease affecting the lungs and liver, characterized by low levels of α-1 antitrypsin (AAT). Glassia (α-1 proteinase inhibitor; α1-PI) is an intravenous augmentation therapy that raises AAT serum concentrations. There is a lack of studies exploring patient characteristics and real-world outcomes associated with AATD treatments in general, and specifically with α1-PI.

OBJECTIVE: To describe demographic and clinical characteristics for patients with AATD treated with α1-PI and assess adherence/persistence for those initiating α1-PI.

METHODS: This retrospective cohort study of US health care encounters used Komodo Health data to describe characteristics of patients with AATD and α1-PI use during the study period (January 2016 to September 2022). The analysis included patients with at least 2 inpatient/outpatient claims at least 30 days apart with an AATD diagnosis and at least 2 α1-PI claims during the identification period. Outcomes included treatment patterns and all-cause/α1-PI–related health care resource utilization. Data were based on the 12-month follow-up period. Mean, median, and IQR were provided for continuous variables, with numbers and percentages for categorical measures.

RESULTS: In this study, a total of 249 patients with AATD were identified as α1-PI users, of whom 159 (63.8%) were new users; mean treatment duration was 13.5 months. Mean age of patients treated with α1-PI was 56.0 years and median follow-up time was 34.9 months. The most common baseline comorbidities were chronic pulmonary disease (95.2%) and hypertension (59.4%). Among α1-PI users, 99.2% had outpatient visits, 33.7% had emergency department visits, and 28.5% had at least 1 inpatient hospitalization during the 12-month follow-up period. New α1-PI users received an average of 22.3 prescription fills. During the 12-month follow-up period, 46.5% of new users persisted on treatment (mean persistence over entire follow-up: 29.2 months). Of new α1-PI patients, 6.9% switched to a different therapy, with most switching to Prolastin-C (5.0%). Among new users, 53.5% discontinued α1-PI, and 28.3% subsequently started another augmentation therapy.

CONCLUSIONS: This study provides novel, descriptive findings on patients with AATD in the United States treated with α1-PI and suggests unmet treatment needs exist in this population. Future studies should explore additional types of health care data to demonstrate the value of α1-PI for patients with AATD.

SPONSORSHIP: Takeda Pharmaceuticals USA, Inc.

J17 Health care costs of hospitalized respiratory syncytial virus cases among adults aged 50 years and older

Singer D[1], *Wang Y*[2], *Wu A*[2], *La E*[1], *Gerber S*[1], *Lee L*[1], *Liu H*[2], *Betts K*[2]; *david.a.singer@gsk.com*
[1]GSK; [2]*Analysis Group*

BACKGROUND: Respiratory syncytial virus (RSV) is a common respiratory virus. Older adults and adults with comorbidities are at increased risk for severe RSV disease, which may include hospitalization.

OBJECTIVE: To estimate the incremental cost of hospitalized RSV cases in adults aged 50 years and older compared with those without a recent acute respiratory illness (ARI).

METHODS: This retrospective cohort study used Optum's deidentified Clinformatics Data Mart Database from October 2015 to June 2023. Adults aged 50 years and older with at least 12 months of continuous enrollment were included and assigned to cohorts based on having an RSV hospitalization (RSV cohort) or not having any recent (defined as the prior 28 days) ARI (control cohort). For the RSV cohort, index date was the start of an episode that included hospitalization and an RSV diagnosis, whereas controls were matched 5:1 with the RSV cohort based on the RSV case index date. Baseline characteristics were measured in the 12 months of continuous enrollment immediately preceding index and outcomes were measured in the time after index for up to 12 months. Outcomes included total, medical, and pharmacy costs per patient per month (PPPM). Costs were compared between the RSV and control cohorts over 1-month, 3-month, and 12-month follow-up, using multivariable linear models to estimate adjusted cost differences and 95% CIs.

RESULTS: The RSV and control cohorts included 14,759 and 73,795 patients, respectively. At baseline, mean age (76.5 vs 69.5 years), mean Charlson Comorbidity Index score (3.3 vs 1.0), and percentage with Medicare insurance (91.3% vs 68.0%) were higher in the RSV vs control cohort. At 1 month, mean (SD) total cost in the RSV cohort was $41,390 ($42,501) PPPM compared with $1,505 ($8,011) PPPM in the control cohort, with an adjusted cost difference of $37,025 (95% CI = $36,345-$37,704). Total cost PPPM and adjusted cost differences PPPM attenuated over time, but the adjusted PPPM cost difference remained $6,699 (95% CI = $6,476-$6,922) over 12-month follow-up. Total costs and total cost differences were driven by medical costs, with an adjusted cost difference between cohorts in medical costs of $6,600 (95% CI = $6,379-$6,822) PPPM over 12 months.

CONCLUSIONS: Hospitalized cases of RSV were associated with substantial incremental costs in adults aged 50 years and older even after adjusting for differences in baseline characteristics with control patients without recent ARI using multivariable modeling. These findings build on previous research demonstrating the high cost of RSV cases involving hospitalization.

SPONSORSHIP: GSK (Study #: VEO-000616)

K00-K93 Diseases of the Digestive System

(eg, Crohn disease, ulcerative colitis)

K1 Development of a claims-based artificial intelligence algorithm for identifying inflammatory bowel disease flares

Visaria J[1], *Quimbo R*[1], *Barron J*[2], *Shao L*[3], *Cozmuta R*[3], *Pollock J*[3], *Morris L*[4], *Fisher V*[4], *Mehta F*[5]; *jay.visaria@carelon.com*; *ralphm.quimbo@carelon.com*
[1]*Carelon Research*; [2]*Carelon Research*; [3]*Elevance Health*; [4]*Elevance Health/CarelonRx*; [5]*Carelon*

BACKGROUND: Episodic flares cause substantial morbidity, loss of productivity, and high medical costs among individuals with inflammatory bowel disease (IBD).

OBJECTIVE: To efficiently identify, through robust identification of flares using administrative claims data, opportunities for clinical interventions to improve patient outcomes.

METHODS: A broad set of IBD-related claims from medical and pharmacy claims for US commercially insured patients diagnosed with ulcerative colitis or Crohn disease with a 5-year continuously eligible period between January 2017 and September 2021 were extracted. Claims from a random sample of 10 patients were jointly reviewed by 2 clinicians to establish agreement and consistency of clinical determination of flare episodes, after which each clinician was provided a random sample of 200 patients from which they identified flare episodes. A random forest artificial intelligence (AI) model was trained on the clinically determined flare episodes. Suspected flare episodes were also scored using claims-based algorithmic criteria. AI predicted flares were then compared with algorithmically determined flares.

RESULTS: A total of 172,177 commercially insured patients with IBD were identified with a mean age of 44 years, and 52% were female. The AI model identified a physician visit accompanied by IBD and flare-related diagnosis codes as the most important and steroid use as the least important predictor of flares. Out of a total of 7.48 million suspected episodes, the AI model identified 247,845 IBD flare episodes with a positive predictive value of 99.97%, specificity of 100.00%, sensitivity of 76.44%, and negative predictive value greater than 99.99%. Seventy-nine percent of false negative episodes involved steroid use and no other IBD diagnosis or related procedure codes.

CONCLUSIONS: The novel AI model identified episodes of IBD flares with fewer false negatives and high positive predictive value and negative predictive value compared with claims-based algorithmic criteria in a large US commercially insured population. The AI model may be used to efficiently identify flares in large IBD populations using claims data, facilitating population-level measurement of treatment outcomes and targeting of interventions by health plans. Future research may validate the model as well as target improvements to model sensitivity without detrimental impact on model performance.

SPONSORSHIP: This AI model development study was sponsored by Elevance Health.

K3 LEAP-002 update: Lenvatinib + pembrolizumab vs lenvatinib + placebo as first-line therapy for patients with advanced hepatocellular carcinoma after an additional 12 months of follow-up

Finn R[1], *Kudo M*[2], *Merle P*[3], *Meyer T*[4], *Qin S*[5], *Ikeda M*[6], *Xu R*[7], *Edeline J*[8], *Ryoo B*[9], *Ren Z*[10], *Cheng A*[11], *Galle P*[12], *Kaneko S*[13], *Kumada H*[14], *Wang A*[15], *Mody K*[16], *Dubrovsky L*[15], *Siegel A*[15], *Llovet J*[17], *Estenson K*[16]; *RFinn@mednet.ucla.edu*; *Kasey_Estenson@eisai.com*

[1]*UCLA*; [2]*Kindai University Hospital*; [3]*Hopital de la Croix Rousse*; [4]*Royal Free London NHS Foundation Trust*; [5]*Cancer Centre of Jinling Hospital, Nanjing University of Chinese Medicine*; [6]*National Cancer Center Hospital East*; [7]*Hunan Cancer Hospital*; [8]*Centre Eugene Marquis*; [9]*Asan Medical Center, University of Ulsan College of Medicine*; [10]*Zhongshan Hospital, Fudan University*; [11]*National Taiwan University Hospital*; [12]*Mainz University Medical Center*; [13]*Kanazawa University Hospital*; [14]*Toranomon Hospital*; [15]*Merck & Co., Inc.*; [16]*Eisai Inc.*; [17]*Tisch Cancer Institute, Icahn School of Medicine at Mount Sinai*

BACKGROUND: The randomized phase 3 LEAP-002 study (NCT03713593) evaluated the efficacy and safety of lenvatinib (len) + pembrolizumab (pembro) vs len + placebo (pbo) in patients with previously untreated advanced hepatocellular carcinoma (HCC). Although the LEAP-002 study did not meet its primary endpoints of overall survival (OS) at final analysis (median, 21.2 vs 19.0 months; hazard ratio [HR] = 0.840; 95% CI = 0.708-0.997) and progression-free survival (PFS) at interim analysis 1 (median, 8.2 vs 8.0 months; HR = 0.867; 95% CI = 0.734-1.024), the late separation of Kaplan-Meier survival curves for OS and PFS between treatment arms from 12 months onward, together with numerical improvements in all efficacy outcomes, warranted extended follow-up.

OBJECTIVE: To report results after 12 months of additional follow-up (median, 43.6 months).

METHODS: Eligible patients had advanced HCC and were randomly assigned 1:1 to len (8 mg/day if body weight [BW] < 60 kg; 12 mg/day if BW ≥ 60 kg) + pembro (200 mg IV every 3 weeks) or len + pbo. OS and PFS per Response Evaluation Criteria in Solid Tumors (RECIST) v1.1 by BICR were dual primary endpoints. Secondary endpoints included overall response rate (ORR) and duration of response (DOR), both per RECIST v1.1 by BICR, and safety.

RESULTS: A total of 794 patients were randomly assigned to receive len + pembro (n = 395) or len + pbo (n = 399). Median follow-up from randomization to data cutoff (June 6, 2023) was 43.6 months (range, 37.3-52.6). Median OS was 21.1 months with len + pembro vs 19.0 months with len + pbo (HR = 0.836; 95% CI = 0.713-0.981); median PFS was 8.2 months vs 8.1 months (HR = 0.810; 95% CI = 0.692-0.949), respectively. ORR was 26.3% for len + pembro vs 17.5% for len + pbo; median DOR was 16.6 months (range, 2.0+ to 45.3+) vs 10.4 months (range, 1.9 to 37.0+), respectively. Grade 3-5 treatment-related adverse event rates were 62.8% with len + pembro and 58.0% with len + pbo. The most common treatment-related adverse events of any grade in the len + pembro vs len + pbo arms were hypertension (43.8% vs 46.8%), diarrhea (40.8% vs 34.2%), and hypothyroidism (40.0% vs 35.9%).

CONCLUSIONS: After an additional 12 months of follow-up, OS and PFS for len + pembro vs len + pbo were consistent with the primary efficacy analyses, and no new safety signals were observed. The median OS of 19.0 months with len monotherapy continues to support its role as a standard-of-care treatment in the first-line setting of advanced HCC. The results of LEAP-002 support the evaluation of TACE ± len + pembro for intermediate-stage HCC in the ongoing phase 3 LEAP-012 study (NCT04246177).

SPONSORSHIP: Eisai Inc., Nutley, NJ, USA, and Merck Sharp & Dohme LLC, a subsidiary of Merck & Co., Inc., Rahway, NJ, USA

K4 Diagnosis rates and prevalence of nonalcoholic steatohepatitis in the United States: Estimates and forecasts based on epidemiological and statistical modeling approaches

Fishman J[1], *Kim Y*[1], *O'Connell T*[2];
jfishman@madrigalpharma.com
[1]*Madrigal Pharmaceuticals*; [2]*Medicus Economics LLC*

BACKGROUND: Historically, treatments were unavailable for nonalcoholic steatohepatitis (NASH)/metabolic dysfunction–associated steatohepatitis. However, the US Food and Drug Administration recently approved resmetirom (REZDIFFRA) as the first treatment for noncirrhotic NASH with moderate to advanced liver fibrosis (~F2-F3). Accordingly, the prevalence of treatment-eligible patients is of interest to decision-makers and requires appraisal of the diagnosis rate of NASH. Evidence suggests that the condition is underdiagnosed, possibly owing to reasons including limited symptoms in early stages, previous lack of incentive to diagnose given the unavailability of treatments, and lack of a standardized diagnosis protocol.

OBJECTIVE: To estimate the diagnosis rates and prevalence of NASH over time, using 2 forecasting methods: (1) an epidemiological, illness-death model and (2) parametric statistical modeling of true prevalence and the diagnosis rate.

METHODS: Two approaches were used, representing distinct classes of forecasting methods used in literature. In Approach #1, an epidemiological, illness-death model was parametrized using estimates of the diagnosed-incidence rate of NASH (from Optum Clinformatics Data Mart, and Veradigm electronic health records linked to Komodo claims–both for 2015-2022) and published mortality data. Parameter-uncertainty impact was tested by varying the current diagnosed-incidence rate and future growth, reflecting estimates of changes in diagnosis using coding intensity in Medicare Advantage vs Traditional, and published analysis of diagnosis increases following new treatments (Oka 2023). In Approach #2, parametric statistical modeling was used to project "true" prevalence (undiagnosed and diagnosed cases) of NASH over time, as well as the diagnosis rate based on a pre/post analogue case. True prevalence was estimated using NHANES 1988-2020 and forecasted linearly (other parametric forms in sensitivities). Future change in the diagnosis rate was estimated using a sigmoidal curve (Auvin 2018, other forms in sensitivities), using diagnosis rate changes in those with elevated low-density lipoprotein cholesterol pre/post introduction of statins.

RESULTS: In Approach #1, diagnosed NASH prevalence was estimated at 0.5%-1.0% in 2025 and projected to grow to 0.8%-1.9% by 2040. In Approach #2, diagnosed NASH prevalence was estimated at 1.0%-1.7% in 2025 and projected to grow to 2.8%-4.6% by 2040.

CONCLUSIONS: Using distinct forecasting methods, diagnosed prevalence of NASH in US adults is projected to remain under 5% by 2040; decision-makers can use the diagnosis-rate and prevalence estimates.

SPONSORSHIP: Madrigal Pharmaceuticals, Inc.

K5 Estimating the underdiagnosis of nonalcoholic fatty liver disease and nonalcoholic steatohepatitis in Medicare claims data

Fishman J[1], *Kim Y*[1], *Nunag D*[2], *Davis M*[2], *Marcum Z*[3]; *jfishman@madrigalpharma.com*
[1]*Madrigal Pharmaceuticals*; [2]*Medicus Economics LLC*; [3]*Medicus Economics*

BACKGROUND: Claims-based epidemiology of nonalcoholic fatty liver disease (NAFLD), or metabolic dysfunction–associated liver disease, and nonalcoholic steatohepatitis (NASH), or metabolic dysfunction–associated steatohepatitis, underestimates disease prevalence. However, claims data remain valuable for understanding disease burden providing large sample sizes and patient-level, longitudinal follow-up. A way to identify the known underdiagnosis of chronic disease prevalence in claims data is to apply a mathematical framework to adjust the estimates.

OBJECTIVE: To quantify the underdiagnosis of NASH and NAFLD prevalence in claims data using a mathematical framework.

METHODS: Using a method by Stocking et al (2023), which identifies latent individuals (those who have not yet been identified with a target condition but are likely to have some degree of condition) to estimate "true" prevalence (ie, accounting for latent individuals) of NAFLD and NASH in a 100% sample of Medicare fee-for-service (FFS) beneficiaries aged 65 years and older continuously enrolled from January 1, 2017, to December 31, 2022 (1-year baseline period and 5 years of follow-up). NAFLD (*International Classification of Diseases, Tenth Revision* [ICD-10]: K76.0 or K75.81) and NASH (ICD-10: K75.81) were defined as at least 1 inpatient claim or at least 2 outpatient claims with diagnosis codes in any position. Using 2018 as the first year of follow-up and assuming patients with an initial NAFLD/NASH diagnosis in 2019 or later also had NAFLD/NASH in 2018, we calculated the ratio of "true" to observed prevalence in 2018. In a separate cohort of continuously enrolled beneficiaries in 2022, we estimated "true" prevalence of NAFLD and NASH in 2022 by multiplying the observed prevalence in 2022 by the estimated ratio.

RESULTS: A total of 15,105,608 beneficiaries (mean age, 74.1 years; 57% female; 84% White) were included in the 5-year

cohort. From 2018 to 2022, 508,609 beneficiaries with NAFLD and 73,220 beneficiaries with NASH were identified, with ~30% of each diagnosed in 2018. Applying the prevalence ratio to the 2022 cohort (N = 22,631,654), NAFLD diagnosed prevalence increased from 1.49% to an estimated "true" prevalence of 5.30% in 2022. Similarly, NASH diagnosed prevalence increased from 0.26% to an estimated "true" prevalence of 0.85% in 2022.

CONCLUSIONS: Using a cohort of Medicare FFS beneficiaries and applying a mathematical approach to correct for underdiagnosis, the "true" prevalence values of NAFLD (5.30%) and NASH (0.85%) were estimated to be ~3.5 times higher than using claims diagnosis codes alone. Decision-makers can use this epidemiologic information to better assess budget impact of new therapeutics for NASH.

SPONSORSHIP: Madrigal Pharmaceuticals

K6 Systematic literature review on the epidemiology, natural history, and disease burden of biliary atresia and its impact on native liver survival in the United States

Lee S[1], *Los A*[2], *Navickas C*[2], *Khankhel Z*[2], *Shamseddine N*[1]; *sonia.lee.ext@ipsen.com*
[1]*Ipsen*; [2]*Genesis Research Group*

BACKGROUND: Biliary atresia (BA) treatment is limited to surgical interventions, primarily Kasai portoenterostomy (KPE), with many patients requiring eventual liver transplantation (LT). There are no approved pharmacologic agents available to slow or prevent complications of BA after KPE.

OBJECTIVE: To describe epidemiology, natural history, and native liver survival (NLS) among patients with BA in the United States via a systematic literature review.

METHODS: We searched Embase and Medline (2014-2024) for English-language studies in US-based patients with BA. Included were clinical trials and observational studies of patients with BA receiving any intervention (eg, KPE or LT) or no treatment. Outcomes focused on epidemiology (incidence, prevalence, survival), natural history (clinical features, abnormalities, comorbidities), and NLS (rates, waitlist time to LT).

RESULTS: We identified 38 studies (n = 40 references; study sample sizes: 10-4,306 patients). Interventions included cohorts receiving KPE alone, primary LT, KPE followed by salvage LT, and combinations of KPE or LT with other interventions (eg, steroids). Analysis of studies with epidemiological data (n = 31 studies) reported incidence rates of 2.85-5.55 per 100,000 children aged younger than 1 year and birth prevalence rates of 6.5-6.8 per 100,000 live births. Pre-LT waitlist mortality was relatively low (4%-6%), whereas posttreatment 1-, 5-, and 20-year overall cohort survival ranged from 93% to 98%, 86% to 98%, and 80% to 91%, respectively. Data on NLS (n = 8 studies) were limited, with most studies having relatively small sample sizes. Reported overall cohort NLS rates at 2 and 5 years ranged from 50% to 69% and 39% to 49%, respectively. Reported time to LT varied based on subgroups and calculation methods (time from BA diagnosis, KPE, or waitlist addition). Reported natural history of BA lacked consistency across studies (n = 24). Hepatic abnormalities were assessed using Model for End-Stage Liver Disease/Pediatric End-Stage Liver Disease scores reported at recommendation for LT (4-18) and at time of LT (6-22). Few studies (n = 6) reported hepatic abnormality frequencies, and patient clinical features and comorbidities varied considerably. Commonly reported clinical features were portal vein thrombosis, portal hypertension, and septicemia/bacteremia, with ascites, cholangitis, esophageal varices, and cirrhosis as frequent comorbidities.

CONCLUSIONS: This systematic literature review highlights heterogeneity in current BA research and demonstrates that significant burden exists as a result of BA-related complications. Future research using real-world data is crucial to identify actionable gaps in the care of BA and to optimize patient management strategies.

SPONSORSHIP: Ipsen

K7 Development of a risk adjustment model in commercially insured patients with inflammatory bowel disease

Moran K[1], *Hellems S*[2], *Munshi K*[3], *Smith I*[2], *Null K*[4]; *kellyn.moran@takeda.com*; *Sarah.hellems@optum.com*; *kiraat.munshi@takeda.com*; *Ian.smith@optum.com*; *kyle.null@takeda.com*
[1]*Takeda Pharmaceuticals U.S.A., Inc*; [2]*Optum Advisory Services*; [3]*Takeda Pharmaceuticals U.S.A., Inc.*; [4]*Takeda Pharmaceuticals, USA, Inc.*

BACKGROUND: Actuarial risk adjuster models are used to explain the variance of health care costs in patients. No model has used clinical-risk groupers specific to patients with inflammatory bowel disease (IBD).

OBJECTIVE: To develop and evaluate clinical-risk groupers to explain the variance of total IBD-related medical costs and to predict future costs in commercially insured patients.

METHODS: Patients with at least 1 claim with a primary diagnosis code for Crohn disease (CD) and/or ulcerative colitis (UC) in 2020-2022 were identified from the Optum database. Clinical-risk groupers, with 3 levels of increasing risk severity, were developed for CD and UC using cluster-based approaches based on per-patient per-month (PPPM)

IBD medical costs associated with *International Classification of Diseases, Tenth Revision* codes. Patients were hierarchically assigned to a severity group for CD and/or UC based on claims for each year of the study. Regression analyses were used with clinical-risk groupers to explain cost variance in the same year (concurrent models) and in the following year (prospective models). Separate models were developed for users and nonusers of targeted IBD therapies (biologics and small synthetic molecules). Clinical variables were included if they improved the explanatory power of models (R^2).

RESULTS: In total, 50,296 commercially insured patients with IBD were identified. For targeted IBD therapy users, the relative PPPM IBD medical costs in concurrent model were 51.5%, 89.8%, and 177.7% of the average for CD severity group 1, 2, and 3, respectively; the relative costs were 29.2%, 62.2%, and 142.1% of the average for UC severity group 1, 2, and 3, respectively ($R^2 = 0.21$). In the prospective model, the relative costs were 75.2%, 98.6%, and 114.0% of the average for CD severity group 1, 2, and 3, respectively; the relative costs were 11.2%, 62.6%, and 103.6% of the average for UC severity group 1, 2, and 3, respectively ($R^2 = 0.12$). In nonusers of targeted IBD therapy, the R^2 for the concurrent and prospective model was 0.02 and 0.07, respectively. Number of hospitalizations and duration of stay increased R^2 when included in concurrent models only (targeted IBD therapy users: 0.42; nonusers: 0.29). No clinical variable increased R^2 in the prospective models.

CONCLUSIONS: We have developed and evaluated the first IBD-specific clinical-risk groupers for commercially insured patients using medical claims data. The applications include risk adjustment for real-world evidence studies, disease state management programs, and capitated arrangement with provider groups.

SPONSORSHIP: Takeda Pharmaceuticals U.S.A., Inc.

L00-L99 Diseases of the Skin and Subcutaneous Tissue

(eg, eczema, psoriasis)

L12 Ruxolitinib cream utilization reduced other topical therapy, corticosteroid, and biologic use in patients with atopic dermatitis: US claims database analysis

Liu J[1], *Bui B*[2], *Odo N*[2], *Bancroft T*[2], *Sturm D*[1];
JinLiu@incyte.com
[1]*Incyte Corporation;* [2]*Optum*

BACKGROUND: Ruxolitinib cream has been shown to be an effective nonsteroidal topical monotherapy in clinical trials of patients with mild to moderate atopic dermatitis (AD). Initial data describing efficacy and safety in real-world settings have demonstrated consistency with clinical trial results.

OBJECTIVE: To characterize treatment patterns in the 6 months before and after initiating ruxolitinib cream for AD.

METHODS: This analysis of administrative claims data from the Optum Research Database included patients treated with ruxolitinib cream for AD between November 1, 2021, and September 30, 2022. The index date was the date of the first claim for ruxolitinib cream; baseline and follow-up periods were 6 months before and after the index date, respectively. Patients were required to have at least 1 medical claim for an AD diagnosis during the 6-month baseline period.

RESULTS: A total of 383 patients (aged ≥12 years) with AD and at least 1 claim for ruxolitinib cream were included. The mean (SD) number of fills of ruxolitinib cream in the 6-month follow-up period was 1.8 (1.3; range, 1-8). Patients had a mean (SD) age of 50.1 (21.4) years; 61.1% were female, and 53.3% were White. Most patients (61.9%) were on commercial plans, and 38.1% were on Medicare Advantage plans. Other topical and systemic therapy decreased after initiation of ruxolitinib cream, including topical corticosteroids (54.1% to 33.2%), topical calcineurin inhibitors (15.1% to 6.0%), topical phosphodiesterase-4 inhibitors (6.8% to 2.6%), and systemic corticosteroids (25.3% to 15.1%). The mean cumulative prednisone-equivalent dose for systemic corticosteroids decreased by 25% (107.1 to 80.7 mg). Of the 319 patients without biologic treatment during the baseline period, 93.1% avoided biologic treatment throughout the 6-month follow-up. Of the 64 patients with biologic treatment during the baseline period, 15.6% did not continue biologic therapy during the follow-up period.

CONCLUSIONS: Following initiation of ruxolitinib cream, there was a marked reduction in claims for other topical therapies and corticosteroids for AD in this analysis. Most biologic-naive patients (>90%) avoided biologics in the 6 months following ruxolitinib cream initiation; approximately 1 in 6 patients who had biologic treatment during baseline did not continue biologic use during follow-up. This analysis confirms previous findings from the Healthcare Integrated Research Database that short-term treatment with ruxolitinib cream may reduce the use of other topical therapies and corticosteroids and has the potential to prevent or decrease use of biologics in patients with AD.

SPONSORSHIP: Incyte Corporation

L13 Unveiling the burden and impact of flare in patients with moderate to severe atopic dermatitis: Results from the Adelphi Real World Disease Specific Programme in the United States

Gray C[1], *Lucats L*[2], *Brookes E*[3], *Rahawi K*[1], *Howell O*[4], *Haughton J*[4], *Anderson P*[4]; *cori.gray@sanofi.com*
[1]*Sanofi*; [2]*Sanofi*; [3]*Sanofi*; [4]*Adelphi Real World*

BACKGROUND: Atopic dermatitis (AD) is a chronic immune-mediated inflammatory skin disease characterized by inflamed, itchy skin with periods of worsening called "flares," which greatly impacts quality of life.

OBJECTIVE: To describe the characteristics, burden, and impact of flares in patients with moderate to severe AD from both the physician and patient perspective.

METHODS: This was a cross-sectional survey of physicians and their adult patients with moderate to severe AD in the United States using data from Adelphi AD Disease Specific Programme conducted between August 2022 and March 2023. Patients were classified as currently flaring or not flaring based on their physician assessment. Baseline demographics; frequency, duration, and severity of flares; associated signs, symptoms, and impacts; and patient-reported outcome measures were summarized using descriptive statistical analyses.

RESULTS: A total of 146 physicians completed patient record forms for 430 patients (mean age: 39.7 years; 50% male; 78% White; mean disease duration: 3.2 years). Based on physician report, 27% of patients were currently experiencing a flare, with the majority of flares classified as moderate (78%) or severe (20%). Eighty-five percent of patients (n/N = 49/58) self-reported having at least 2 flares in the past year (mean [SD] = 3.3 [2.32] flares/year), whereas physicians reported 69% of patients (n/N = 113/163) having at least 2 flares in the past year (mean [SD] = 2.6 [2.69] flares/year). The most common signs and symptoms experienced by greater than or equal to 80% of patients during the current flare episode as reported by both physicians and patients were itching, skin pain, scratching, dry skin, cracking/raw areas, scaling/scaly skin, and sleep disturbance. Swelling was also common in more than 80% patients when reported by physicians. Itch appeared to be the most severe symptom of current flare, with physicians characterizing this symptom as severe for 34% of patients and 53% of patients doing so themselves. Mean scores of EuroQol 5-Dimension Visual analog scale, Dermatology Life Quality Index, and Patient-oriented Eczema Measure in patients who were currently flaring were 67.2 (n = 27), 9.1 (n = 28), and 12.0 (n = 29), respectively, and 82.9 (n = 106), 4.1 (n = 106), and 5.7 (n = 105), respectively, in those who were not currently flaring.

CONCLUSIONS: The burden of flare and differences between patient and physician perception of flare identified in this research highlights the importance of developing new therapies that better address flare and defining patient-centered measures to assess flares in clinical trials and practice to advance patient care.

SPONSORSHIP: Sanofi

L14 Brand-to-brand nonmedical switching among interleukin-17 inhibitors or other biologics: Implications of a formulary change

Fendrick A[1], *Mittal M*[2], *Peng Y*[2], *Johns B*[2], *Holmes C*[2], *Liu Y*[3]; *amfen@med.umich.edu*; *liuyif@umkc.edu*
[1]*University of Michigan*; [2]*AbbVie Inc.*; [3]*University of Missouri-Kansas City School of Pharmacy*

BACKGROUND: In January 2021, a large pharmacy benefit management (PBM) organization under one group purchasing organization (GPO) changed preferred agents on its national formulary from one branded interleukin (IL)-17 inhibitor (TxA) to another (TxB). This change thereby prompted a nonmedical switch (NMS) for existing TxA patients.

OBJECTIVE: To evaluate the impact of this change on IL-17 inhibitor use among patients with psoriasis, psoriatic arthritis, or ankylosing spondylitis.

METHODS: Using PBM-specific data from the Symphony Health Analytics database, treatment patterns following the formulary change were assessed. Eligible patients were treated with TxA for at least 84 days (July to December 2020), with at least 1 day treated in December, and had no other biologic since initiation of TxA. Within the same GPO, 2 comparator groups were established: patients affected by the formulary change (PBM-1 group) and patients not affected (PBM-2 group). Outcomes were (1) the proportion of patients with a treatment disruption in the first 6 months

of 2021 (ie, discontinuation of TxA or NMS from TxA to TxB) in the PBM-1 and PBM-2 groups and (2) for the PBM-1 group only, medication-taking behaviors (ie, adherence, discontinuation or switching rates) were compared between patients who continued TxA vs NMS to TxB.

RESULTS: Baseline demographics were generally similar between PMB-1 (N = 1,703) and PMB-2 (N = 462) groups. In the 6 months after the formulary change (December 2020 to June 2021) TxA use decreased (17% to 10%) and TxB use increased (8% to 16%) for patients in the PBM-1 group. Patients in the PBM-1 vs PBM-2 group had significantly higher ($P < 0.001$) rates of NMS to TxB (19% vs 1%), switching to another biologic (8% vs 2%), and discontinuing treatment (27% vs 14%); hence, 46% and 83% of patients in the PBM-1 and PBM-2 groups remained on TxA, respectively. In the PBM-1 group, those with NMS to TxB vs those continuing TxA had significantly ($P < 0.05$) lower adherence rates (46% vs 63%) and higher rates of discontinuation (20% vs 14%) or switching (14% vs 6%). Among patients with NMS to TxB who subsequently switched treatment, 41% switched back to TxA. In the PBM-1 group, 36% of Black patients discontinued treatment after the formulary change, further widening health care disparity.

CONCLUSIONS: These data demonstrate the detrimental clinical and equity impacts of NMS, in that patients who did switch to TxB experienced greater treatment disruption, including worse adherence and higher rates of discontinuation or switching. Such policy changes should be carefully considered so as to not cause undue burden to patients.

SPONSORSHIP: AbbVie, Inc.

L15 Impact of a managed care pharmacist clinical management tool: Dupilumab oversupply identification and intervention outcomes

Friedlander N, Curtis K, Gleason P;
nicholas.friedlander@primetherapeutics.com
Prime Therapeutics

BACKGROUND: Typical pharmacy benefit design allows members to refill medications after 75% of their estimated on-hand supply is exhausted. Persistent medication refilling, such as dupilumab, at this threshold can result in excessive supply, increasing both member and payer cost. Dupilumab annual wholesale acquisition cost is ~$50,000. Providing managed care pharmacists (MCPs) with identified cases of oversupply from pharmacy claims data can assist in managing excessive on-hand drug supply (aka stockpiling).

OBJECTIVE: To reduce stockpiling and associated medication waste, we developed cumulative oversupply detection logic targeting dupilumab into an MCP tool designed to facilitate MCP outreach.

METHODS: From the 14.5 million commercially insured members enrolled in the MCP outreach program, 6 months of pharmacy claims history was used to quantify a member's on-hand drug supply and next anticipated fill date. Identification of excess supply cases of more than 28 days was conducted on a weekly basis, from April 2023 through April 2024 (assessment period), and cases were made available to MCPs through a web tool. Once oversupply was confirmed through claims history review, MCPs outreached to pharmacies, members, and/or other health professionals to confirm fill history and delay subsequent fills as appropriate, minimizing waste and generating payer and member savings. Notes regarding outreach and case review were documented for each reviewed case in the web tool. Payer savings were calculated and documented for each successful case based on drug cost per day, anticipated refill date without intervention, and actual refill date.

RESULTS: There were 29,943 unique dupilumab-using members, 1 in 500 members, with at least 1 paid dupilumab claim in the assessment period. Of these members, 536 cases of oversupply involving dupilumab were identified and reviewed by MCP. Three hundred five (56.9%) identified cases resulted in validated savings during the assessment period, generating a total of $1,049,032 in payer savings. An additional 98 (18.2%) cases are currently in progress, with estimated per-case savings of $4,470. Outreach was unsuccessful for 56 (10.4%) of reviewed cases, and 77 (14.4% cases were considered inactionable based on MCP review.

CONCLUSIONS: MCP outreach minimized dupilumab oversupply with more than $1M in documented savings. Additional factors that could help to minimize oversupply include adjustment in the benefit design threshold for refill allowance or institution of a maximum annual fill count to be dispensed annually per drug, per member.

SPONSORSHIP: Prime Therapeutics LLC

L16 Understanding the burden of flares in patients with moderate to severe atopic dermatitis: A targeted literature review

Gray C[1], Joseph J[2], Cholasamudram S[2]; cori.gray@sanofi.com
[1]Sanofi; [2]Sanofi, Health Economics & Value Assessment

BACKGROUND: Atopic dermatitis (AD) is a chronic, immune-mediated, inflammatory condition of the skin, characterized by intense pruritus and recurrent eczematous lesions. Patients with AD experience flares associated with worsening signs and symptoms, often requiring intensification of treatment.

OBJECTIVE: To summarize the clinical, humanistic, and economic burden of flares in adult patients with moderate to severe AD.

METHODS: A targeted literature review of English-language publications was conducted using Embase and Medline on January 26, 2024. Additionally, American Academy of Dermatology and European Academy of Dermatology and Venereology conference websites were reviewed to supplement database searches. Real-world evidence studies including patients with moderate to severe AD (≥60% of study cohort) aged 12 years and older were included. Outcomes of interest encompassed proportions of patients experiencing flares; frequency and duration of flares; and flare-associated health care resource utilization (HCRU), costs, and impact on quality of life (QoL).

RESULTS: A total of 26 studies (United States: 8; Europe: 12; others: 6) were included in the review. The definition of flares differed across studies. Proportion of patients currently experiencing flares ranged from 36.4% to 58.4%. Average number of flares over the past year ranged from 2.1 to 11.3, with duration ranging from 1 day to greater than or equal to 2 months. The frequency of flares was higher among patients with severe AD compared with moderate AD. Although a reduction is seen among patients treated with a systemic therapy, some patients still continue to experience flares. The proportion of patients with AD experiencing frequent severe flares was higher for those with a Dermatology Life Quality Index score greater than 10 than for those with a score less than or equal to 10, with frequent severe flares as a strong predictor of AD-related QoL. The mean Patient-Oriented Eczema Measure, Dermatology Life Quality Index, peak pruritis numeric rating scale, and Work Productivity and Activity Impairment scores were worse for currently flaring patients than those who were not flaring. An increase in median out-of-pocket costs was observed with an increase in flare days. Higher HCRU was observed in patients with AD with flares than in those without flares, such as days off from regular activities (nonwork), health care provider office visits, and sick leaves.

CONCLUSIONS: This literature review found that flares in patients with moderate to severe AD are associated with a higher clinical, humanistic, and economic burden highlighting the need for treatments that prevent or reduce flares in this patient population.

SPONSORSHIP: Sanofi

L18 Tapinarof cream 1% once daily: Real-world clinical experience and patient satisfaction in adults with plaque psoriasis

Fredericks D[1], *Fournier J*[1], *Bin Sawad A*[2], *Leiras C*[3], *Gomez Rey G*[4], *Vazquez M*[2], *Tallman A*[1]; *doral.fredericks@dermavant.com*; *janine.fournier@dermavant.com*
[1]*Dermavant Sciences, Inc.*; [2]*Formerly of Dermavant Sciences, Inc.*; [3]*Genesis Research Group*; [4]*Formerly of Genesis Research Group*

BACKGROUND: Psoriasis is an inflammatory skin disease that substantially impacts quality of life. Key treatment goals include symptom relief and achieving/maintaining clear skin. More than 50% of patients may be dissatisfied with their current psoriasis treatment. Dissatisfaction with topical therapies and frequent application may lead to low adherence, medication switching, and poor outcomes. In the PSOARING phase 3 program, tapinarof cream 1% (VTAMA, Dermavant Sciences, Inc.) once daily was efficacious and well tolerated in adults with plaque psoriasis, and patients had highly positive perceptions of tapinarof.

OBJECTIVE: To assess real-world experience and patient satisfaction with tapinarof cream in adults with plaque psoriasis.

METHODS: An online survey was conducted based on a US proprietary patient panel. Eligible adults (aged ≥18 years) had plaque psoriasis and had used tapinarof for at least 1 month. Body surface area affected was estimated using number of palm-sized areas (<3, 3-10, or >10). The survey included 28 questions related to demographics, plaque psoriasis treatment experience prior to tapinarof, and satisfaction with tapinarof.

RESULTS: A total of 354 participants completed the survey; 51.4% were male, mean age was 39.8 years (range 18-80), and 76.3% were White. Mean duration of psoriasis was 6.4 years. Body surface area affected was as follows: less than 3 palm-sized areas, 18.6%; 3-10, 64.7%; and more than 10, 16.7%. Prior to tapinarof, the proportions who had tried other therapies were as follows: topicals, 92.1%; orals, 63.0%; biologics, 32.2%; and other treatments, 31.4%. Most participants (84.7%) discontinued other topicals when tapinarof was initiated. Tapinarof relieved participants' psoriasis symptoms, including itching (80.2%), flaking (57.3%), and scaling (52.5%). A high proportion strongly agreed or agreed that they could easily manage their psoriasis with tapinarof (82.8%); were satisfied with how well tapinarof worked (81.4%); were satisfied with time spent applying tapinarof (86.4%); and considered tapinarof easy to apply (88.7%), not greasy (71.5%), and quickly absorbed into the skin (80.5%).

CONCLUSIONS: In this real-world survey, most participants considered tapinarof cream 1% once daily to be an effective treatment for their plaque psoriasis. High rates of satisfaction and positive perceptions of tapinarof were consistent with findings from the PSOARING program. The efficacy of tapinarof together with its optimized formulation properties and high treatment satisfaction may lead to improved adherence and favorable outcomes for patients with plaque psoriasis.

SPONSORSHIP: Dermavant Sciences, Inc.

L19 Treatment utilization and switching patterns among patients in the United States with psoriatic arthritis initiating biologics

Batech M[1], *Coakley C*[1], *Song C*[2], *Novatski J*[2]; *michael.batech@aetion.com*; *chao.song@ucb.com*
[1]*Aetion, Inc.*; [2]*UCB Pharma*

BACKGROUND: Psoriatic arthritis (PsA) is a heterogeneous inflammatory disease treated with different biologics. Biologic treatments and switching patterns among patients with PsA may change over time.

OBJECTIVE: To described treatment utilization and switching patterns within 1 year of biologic initiation in patients with PsA.

METHODS: MarketScan claims data (January 1, 2015, to October 31, 2022) were analyzed for patients (aged 18-65 years) in the United States with at least 1 claim for PsA diagnosis on their index date. Index was the date that patients initiated a new treatment in one of the following classes: interleukin (IL)-17 inhibitor (i), IL-23i, tumor necrosis factor (TNF)i, IL-12/23i, and targeted synthetic disease-modifying antirheumatic drug. To evaluate treatment utilization, 2 cohorts were considered (2015-2017 and 2018-2022), based on the years IL-17i and IL-23i were approved by the US Food and Drug Administration, respectively. To evaluate switching patterns, patients were followed less than or equal to 1 year after initiation; the number who switched to another treatment within or outside a biologic class was measured.

RESULTS: Among patients with new treatments, new IL-17i users increased from 1,183 (9.0%) in 2015-2017 (N = 13,118) to 2,566 (23.5%) in 2018-2022 (N = 10,938). Of these, 829 (70.1%) and 1,560 (60.8%) had prior exposure to at least 1 unique biologic class at baseline in 2015-2017 and 2018-2022, respectively. From 2015-2017 to 2018-2022, the proportion of new TNFi users decreased (55.4% [n = 7,269] to 41.9% [n = 4,581]). Of 35,950 patients with an initial treatment, 3,467 (9.6%) used an IL-17i, with 2,487 and 958 patients on secukinumab (SEC) and ixekizumab (IXE), respectively; 23,498 (65.4%) used a TNFi. A total of 3,253 (9.0%) patients switched to another treatment during follow-up. Patients most frequently switched to adalimumab (22.5%), etanercept (19.7%), or SEC (15.0%). Among IL-17i users, 314 (9.1%) switched; 72 (22.9%) switched within the IL-17i class and 242 (77.1%) patients switched out of the IL-17i class. Of 226 and 88 patients who switched from SEC and IXE, respectively, most (SEC: n = 179 [79.2%]; IXE: n = 63 [71.6%]) switched out of class. Among TNFi users, 2,058 (8.8%) switched; 481 (23.4%) switched to IL-17i, consisting of 335 (69.6%) and 144 (29.9%) patients switching to SEC and IXE, respectively.

CONCLUSIONS: Among patients with PsA who initiated treatment from 2015-2017 to 2018-2022, IL-17i use increased over time, whereas TNFi use decreased. TNFi remained the most used biologic class. Treatment switching within 1 year after initiation among IL-17i users was infrequent.

SPONSORSHIP: UCB Pharma; medical writing support: Costello Medical.

M00-M99 Diseases of the Musculoskeletal System and Connective Tissue

(eg, osteoarthritis, osteoporosis, rheumatoid arthritis)

M4 Real-world switching patterns for patients with psoriatic arthritis on first-line advanced therapies

Walsh J[1], *Wu J*[2], *Ye X*[3], *Patel M*[4], *Li C*[4], *Saffore C*[4], *Vora J*[4], *Ogdie A*[5]; *Jessica.Walsh@hsc.utah.edu*; *xiaolan.ye@abbvie.com*
[1]*University of Utah*; [2]*Dermatology Research and Education Foundation*; [3]*AbbVie Inc*; [4]*AbbVie Inc.*; [5]*University of Pennsylvania*

BACKGROUND: Advanced therapies such as interleukin (IL)-23, IL-12/23, IL-17, phosphodiesterase-4 (PDE-4), and tumor necrosis factor (TNF) inhibitors (i) have been approved for first-line treatment of active psoriatic arthritis (PsA). Treatment goals for PsA include achieving the lowest possible disease activity, optimizing functional status, and avoiding treatment complications. A treatment switch is recommended when those goals are unmet.

OBJECTIVE: To evaluate the real-world switching patterns among patients with PsA taking first-line advanced therapies (1LAT) over 12 months.

METHODS: Adults with at least 1 PsA diagnosis in baseline who initiated a new 1LAT between January 21, 2022, and January 31, 2023, were identified using the Merative Marketscan databases covering January 1, 2016, to January 31, 2024.

Patients had at least 6 months of continuous enrollment pre-index date (baseline) and at least 12 months of continuous enrollment post-index date. Switching was defined as the proportion of patients who switched to a new advanced therapy in the 12-month follow-up after treatment initiation. Multivariate logistic regression was used to compare switching when accounting for differences in baseline characteristics. Switching was evaluated for the overall population, stratified by the mechanism of action (MOA; reference IL-23i) and individual drugs (reference: risankizumab [RZB]).

RESULTS: A total of 1,309 patients were included in the analysis. Baseline characteristics were similar between MOAs. Overall, 24.2% of patients switched therapies over 12 months. Switching was least common in patients initiating IL-23i (8.1%) compared with TNFi (31.5%), IL-17i (19.9%), and PDE-4i (26.9%). Compared with IL-23i, the odds of switching were 5.22 (95% CI = 3.15-8.67), 4.11 (95% CI = 2.43-6.96), and 2.75 (95% CI = 1.53-4.96) for TNFi, PDE-4i, and IL-17i, respectively (all $P < 0.001$). RZB was associated with significantly lower proportion of switchers (3.8%) than guselkumab (12.9%), followed by secukinumab (18.8%), ixekizumab (20.8%), apremilast (26.9%), etanercept (30.0%), and adalimumab (32.4%) (all $P < 0.05$). Compared with RZB, the odds of treatment switch were 11.87 (95% CI = 4.68-30.11), 10.77 (95% CI = 3.91-29.68), 8.87 (95% CI = 3.48-22.56), 6.46 (95% CI = 2.33-17.91), 5.52 (95% CI = 1.91-15.94), and 3.79 (95% CI = 1.33-10.82) for adalimumab, etanercept, apremilast, ixekizumab, secukinumab, and guselkumab, respectively (all $P < 0.05$).

CONCLUSIONS: The proportion of patients with PsA taking 1LAT who switched over 12 months was the lowest with IL-23i. At the individual drug level, RZB was associated with the lowest odds of switching.

SPONSORSHIP: AbbVie Inc.

M5 Real-world dose escalation of biologic treatments for psoriatic arthritis

Walsh J[1], *Ye X*[2], *Peng Y*[3], *Patel M*[3], *Clewell J*[3], *Wu J*[4]; *Jessica.Walsh@hsc.utah.edu*; *xiaolan.ye@abbvie.com*
[1]*University of Utah*; [2]*AbbVie Inc*; [3]*AbbVie Inc.*;
[4]*Dermatology Research and Education Foundation*

BACKGROUND: Biologics such as interleukin (IL)-23, IL-12/23, IL-17, or tumor necrosis factor (TNF) inhibitors (i) are used to treat psoriatic arthritis (PsA). Dose escalation (DE) may be required for patients to achieve treatment goals. However, there is limited real-world evidence on DE in patients with PsA using recently approved biologics.

OBJECTIVE: To compare DE of patients with PsA on risankizumab (RZB) with other biologics in the maintenance dosing period.

METHODS: The Merative MarketScan databases identified adult patients with PsA with at least 3 maintenance claims of the index biologic medication from January 21, 2022, to November 30, 2023. Patients had at least 6 months of continuous enrollment pre-induction and at least 6 months of post-start of the maintenance dose. DE was defined as at least 2 dosing intervals during the maintenance period where the average daily dose (total strength [milligrams] divided by days between subsequent doses) was greater than or equal to 30% higher than the expected daily dose (per US Food and Drug Administration–approved dosing). DE was evaluated for the full maintenance period (until the end of treatment duration and/or continuous enrollment) and the first 6 and 12 months of the maintenance period. Comparisons in DE between RZB and other cohorts were made using chi-square tests. Logistic regressions adjusting for age, sex, region, Charlson Comorbidity Index, and previous biologic use were also conducted.

RESULTS: Overall, 1,558 patients with PsA were included in the analysis. Baseline characteristics were similar across treatment groups. During the full maintenance period, DE rate was significantly lower for RZB (9.6%) vs TNFi (21.0%), IL-12/23i (58.1%), and IL-17i (57.6%) (all $P < 0.01$) and numerically lower vs other IL-23i (14.9%; $P = 0.11$). The proportion of RZB patients with DE was significantly lower than that for patients receiving all other biologics (9.6% vs 33.0%, $P < 0.0001$). Similar trends were observed for the first 6 and 12 months of maintenance treatment. Results were consistent after adjusting for baseline characteristics. Compared with RZB, adjusted odds ratios were 2.63 for TNFi, 13.18 for IL-12/23i, 12.39 for IL-17i (all $P < 0.01$), and 1.56 for other IL-23i ($P = 0.16$). In addition, at the drug level, DE rate was significantly lower for RZB when compared with adalimumab (24.4%), ixekizumab (39.8%), secukinumab (78.9%) and ustekinumab (58.1%) (all $P < 0.01$) and numerically lower vs etanercept (13.8%; $P = 0.27$) and guselkumab (14.9%; $P = 0.16$).

CONCLUSIONS: A significantly lower proportion of RZB-treated patients with PsA had dose escalations compared with patients receiving other biologics, suggesting reliable dosing among RZB-treated patients.

SPONSORSHIP: AbbVie Inc.

M9 Treatment utilization and switching patterns among bio-naive and bio-experienced patients with axial spondyloarthritis initiating biologics in the United States

Batech M[1], *Coakley C*[1], *Song C*[2], *Novatski J*[2]; *michael.batech@aetion.com*; *jeannette.novatski@ucb.com*
[1]*Aetion, Inc.*; [2]*UCB Pharma*

BACKGROUND: Axial spondyloarthritis (axSpA) is a chronic immune-mediated inflammatory disease affecting the sacroiliac joints and spine. Tumor necrosis factor (TNF)-α, interleukin (IL)-17, and IL-23 are the major proinflammatory cytokine pathways in axSpA; biologics have been effective in treating axSpA.

OBJECTIVE: This study aimed to describe annual biologic utilization patterns and frequency of initial treatment switching among patients with axSpA in the United States.

METHODS: Retrospective data for patients with axSpA were extracted from MarketScan (January 1, 2015, to October 31, 2022). Eligible patients (aged 18-65 years) had at least 1 claim with axSpA diagnosis on the biologic initiation date. Index was the date that patients initiated a new treatment within one of the following classes: TNF inhibitor (i), IL-17i, IL-23i, IL-12/23i, and targeted synthetic disease-modifying antirheumatic drug (tsDMARD). Annual biologic utilization was evaluated as the proportion of patients (n [%]) using each treatment and class. Biologic switching patterns were assessed less than or equal to 1 year after index as the number of patients who switched to another treatment within or outside the same class.

RESULTS: Among patients with axSpA, TNFi was the most utilized medication class from 2015 to 2022, with adalimumab as the most frequently used. The second highest used biologic class until 2018 was tsDMARD, after which this was surpassed by IL-17i. IL-17i utilization increased from 20 (<0.1%) patients in 2015 to 1,023 (1.0%) patients in 2022, with secukinumab as the most commonly used IL-17i. After 2015, IL-23i and IL-12/23i were the least common treatment classes. Of patients with an index treatment (n = 16,752), 1,223 (7.3%) switched within 1 year to another treatment within or outside the same class. From 2015 to 2022, apremilast had the highest proportion of patients who switched within 1 year (n = 50 [9.7%]), followed by ixekizumab (n = 16 [9.2%]). Among patients initiating a TNFi (n = 14,476), IL-17i (n = 817), IL-23i (n = 69), IL-12/23i (n = 487), or tsDMARD (n = 903) at index, 255 (1.8%), 57 (7.0%), 4 (5.8%), 17 (3.5%), and 73 (8.1%) patients switched to a different class, respectively, whereas 803 (5.5%), 11 (1.3%), 1 (1.4%), 0, and 2 (0.2%) patients switched to another biologic treatment in the same class.

CONCLUSIONS: Although TNFi was the most common medication class among patients with axSpA in the United States from 2015 to 2022, use of IL-17i biologics increased over time. The proportion of patients switching treatments within 1 year after treatment initiation was low across biologic classes.

SPONSORSHIP: UCB Pharma; medical writing support: Costello Medical

M17 Influence of clinical scenarios on cost-effectiveness model results for biosimilar denosumab in women with postmenopausal osteoporosis

Flanigan J[1], *Kane S*[2], *Fashami F*[2], *Zhou A*[2], *Almuallem L*[3], *Tindal M*[1], *Schauf M*[4], *Lyman G*[5]; *jeanine.flanigan_ext@sandoz.com*
[1]*Sandoz*; [2]EVERSANA *Value & Evidence*; [3]EVERSANA; [4]*Sandoz International GmbH*; [5]*Fred Hutchinson Cancer Center*

BACKGROUND: Denosumab is an initial and effective therapy for treating osteoporosis in postmenopausal women with high risk of fracture. Previous economic evaluations assessed the cost-effectiveness (CE) of reference denosumab compared with bisphosphonates; the availability of biosimilar denosumab may improve its CE vs bisphosphonates. However, published denosumab CE studies apply different scenarios to their respective models, leading to varying results. Therefore, these scenarios must be carefully considered when building a contemporary CE model for biosimilar denosumab.

OBJECTIVE: To evaluate the influence of various clinical scenarios on the overall results of a CE model for biosimilar denosumab.

METHODS: A Markov model was developed to compare costs and clinical outcomes of biosimilar denosumab vs bisphosphonates for postmenopausal osteoporosis. Model inputs, including rates of hip and vertebral fractures, were sourced from publicly available data and published literature. The incremental CE ratio (ICER) was calculated per quality-adjusted life-year (QALY) gained from a US payer perspective. Multiple scenario analyses were conducted to evaluate the impact to the ICER of modifying variables associated with uncertainty.

RESULTS: Four scenarios that influenced the CE of biosimilar denosumab were drug adherence, the mix of therapies administered as subsequent treatment, the wrist fracture efficacy data source, and treatment duration for all therapies. In the model reference case, adherence was excluded, subsequent therapy consisted solely of alendronate, wrist fracture efficacy was informed by a 2016 study of 84,070 patients, and treatment duration was 10 years, resulting in an ICER of

$101,017 per QALY for biosimilar denosumab vs alendronate. Modifying each variable independently impacted the CE for biosimilar denosumab vs alendronate as follows: including adherence increased the ICER to $103,122, including a mix of bisphosphonates for subsequent treatment increased the ICER to $106,519, using a 2013 study with 73,464 participants as an alternate wrist fracture efficacy data source increased the ICER to $106,909, and shortening the treatment duration to 5 years decreased the ICER to $64,123.

CONCLUSIONS: Because different scenarios influence the final CE results, the final model should reflect realistic treatment practices as garnered from other published economic models. This evaluation provides transparency in economic model development, in alignment with ISPOR Good Practices.

SPONSORSHIP: Sandoz, Inc.

M21 Health care resource utilization among patients with hypophosphatasia treated with Strensiq (asfotase alfa)

Lyons G[1], *Bates T*[1], *Vlasnik J*[1], *Wakeford C*[1], *Donckels E*[2], *Chan P*[2], *Robinson S*[2], *Dahir K*[3]; *genevieve.lyons@alexion.com*; *jon.vlasnik@alexion.com*
[1]*Alexion Pharmaceuticals, Inc.*; [2]*Inovalon*; [3]*Vanderbilt University Medical Center*

BACKGROUND: Hypophosphatasia (HPP) is a rare, inherited, systemic disease characterized by bone mineralization defects, fragility fractures, and systemic manifestations. Asfotase alfa (AA), an enzyme replacement therapy, is the only US Food and Drug Administration–approved treatment for perinatal/infantile- and juvenile-onset HPP. Real-world data are lacking on health care resource utilization (HCRU) in patients treated with AA.

OBJECTIVE: To determine HCRU using a US claims database among patients with HPP after AA initiation.

METHODS: A retrospective analysis of patients with HPP was conducted using the Inovalon MORE2 closed claims and Medicare fee-for-service databases from January 2016 to December 2022. Patients (aged ≥2 years) treated with AA (at least 1 pharmacy claim for AA between January 1, 2017, and December 31, 2021 [identification period]) and with at least 12 months of continuous enrollment with medical and pharmacy coverage before and after AA initiation were included. All-cause and fracture-related HCRU and expenditure were assessed. Statistical testing was performed using Wilcoxon signed rank and paired t-tests as appropriate.

RESULTS: A total of 149 patients (65.1% female, mean age 39.6 years) met the inclusion criteria; 69.1% were adults aged 18 years and older (mean age 53.8 years). During baseline, HCRU included imaging (81.9% of patients), pain management–related visits (70.5%), mental/behavioral health visits (43.0%), physical/occupational therapy visits (41.6%), emergency department (ED) visits (36.9%), and physical medicine and rehabilitation visits (17.4%). Fewer patients had a fracture-related pain management visit from pre- to post-AA initiation (18 vs <11; $P = 0.033$). The mean per-patient per-year (PPPY) pre- and post-AA initiation costs for all-cause ED visits were comparable ($1,250 vs $932; $P = 0.292$). The mean PPPY costs significantly decreased from pre- to post-AA initiation for fracture-related outpatient care ($228 vs $133; $P = 0.0496$).

CONCLUSIONS: The number of patients with fracture-related pain visits and fracture-associated costs decreased after AA initiation, highlighting the potential benefits of this treatment in patients with HPP.

SPONSORSHIP: Alexion, AstraZeneca Rare Disease

M22 Real-world treatment patterns of phosphorodiamidate morpholino oligomer therapies in patients with Duchenne muscular dystrophy: An administrative claims–based analysis

Grabich S[1], *Ung B*[1], *Nadkar A*[1], *DeYoung K*[1], *Khachatourian A*[2], *Signorovitch J*[3]; *sgrabich@sarepta.com*
[1]*Sarepta Therapeutics, Inc.*; [2]*Sarepta Therapeutics, Inc.*; [3]*Analysis Group*

BACKGROUND: Data on treatment patterns of phosphorodiamidate morpholino oligomers (PMOs) in patients with Duchenne muscular dystrophy (DMD) outside of clinical trials are limited.

OBJECTIVE: To assess PMO treatment patterns in administrative claims data, characterizing continuous claims coverage as well as the frequency and timing of claims after gaps.

METHODS: Data from June 1, 2016, to September 30, 2023, were analyzed using Inovalon closed claims data, including private and public administrative retrospective claims. The index date was defined as the first medical or pharmacy PMO therapy claim (eteplirsen, golodirsen, viltolarsen, casimersen). Primary analyses evaluated claims within 1 year of continuous enrollment from index. Secondary analyses used all available follow-up. Outcomes included continuous claims coverage (defined by days supply plus 60/30-day allowance through follow-up) and at least 1 subsequent claims after a gap.

RESULTS: A total of 315 male patients were included in the primary analysis, with a mean (SD) age of 12.6 (5.99) years at index: eteplirsen, n = 178 (56.5%); golodirsen, n = 32 (10.2%); viltolarsen, n = 17 (5.4%); casimersen, n = 88 (27.9%).

Continuous claims coverage on therapy at the end of 1-year follow-up occurred in 208 (66.0%) and 165 (52.4%) patients using the 60- and 30-day claim gap definition, respectively. Of the 107 patients who had a 60-day gap in claims, 60 (56.1%) had a subsequent claim after the gap within a mean (SD) time of 66.7 (65.36) days; 108/150 (72.0%) patients with a 30-day gap had a subsequent claim after the gap within a mean (SD) time of 54.0 (64.56) days. At least 85% of patients, regardless of gap definition, had either continuous claims coverage or at least 1 subsequent claim after a gap over 1-year follow-up.

CONCLUSIONS: These real-world data demonstrate that most patients with DMD included in this analysis had continuous claims coverage or subsequent claims after a claim gap of PMO therapy through 1 year of follow-up. Analyses of gaps in claims coverage should consider factors such as patient access, insurance reauthorization periods, and change in coverage. More than half of patients with a 30- or 60-day gap in claims had a subsequent PMO claim, exemplifying the challenge for characterizing treatment patterns using claims data alone. Additional data sources and methodologies could offer deeper insights into actual treatment patterns in real-world settings.

SPONSORSHIP: This study was funded by Sarepta Therapeutics, Inc.

M23 Work, productivity, and activity impairment for caregivers of adults with Duchenne muscular dystrophy and other conditions in the United States: Combined real-world evidence and literature review

Strober J[1], *Posner N*[2], *Cappelleri J*[3], *Talaga A*[2], *Aslam Z*[2], *Dukacz S*[2], *Morton E*[4], *Iqbal H*[4], *Chatterton E*[4], *DeCourcy J*[4]; *Jonathan.Strober@ucsf.edu*; *Nathaniel.Posner@pfizer.com*
[1]*UCSF Benioff Children's Hospital*; [2]*Pfizer Inc.*; [3]*Pfizer Inc*; [4]*Adelphi Real World*

BACKGROUND: Duchenne muscular dystrophy (DMD) is a genetic disorder associated with progressive muscle degeneration due to a lack of dystrophin. DMD primarily affects young male individuals, who require increasingly intensive caregiver support as they progress into adulthood.

OBJECTIVE: To evaluate how caring for someone with DMD affects work productivity, especially when compared with caregiving for people with other conditions, or with the productivity of noncaregivers.

METHODS: Data were drawn from the Adelphi Real World DMD Disease Specific Programme, a cross-sectional survey with retrospective data collection of caregivers conducted in the United States from October 2022 to November 2023. Caregivers voluntarily completed a survey containing questions on demographics and the Work, Productivity, and Activity Impairment questionnaire. A literature search identified a study from the 2013 US National Health and Wellness Survey, from which family caregiver and noncaregiver Work, Productivity, and Activity Impairment data were analyzed. Caregivers were caring for at least 1 adult (aged 18 years and older) relative, and noncaregivers were not caring for any adult relatives. Analyses were descriptive.

RESULTS: Data were reported by 27 caregivers of adult (aged 18 years and older) male patients with DMD. Mean (SD) caregiver age was 51.0 (4.9) years, 78% were female, 89% were caring for their child, and 63% were employed full- or part-time. Mean (SD) patient age was 21.1 (3.3) years and 93% were nonambulatory. Caregiver-reported absenteeism, presenteeism, and overall work productivity loss (n = 17) were 8.5%, 35.3%, and 38.9%, respectively. Caregiver-reported activity impairment (n = 27) was 49.3%. The US study reported caregiver vs noncaregiver absenteeism (8.2% vs 3.6%), presenteeism (24.1% vs 13.6%), overall work productivity loss (27.4% vs 15.7%), and activity impairment (27.1% vs 15.8%), respectively.

CONCLUSIONS: These data highlight the burden that caregiving for adults with DMD has on employment and daily life in the United States. There is potential for this burden to be reduced with the provision of better support for the caregivers of adults with DMD and treatments that effectively control DMD symptoms.

SPONSORSHIP: None

M24 Work, productivity, and activity impairment for caregivers of pediatric patients with Duchenne muscular dystrophy and other conditions in the United States: Combined real-world evidence and literature review

Strober J[1], *Posner N*[2], *Cappelleri J*[3], *Talaga A*[2], *Aslam Z*[2], *Dukacz S*[2], *Morton E*[4], *Iqbal H*[4], *Chatterton E*[4], *DeCourcy J*[4]; *Jonathan.Strober@ucsf.edu*; *Nathaniel.Posner@pfizer.com*
[1]*UCSF Benioff Children's Hospital*; [2]*Pfizer Inc.*; [3]*Pfizer Inc*; [4]*Adelphi Real World*

BACKGROUND: Duchenne muscular dystrophy (DMD) is a genetic disorder associated with progressive muscle degeneration due to a lack of dystrophin.

OBJECTIVE: DMD caregiver employment has been investigated for pediatric patients; however, there is a lack of descriptive analyses comparing this with other diseases involving pediatric patients, which we aim to explore.

METHODS: Data were drawn from the Adelphi Real World DMD Disease Specific Programme, a cross-sectional survey

with retrospective data collection of caregivers in the United States from October 2022 to November 2023. Caregivers voluntarily completed a survey containing questions on demographics and the Work, Productivity, and Activity Impairment (WPAI) questionnaire. A literature review was conducted to identify multiple sources assessing caregiver WPAI of pediatric patients in the United States. Analyses were descriptive.

RESULTS: Data were reported by 65 caregivers of pediatric (aged 17 years and younger) male patients with DMD. Mean (SD) caregiver age was 40.0 (6.6) years, 89% were female, and 63% were employed full- or part-time. Mean (SD) patient age was 10.5 (4.2) years and 69% were ambulatory. Caregiver-reported absenteeism (n = 43), presenteeism (n = 43), and overall work productivity loss (n = 42) were 12.8%, 35.6%, and 42.2%, respectively. Caregiver-reported activity impairment (n = 65) was 40.9%. Previous literature surrounding caregiver WPAI data for pediatric patients with primary hyperoxaluria reported absenteeism (7.2%), presenteeism (27.8%), overall work impairment (31.1%), and activity impairment (30.0%). Caregivers of pediatric patients with uncontrolled asthma reported absenteeism (4.5%), presenteeism (12.7%), overall work impairment (16.9%), and activity impairment (19.1%). Moreover, caregivers of pediatric patients with neurofibromatosis type 1 and plexiform neurofibroma reported absenteeism (6.9%), presenteeism (17.3%), overall work impairment (22.3%), and activity impairment (17.2%).

CONCLUSIONS: Caregivers of pediatric male patients with DMD reported high impairment across all 4 WPAI domains. This highlights the impact to caregivers when balancing their caregiving requirements with employment and personal activities, indicating an unmet need for better support for caregivers and treatments that effectively control DMD symptoms.

SPONSORSHIP: None

N00-N99 Diseases of the Genitourinary System

(eg, chronic kidney disease)

N3 Impact of glucagon-like peptide-1 receptor agonists on renal outcomes among patients with diabetes with chronic kidney disease: A logistic regression using real-world data

Strand M, Watanabe J; mwstrand@uci.edu
Center for Data-Driven Drugs Research and Policy

BACKGROUND: Recent clinical studies have recognized potential renal benefits associated with the use of glucagon-like peptide-1 receptor agonists (GLP-1 RAs) for patients with diabetic kidney disease including several related to chronic kidney disease (CKD) progression. However, limited real-world data have been available to study this impact among a general population of patients with diabetes.

OBJECTIVE: To estimate the odds for new GLP-1 RA users with diabetes with CKD of progression to incident end-stage renal disease (ESRD) as well as a reduction of estimated glomerular filtration rate (eGFR) greater than 50%.

METHODS: We used a logistic regression model to estimate the odds of a negative renal outcome during the first 2 years for new GLP-1 RAs users compared with new dipeptidyl peptidase-4 inhibitor users. Patients were considered "new users" if they had at least 3 refills or a proportion of days covered greater than 0.8 during their first 3 months of drug use. Adjustments were made for race, sex, ethnicity, obesity, and stage of CKD at the time of their first prescription.

RESULTS: Our study included 3,340 total patients with 1,491 in the exposure group. Of the GLP-1 RA patients, 58 developed ESRD and 3 experienced a decrease in eGFR greater than 50% during the first 2-year period since initializing. Adjusted odds ratios for developing ESRD of 0.57 (95% CI = 0.40-0.81) and 0.22 (95% CI = 0.05-0.75) of experiencing a decrease in eGFR greater than 50% were observed for the GLP-1 RA patients when compared with new dipeptidyl peptidase-4 users.

CONCLUSIONS: These results including real-world data from a diverse population lend significant observational evidence to the findings of other studies and support the notion that GLP-1 RAs may be linked to improved renal outcomes. Further randomized studies are warranted and necessary to confirm a causal relationship.

SPONSORSHIP: None

N5 Real-world treatment patterns of metastatic urothelial cancer and utilization of sacituzumab govitecan in the United States

Wang X[1], *Chin A*[2], *Popadic L*[2], *Devine F*[2], *Poretta T*[1], *Schmidt K*[1], *Sierecki M*[1], *Hoffman-Censits J*[3]; *xue.wang@gilead.com*
[1]*Gilead Sciences, Inc*; [2]*Komodo Health*; [3]*Department of Medical Oncology & Department of Urology, The Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins, Johns Hopkins Greenberg Bladder Cancer Institute*

BACKGROUND: Despite the availability of platinum-based chemotherapy (PT) for metastatic urothelial cancer (mUC), prognosis remains poor. US Food and Drug Administration approvals of immunotherapy (IO) and antibody drug conjugates such as enfortumab vedotin (EV) and sacituzumab govitecan (SG) in recent years have transformed the treatment landscape, with improved clinical outcomes.

OBJECTIVE: To examine real-world treatment patterns in mUC by line of therapy and to assess SG use.

METHODS: This retrospective cohort analysis used open and closed administrative claims data from Komodo's Healthcare Map. Adult patients with at least 2 UC diagnostic claims and at least 2 secondary neoplasm claims within 30 days of the first UC claim, between January 1, 2016, and September 30, 2023, were selected. Index date was the earliest date of a secondary neoplasm claim after UC diagnosis on or after January 1, 2017. In closed claims, patients were required to have continuous enrollment during the 6-month baseline period; patients in open claims were required to have at least the median number of claims among all patients in open claims. Patients were observed until death or end of study, whichever occurred first. Treatment patterns were examined in patients who initiated first-line (1L) treatment on or after January 1, 2020. SG utilization patterns including granulocyte-colony stimulating factor (G-CSF) use were assessed. A sensitivity analysis was conducted in closed claims alone.

RESULTS: In this analysis of 35,028 patients with mUC, median age was 75 years; 76% were male; 79% were Medicare recipients; and 65% had de novo mUC. PT with/without IO maintenance was the most common 1L regimen (50%), whereas IO-based (36%) and EV (21%) were the dominant 2L regimens. In 3L, IO (25%), EV (19%), and SG (17%) were most commonly used. Of the 359 SG-treated patients, 28% received SG in 2L with a median of 5.5 cycles; those who received SG in 3L+ had a median of 4 cycles. G-CSF was used in 41% of SG patients, of whom 79% in 2L and 63% in 3L+ initiated G-CSF during cycle 1 (≤21 days). Sensitivity analysis in closed claims data showed similar findings.

CONCLUSIONS: Although PT with/without IO maintenance was the predominant 1L treatment in the study period, the mUC treatment landscape continues to evolve as EV+pembrolizumab becomes a 1L standard of care. Further studies are needed to assess the evolving treatment patterns in mUC and the optimal sequence of therapeutic agents, to achieve the best possible outcomes for patients.

SPONSORSHIP: Gilead Sciences, Inc.

N6 Health care resource utilization and direct medical costs among patients with metastatic urothelial carcinoma receiving first-line systemic treatment and avelumab first-line maintenance: Results from the IMPACT UC-III study

Ike C[1], *Wangia Dixon R*[2], *Crowe C*[2], *Venkataraman M*[2], *Moon H*[3], *Morris V*[1], *Kearney M*[4], *Tonnu-Mihara I*[2], *Barron J*[2]; *chiemeka.ike@emdserono.com*
[1]*EMD Serono, Inc.*; [2]*Carelon Research*; [3]*Kaiser Permanente*; [4]*The Healthcare Business of Merck KGaA*

BACKGROUND: Avelumab (ave) first-line (1L) maintenance (1LM) is a standard of care for patients with metastatic urothelial carcinoma (mUC) with nonprogressive disease after 1L platinum-based chemotherapy (PBC). Recent guideline updates do not recommend 1L immuno-oncology (IO) monotherapy for routine use in mUC.

OBJECTIVE: To examine health care resource utilization (HCRU) and costs in patients with mUC receiving 1L systemic treatment ± ave 1LM and subsequent second-line (2L) treatment.

METHODS: Administrative claims data from the Healthcare Integrated Research Database (July 1, 2019, to August 31, 2023) were analyzed. Eligible patients had bladder cancer stage IIIB/IV or at least 2 *International Classification of Diseases, Tenth Revision* diagnoses of metastatic cancer, were aged 18 years and older, and had initiated 1L treatment (index date: January 1, 2020, to July 31, 2023). HCRU and medical costs for inpatient (IP) stays and emergency department (ED) or outpatient (OP) visits were evaluated. All-cause HCRU/costs were assessed per patient per month (PPPM) by 1L treatment type.

RESULTS: Of 2,820 treated patients with mUC, 37.0% (n = 1,044) received 1L PBC, 39.0% (n = 1,099) 1L IO monotherapy, and 24.0% (n = 677) other 1L treatment. Mean (SD) age was 69.9 (11.6) years; 69.2% were male. A total of 32.8% (n = 926) received 2L treatment, of whom 46.0% (n = 408) received 1L PBC only, 22.2% (n = 244) 1L IO, and 30.1% (n = 204) other 1L treatment. After completing 1L PBC, 15.0% (n = 157/1,044) initiated ave 1LM within 90 days, of whom 44.6% (n = 70) received 2L treatment. In 2L patients treated with 1L PBC

only, 1L IO, or other 1L treatment, respectively, 78.7%, 76.2%, and 68.1% had IP stays and 55.9%, 48.4%, and 52.0% had ED visits. In patients treated with 1L PBC + ave 1LM, 68.6% had IP stays and 61.4% had ED visits. In 1L PBC only, 1L IO, and other 1L treatment groups, respectively, mean (SD) IP stays were 0.20 (0.26), 0.18 (0.25), and 0.18 (0.23) PPPM and ED visits were 0.09 (0.15), 0.06 (0.10), and 0.08 (0.14) PPPM. In patients treated with 1L PBC + ave 1LM, mean (SD) IP and ED visits were 0.13 (0.16) and 0.11 (0.17) PPPM. Median (IQR) all-cause total medical costs PPPM for 1L PBC only, 1L IO, and other 1L treatment were $15,859 ($8,400-$27,121), $11,346 ($3,164-$23,402), and $9,516 ($2,422-$21,625), respectively, and for 1L PBC + ave 1LM were $19,781 ($14,134-$30,550). Most costs were from OP visits.

CONCLUSIONS: mUC is associated with substantial HCRU and costs, driven primarily by OP visits in a mostly older male patient population. Low 2L treatment rates persist. Ave 1LM slightly increased costs vs 1L PBC only; however, mean IP stays and length of stay were lower vs those treated with 1L IO monotherapy. Further studies are needed to assess the economic impact of novel agents and treatment sequences in mUC.

SPONSORSHIP: EMD Serono (CrossRef Funder ID: 10.13039/100004755)

N7 Costs associated with novel first-line treatments in patients with locally advanced or metastatic urothelial carcinoma from a US Medicare and commercial payer perspective

Ike C[1], *Kongnakorn T*[2], *Tichy E*[3], *Sanchez Alvarez J*[4], *Kearney M*[5]; *chiemeka.ike@emdserono.com*
[1]*EMD Serono, Inc.*; [2]*Evidera*; [3]*Evidera*; [4]*Evidera*; [5]*The Healthcare Business of Merck KGaA*

BACKGROUND: Recent drug approvals in the 1L treatment setting have expanded options for patients with locally advanced (la)/metastatic urothelial carcinoma (mUC). It is important to understand the economic burden of these novel therapies within the current treatment landscape.

OBJECTIVE: To estimate the first-year cost of care in the United States from a Medicare and commercial payer perspective for patients with la/mUC treated with the following first-line (1L) treatment: enfortumab vedotin (EV) + pembrolizumab (PEM) in platinum-eligible patients, nivolumab (NIV) + platinum-based chemotherapy (PBC) in cisplatin-eligible patients, and 1L PBC with or without avelumab (AVE) 1L maintenance (1LM).

METHODS: A cost-of-care model was developed to estimate direct medical care costs in the first year of treatment with EV + PEM, NIV + PBC, and PBC with and without AVE 1LM in patients with la/mUC who are eligible for 1L PBC; patients treated with NIV + PBC were eligible for cisplatin. The latest available costs (2023 USD)—including drug acquisition and administration, disease management, adverse event (AE) management, and subsequent therapy (second-line or later-line) costs—were calculated based on treatment duration, progression-free survival, overall survival, and AE incidence with various therapies. Efficacy and safety data were sourced from key published trials (EV-302, CheckMate-901, JAVELIN Bladder 100) and product prescribing information.

RESULTS: Estimated total first-year costs per treated patient for 1L EV + PEM, NIV + PBC, PBC with AVE 1LM, and PBC only, respectively, were $438,660, $154,731, $120,494, and $56,952 for Medicare and $457,390, $194,866, $156,298, and $90,918 for commercial payers. Drug acquisition and administration costs in the 1L represented the majority of the costs except for PBC only, for which subsequent treatment and administration costs represented the largest cost component. Estimated annual costs for drug acquisition and administration per treated patient for 1L EV + PEM, NIV + PBC, PBC with AVE 1LM, and PBC only, respectively, were $424,865, $119,928, $96,402, and $3,719 for Medicare and $422,304, $125,883, $101,760, and $7,785 for commercial payers. Subsequent treatment and administration costs for PBC only were $30,971 for Medicare and $33,027 for commercial payers.

CONCLUSIONS: Costs per treated patient were lower for PBC with AVE 1LM than for EV + PEM and NIV + PBC. As value-based oncology care is becoming increasingly important in the United States, comprehensive understanding of costs across different treatment options and sequences can facilitate informed treatment choice.

SPONSORSHIP: EMD Serono (CrossRef Funder ID: 10.13039/100004755)

N8 Informing optimal treatment sequences for platinum-eligible patients with locally advanced or metastatic urothelial carcinoma in the United States: Results of a clinical simulation modeling exercise

Sanchez Alvarez J[1], *Benedict A*[2], *Tichy E*[2], *Kongnakorn T*[3], *Ike C*[4], *Kearney M*[5]; *Javier.SanchezAlvarez@evidera.com*; *chiemeka.ike@emdserono.com*
[1]*Evidera*; [2]*Evidera*; [3]*Evidera*; [4]*EMD Serono, Inc.*; [5]*The Healthcare Business of Merck KGaA*

BACKGROUND: Platinum-based chemotherapy (PBC) followed by avelumab (AVE) first-line (1L) maintenance for patients with nonprogressive disease (PBC ± AVE) is a recommended treatment option in locally advanced (la)/metastatic urothelial carcinoma (mUC).

OBJECTIVE: To compare overall survival (OS) and cost-effectiveness with the treatment sequence PBC ± AVE followed by enfortumab vedotin (EV) or other therapies, including immuno-oncology (IO) agents, in second line (2L; ie, PBC + 2L EV/IO or PBC + AVE + 2L EV/IO) vs 1L EV + pembrolizumab (PEM) for PBC-eligible patients (PBCp) from a US commercial payer perspective.

METHODS: A partitioned survival model compared outcomes with PBC ± AVE and EV + PEM in PBCp. Inputs included progression-free survival and OS, time to treatment discontinuation, adverse event (AE) incidence, and health utilities and costs. Efficacy inputs were sourced and extrapolated from patient data in JAVELIN Bladder 100 (data cut: June 4, 2021), AVENANCE real-world study of AVE 1L maintenance, and published data (EV-302). Safety data were sourced from US prescribing information. Drug, subsequent treatment, disease management, and AE management costs (2023 USD) were calculated. The optimal treatment sequencing scenario assumed 80% of patients starting PBC were eligible to receive AVE (per 2023 Flatiron data), 70% of patients progressing on PBC ± AVE would start 2L, and 70% of those would be eligible for EV. Outcomes included life-years (LYs), quality-adjusted LYs (QALYs), costs, and incremental cost-effectiveness ratio (ICER). Scenarios and probabilistic sensitivity analyses (PSAs) were conducted to assess uncertainty.

RESULTS: Use of 2L EV after PBC ± AVE is associated with 0.76 lower QALYs vs 1L EV + PEM (2.63 vs 3.39) but a large cost saving ($597,066 vs $981,069); thus, it represents a cost-effective use of resources at the $150,000/QALY willingness-to-pay (WTP) threshold. The ICER for 1L EV + PEM vs PBC ± AVE was $505,231. PSAs showed that PBC ± AVE is likely to be more cost-effective over a broad and relevant WTP threshold range ($0-500,000/QALY). Results were sensitive to drug acquisition costs and eligibility to receive AVE and 2L EV.

CONCLUSIONS: Use of 2L EV after PBC ± AVE achieves considerable survival benefit at substantially lower costs vs 1L EV + PEM in PBCp with la/mUC. Given the shift toward value-based care in oncology, such analyses are increasingly relevant to guide clinical decision-making. There is a need to assess treatment alternatives that balance health care costs while optimizing patient outcomes.

SPONSORSHIP: EMD Serono (CrossRef Funder ID: 10.13039/100004755)

N9 Burden of sleep disturbances and vasomotor symptoms on work productivity and health care resource utilization among women experiencing menopause in the United States

Charafi L[1], *Schoof N*[2], *Nguyen J*[1], *Modi K*[3], *Drakeley S*[3], *Yang L*[3], *Banks V*[4], *Genga K*[1], *Halvorson L*[5], *Bolling K*[6], *Moeller C*[2], *Dinkel-Keuthage C*[1]; *lena.charafi@bayer.com*
[1]*Bayer*; [2]*Bayer AG*; [3]*Oracle Life Sciences, Oracle Corporation*; [4]*Bayer AG, UK*; [5]*Bayer Pharmaceuticals*; [6]*Bayer U.S. LLC*

BACKGROUND: Sleep disturbances (SDB) and vasomotor symptoms (VMS) are commonly experienced by women during menopause, but impact on work-related productivity and activity impairment (WPAI) and health care resource use (HCRU) is not well studied.

OBJECTIVE: To assess SDB and VMS effect using the US National Health and Wellness Survey (NHWS) among postmenopausal women.

METHODS: Women aged 40-65 years who completed the online NHWS and reported experiencing menopause symptoms were included. Postmenopausal status was defined if menstrual bleeding ceased more than 12 months ago. Women were grouped by the presence/absence of VMS (defined by reporting hot flashes/night sweats in the past 12 months) and SDB (defined as sleep difficulties in the past 12 months or select sleep symptoms experienced at least weekly).WPAI scores were assessed among employed respondents only for absenteeism, presenteeism, and work productivity; activity impairment included all respondents. Higher percentage scores indicated increased WPAI. HCRU outcomes included self-reported health care provider (HCP) visits among others.

RESULTS: This study population consisted of 21,616 women experiencing menopause (mean age: 57.6 years, SD: 5.7). Among respondents, 43.4% reported VMS, of whom 66.7% had SDB; 20.1% reported SDB without VMS. A total of 55.9% of the study population was employed. In multivariable regression analysis, employed women experiencing both SDB and VMS showed highest absenteeism (estimated marginal means [EMM]: 2.6%), presenteeism (EMM: 11.8%), and work productivity impairment (EMM: 12.8%), followed by SDB only (EMM: 2.4%, 10.9%, 11.7%, respectively), then VMS only (EMM: 1.9%, 10.7%, 11.5%, respectively). Among all respondents, both SDB and VMS and SDB only had most activity impairment (EMM: 20.3%, 20.8% respectively) compared with VMS only or neither SDB nor VMS (EMM: 18.1%, 16.2%). Women experiencing SDB only (odds ratio [OR] = 1.35), VMS only (OR = 1.18), and both (OR = 1.26) were more likely to have an HCP visit, compared with those experiencing neither SDB

nor VMS. Additionally, all groups showed a higher adjusted mean HCP visit in the past 6 months than those with neither SDB nor VMS (SDB+VMS = 3.94, SDB only = 3.95, VMS only = 3.50, neither SDB nor VMS = 3.24).

CONCLUSIONS: In this study population, SDB was experienced by nearly half of all postmenopausal women and was a larger driver in WPAI and HCRU burden than VMS. However, both SDB and VMS showed increased burden among women experiencing menopause compared with those experiencing neither, highlighting the individual and combined symptom impact of SDB and VMS on WPAI and HCRU burden among postmenopausal women.

SPONSORSHIP: Bayer

N10 Demographic and clinical characteristics of patients with uterine fibroids and heavy menstrual bleeding treated with gonadotropin-releasing hormone antagonists and hormonal therapy

Lickert C[1], *Chen J*[2], *Leslie S*[1], *DeKoven M*[2], *Coyle K*[2], *Shi L*[2], *Cole M*[1]; *Cassandra.lickert@us.sumitomo-pharma.com*; *michele.cole@us.sumitomo-pharma.com*
[1]*Sumitomo Pharma America*; [2]IQVIA

BACKGROUND: Uterine fibroids (UF) are common, benign uterine tumors that affect approximately 70% of women of reproductive age. The most common symptom is heavy menstrual bleeding (HMB). Gonadotropin-releasing hormone (GnRH) antagonists were recently approved by the US Food and Drug Administration (FDA) to treat HMB associated with UF. Currently, limited information exists on the real-world utilization of these new therapies.

OBJECTIVE: To describe baseline demographic and clinical characteristics of patients with UF and HMB treated with GnRH antagonists and hormonal therapy (HT).

METHODS: Women who had at least 1 medical claim with an *International Classification of Diseases, Tenth Revision* (ICD-10) diagnosis code for UF (ICD-10: D25.0, D25.1, D25.2, D25.9) between May 1, 2021, and August 31, 2022 (index date), were identified in IQVIA's New Data Warehouse (deterministically linked professional fee [Dx], hospital charge master data [CDM], and pharmacy claims [LRx]). Of these, women with at least 2 claims for UF at least 30 days apart in Dx and/or CDM and with at least 1 claim for HMB in Dx and/or CDM (ICD-10: N92.0-6, N93.8, N93.9) over the 12-month pre- or post-index periods were included in the analysis. Patients with evidence of UF during the 12-month pre-index period were excluded (only incident patients remained). Women were stratified into 2 cohorts based on the therapy received following the UF diagnosis (index date): relugolix combination therapy (RCT) and HT (aromatase inhibitors, danazol, etonogestrel implant, GnRH agonists/antagonists, medroxyprogesterone, contraceptive patches, oral contraceptives, and progestins). Baseline clinical and demographic characteristics were descriptively reported.

RESULTS: Following the application of exclusion/inclusion criteria, 143 RCT- and 4,694 HT-treated women were identified. Mean (SD) age was 44.1 (5.9) years for the RCT and 41.9 (7.1) years for the HT cohort. The following comorbidities were reported at baseline for the RCT and HT cohorts, respectively: obesity (26.4%, 31.0 %), endometriosis (7.9%, 7.2%), pregnancy (2.9%, 7.5%), and infertility (1.4%, 3.1%). The RCT and HT cohorts experienced a high clinical burden: bulk symptoms (46.4%, 53.6%), anemia (33.6%, 24.6%), and fatigue (12.9%, 14.6%). The most frequently prescribed hormonal therapies were oral contraceptives (24.3%, 30.6%), progestins (21.4%, 18.9%), and GnRH antagonists (12.9%, 0.6%), respectively.

CONCLUSIONS: Women with UF and HMB experience a high clinical burden, including bulk symptoms and anemia. Use of an FDA-approved medication to treat HMB associated with UF should be included in the treatment selection.

SPONSORSHIP: Sumitomo Pharma America Pfizer, Inc

O1 Trends in utilization of different types of progesterone for prevention of preterm birth

Wang X, Djibo D, McMahill-Walraven C;
xi.wang@cvshealth.com
CVS Health

BACKGROUND: Intramuscular 17a-hydroxyprogesterone caproate (17-OHPC) was the only US Food and Drug Administration (FDA)–approved medicine to reduce the risk of preterm birth (PTB) in pregnant women with history of spontaneous PTB. The FDA held a committee meeting in late 2019 to review results from an FDA-requested randomized trial ("PROLONG trial") that failed to confirm the benefit of 17-OHPC. A final decision to withdraw approval of generic 17-OHPC was issued by the FDA in 2023. The removal of generic 17-OHPC was speculated to result in more administering of compounded 17-OHPC. Vaginal progesterone may also be an alternative option for PTB prevention endorsed by the American College of Obstetricians and Gynecologists.

OBJECTIVE: To determine the trend in use rates of generic 17-OHPC, compounded 17-OHPC, and vaginal progesterone for patients at high risk for PTB during 2016-2023.

METHODS: A retrospective study was conducted among pregnant people enrolled in a large national commercial health plan. Study population were pregnant women (1) whose pregnancies ended in live birth, (2) with a delivery age between 10 and 54 years, (3) who had at least 6 months

of continuous medical and drug coverage with allowable 45-day gap from pregnancy start date to delivery date, and (4) who had a diagnosis of history of PTB. Pregnancy outcomes and duration were estimated using a validated algorithm. Dispensings of generic 17-OHPC, compounded 17-OHPC, and vaginal progesterone were ascertained through National Drug Code numbers.

RESULTS: This study included 5,412 live births to people with history of PTB. The percentage of included pregnancies receiving generic 17-OHPC increased from 16.5% in 2016 to 27.6% in 2018 and decreased to 15.0% in 2019, 8.7% in 2020, 5.6% in 2022, and 2.4% in 2023. The use rate of compounded 17-OHPC peaked in 2019 (13.8%) and decreased significantly from 2020 (8.2%) to 2023 (1.1%). The use rate of vaginal progesterone remained stable and low, ranging from 2.2% to 4.0% during 2016-2023. In 2023, 93.7% of pregnant women with PTB history did not receive any type of progesterone.

CONCLUSIONS: Obstetrical providers reduced dispensing of generic and compounded 17-OHPC for patients at risk for PTB after the publication of PROLONG trial. We did not observe significant change in vaginal progesterone use in this sample. The majority of at-risk pregnancies did not receive any progesterone for PTB prevention. Given the important public health impact of PTB, there is an opportunity to update clinical guidelines and to invest in research and development for new medicines for PTB prevention.

SPONSORSHIP: CVS Healthspire Life Science Solutions

O2 Trend in utilization of telehealth for prenatal care from 2018 to 2024

Wang X, Djibo D, McMahill-Walraven C;
xi.wang@cvshealth.com; audrey.djibo@cvshealth.com
CVS Health

BACKGROUND: Evidence suggests that telehealth provides comparable health outcomes when compared with traditional methods of health care delivery, enhances patient satisfaction, and improves patient engagement. The American College of Obstetricians and Gynecologists endorses telehealth to reduce maternal morbidity and mortality.

OBJECTIVE: To describe the trend in use of telehealth prenatal care during 2018-2024.

METHODS: This cross-sectional study used administrative claims data from a large national commercial health plan. We identified prenatal care encounters occurred between January 2018 and April 2024 for individuals with medical coverage. A prenatal care encounter was defined as a patient having any claims with primary diagnosis code of pregnancy supervision on a specific date. For each prenatal care encounter, we determined whether it was provided in-person or via telehealth and the modality of telehealth (such as video or telephone), using a combination of procedure codes, place of service codes, and modifiers. To describe the trend of use rate over time, we then aggregated data to monthly level: all pregnant people in each month (ie, individuals with any prenatal care encounter during that month) were used as denominators and the number of pregnant people with at least 1 prenatal care encounter provided via telehealth during that month as numerators.

RESULTS: We identified 14,321,649 prenatal care encounters and classified 44,143 (0.3%) of them as a telehealth visit. Among these telehealth prenatal encounters, 37,982 (86%) were via real-time video, 4,081 (9%) were via telephone, and 646 (1%) were remote monitoring. Monthly telehealth utilization rates for pregnant people remained as 0.1% from January 2018 through February 2020. The monthly rate dramatically increased after the outbreak of the COVID-19 pandemic: 2.4% in March, 6.9% in April, 5.7% in May, and 4.5% in June of 2020. After the second year of the pandemic, the monthly telehealth prenatal care use rate gradually decreased: 3.8% in March 2021, 3.5% in March 2022, 3.1% in March 2023, and 2.6% in March 2024.

CONCLUSIONS: The use of telehealth services for prenatal care increased substantially during the first year of the COVID-10 pandemic, with real-time video as the majority modality. The use rates slightly decreased in later phases of the pandemic but remained significantly higher than the prepandemic level. Policymakers, payers, professional societies, and providers should assess and modify barriers and facilitators for telehealth utilization to improve access to prenatal care services during this digital age.

SPONSORSHIP: CVS Healthspire Life Science Solutions

Q1 Patient characteristics and utilization patterns in early adopters of the novel glucocorticoid vamorolone in the treatment of Duchenne muscular dystrophy in the United States

Abdul Hamid O[1], Woods S[2], Ecker K[2], Habkouk K[3], Badgett R[4], Monduy M[5]; Omer.Abdulhamid@nemours.org; steve.woods@catalystpharma.com
[1]Nemours Children's Hospital; [2]Catalyst Pharmaceuticals; [3]AnovoRx, Inc; [4]BrightScale, Inc; [5]Nicklaus Children's Hospital

BACKGROUND: Duchenne muscular dystrophy (DMD) is a childhood neuromuscular disease with an incidence of 1 in every 5,000-6,000 live male births. Treatment with glucocorticoids (GCs) increases muscle strength and function and should be considered in all patients to align with the expert care guidance in DMD. Vamorolone (VAM) is a structurally unique steroidal anti-inflammatory drug

recently approved in the United States for patients aged 2 years and older with DMD.

OBJECTIVE: To describe the patient characteristics and early utilization patterns of vamorolone, a new oral anti-inflammatory steroidal drug.

METHODS: Patient characteristics and VAM utilization were evaluated using data sourced through a specialty pharmacy and stored in a cloud-based data warehouse. This point-in-time analysis includes the first 100 patients to receive VAM from February 1 to March 30, 2024. Available data were pooled to provide this early evaluation of patient demographics, past and current DMD treatments, VAM dosing, geographic vicinity, and payer type.

RESULTS: One hundred patients with DMD met the criteria for on-label VAM utilization with a mean age of 12.1 years (median 11; min 2; max 59) at initiation. The cohort mean weight was 45 kg (median 44.5), with 98 male subjects and 2 not reported. Seven boys received gene therapy prior to VAM initiation (mean age 8 years) with 2 boys being steroid naive. Sixteen boys were currently receiving exon skipping therapy at VAM initiation with a mean age of 11.7 years. Prior glucocorticoid use was common in this cohort, with 80% (n = 80) receiving any GC prior to VAM initiation. Sixteen patients were naive to GC therapy, whereas 43 and 42 were receiving deflazacort and prednisone, respectively. The initial mean dose of VAM was 4.7 mg/kg/day (median 5.45). The vast majority of patients (95%) were actively receiving VAM at the March 30, 2024, endpoint with 5 discontinuations (2 patient request, 2 MD request, and 1 adverse event). Payer types were 62% commercial, 35% Medicaid, and 3% other with mean ages of 11.8, 12.5, and 15 years, respectively.

CONCLUSIONS: Among patients in the United States receiving on-label doses of the new steroidal anti-inflammatory drug vamorolone, prior GC use was common, with a majority of boys switching from a traditional GC. Previous or current gene therapy or exon skipping therapy was not uncommon. The mean VAM starting dose correlated with a cohort of patients previously receiving GC therapy. Given the limitations of this retrospective analysis, these findings warrant additional review as VAM use increases.

SPONSORSHIP: Catalyst Pharmaceuticals

Q2 Real-world study of the burden of hypochondroplasia on children and their caregivers

Zincavage R[1], *Zhang* Y[2], *Ito* D[1], *Butt* T[2], *Sussman* M[1]; *rebekah@stratevi.com*; *tom.butt@bmrn.com*
[1]*Stratevi*; [2]*BioMarin*

BACKGROUND: Hypochondroplasia (HCH), a skeletal dysplasia genetic bone growth disorder, is a form of short-limbed dwarfism resulting in disproportionate short stature. The burden associated with HCH among youth aged 0–7 years and their caregivers is undefined.

OBJECTIVE: To assess the burden associated with HCH among children and evaluate the impact it has on caregivers' lives in a real-world US setting.

METHODS: A cross-sectional survey was administered to US adults who were primary caregivers to a child aged 0–7 years with HCH. Participants were recruited through patient and provider panels and patient advocacy groups. Caregiver demographics, child clinical characteristics, and health care resource utilization in the past 12 months are reported.

RESULTS: Sixteen caregivers (mean [SD] age 35.9 [5.3] years, 88% female, all married and identified as the parent) of children with HCH (mean age 5.1 [1.2] years, 81% male) completed the survey. Frequent fatigue (69%), chronic middle ear infections (50%), and sleep apnea (44%) were the most common comorbidities. Eighty-one percent of children received physical therapy and more than 50% visited a health care professional at least once per month in the past year. Fifty percent of caregivers made home modifications because of their child's HCH, all of whom characterized the associated out-of-pocket costs as burdensome.

CONCLUSIONS: The comorbidities experienced by children with HCH and the burdensome costs associated with health care services and home modifications reported by caregivers highlight the high burden of HCH, suggesting a substantial unmet need to be addressed in these patients and their families.

SPONSORSHIP: BioMarin Pharmaceutical Inc.

Q3 Real-world study of the burden of achondroplasia on children aged 0-7 years and their caregivers

Zincavage R[1], *Zhang Y*[2], *Ito D*[1], *Butt T*[2], *Sussman M*[1]; *rebekah@stratevi.com*; *tom.butt@bmrn.com*
[1]*Stratevi*; [2]*BioMarin*

BACKGROUND: Achondroplasia (ACH) is a skeletal dysplasia that results in disproportionate short stature. The burden associated with ACH among youth aged 0-7 years and their caregivers is undefined.

OBJECTIVE: To assess the burden associated with ACH among children aged 0-7 years and evaluate the impact it has on caregivers' lives in a real-world US setting.

METHODS: A cross-sectional survey was administered to US adults who were primary caregivers to a child aged 0-7 years with ACH and not previously or currently treated with vosoritide. Participants were recruited through patient and provider panels and patient advocacy groups. The survey assessed caregiver demographics, child clinical characteristics, health-related quality of life including PedsQL Generic Core scale and PedsQL Family Impact Module, health care resource utilization in the past 12 months, and Work Productivity Activity Impairment.

RESULTS: Thirty-four caregivers (mean [SD] age 37.8 [8.9] years, 77% female, 82% married, 88% identified as the parent) of children with ACH (mean current age 4.8 [1.5] years, 59% male) completed the survey. Frequent fatigue (59%), chronic middle ear infections (50%), and nightly snoring (47%) were the most common comorbidities. Forty-four percent of children visited a health care professional at least once per month in the past year, with pediatricians (68%) the most visited. Forty-four percent of caregivers made home modifications because of their child's ACH, and more than half of these described the associated out-of-pocket (OOP) costs as burdensome. Caregivers spent an average of 10.6 hours/week on care related to their child's ACH, and 38% sought mental health care because of their child's diagnosis or care. Caregivers reported a mean PedsQL total score of 59.1 (22) and 55.4 (16.9) for youth aged younger than 5 years and 5-7 years, respectively, which was considerably lower than children in the general population aged 0-7 years (mean = 83.9). Caregivers also reported lower family quality-of-life scores in all categories, with the greatest difference in communication and worry domains when compared with caregivers of children in the general population.

CONCLUSIONS: Children with ACH can experience a lower quality of life compared with children in the general population. Caregivers spend substantial time providing care related to ACH and face burdensome OOP costs for health care services and home modifications because of ACH. This research highlights the substantial burden associated with ACH experienced by children and their caregivers.

SPONSORSHIP: BioMarin Pharmaceutical Inc.

R1 Safety screening completion in patients initiating immune response mediator therapy for rheumatologic, dermatologic, and inflammatory bowel disease conditions: Multisite prospective analysis

Zuckerman A[1], *Godley E*[2], *Thomas K*[3], *Gargurevich N*[4], *Tong K*[5]; *autumn.zuckerman@vumc.org*
[1]*Vanderbilt Specialty Pharmacy*; [2]*Novant Health*; [3]*University of Illinois Chicago*; [4]*VUMC Department of Biostatistics*; [5]*Yale New Haven Health*

BACKGROUND: Treatment of chronic autoimmune disorders often requires agents that target immune responses. Despite the recommendation that patients initiating these agents be screened for tuberculosis (TB) and hepatitis B virus (Hep B) prior to initiation, previous studies have found disparities in screening rates.

OBJECTIVE: To assessed TB and Hep B screening completion rates prior to immune response mediator initiation and reasons for incomplete screening in health system specialty pharmacies.

METHODS: A multisite, prospective, observational cohort study was performed including adult patients initiating a medication with risk for TB or Hep B reactivation indicated for a rheumatologic, dermatologic, or inflammatory bowel diseases condition between March 1 and May 31, 2023. Patients with a specialty medication within 1 year prior to the index referral date or with an immune-modulating medication referral from a non–health system provider were excluded. Outcomes included the rate at which a composite of both TB and Hep B screening (termed complete safety screening) was completed prior to the first immune-modulating treatment fill, the rate at which TB and Hep B screenings were ever completed separately, and the rate at which screenings were completed within 2 years and 1 year before or after treatment initiation (if first fill date available). Reasons for incomplete screenings were evaluated. For patients who had both TB or Hep B laboratory results dates and a fill date, the time between laboratory results and first fill was calculated.

RESULTS: Across 12 sites, 738 patients were referred for immune-modulating treatment. Of those, 61% had complete safety screening documented. In patients with first fill date available (n = 517), 87% had complete safety screening before the first fill and 2% had neither screening completed before the first fill. For the entire sample, TB screening was

completed in 96% of patients at any time. For patients with a first fill date available, 91% had TB screening within 2 years and 86% within 1 year. HBV screening was deemed unnecessary by the provider or completed in 97% of patients at any time prior to therapy initiation. For patients with first fill date available, 86% were screened for Hep B within 2 years and 80% within 1 year. The most common reason for incomplete TB and Hep B screening was provider driven (38% and 48%). Median time from TB result to first fill was 20 days (IQR = 6-98) and Hep B result to first fill was 27 days (IQR = 8-158).

CONCLUSIONS: Most patients with a health system specialty pharmacy referral are screened for TB and Hep B prior to treatment. Foregone safety screening is commonly driven by the prescribing provider.

SPONSORSHIP: None

R2 Pharmacist involvement in safety screening for patients starting immune-modulating therapies: Results from a multisite, prospective, observational cohort study

Thomas K[1], *Rhudy C*[2], *Loucks J*[3], *Plaia G*[4], *Zuckerman A*[5], *Tong K*[6], *Rizkalla N*[7]; *kct@uic.edu*
[1]*University of Illinois Chicago*; [2]*University of Kentucky HealthCare*; [3]*The University of Kansas Health System*; [4]*Dartmouth Health*; [5]*Vanderbilt Specialty Pharmacy*; [6]*Yale New Haven Health*; [7]*Northwell Health*

BACKGROUND: Most self-administered biologic or oral small molecule medications for inflammatory conditions modulate the immune system, increasing the risk of tuberculosis or hepatitis B virus reactivation, so screening is recommended prior to therapy.

OBJECTIVE: To determine the impact of health system specialty pharmacies (HSSPs) on safe initiation of self-administered immune-modulating therapies and what types of HSSP interventions occur during the safety screening process.

METHODS: This is a multisite, prospective, observational cohort study to determine how HSSP pharmacists impact the safe initiation of new immune-modulating therapies in patients with dermatology, gastroenterology, or rheumatology specialty conditions. Adult patients were included if they were prescribed and started a new immune-modulating medication between March 1, 2023, and May 31, 2023. Patients were excluded if they received immune-modulating therapy in the 12 months prior to referral, if they were aged younger than 18 years, or if their provider was not serviced by the HSSP. Documented pharmacist interventions were collected prior to and after laboratory safety screening completion and quantified at each site. Descriptive statistics were used to report the rate of pharmacist interventions by category.

RESULTS: Across 12 health systems, 738 patients were included in the analysis. The overall study population was 63.7% (470) female and 66.8% (493) White, with a median age of 48 years. Patients had rheumatology (55.6%, 410), dermatology (29%, 214), or gastroenterology conditions (15.4%, 114). Pharmacists reviewed and noted laboratory results were appropriately collected with no need for intervention in 23.2% (171) of patients. Pharmacists were not involved in screening for 63.3% (467) of patients. Pharmacists intervened in the safety screening process for 37% of patients (271). Interventions included identifying the need for a safety laboratory to be completed prior to screening 41.3% (112), coordinating ordering of the safety laboratory for 26% (71), and/or communicating with the patient to coordinate laboratory collection for 31.7% (86). After screening was completed, pharmacists identified abnormal safety laboratory results for 11.4% (31) and treatment plans were changed for 3.3% (9) of patients.

CONCLUSIONS: HSSP pharmacists caring for patients on immune-modulating therapies have an important role in preventing serious infection by ensuring patients complete safety screening and guiding response to abnormal screening results.

SPONSORSHIP: None

S1 Assessment of the potential clinical and economic impact of the acellular tissue engineered vessel in the treatment of patients with high-risk traumatic vascular injury

Velez F[1], *Pascarella L*[2], *Malone D*[3], *Sun L*[4], *Nicklason L*[1]; *fvelez@humacyte.com*
[1]*Humacyte Global, Inc.*; [2]*University of North Carolina*; [3]*Strategic Therapeutics, LLC*; [4]*Curta, Inc.*

BACKGROUND: Traumatic vascular injury is an important cause of morbidity and mortality. According to the PROspective Observational Vascular Injury Trial (PROOVIT), which is the largest civilian vascular trauma registry, approximately 20% of patients are treated with synthetic or other grafts rather than the "gold standard," which is harvested autologous vein. In the setting of trauma, vascular repair using nonvein conduit has higher rates of infection and amputation than with autologous vein. However, drawbacks of autologous vein include the additional injury incurred during harvest and the time required for harvest, which delays reperfusion. The Acellular Tissue Engineered Vessel (ATEV) is a bioengineered, universally implantable,

and off-the-shelf conduit that has shown low amputation and infection rates in clinical trials of patients with extremity arterial trauma without adequate vein.

OBJECTIVE: To evaluate the potential clinical and economic impact of the ATEV in patients with vascular trauma.

METHODS: Using the perspective of a Level 1 Trauma Center, a decision model comparing the expected frequency and cost of early complications (amputation, conduit infection, fasciotomy, shunt use, rhabdomyolysis) and late complications (amputations and reinterventions due to thrombosis/stenosis) between 2 treatment scenarios was developed. Based on PROOVIT data, 2 "current treatment scenario" groups were constructed: (1) those having ischemia time longer than 5 hours (42.7% of patients) and (2) patients lacking autologous vein (57.3% of patients—comprising 22.8% receiving synthetic graft, 17.2% cryopreserved vein, and 17.2% bovine xenograft). In the "ATEV treatment scenario," 2 simulations of ATEV use were conducted: 25% and 100% ATEV use in each of the "current treatment scenario" groups.

RESULTS: Early complications were reduced in the "ATEV treatment" scenario. Amputations decreased between 13.6% and 54.6% when the ATEV was used; conduit infections decreased between 11.7% and 46.8%; fasciotomies decreased between 9.5% and 37.9%; initial shunt use decreased between 6.7% and 27.8%; and rhabdomyolysis decreased between 6.9% and 27.8%. Late complications leading to readmissions were also reduced: amputations decreased between 13.6% and 54.6%, and reinterventions decreased between 4.7% and 18.9%. There was a 10% to 40.1% reduction in complication-related costs per patient.

CONCLUSIONS: In this clinical and economic evaluation of patients with extremity arterial trauma, the rate of early and late complications and associated direct costs were lower with ATEV use compared with treatment with non-vein grafts.

SPONSORSHIP: Humacyte Global, Inc.

U00-U99 Codes for Special Purposes and AMCP Unclassified Abstracts

(eg, benefit management, care management, multidisease studies, pharmacist services, Part D, specialty pharmacy, star ratings)

U7 Adherence care coordination to improve medication adherence

Green G[1], *Haskell J*[1], *Patel R*[1], *DiGeronimo J*[2], *Erxleben T*[2]; *geannette.green@centene.com*; *JDiGeronimo@rxante.com*; *TErxleben@rxante.com*
[1]*Centene*; [2]*RxAnte*

BACKGROUND: Medication adherence is a challenge for Medicare Advantage Prescription Drug (MAPD) members with chronic conditions. Centene's Adherence Care Coordination (ACC) program, launched in July 2023, aims to improve medication adherence for cholesterol, blood pressure, and diabetes (DIAB, RASA, STAT) by addressing social drivers of health (SDOH).

OBJECTIVE: To evaluate the effectiveness of the live member barrier calls coupled with escalation in improving medication adherence rates among MAPD members with SDOH barriers.

METHODS: Predictive analytics targets members weekly at highest risk for nonadherence to receive a live call from a Centene Pharmacy Technician and/or Pharmacist. Adherence calls are timed to reach members before, when, and after a medication is due for refill. Motivational interviewing and personalized adherence support is used to evaluate each member's unique health barrier(s). Members who complete a technician-driven barrier assessment to identify medication adherence barriers are receptive. If the Technician is unable to address the member's barrier(s), the member is escalated to a Clinical Pharmacist for consultation or further escalation. Twelve ACC opportunities support members and help them overcome the identified barrier(s) including direct outreach to a member's pharmacy or providers to obtain medication refills and referrals to resources such as community advocacy services, case management, and member services. Pharmacy claims data were analyzed for Centene MAPD members that met specifications for the Pharmacy Quality Alliance adherence measures (DIAB, RASA, STAT) in 2023. Adherence (proportion of days covered ≥ 0.80) intervention lift rates were calculated as the average change in adherence rates from baseline to year-end, controlling for difference in receptive vs never-reached members.

RESULTS: Centene completed telephonic outreach to 33,000 members at highest risk for medication nonadherence from July to December 2023. Members that received a barrier assessment resulting in care coordination with the appropriate health plan and community resources had a 6.2% increase in adherence rate compared with members that were not reached. Members who were receptive and were escalated to receive additional services showed an adherence improvement of 9.2%.

CONCLUSIONS: Adherence calls coupled with barrier assessment and escalation effectively addresses SDOH, leading to improved medication adherence among MAPD members. By identifying barriers, members received central and/or local support for clinical and non–health plan–related needed resources.

SPONSORSHIP: Centene Corporation and RxAnte

U8 Evaluation of health care decision-maker perspectives on artificial intelligence workflow implementation in health care and its utilization in the formulary decision-making process

Le D, Ng J, Nielsen M, Berns C, McCart M, Ruban C, Abdel-Sattar M; darlena.le@cencora.com
Cencora

BACKGROUND: Artificial intelligence (AI) has been used across different health care applications such as in clinical evaluations, administrative processes, and drug discovery. In previous research, health care decision-makers (HCDMs) have expressed optimism around potential time and cost savings but were also concerned about the ethical and legal ramifications of implementing AI in health care. There is limited research on assessing the continuous evolution of HCDM sentiment toward AI workflow implementation in health care and perspectives around the use of AI in the formulary decision-making process.

OBJECTIVE: To assess HCDM perspectives on the workflow implementation of AI in health care and its use in the formulary decision-making process.

METHODS: An online survey was fielded between March and April 2024 to HCDMs who met the study inclusion criteria and use FormularyDecisions, an online platform that connects biopharma companies to HCDMs.

RESULTS: Surveyed HCDM respondents (N = 30) represented health plans (40%), health systems (33%), pharmacy benefit managers (13%), specialty pharmacies (3%), and other health care settings (11%). Seventy-three percent of HCDMs reported having access to AI tools at their organization. HCDMs said preparation of monographs (37%) and summarizing research papers (37%) were the most common processes for which AI has been leveraged. Most respondents agreed that AI tools should be somewhat utilized (67%) to extremely/very utilized (27%) to aggregate evidence on a pharmaceutical product in the formulary evaluation process. Among the benefits of using an AI tool, increasing productivity (80%) and decreasing cost (80%) were viewed as extremely/very important. Twenty-seven percent of HCDMs believed that third-party consulting/biopharma companies have a role in providing support for the use of AI tools at their organization, whereas 60% were unsure. Among the remaining HCDMs (n = 4) who did not believe third-party consulting/biopharma companies should provide support for AI tool use, 75% said they were extremely/very concerned about privacy of information shared with an AI tool.

CONCLUSIONS: HCDMs acknowledge the value of integrating AI in health care; however, concerns with AI security and privacy highlight the need for further AI technological development, guidance, and education. As AI continues to evolve, understanding HCDM sentiment toward implementing AI into their formulary evaluation process will provide valuable insight into how third-party consulting/biopharma companies can best partner with HCDMs.

SPONSORSHIP: Cencora

U9 The role of patient experience data in payer decision-making

Yoshida S[1], Chiang T[1], Sugarman R[2], Hsu L[3]; shaila.yoshida@cencora.com; tina.chiang@cencora.com; rebecca_sugarman@yahoo.com
[1]Cencora; [2]Cencora (at time of research); [3]HMSA (BCBS of HI)

BACKGROUND: As defined by the 21st Century Cures Act, patient experience data (PED) include data collected by any person to provide information about patients' experiences with a disease or condition including the impact on patients' lives and patient preferences with respect to treatment. Understanding how PED are currently being used by payers in decision-making, what challenges are encountered during the implementation process, and how those challenges are being addressed will be key to ensuring the patient voice is captured in coverage and management decisions.

OBJECTIVE: To understand how US payers are using PED to inform their decision-making.

METHODS: A web-based survey was fielded to US payers in October 2023. A subset of those who completed the survey also participated in a telephone interview to provide rationale and additional insights on PED.

RESULTS: A total of 43 payers from health plans (n = 26), integrated delivery networks (n = 7), and pharmacy benefit managers (n = 10) completed the survey, and 5 also

participated in telephone interviews. Twenty-one percent report their organization has already incorporated PED in coverage decisions, 14% have plans to begin incorporating PED, and 40% are in the discussion stages. Among types of PED, clinician-reported outcomes and patient-reported outcomes are most often used (63% and 56% respectively). Scenarios in which PED are likely or extremely likely to be used in decision-making include when patient adherence is a concern (72%), a clinical outcome assessment is a primary endpoint (65%), cell and gene therapy is being evaluated (45%), or a digital therapeutic is being evaluated (44%). Data quality and reliability and lack of understanding and interpretability of PED were stated as barriers for incorporating PED into their decision-making process. Types of data that payers would like to receive from manufactures include those demonstrating improved patient adherence to therapy (67%), those that are published in relevant peer-reviewed journals (67%), and those that explain PED used in clinical trials (58%). Importantly, roughly half (53%) expect PED to play a larger role in their decision-making over the next 5 years.

CONCLUSIONS: Payers are incorporating or considering incorporating PED into their coverage decisions, especially where patient adherence is a concern. Although they currently face challenges in using PED in decision-making, they still believe PED will play a larger role in the next 5 years. Ways of overcoming current barriers to incorporating PED should be the focus of future research.

SPONSORSHIP: Cencora

U10 Identification of factors associated with prescription claim rejections in the United States using integrated closed and open insurance claims data

Gagnon-Sanschagrin P, Maitland J, Bedard J, Trinh L, Guerin A; patrick.gagnon-sanschagrin@analysisgroup.com
Analysis Group, Inc.

BACKGROUND: Utilization management mechanisms (eg, formulary restrictions) are increasingly enforced. The resulting prescription claim rejections may lead to treatment delays and impair patient outcomes. Data on factors associated with these rejections are limited; new methodologies/data opportunities are needed to understand the clinical impact.

OBJECTIVE: To leverage a novel integrated data source to assess the extent of prescription claim rejections in the United States, characterize factors associated with rejections, and describe the associated clinical impact.

METHODS: Retrospective analysis of integrated closed and open claims from Komodo Research Data + (2016-2022). Closed claims data include patient medical and pharmacy health plan eligibility, plan type, region of residence, and health care costs; open claims data include pharmacy claims lifecycle information (paid, rejected, reversed) and drug details (drug class, branded/generic). For each calendar year, a random sample of patients with continuous health plan enrollment and at least 1 pharmacy claim with lifecycle information in each quarter were identified. Proportion of rejections and unadjusted odds ratio of rejection (OR) were reported by year, drug class, branded/generic, plan type, and patient region of residence.

RESULTS: In total, 2,307,893 patients with 210,015,388 prescription claims were observed (mean 91 claims/patient), of which 173,341,361 (82.5%) had lifecycle information: 93.2% paid, 4.1% rejected, and 3.7% reversed. Reasons for rejection included refill too soon (30.2%), product/service not covered (23.8%), and prior authorization required (9.1%). On average, the proportion of rejected claims increased 3.7% year-over-year (YOY) from 3.6% in 2016 to 4.4% in 2022, with large YOY increases observed in rates of rejection for Medicaid (5.8%) and trademarked drug (4.5%) claims. The proportion of rejected claims varied by drug class–antiseptics/disinfectants had the highest rejection rate (43.0%) and penicillin had the lowest (1.0%). Rates of rejection were higher for branded drugs compared with generics (7.3% vs 3.4%; OR = 2.3) and for Medicare/Medicaid compared with commercial (4.8% vs 3.5%; OR = 1.4) claims. Further variation was observed by region of residence (from 3.9% in the West to 4.4% in the Northeast).

CONCLUSIONS: Prescription claim rejections are increasingly common. The extent of rejections varied over time, by drug class, plan type, and region of residence. Ongoing work will better understand the interplay between factors associated with increased rates of rejections and characterize the clinical and health care cost impact.

SPONSORSHIP: None

U11 A systematic literature review of adherence, economic burden, and clinical outcomes associated with subcutaneous vs intravenous treatments in the United States

Casañas i Comabella C[1], Burnett H[1], Epstein R[2], Sarocco P[3]; Carolina.CasanasComabella@evidera.com; psarocco@halozyme.com
[1]Evidera; [2]Epstein Health, LLC; [3]Halozyme Therapeutics, Inc.

BACKGROUND: Despite evidence suggesting various benefits associated with subcutaneous (SC) vs intravenous (IV) therapies, studies have shown SC treatments are underutilized in the United States in comparison with the European Union. It is therefore of interest to comprehensively summarize the

impacts of SC drug administration compared with IV from real-world (RW) studies conducted in the United States.

OBJECTIVE: To identify all recently published US studies that assess the impact of SC vs IV mode of administration on medication adherence, economic outcomes, and RW effectiveness and safety.

METHODS: Systematic searches were conducted in Embase, Medline, EconLit, and PsycINFO. Observational US studies in any disease published between 2014 and 2024 were included. Two independent reviewers screened titles/abstracts and full-text articles. Data extraction was performed by one reviewer and validated by a second.

RESULTS: Of 3,294 publications screened, 18 were included, focused on treatments for cancer (n = 4), COVID-19 (n = 4), autoimmune diseases (n = 3), diabetes (n = 3), and other diseases (n = 6). Adherence was reported in only 1 study, where SC biologics improved compliance in patients with rheumatoid arthritis. Two studies assessed health care resource utilization (HCRU) in oncology and both reported HCRU savings in chair time savings, including a reduction by 62% (133.4 minutes; $P < 0.001$) with SC vs IV rituximab in patients with hematologic cancer. Only 2 studies compared costs. One showed mean total health care costs were lower with SC ($40,434) vs IV ($44,387) abatacept in rheumatoid arthritis. In the other study, SC vs IV immunoglobulin mean disease-related costs were higher for SC ($43,266 vs $38,064, $P = 0.002$) in primary immune deficiency. Fourteen studies compared the effectiveness of SC vs IV: 3 showed favorable outcomes for SC, 4 favorable outcomes for IV, and 7 comparable outcomes. Four studies compared the safety of SC vs IV: 3 showed favorable outcomes for SC, and 1 reported comparable outcomes.

CONCLUSIONS: Findings from RW studies demonstrated comparable effectiveness of SC vs IV administration and a potentially improved side-effect profile. Although our findings from 4 studies suggest there may be cost and HCRU advantages, additional studies are needed to demonstrate the RW impact of mode of administration on cost, resource use, and adherence across different diseases. Considerably more RW studies in the European Union have demonstrated cost and HCRU of SC IV. Findings from this systematic literature review can be used to inform RWE generation, policy, and healthcare decision-making in the United States, where SC is underutilized.

SPONSORSHIP: Halozyme Therapeutics, Inc.

U12 Trends in utilization of copay assistance for specialty medications in a commercially insured population

Gauni R[1], *Jhamb S*[2], *Kumar D*[2], *Mazumder D*[2];
ranjeet.gauni1@optum.com;
dibyajyoti_mazumder@optum.com
[1]*Optum*; [2]*Optum Global Solutions*

BACKGROUND: Copay assistance have been instrumental in addressing out-of-pocket expenses for high-cost prescription drugs. Understanding patient and claim characteristics that influence utilization pattern might help in addressing gaps and fulfillment of copay assistance.

OBJECTIVE: To evaluate utilization pattern and patient/claim characteristics in a population receiving specialty medication.

METHODS: A retrospective study was conducted using Optum pharmacy claims data for patients with claims between January 1 and December 31, 2023. Pharmacy claims were descriptively analyzed to understand copay assistance utilization and patient and claim characteristics. Members with complete demographics and claims characteristics were included in the analysis. Copay assistance was defined as use of copay card or other noncash measures used to pay toward copay.

RESULTS: A total of 213,300 members with 1,312,928 claims were identified. Nearly 80% (~1.05M; n = 165,698) of these claims were for maintenance drugs, which was the subset used for further analysis. Among maintenance drugs, 54% claims had a copay assistance comprising 115,673 (70%) patients. The mean age was 44.4 vs 49.1 years for patients who received at least 1 copay assistance vs those who did not. The majority of patients were female in either cohort (57% in both). Copay assistance was highest (56%) in the 41-64 years age group, followed by 21-40 years (27.9%). Based on therapy area, chronic inflammatory disease reported the highest copay assistance (~$178M, 75%). Further, we analyzed the trend in patients using anti–tumor necrosis factor (anti-TNF) agents, the subset of chronic inflammatory disease with the highest (33%) copay assistance. Among anti-TNF users, copay assistance was more frequent when the deductible was not met (88%) vs when it was met (58%). Copay assistance was predominantly observed for a copay value of $1-1,000 (93%). The mean out-of-pocket (OOP) cost was $659 vs $890 for patients with copay vs non–copay assistance cohorts. Mean OOP cost decreased with increasing number of copay assistance (1-5: $942, 6-10: $331, 11-15: $231). Further, average days supply for anti-TNFs was 157 vs 146 days for patients with and without copay assistance.

CONCLUSIONS: The results indicated that in certain therapy areas, patients from specific age groups, sexes, and copay thresholds may show higher likelihood to avail copay assistance. Thus, it is imperative to identify the features and assess their correlation with copay assistance needs. Future research would help design robust models to predict patients' need for copay assistance and in turn help health care stakeholders design custom programs for such patients.

SPONSORSHIP: All authors are employees of Optum.

U13 Finding a niche? Role of real-world evidence in US Food and Drug Administration expedited development and review pathways

York M, Rojubally A, Gdovin Bergeson J, Franklin M;
myork@franklin-pharma.com
FPC

BACKGROUND: The inclusion of real-world evidence (RWE) within US Food and Drug Administration (FDA) approval reviews has been increasing, with further guidelines expected. The FDA uses a variety of expedited development and review pathways for novel drugs deemed promising or with potentially significant benefits over existing treatments. Exploring the role of RWE in the different expedited processes by the FDA warrants further scrutiny.

OBJECTIVE: To evaluate the recent use of RWE within novel drug approvals classified as expedited in the FDA's development and review process.

METHODS: Publicly available FDA application review files for novel drug approvals classified for expedited review during 2020-2023 were systematically reviewed for references of RWE. The inclusion of RWE in the approvals were assessed by expedited approval types and review priority. RWE was categorized by the intended purpose including natural disease history, benchmark/contextualization, formal comparison, or postauthorization to support efficacy and/or safety or demonstrate unmet need. The influence of RWE on the outcome of the FDA's approval decision was categorized as rejected, supported, neutral, or included as a postmarket requirement.

RESULTS: Of the 131 evaluable approvals, 99 (76%) included RWE within the application review files. RWE was included in 85/114 (75%) of drugs categorized as priority review, 54/72 (75%) as fast track, 38/57 (67%) as breakthrough therapy, and 27/39 (69%) as accelerated review. Trends in the inclusion of RWE across all approvals were 66% (2020), 69% (2021), 79% (2022), and 89% (2023). Of these priority level groups, RWE was most frequently included in standard-orphan (83%), priority (79%), and priority-orphan reviews (75%). RWE was most often requested as part of a postmarketing requirement (42%) or used as a benchmark/disease history (41%), followed by use as a formal comparison (16%). RWE was often used to support safety (49%), then efficacy (28%), safety and efficacy (8%), and demonstrate an unmet need (14%). Finally, RWE was requested as part of the postmarketing requirements in 42% of reviews, considered supportive in 31%, rejected in 19%, and neutral in 7%.

CONCLUSIONS: Between 2020 and 2023, RWE was commonly included for consideration by the FDA in review files evaluated in the expedited development program. An upward trend in the percentage of review files including RWE was observed across each of the 4 years evaluated. This study provides valuable data in assessing the continued inclusion of RWE within novel drug approvals.

SPONSORSHIP: None

U14 Alignment of commercial health plan utilization management criteria with clinical treatment guidelines

Rucker J[1], Clifford K[1], Beinfeld M[2], Campbell J[3], Motyka J[3], Chambers J[2]; jrucker@tuftsmedicalcenter.org
[1]Tufts Medical Center; [2]Center for the Evaluation of Value and Risk in Health, Institute for Clinical Research and Health Policy Studies, Tufts Medical Center;
[3]National Pharmaceutical Council

BACKGROUND: Utilization management (UM) criteria are pivotal in regulating patient access to specialty drugs. Despite calls for greater transparency, a significant gap remains in understanding how plans derive UM criteria from clinical treatment guidelines. This study investigates the relationship between commercial health plans' UM criteria and the favorability of clinical guidelines.

OBJECTIVE: To investigate how commercial plans UM criteria for specialty drugs align with clinical treatment guidelines.

METHODS: We identified publicly available US-based clinical guidelines for all drug-indication pairs, excluding oncology and biosimilar treatments, in the Tufts Medicine Specialty Drug Evidence and Coverage database as of December 2023. The Tufts Medicine Specialty Drug Evidence and Coverage database includes details on UM criteria, including step therapy protocols and patient subgroups, from the specialty drug coverage policies issued by 18 large commercial health plans. We identified and categorized guidelines as (1) favorable (recommended use was consistent, or more generous than, the drug's US Food and Drug Administration [FDA] label) and (2) unfavorable (recommended use was narrower than the drug's FDA approval, eg, guideline-recommended

use only after failure of alternative therapies). We evaluated each coverage decision for consistency with the available guidelines.

RESULTS: Our sample included 389 drug-indication pairs representing 5,699 coverage decisions. We identified at least 1 guideline for 195 of the 389 drug-indication pairs (50%), with 132 (68%) categorized as "favorable" and 63 (32%) as "unfavorable." When guideline recommendations were favorable, 1,268 of 2,002 (63%) coverage decisions included additional UM. When guideline recommendations were unfavorable, 607 of 905 (67%) coverage decisions included additional UM. When health plans added additional UM criteria, they were most often step therapy protocols (46% of decisions), followed by patient subgroup restrictions (26% of decisions).

CONCLUSIONS: We found a misalignment between clinical guideline recommendations and health plans' use of UM in specialty drug coverage policies. Although health plans imposed UM less often for drugs with favorable clinical guideline recommendations than for those with unfavorable recommendations, we found that plans still applied additional UM criteria in most decisions for drugs despite favorable guidelines. Future research should explore the potential impact of UM criteria on patient access and outcomes.

SPONSORSHIP: National Pharmaceutical Council

U15 Insights on the evolving role and educational needs of managed care learners

Mills O[1], *Wetstein A*[2]; *oliver.d.mills@gmail.com*; *awetstein@pharmacytimes.com*
[1]*Pharmacy Times Continuing Education*; [2]*MJH Lifesciences*

BACKGROUND: The health care industry's shift to value-based care demands a reevaluation of managed care (MC). Traditionally associated with payer organizations, MC principles hold immense value beyond just payment structures. Understanding the evolving roles of MC pharmacists across practice settings is essential for tailoring educational content. Equipping them with in-depth knowledge on applying MC concepts to their respective practice settings will be crucial. By recognizing these specific needs, education can be developed that empowers MC pharmacists to navigate the health care landscape and optimize patient outcomes while ensuring cost-effectiveness.

OBJECTIVE: To gain insight on the evolving roles of MC learners and their specific educational needs.

METHODS: Pharmacy Times Continuing Education delivered 8 satellite symposia at the 2024 AMCP Annual Meeting. The programs were 1.5 credit live symposia that were virtually broadcasted nationally. Baseline knowledge/confidence were assessed through a pretest survey. Learners also identified if they had roles specific to MC. After completing the activity, pharmacists completed a posttest evaluation and selected planned practice changes and practice-related challenges. Data across all programs were aggregated and learners identifying as having MC roles were segmented. We hypothesized that the MC learners' practice settings span beyond payer organizations and their educational needs differ from the general pharmacist audience.

RESULTS: MC roles were identified in the following practice settings: payers, retail, specialty, health systems, long-term care, academia, and infusion centers. The top 3 practice settings for MC learners were payers, retail, and health system (24%, 26%, and 20%, respectively). MC-focused education was more effective at increasing confidence for MC learners (52% increase), compared with non-MC learners (36% increase). The top educational need for all learners was "familiarity with clinical evidence," but "benefit design recommendations" and "considerations for formulary inclusion" were needs specific to MC learners (14.7% and 23.4%, respectively). The top challenge for MC learners was "Need for additional efficacy/safety data to implement changes" (17%); the top barrier for non-MC learners was "Difficulties following up with patients after therapy is initiated" (11.5%).

CONCLUSIONS: The roles of MC pharmacists are evolving and MC-focused education needs to be applied across all pharmacy practice settings.

SPONSORSHIP: These activities were supported by educational grants from Janssen, BMS, Genentech, Novartis, ViiV, Boehringer Ingelheim, Lilly, and BeiGene.

U16 Forecasting the impact of the Inflation Reduction Act on pharmaceutical innovation

Gratie D[1], *Bandy S*[1], *Stokes N*[2], *Chen W*[1], *Pistilli T*[2], *Warren G*[2], *Masseria C*[1]; *Dan@aesara.com*
[1]AESARA; [2]*Axene Health Partners*

BACKGROUND: The Inflation Reduction Act (IRA) allows Medicare to negotiate prices for high-cost prescription drugs; it is not yet known what the potential downstream implications on lost revenue will have on future pharmaceutical research.

OBJECTIVE: To provides estimates of the potential revenue decrease following the negotiated prices for the 10 selected drugs and the potential impact on research and development (R&D).

METHODS: Following Centers for Medicare & Medicaid Services (CMS) guidance in determining the maximum fair price (MFP) for negotiated drugs, we estimated how

implementing these costs could have impacted 2022 CMS drug spend. MFP was estimated by the lowest of a calculated average net price in CMS plans or the estimated nonfederal average manufacturer price with applicable monopoly percentages and inflation applied. Claims data from Merative MarketScan and proprietary data sources were used to identify a range of discounts (14%-48% for specialty drugs and single-source drugs). An average discount (18%) was applied to wholesale acquisition costs to estimate the nonfederal average manufacturer price. The MFPs were then used to calculate total CMS spend when considering the reported dosage units dispensed. After new CMS spend was calculated, the difference was multiplied by a range of industry revenue that has been invested in R&D of 18.6%-25.8%, as estimated by a Congressional Budget Office report.

RESULTS: Had the IRA prices gone into effect in 2022, the estimated spend by CMS could have decreased from $44.8B to between $24.1B and $27.9B. Assuming a range of possible MFPs, this could lead to a decrease of $20.7B to $16.9B in CMS spend. This potentially lost pharmaceutical manufacturer revenue could then lead to an estimated $5.3B to $3.1B reduction of reinvestment in further drug research over the course of a calendar year.

CONCLUSIONS: A potential $5B loss of R&D investment may have a substantial impact on the innovation strategies in the pharmaceutical industry and may impact indication sequencing or label expansion efforts. These calculations represent an approximation as the relationship between CMS and manufacturer liability will change over time. Additionally, these MFPs show the ceiling negotiated prices and may underestimate the revenue impact once final negotiated prices are applied. Our findings reveal that a reduction in pharmaceutical manufacturer revenue could adversely impact investment in R&D and hinder innovation.

SPONSORSHIP: None

U17 Does the maximum fair price accurately represent the value of multi-indication products?

Gratie D[1], *Buikema A*[2], *Bandy S*[1], *Brooks E*[1], *Hulbert E*[2], *Moore K*[2], *Masseria C*[1]; *Dan@aesara.com*
[1]AESARA, *Chapel Hill*, NC; [2]*Optum*

BACKGROUND: The Inflation Reduction Act (IRA) of 2022 allows Medicare to negotiate prices for high-cost prescription drugs. Current guidance estimates average drug expenditure across dosages and formulations; no information has been provided on how variability across indications will be considered and the impact this will have on the inherent value of a treatment.

OBJECTIVE: To investigate high-spend Medicare drugs with multiple indications that are eligible for IRA negotiations in 2025 and estimate the distribution of dosage/formulations tied to different indications.

METHODS: Using 2022 Medicare expenditures, we identified high-spend drugs with more than 4 indications covered by Centers for Medicare & Medicaid Services (CMS), not previously considered for IRA negotiations (XTANDI, JAKAFI, TAGRISSO, REPATHA, COSENTYX, OTEZLA, and VRAYLAR). Claims data from the Optum Research Database of Medicare Advantage with Part D enrollees from January 1 to December 31, 2022, were used. Patients were eligible for inclusion with at least 1 fill for a potential drug (index date), enrollment for at least 1 day, and a related diagnosis code. The distribution of dosage/formulations by drug (National Drug Code-9 numbers) and label-consistent indications based on *International Classification of Diseases, Tenth Revision* codes within 1 year pre-index were described and analyzed.

RESULTS: There were 5,586,290 MAPD beneficiaries with at least 1 day of eligibility and a health care encounter (visit or prescription) in 2022. Samples of patients with a prescription fill in 2022 ranged from 992 (TAGRISSO) to 34,466 (REPATHA), with an average of 2.71 (SD = 1.38) dosage and formulations compared with 4.14 (SD = 0.38) indications per drug. For example, weighting XTANDI (n = 2,625) by formulation would show 28.5% of patients were prescribed 40-mg capsules, 53.7% 40-mg tablets, and 17.8% 80-mg tablets. However, this would not show that 69.7% of these patients were being treated for metastatic disease compared with 30.3% for nonmetastatic disease, as dosage did not vary widely by metastatic status.

CONCLUSIONS: Our findings highlight variability in drug utilization based on dosage/formulation and indications. Medicare should consider a weighting approach reflecting the clinical value of medications across different indications, beyond just formulation options. The value of these drugs depends on the unmet needs and clinical outcomes for each indication. These results demonstrate that the current IRA calculations may not accurately represent a product's clinical value when establishing a maximum fair price.

SPONSORSHIP: None

U18 How does preapproval information exchange affect formulary decision-making?

Penley B, Brisibe T, Dragovich C; ben.penley@xcenda.com
Cencora

BACKGROUND: In 2022, the Consolidated Appropriations Act, 2023 was passed and codified a permanent safe harbor for manufacturers to proactively engage in preapproval information exchange (PIE) with health care decision-makers

(HCDMs). In prior market research, HCDMs claimed that PIE can improve their formulary decision-making ability.

OBJECTIVE: To understand HCDM appetites for PIE, its impact on their decision-making, and its implications in patient access.

METHODS: A double-blinded, web-based survey was fielded with advisors from Cencora's Managed Care Network from May 6 to 16, 2024.

RESULTS: A total of 41 HCDMs responded to the survey, representing health plans (51%), pharmacy benefit managers (32%), and integrated delivery networks (17%), with roles as pharmacy directors (66%), medical directors (24%), and pharmacy managers/trade relations (10%). Of the HCDM respondents, 61% perceived a gap in preapproval information needed for their organization vs what was available in literature and/or supplied by the manufacturer (up from 47% in 2023). Among products that were US Food and Drug Administration (FDA) approved in 2023, 56% of HCDMs received preapproval information on few or none of them. HCDMs noted that if they were to receive preapproval information, their formulary decision-making would be better informed (80%), budget forecasting would be improved (68%), and Pharmacy and Therapeutics (P&T) review (59%) and contracting agreements would be expedited (39%). Specifically, if an estimated price range was available before approval, HCDMs would be better able to forecast a product's budget impact (93%) and make faster decisions upon FDA approval (59%), among other benefits. A total of 61% of HCDMs said that patients would gain access to products sooner if PIE were conducted. More than 1 in 4 HCDMs said that the outcome of a product or class review was changed because their organization had access to preapproval information. In free responses, the implications of not sharing preapproval information with HCDMs were largely negative, including "delayed P&T review," "delayed strategic planning," "less informed decisions," and "lower quality analysis of clinical data."

CONCLUSIONS: In 2024, HCDMs perceived a widening gap relative to 2023 in preapproval information needed vs what was provided by manufacturers. HCDMs noted that PIE can result in expedited P&T review and contracting agreements, among other benefits, potentially leading to earlier patient access to new therapies. To facilitate patient access, manufacturers should actively engage with HCDMs in PIE.

SPONSORSHIP: Cencora

U19 Optimizing the timing of preapproval information exchange for oncology products, products for rare diseases, and cell and gene therapies

Penley B, Hydery T, Dragovich C; ben.penley@xcenda.com
Cencora

BACKGROUND: In 2022, the Consolidated Appropriations Act, 2023 was passed and codified a permanent safe harbor for manufacturers to proactively engage in preapproval information exchange (PIE) with health care decision-makers (HCDMs). In prior market research, HCDMs indicated that, for certain therapeutic categories, including oncology products, products for rare diseases, and cell and gene therapies (CGTs), early PIE is critical for formulary decision-making.

OBJECTIVE: To investigate when HCDMs desire certain types of preapproval information for oncology products, products for rare diseases, and CGTs and what impact PIE has on patient access.

METHODS: A double-blinded, web-based survey was fielded with advisors from Cencora's Managed Care Network from May 6 to 16, 2024.

RESULTS: A total of 41 HCDMs responded to the survey, representing health plans (51%), pharmacy benefit managers (32%), and integrated delivery networks (17%), with roles as pharmacy directors (66%), medical directors (24%), and pharmacy managers/trade relations (10%). Of the HCDM respondents, 49% indicated that patients would gain access to a product sooner if PIE were conducted for specific product categories. Among these respondents, categories included oncology products (85%), products for rare diseases (85%), and CGTs (80%). For all products, 47% of HCDMs said that the latest manufacturers should engage in PIE was 3 months before anticipated product approval. Relative to all products, HCDMs desired most types of preapproval information earlier for products in the specified categories; the types of preapproval information most often desired more than 12 months before anticipated US Food and Drug Administration (FDA) approval were product information (all products: 15%; oncology products: 29%; products for rare diseases: 37%; CGTs: 34%), anticipated FDA approval timeline (19%; 20%; 27%; 34%), and indication sought (19%; 31%; 32%; 31%). HCDMs noted that phase 2 data prior to approval were extremely important/important for oncology products (76%), products for rare diseases (76%), and CGTs (73%) compared with 29% for all products.

CONCLUSIONS: Relative to all products, HCDMs desired preapproval information sooner for oncology products, products for rare diseases, and CGTs. Preapproval communication of phase 2 data was more highly valued for these

products. Some HCDMs indicated that patients could gain access to these products sooner if preapproval information were communicated. To facilitate patient access to products in these therapeutic categories, manufacturers should consider earlier engagement in PIE with HCDMs.

SPONSORSHIP: Cencora

U20 Promoting health equity through enhanced, personalized digital outreach

Grevenitz N, Makanji S, Santilli M, Bisceglio V, DiMaio M; nrusso211@gmail.com; mark.santilli@primetherapeutics.com
Prime Therapeutics

BACKGROUND: The Centers for Medicare & Medicaid Services (CMS) have implemented a 5-Star quality rating system for Medicare plans to drive quality improvement for beneficiaries. To assist a 14,000-member-life Medicare plan in improving the quality of care delivered to their beneficiaries and maximizing Star performance, Prime Therapeutics Management (Prime) collaborated on the development and implementation of a pharmacist-led clinical program designed to specifically address the CMS Star diabetes, RAS antagonists, and statins adherence measures. The plan's membership was 25% Spanish speaking.

OBJECTIVE: To improve performance for the Star Adherence measure and promote health equity by translating Short Message Service (SMS) outreach for Spanish-speaking members.

METHODS: Prime's outreach for the adherence measures is primarily made via pharmacist-led, live telephonic calls with SMS supplemental to the calls. Once we engage the member, pharmacists have in-depth conversations including, but not limited to, disease state education and the importance of medication adherence. They uncover barriers to adherence and implement clinical interventions to overcome those barriers. Members are targeted for outreach based on various factors such as proportion of days covered (PDC), risk for nonadherence, and refill due dates. Through enrollment data and live outreach, language preferences for members were identified. Approximately 25% of the population was Spanish speaking. The SMS workflow was translated to Spanish in 2023. To improve engagement, if a member's preferred language was Spanish, the SMS workflow that they received was in Spanish instead of English.

RESULTS: SMS participation increased 9% from 2022 to 2023 in the Spanish-speaking population as a result of the SMS workflow translation in 2023. We saw a positive impact on telephonic engagement, 90-day supply conversion rates, and PDC for members who participated in the SMS campaign. Given the Spanish-speaking population was a quarter of the population, this resulted in an increase in performance in the overall population across all 3 measures by 1.2%-3.7%.

CONCLUSIONS: Promoting health equity and connecting with members in their primary language can drive increased engagement. Active clinical engagement that is personalized to the member is an important tool in improving the treatment rates for Star measures such as adherence.

SPONSORSHIP: Conducted by Prime Therapeutics Management, an affiliate of Prime Therapeutics, without external funding

U21 Maximizing synergies in the AMCP 5.0 dossier guidance and the Centers for Medicare & Medicaid Services guidance for the Medicare Drug Price Negotiation Program

Patel M, Rojubally A, Franklin M, Gdovin Bergeson J; mpatel@franklin-pharma.com; jgdovin@franklin-pharma.com
FPC

BACKGROUND: The enhancements in the Academy of Managed Care Pharmacy Formulary Submissions version 5.0 (AMCP 5.0) guidance provide a structured platform to compile evidence. The Centers for Medicare & Medicaid Services guidance (CMS guidance) for the Medicare Drug Price Negotiation Program under the Inflation Reduction Act (IRA) informs on manufacturer submissions (dossiers) for selected drugs. The standardized structure, enhanced data presentation, and improved usability of AMCP 5.0 are critical for developing IRA dossiers, particularly for older medicines.

OBJECTIVE: To compare evidence requirements in AMCP 5.0 and the CMS guidance, identifying similarities and differences to create a framework for maximizing efficiencies in dossier development.

METHODS: The CMS guidance for Medicare Drug Price Negotiation Program (June 2023) was reviewed and sections that will require manufacturer submissions were identified. From the new AMCP 5.0, the framework for Evidence Recommendations for Approved Product Dossiers was reviewed. Similarities, differences, and gaps were identified and mapped.

RESULTS: Both AMCP 5.0 and CMS guidance require thorough documentation of a drug's properties, therapeutic role, clinical benefits, and comparative effectiveness and seek information on addressing health disparities and integrating real-world evidence. This real-world evidence focus benefits older drugs in CMS negotiations owing to the abundance of real-world data. A key difference lies in

documentation approaches: AMCP 5.0 offers detailed guidance on the evidence needed for each section, whereas CMS guidance requires specific evidence types but not in a structured format. Additionally, for economic evidence AMCP 5.0 emphasizes health economic evaluations, such as cost-effectiveness analyses, budget impact models, and quality-adjusted life-year (QALY) data, whereas CMS focuses on broader economic evidence to determine a fair price, excluding QALY evaluations.

CONCLUSIONS: Understanding and utilizing the synergies between AMCP 5.0 and CMS requirements can streamline dossier preparation, reduce redundancy, and provide a robust evidence dossier of a drug's effectiveness and impact on patients. Additionally, where gaps exist, leveraging AMCP 5.0's methodologies and standardized structure can be useful for developing IRA dossiers, particularly for older medicines where an updated AMCP dossier may not exist. The evolving guidance for evidence synthesis and summaries in the form of a dossier warrant ongoing discussion and continued research.

SPONSORSHIP: None

U22 Variation in payer prior authorization rates is an unequal barrier to patient care

Malinowski J[1], *Edwards A*[2], *Son M*[3], *Monaghan K*[4], *Fahy S*[5]; *jmalino2@its.jnj.com*
[1]*Johnson & Johnson Innovative Medicine*; [2]*Symphony Health, an ICON plc company*; [3]*Symphony Health*; [4]*ICON Symphony Health*; [5]*Symphony Health, an ICON plc company*

BACKGROUND: Prior authorization (PA) is intended to reduce inappropriate health care utilization; however, variability in its application by different payers (pharmacy benefit managers [PBMs] and managed care organizations [MCOs]) may contribute to inconsistencies in patient access to treatment.

OBJECTIVE: To quantify variation in PA-related metrics for a variety of specialty pharmacy products.

METHODS: We performed a retrospective analysis of ICON's Symphony Health Integrated Dataverse to compare differences in PA-associated rates for a broad selection of specialty pharmacy products in 7 therapeutic areas (adjuvant treatment, autoimmune, blood oncology/hematology, hepatitis C, hypercholesterolemia, migraine, and solid tumor). Patients were included if they had at least 3 claims for at least 1 product with the index claim occurring between January 1, 2020, and December 31, 2022. Primary endpoints were PA rejection rate, abandonment rate, time to overcome a PA, and pull-through rate (ability of patients to overcome PA). Ten PBMs and 25 MCOs were selected as having the highest claims volumes for included products. Demographics and primary endpoints are reported using descriptive statistics for the PBMs and MCOs.

RESULTS: Overall, 12.69% of all claims were subjected to PA. The included PBMs represented coverage of 1,897,872 patient lives (12,095,152 claims), and the included MCOs covered 1,588,049 patient lives (9,440,430 claims). The percent of rejected claims due to PAs ranged from 0.14% to 48% for PBMs and 22.34% to 57.15% for MCOs. Abandonment rates were less than 10% for all PBMs, and MCOs and ranged from 3.11% (MCO25) to 8.33% (MCO21). Pull-through rates greater than 50% were found in 40% of PBMs and 20% of MCOs. The lowest PA pull-through rate observed was 26.44% (MCO25) and the highest rate was 67.33% (PBM6). The mean (SD) time to overcome a PA ranged from 6.72 (5.68) days (PBM6) to 9.33 (6.12) days (MCO11). Overall, time to overcome a PA ranged from 0 to 21 days.

CONCLUSIONS: Rates associated with PA were variable across more than 12 million claims in 10 PBMs and 25 MCOs. Although abandonment was not frequent, pull-through rates less than 50% suggest an inability of patients and their providers to overcome strict PA criteria. Even for PAs that were ultimately approved or reversed, the PA process delayed access to treatment up to 3 weeks for specialty products included in this analysis. Variability in PA adjudication rates by payers may lead to unequal patient access to treatment, which may further amplify existing health disparities.

SPONSORSHIP: Janssen Scientific Affairs, LLC, a Johnson & Johnson company

U23 From policy to practice: US pharmacy benefit managers' implementation of biosimilar coverage decisions

Lin S, Shetty A, Brown J, McCoy M, Zenker G; samuel.lin@iconplc.com; Amith.Shetty@iconplc.com
ICON plc

BACKGROUND: The introduction of biosimilars has allowed payers increased flexibility in the formulary decisions regarding inclusion and exclusion of specific drugs. There are arguments both for and against whether biosimilars have resulted in cost savings for the health care systems and improved patient access.

OBJECTIVE: To understand and assess trends and practices for formulary management of select biosimilar(s) and their reference product across 3 large national pharmacy benefit managers (PBMs).

METHODS: This study compared the 2024 standard formularies from the top 3 national PBMs: CVS Caremark's Performance Drug List, Express Scripts National Preferred Formulary, and Optum Rx's Premium Standard Formulary.

The initial analysis compared coverage status of biologic categories for any reference product with 3 or more biosimilars approved by the US Food and Drug Administration (FDA). Coverage status was classified into 2 categories: Covered and Excluded/Assumed to be excluded (due to lack of commentary). A follow-up analysis was conducted to identify trends and/or practices regarding coverage of reference products and their biosimilars.

RESULTS: Seventeen reference products were identified to have FDA-approved biosimilars; 7 of these reference products were identified to have 3 or more biosimilars approved by the FDA. These products were Humira, Herceptin, Avastin, Neulasta, Neupogen, Remicade, and Rituxan; all were included in this study. Any biosimilars that were not launched at the time of this study were excluded from the analysis. Comparing coverage of these reference products and their associated biosimilars, Optum Rx on average covered the most products of all the PBMs, 2.14 products per biologic category. This compares with Express Scripts with 1.57 products biologic category and CVS Caremark with 1.43 products per biologic category. The overall analysis showed that of the 41 products included in this study, Optum Rx covered 15 of 41 products (37%), Express Scripts covered 11 of 41 products (27%), and CVS Caremark covered 10 of 41 products (24%). In most instances, reference products did not receive any PBM coverage. Humira (adalimumab) is the only reference product that is covered by 2 PBMs.

CONCLUSIONS: Overall, PBMs appear more likely to cover biosimilar options over their reference products. However, PBMs showed variability in the number and selection of products covered in each biologic category. These formulary differences may become increasingly diverse as more biosimilars enter the market, allowing PBMs an advantage when negotiating biosimilar formulary coverage.

SPONSORSHIP: ICON Market Access

U24 Pharmacist involvement in specialty medication discontinuation and dose changes

Perciavalle K[1], *Edelman M*[1], *Zuckerman A*[2], *DeClercq J*[3], *Choi L*[3], *Renfro C*[1]; *kristin.perciavalle@vumc.org*; *chelsea.renfro@vumc.org*
[1]*Vanderbilt Specialty Pharmacy, Vanderbilt Health*; [2]*Vanderbilt Specialty Pharmacy*; [3]*Vanderbilt University Medical Center*

BACKGROUND: Health system specialty pharmacy (HSSP) pharmacists monitor medication effectiveness and safety. Data describing HSSP pharmacists' role and financial implications in intervening to discontinue or switch ineffective or unsafe treatments are needed.

OBJECTIVE: To evaluate HSSP pharmacists' involvement in specialty medication discontinuations, switches, and dose changes and the resulting cost avoidance.

METHODS: A single-center, retrospective cohort analysis of data collected from electronic health records and specialty pharmacy management system was conducted. Patients were included if they had an intervention from January to December 2022 where the HSSP pharmacist indicated the discontinuation, dose change, or switch (termed "medication outcomes") resulted from the pharmacist's recommendation. Included patients had at least 1 specialty medication fill with the HSSP. Interventions due to insurance, finances, therapy completion, medication availability, patient deceased, or therapy not started were excluded. Primary outcomes were number of medication outcomes resulting from pharmacist intervention. Secondary outcomes were intervention reasons and direct costs avoided. A 3-investigator panel categorized each intervention on the probability (Pr) that the medication outcome would not have occurred without the pharmacist (from absolutely not [Pr = 0] to very likely [Pr = 0.75]). Medication costs were calculated using wholesale acquisition cost, average wholesale price, and average wholesale price minus 20%. Cost avoidance was determined by multiplying one fill of the medication costs by the assigned probability of the medication outcome.

RESULTS: Pharmacists completed 113 interventions for 91 patients. Patients were predominantly White (n = 76, 84%) and female (n = 55, 60%), with a median age of 53 (IQR = 27-65). Most interventions were completed in neurology (n = 42, 37%) and rheumatology (n = 26, 23%) clinics. There were 67 (59%) dose changes, 25 (22%) switches, and 21 (19%) discontinuations. The most common intervention reasons were suboptimal therapy response (n = 36, 32%) and side effects (n = 23, 21%). The total monthly estimated costs avoided ranged from $97,824 to $122,281 for dose changes, $26,591 to $33,239 for medication switches, and $5,290 to $6,613 for medication discontinuations.

CONCLUSIONS: HSSP pharmacists intervene to support safe and effective specialty medication use and avoid unnecessary costs. These results can be used to rationalize the employment and support for pharmacists in the specialty pharmacy setting beyond prescription fulfillments.

SPONSORSHIP: None

U25 Access to specialty medicines with alternative funding programs: A descriptive survey of patient experiences

Dalglish H[1], *Gibbs S*[1], *Yermilov I*[1], *Bienvenu L*[2], *James J*[2], *Wong W*[3]; *hannah@pharllc.com*; *wong.william@gene.com*
[1]*Partnership for Health Analytic Research*; [2]*Hope Charities, Prairieville, LA*; [3]*Genentech*

BACKGROUND: Alternative funding programs (AFPs) attempt to lower plan sponsor costs by excluding specialty medicines from a beneficiary's plan coverage and obtaining those medicines through alternative sources (typically, manufacturer patient assistance programs) via a third party (ie, AFP vendor). Patients' experiences with and access to medicines through these programs have not been previously described.

OBJECTIVE: To describe patients' experiences and medication access with AFPs.

METHODS: A US national survey consisting of optional single- and multiple-choice questions with branching logic was administered (October to December 2023) to patients who had reported experience with AFPs. Patients were recruited online from a patient panel and patient advocacy group. Broadly, the survey evaluated patients' (1) awareness of AFPs, (2) experience with the patient assistance programs application process via the AFP vendor, and (3) timeliness of medication access if granted and/or the health impact of any delay in medication access. All responses were analyzed descriptively (proportions, means) and reported only for patients who responded to the question(s).

RESULTS: A total of 227 patients were included in the final sample. Most (61%) did not learn about AFPs through their employer and instead first learned about them as part of their health plan benefit when trying to obtain their specialty medication. More than half of patients (54%) reported being uncomfortable with the representative from the AFP vendor, including feeling hesitant providing them with sensitive information. Patients reported a mean wait time of approximately 2 months (68.2 days) to receive their medication and a negative impact on their health (24% reported waiting worsened their condition and 64% reported that it led to stress and/or anxiety). Those who reported that waiting led to a negative impact on their health considered leaving or had left their job at a rate 3 to 5 times higher than those who did not. Eighty-eight percent of patients reported being stressed or anxious because of the medication coverage denial and the uncertainty of being able to obtain their medication.

CONCLUSIONS: Most patients obtaining their specialty medicines via AFPs reported being uncomfortable with the process and had delays in obtaining their medication, which may be linked to worse mental well-being, worsening disease progression, and consideration of a job change. Employers should consider delays in medication access along with the potential downstream impacts on employee retention and employee-employer relationships when considering implementing an AFP into their health plans.

SPONSORSHIP: Genentech, Inc.

U26 Trends in commercial health plan costs and patient liability in states with or considering prescription drug affordability boards

Sheinson D[1], *Xia Z*[2], *Wong W*[1]; *sheinson.daniel@gene.com*; *wong.william@gene.com*
[1]*Genentech*; [2]*Genentech, Inc.*

BACKGROUND: A number of states have enacted or considered legislation enabling the formation of prescription drug affordability boards (PDABs) to assess the affordability of specific drugs. One goal of PDABs is to address affordability for consumers; however, the opportunity and potential for PDABs to do so is unknown, especially in commercial markets where less is known about plan and patient cost trends at the state level.

OBJECTIVE: To describe the state-level trends in commercial health plan spend and patient costs (out-of-pocket costs including financial assistance) by drugs vs nondrug services.

METHODS: A retrospective analysis of 2022 pharmacy and medical claims from the IQVIA PharMetrics Plus database assessed health plan spend and patient costs in states that have either enacted or considered PDAB legislation in 2023 (MI, IL, WI, VA, VT, PA, NE, WA, OR, CO, MN, and MD). Patients were required to be continuously enrolled in a self-funded or fully insured commercial plan during 2022. Costs were categorized into nondrug health care services (inpatient, emergency department, ancillary, provider services) and drug (pharmacy and office administered) and analyzed descriptively as a percent of health plan spend or patient liability for each state. Summary means were estimated by averaging the proportions across the states. Cost sharing was defined as the proportion of the total cost of a health care service or drug that is the patient's financial responsibility.

RESULTS: A total of 7,070,286 patients were included, with the number included in each state varying from 1.5 million (PA) to 77,000 (VT). Nondrug health care services made up two-thirds of total health plan spend (drugs + nondrugs) in each state (mean 65.6%; range: 57.6% [MD] to 73.1% [WI]) and approximately 80% of all patient costs (drugs + nondrugs) in each state (mean 79.4%, range: 72%-88%). In each

state, patients' share of the cost for nondrug health care services was larger than that for drugs (mean nondrug vs drug: 17.2% vs 9.3%), with the largest percentage point differences (nondrug vs drug) in VT (12.8%), CO (9.7%), and MD (9.3%). Nondrug cost sharing in fully insured plans was larger vs self-funded in each state, with percentage point differences ranging from 1% to 9.8%.

CONCLUSIONS: Compared with drugs, nondrug health care services represent a larger share of health plan spend and patient costs in each state, and thus, the potential to reduce patients' costs for specific drugs via PDABs may be relatively limited. Future policy efforts to reduce patients' nondrug health care costs (ie, via benefit design reform) may result in more savings to patients relative to PDABs.

SPONSORSHIP: Genentech, Inc.

U27 Real-world familiarity with US biosimilar regulatory guidelines and interchangeability state laws among pharmacists and physicians treating immunological disorders

Ling Y[1], *Gentile D*[2], *Kotomale A*[2], *Sharpe J*[2], *Geli D*[1], *Gruben D*[1], *Omary C*[2], *Brackin C*[2], *Bailey M*[2]; *you-li.ling@pfizer.com*
[1]*Pfizer Inc*; [2]*Cardinal Health*

BACKGROUND: The US Food and Drug Administration (FDA) considers an interchangeable biosimilar to produce the same clinical result as the reference product and can be automatically substituted by pharmacists, where state law permits. There is little research describing pharmacists' and physicians' perceptions of and experience with biosimilars in real-world settings.

OBJECTIVE: To assess US providers' perceptions and decision-making around prescribing and dispensing of biosimilars, including those with interchangeability designation.

METHODS: US outpatient pharmacists and physicians who prescribe biologics for dermatological, gastroenterological, rheumatological, or other immunological disorders responded to a cross-sectional electronic survey. Professional characteristics, familiarity with FDA guidelines, treatment substitutions workflow, and perceived barriers to dispensing interchangeable biosimilars were summarized descriptively.

RESULTS: One hundred fifty physicians and 99 pharmacists (total n = 249) from diverse practice settings responded to the survey. Continuing education units related to biosimilars were obtained by 65.7% of pharmacists and 50.7% of physicians. A higher percentage of pharmacists (35.4%) than physicians (20.0%) rated themselves as "extremely familiar" with pharmacy retention of communication records. Most pharmacists (59.6%) had a workflow in place for carrying/dispensing biosimilars with an interchangeability designation. A greater proportion of pharmacists (47.5%) than physicians (31.3%) were "extremely likely" to recommend a biosimilar product to new-start patients (ie, never treated with a reference biologic and/or biosimilar). Among all providers the barriers to biosimilars most often perceived to be "extremely significant" were payer coverage/formulary placement (51.0%) and cost to the patient (41.0%). Across all providers, the perceived strategies "extremely likely" to improve biosimilar uptake were FDA guidance for switching patients (ie, receiving a biosimilar switched from a reference biologic) between biosimilars with interchangeability designation (40.2%) and reducing patient cost (38.6%).

CONCLUSIONS: Pharmacists reported higher rates of familiarity and training with biosimilars and recommendation of biosimilars to patients than physicians. A diverse sample of physicians and pharmacists expressed perceived barriers and strategies to improve biosimilars uptake. Further research is needed to determine whether providers' perceptions of biosimilars are associated with actual biosimilars uptake.

SPONSORSHIP: Pfizer Inc

U28 Meta-analysis of all-cause mortality in inpatients with COVID-19 treated with remdesivir vs not treated with remdesivir

Papadakis-Sali A[1], *Thaliffdeen R*[1], *Curteis T*[2], *Jafry Z*[3], *Zolotor A*[3], *Jarrett J*[4]; *arman.psali@gilead.com*
[1]*Gilead Sciences*; [2]*Costello Medical Ltd.*; [3]*Costello Medical, Inc.*; [4]*Gilead Sciences, Inc.*

BACKGROUND: Remdesivir is an intravenously administered nucleotide prodrug used to treat patients with COVID-19. The comparative effectiveness of remdesivir vs no remdesivir in preventing all-cause mortality among inpatients with COVID-19 in real-world settings requires further investigation.

OBJECTIVE: To conduct a meta-analysis (MA) of observational studies to estimate the difference in risk of mortality in inpatients with COVID-19 taking remdesivir vs those not treated with remdesivir.

METHODS: A systematic literature review adhering to the Cochrane Principles was conducted in December 2022. The SLR identified 7 observational studies reporting on all-cause mortality at Week 4 in remdesivir-treated inpatients with COVID-19 and those not treated with remdesivir. Following a heterogeneity assessment of study and patient characteristics, an MA in R generated a pooled estimate of the odds ratio of all-cause mortality for patients treated vs not treated with remdesivir.

RESULTS: Of the 7 studies identified in the systematic literature review, 2 were excluded owing to overlap in patient populations; 5 unique studies reporting on a total of 74,066 remdesivir-treated patients and 64,242 patients not treated with remdesivir were included in analyses. Patient age and sex were homogeneous across studies. However, studies varied in design (eg, prospective vs retrospective cohorts) and propensity score matching methods; sample size, race and ethnicity, baseline disease severity, and oxygen status were also potential sources of heterogeneity. Additionally, information on treatments received by patients not receiving remdesivir, which may have differed based on geography and date of hospital admission, was not well reported and likely introduced clinical heterogeneity. In the MA, the random effects model was selected over the fixed effects model given the substantial between-study heterogeneity (I squared = 90%). The pooled random effects estimate for the odds ratio was 0.83 (95% CI = 0.75-0.92), a statistically significant result suggesting that, compared with inpatients not treated with remdesivir, those taking remdesivir had 17% lower odds of all-cause mortality at Week 4.

CONCLUSIONS: Compared with no remdesivir, remdesivir is effective in reducing the risk of all-cause mortality by Week 4 in inpatients with COVID-19 in real-world settings. Despite the presence of within- and between-study heterogeneity, this finding aligns with clinical trial results.

SPONSORSHIP: Gilead Sciences, Inc.

U29 Health-related social needs and quality measure attainment among dual-eligible Medicare Advantage beneficiaries

Suehs B[1], *Cowburn S*[2], *Canterberry M*[2], *Swankoski K*[3], *Rastegar J*[2], *Franklin S*[4]; *bsuehs6@humana.com*
[1]*Humana Healthcare Research, Inc*; [2]*Humana Healthcare Research*; [3]*Humana*; [4]*Humana, Inc.*

BACKGROUND: The dual-eligible (DE) Medicare Advantage (MA) population is socially and clinically vulnerable, accounting for a disproportionate share of acute care use and costs. Unmet health-related social needs (HRSNs) may increase the risk of developing chronic conditions and reduce an individuals' ability to manage these conditions. As health plans prepare for the new Health Equity Index measure for Star Ratings, which holds MA contracts accountable for quality measure (QM) performance among members with social risk factors (such as dual eligibility), understanding the impact of HRSNs on QM attainment in this population is necessary.

OBJECTIVE: To explore the relationship between HRSNs and QM attainment in a DE MA population.

METHODS: This was a cross-sectional retrospective study of DE MA members enrolled during calendar year 2019 and who completed a self-reported HRSN survey between November 2019 and February 2020. QM attainment was assessed during calendar year 2019 on 6 measures: medication adherence for diabetes, statins, hypertension, diabetes eye examination, colorectal cancer screening, and breast cancer screening. Bivariate and adjusted logistic regression analyses controlling for sociodemographic and clinical factors were conducted to examine the relationship between the presence of HRSNs and QM attainment.

RESULTS: The study included 10,321 DE individuals, with 36.2% aged younger than 65 years, 68.5% female, and 62.2% White. Eligibility of patients for each of the QM-specific analyses ranged from 2,031 (diabetes medication adherence measure) to 5,752 (statin medication adherence measure). Overall, 80.6% of individuals reported at least 1 HRSN and 38.4% reported at least 3 HRSNs. In adjusted analyses, greater HRSN burden (having a greater number of HRSNs vs no HRSNs) was significantly associated with lower odds of attainment of each of the 6 QMs assessed. When separately assessing the individual HRSNs, having unreliable transportation was consistently associated with lower odds of QM attainment for all outcomes. The other HRSNs had more varied relationships. Adherence to statin and hypertension medications were associated with the greatest number of statistically significant relationships with the individual HRSNs assessed.

CONCLUSIONS: These study results offer insights to payers, providers, and policymakers on the relationship between HRSNs and beneficiaries' receipt of evidence-based and appropriate care. These insights are timely with the 2023 announcement of a new Health Equity Index for the Star Ratings to incentivize plans to focus on improving QM performance among this vulnerable population.

SPONSORSHIP: Johnson & Johnson Services, Inc.

U30 Medical drug coding and adjudication review

Carlson D; Debra.Carlson@healthalliance.org
Health Alliance Medical Plans

BACKGROUND: Medical drug spend has continued to trend upward over the past several years. There is often limited options to manage medical drugs effectively. Health Alliance Medical Plans spend related to medical drugs was more than $140 million in 2021 and raised to more than $158 million in 2022. One of the difficulties around spend control is related to the medical drug coding and claim auditing process.

OBJECTIVE: To review medical drug codes to assess coding errors and identify opportunities for process improvement that could lead to better management of medical drug codes and potential plan savings.

METHODS: A total of 990 Healthcare Common Procedure Coding System codes were identified as defining a medically billed drug and included within scope of the assessment. These codes were compared with the pharmacy benefit formulary files to identify any type of coding error. Pharmacy benefit formulary files served as the source of truth to reflect coverage intent as determined by the plan's Pharmacy and Therapeutics Committee. A coding error included any incorrect setup including missing prior authorization status or location, wrong tier adjudication, missing vendor designation, etc. In addition to assessing coding, the process for code updates and departmental communication was also evaluated in attempt to identify any areas to improve efficiency or accountability.

RESULTS: Of the 990 codes included, 124 codes with usage in 2022 were determined to have some type of coding error, establishing an error rate of ~12.5%. Based on internal discussion, there were 49 codes prioritized to be corrected for January 1, 2024. These corrections decreased the error rate to 7.58%. Corrections were estimated to lead to a plan savings of more than $900,000. There were also several procedural updates made to improve communication among departments and allow for more inclusive problem resolution.

CONCLUSIONS: Medical drug management continues to be difficult given the differences between medical and pharmacy benefit structure. A regular and thorough comparison of both benefits can lead to improved consistency and better cost management.

SPONSORSHIP: None

U31 Adherence to opioid use disorder treatment with BRIXADI, an extended-release weekly and monthly injectable buprenorphine

Budilovsky-Kelley N[1], *Frost* M[2], *Lovvoll* V[1], *Cohen* J[1]; *nbudilovsky@braeburnrx.com*
[1]*Braeburn*; [2]*Wayspring*

BACKGROUND: Buprenorphine treatment for opioid use disorder (OUD) success depends on maintaining therapeutic plasma levels. In OUD, physical changes occur to brain areas critical to judgment, decision-making, memory, and behavior control, so choosing or remembering to take daily buprenorphine may be an obstacle. Clinical consequences of nonadherence may involve return to nonprescribed opioid use. Prior studies found sublingual buprenorphine adherence to range between 21% and 43%. In the treatment of conditions other than OUD, poor adherence has been associated with increased dosing frequency. Long-acting injectables have been used successfully in difficult-to-treat psychiatric diseases to address adherence challenges. BRIXADI, known as CAM2038 during development, is an extended-release buprenorphine that has been available in the United States in multiple dosage strengths in weekly and monthly formulations since September 2023.

OBJECTIVE: To evaluate adherence among patients initiating OUD treatment with CAM2038 weekly or monthly.

METHODS: Patients who had received at least 2 shipments of CAM2038 weekly or monthly between September 5, 2023, and February 23, 2024, and where longitudinal shipment history was available were included in the analysis. Adherence to CAM2038 was determined using the Medication Possession Ratio (MPR), calculated based on total days supplied divided by total days lapsed between product ship dates. MPR for patients initiating treatment with CAM2038 weekly, CAM2038 monthly, and pooled was evaluated.

RESULTS: A total of 4,928 patients met inclusion criteria for the analysis; 1,704 patients were initiated with CAM2038 weekly and 3,224 patients with CAM2038 monthly. The MPR for CAM2038 weekly and monthly was 75% and 94%, respectively. The MPR for the pooled population was 88%.

CONCLUSIONS: Both weekly and monthly CAM2038 demonstrated high adherence. Consistent with longer dosing intervals supporting adherence, patients initiating with CAM2038 monthly exhibited higher adherence than patients initiating treatment with CAM2038 weekly.

SPONSORSHIP: Braeburn, Inc

U32 Impact of copay maximizer programs on total patient liability

Sheinson D, *Patel* A, *Wong* W; *sheinson.daniel@gene.com*; *wong.william@gene.com*
Genentech

BACKGROUND: Copay maximizers (CMs) prevent the financial value of a drug copay card from counting toward a patient's deductible or out-of-pocket (OOP) maximum, while also setting a patient's liability to a value unrelated to the cost of the drug (ie, the maximum value of the copay card applied evenly throughout the year). Although patients may not face low or no OOP costs for the CM drug, it is unknown how CMs may impact patients' liability for other health care services.

OBJECTIVE: To assess the impact of CMs on non-maximizer-drug (NMD) patient liability.

METHODS: This was a retrospective analysis of pharmacy and medical claims from the IQVIA PharMetrics Plus database. Patients were required to have at least 3 prescriptions from a select list of autoimmune, multiple sclerosis, or oral oncolytic drugs in a given calendar year between 2018 and 2022 and be continuously enrolled in a commercial plan during the calendar year. CM patients were identified based on specified patterns of drug patient liability throughout a calendar year. Incident CM patients were identified via lack of exposure to a CM program in the year immediately preceding exposure to a CM program (baseline period), and were then matched 1:1 to non-CM patients. A difference-in-differences (DiD) approach was employed to assess the effect of CM programs on all NMD patient liability using a generalized linear mixed-effects model adjusted for baseline factors including baseline maximizer drug patient liability (BMDPL). Three-way interactions between groups (CM or non-CM), baseline NMD liability, and BMDPL were used to assess changes in the CM effect on NMD patient liability. A second model accounting for changes in utilization allowed for a scenario analysis assuming no change in total costs from baseline to follow-up.

RESULTS: A total of 6,366 patients were included in the analysis (3,183 matched patients per group). Overall, the effect of CMs resulted in 1.23- to 1.37-fold increases in NMD liability for BMDPLs of $500-$2,000. Assuming a baseline NMD liability of $1,000, this represented an increase in NMD of $199 to $257. Assuming no change in total costs from baseline to follow-up, the effect of CMs increased as the BMDPL increased, ranging from no effect at a BMDPL of $125 (DiD [95% CI] = 1.0 [0.71-0.14]) to a 47% increase in NMD liability at a BMDPL of $4,000 (1.47 [1.15-1.87]). This represented up to a $243 increase in NMD liability (assuming a baseline NMD liability of $1,000).

CONCLUSIONS: CMs may increase patient liability for other health care services and should be factored into decisions and policies on implementing and regulating these programs.

SPONSORSHIP: Genentech, Inc.

U33 Methodological framework for propensity-adjusted benchmarks for pharmacy key performance indicators

Hunter B, Brown-Gentry K, Scripture J, Eckwright D, Prasla K;
brooke.hunter@primetherapeutics.com
Prime Therapeutics

BACKGROUND: Risk profiles can differ across populations because of variations in characteristics like disease prevalence and demographics. Many industries (eg, health care and insurance) control for these extraneous influences by applying risk adjustments when comparing a population of interest to a benchmark on key performance indictors (KPIs). This method could help pharmacy organizations provide more accurate insights when comparing different populations.

OBJECTIVE: To create a methodological framework for using propensity weights to adjust for varying risk profiles when comparing an index group with a benchmark group on pharmacy KPIs

METHODS: A commercial sample was derived from continuously enrolled members in a medical drug management program (mean age = 35 years; SD = 20) serving diverse customers during 2022. The program sought to reduce spend through a set of services (eg, fee schedule, prior authorization, and step therapy). Customer A (n = 4,746) and customer B (n = 2,030,112) were used as index groups in 2 separate comparisons. Propensity weights, which are the probability that a particular individual matches to a population of interest based on a set of covariates, were created to identify a matched benchmark for each customer. Covariates were chosen based on the following criteria: significantly predicted group membership, had a theoretical relationship to the dependent measure, and had overlap between the index group and benchmark group. A logistic regression model was used to create the propensity weights based on covariates (disease prevalence, demographics) predicting group membership. The index group weights were recoded to 1 so that only the benchmark outcomes were weighted. The dependent measure for the benchmark comparison was the per-member per-month (PMPM) cost for medical drugs.

RESULTS: After applying the weights to the benchmark sample, all differences in covariates between the index and benchmark were reduced to Cohen's $d < 0.10$. Customer A's PMPM allowed amount was $18.60, the unweighted benchmark PMPM was $39.67, and the weighted benchmark was $23.03. Customer B's PMPM allowed amount was $46.46, the unweighted benchmark PMPM was $37.33, and the weighted benchmark was $47.33.

CONCLUSIONS: When comparing an index group with a benchmark with varying risk profiles, KPIs may not reflect the index group's performance but instead reflect differences in population composition. Accounting for confounding differences through a propensity weight can control for varying risk across groups and distill a more meaningful comparison by comparing the index group with a benchmark with a similar risk profile.

SPONSORSHIP: Prime Therapeutics

U34 Trends in US health plan coverage for adalimumab products

LaMountain F, Beinfeld M, Chambers J; Fariel.LaMountain@tuftsmedicine.org; molly.beinfeld@tuftsmedicine.org
Center for the Evaluation of Value and Risk in Health, Institute for Clinical Research and Health Policy Studies, Tufts Medical Center, Boston, MA

BACKGROUND: The first adalimumab biosimilar launched in the United States in January 2023, ending Humira's market exclusivity. Throughout 2023, 7 additional biosimilars entered the market, with more expected in 2024. Despite the increase in competition and potential cost savings, there are concerns about the limited uptake of adalimumab biosimilars, with health plans playing a key role.

OBJECTIVE: To examine the frequency with which US commercial health plans have granted Humira preferred coverage since the introduction of adalimumab biosimilars.

METHODS: We created a dataset of adalimumab coverage policies issued by 15 large US commercial health plans at 3 timepoints in 2023, using the Tufts Medicine Specialty Drug and Evidence database. The dataset included 8 biosimilars and Humira. We analyzed each US Food and Drug Administration–approved indication separately, considering only coverage decisions where payers covered both Humira and at least 1 biosimilar. We categorized each decision as follows: (1) "Non-Preferred: ≥1 Biosimilar Preferred Over Humira," meaning the plan covered 1 or more biosimilars as a preferred treatment prior to granting access to Humira, (2) "Co-Preferred: Humira Same Line as ≥1 Biosimilar," indicating the plan covered 1 or more biosimilars AND Humira as preferred treatments, or (3) as "Preferred: Humira Sole Preferred," where the plan covered Humira as the sole preferred treatment before granting access to biosimilars. We examined the frequency that plans granted Humira preferred status and how it changed over 2023.

RESULTS: The number of coverage policies for adalimumab products increased from 88 in April to 126 in December 2023 as additional biosimilars became available. Plans increasingly preferred biosimilars alongside or over Humira, while the frequency of granting Humira sole preferred status declined. In April, 27% of policies included Humira as the "sole preferred" treatment, but by December, none did. By December, 14 of 15 plans copreferred Humira and 1 or more biosimilars and 1 plan preferred at least 1 biosimilar over Humira.

CONCLUSIONS: As adalimumab biosimilar competition has grown, health plan coverage has evolved to offer patients a choice between multiple biosimilars and Humira. The low utilization of adalimumab biosimilars so far may be attributed to patient and physician preference for brand-name products given the option, as well as declines in the net price of Humira. Further research on the relationship between prices, utilization, and coverage may provide more insights into future trends in the US biosimilars market.

SPONSORSHIP: None

U35 Evaluation of vilobelimab as a cost-effective option to treat severely ill mechanically ventilated patients with COVID-19

Biskupiak J[1], Malone D[1], Brixner D[1], Oderda G[1], Seheult R[2], Burnett B[3]; Dan.Malone@utah.edu; diana.brixner@utah.edu
[1]University of Utah College of Pharmacy; [2]UC Riverside School of Medicine; [3]InflaRx Pharmaceuticals Inc

BACKGROUND: Gohibic (vilobelimab) has been authorized for emergency use by the US Food and Drug Administration under an Emergency Use Authorization for the treatment of COVID-19 in hospitalized adults when initiated within 48 hours of receiving invasive mechanical ventilation or extracorporeal membrane oxygenation. Vilobelimab is an anti-C5a complement factor blocker that demonstrated a significant reduction in 28-day all-cause mortality in the PANAMO phase 2 global study (N = 368) in adult critically ill patients with COVID-19 in addition to standard of care (SoC) of 32% compared with placebo at 42% (hazard ratio [HR] = 0.67, 95% CI = 0.48–0.96, *P* = 0.027). In PANAMO, SoC included use of corticosteroids (97%), antithrombotic agents (98%), and immunomodulators (~20%; tocilizumab or baricitinib). In a post hoc analysis to evaluate whether any prior or concomitant treatment with immunomodulators provided additional survival benefit on top of vilobelimab (n = 34) and SoC vs placebo and SoC (n = 37), the point estimate for 28-day all-cause mortality was 6.3% vs 40.9% (HR = 0.13, 95% CI = 0.03–0.56, *P* = 0.006).

OBJECTIVE: To evaluate the cost-effectiveness of vilobelimab added to SoC based on the PANAMO clinical study data in both the total study population and the immunomodulator subpopulation.

METHODS: A short-term acute care decision tree followed by a postdischarge 2-state Markov cohort model was used to estimate quality-adjusted life-years (QALYs) and the incremental cost-effectiveness ratio (ICER) of the treatment arms. The model simulated progression from severe COVID-19 to survival or death over a lifetime. Outcomes data (COVID-19 all-cause mortality) were incorporated from PANAMO. Post–COVID-19 mortality was based on Centers for Disease Control and Prevention age-specific survival data. Utility values and hospital costs came from the literature. Vilobelimab cost was obtained from RED BOOK online.

RESULTS: For the total study population, total costs of care were $103,414 (SoC) and $132,247 (SoC plus vilobelimab), respectively (incremental cost $28,833). SoC provided 6.70 QALYs vs 7.99 QALYs for vilobelimab (additional 1.29 QALYs). The ICER for vilobelimab plus SoC compared with SoC alone was $22,287/QALY. Probabilistic sensitivity analysis (PSA) demonstrated the robustness of the cost-effectiveness result as vilobelimab plus SoC was favored at a willingness-to-pay (WTP) threshold of $50,000 in more than 81% of iterations. In the immunomodulator (IM) subpopulation, the ICER for vilobelimab plus SoC and IM compared with SoC and IM alone was $7,892/QALY.

CONCLUSIONS: Vilobelimab provides a cost-effective option to treat patients with severe COVID-19 in the intensive care unit receiving invasive mechanical ventilation compared with SoC, at well below the commonly accepted $50,000 US WTP threshold.

SPONSORSHIP: InflaRx Pharmaceuticals Inc.

Z00-Z99 Factors Influencing Health Status and Contact With Health Services

Z1 Specialty medication adherence improvements via a targeted, telehealth motivational interviewing program: Scalability across diverse, at-risk therapeutic populations

Pitts S[1], *Amato D*[2], *Martin N*[1], *Mager D*[3], *Bridges G*[1]; *stefanie.pitts@accredo.com*
[1]*Accredo*; [2]*Evernorth Health, Inc.*; [3]*Evernorth Inc*

BACKGROUND: Motivational interviewing programs show benefit in numerous limited-scope trials in single therapeutic areas. Our study is one of the largest motivational interviewing studies to date, expanding the evidence across more than 300 high-acuity specialty therapy classes in a telehealth practice setting.

OBJECTIVE: To evaluate the impact of supplemental, risk-stratified motivational interviewing on proportion of days covered (PDC) in specialty patients at risk for worsening adherence, despite preexisting specialty clinical support.

METHODS: An adherence-risk score was deployed to identify patients with negative adherence trends across eligible therapeutic classes. Patients scored at risk as of the study index period (December 1, 2022, to March 31, 2023) were followed proactively for 6 months. Patients transferring pharmacies, deceased, or opting out of clinical support in the index period were excluded. A matched, scored control population was compared with those receiving clinician-led motivational interviewing outreach. Six-month PDC was measured before and after the first motivational interview outreach (completed interventions, attempted left message) or score date (controls).

RESULTS: In all, 40,908 eligible patients scored at risk; 19,137 controls and 21,771 stratified to outreach, of which 5,055 completed clinical intervention. The average age was 48.4 (16.3) years for controls, 48.6 (16.4) for interventions, and 46.1 (16.0; $P<0.05$) for attempts, with 41.6%, 45.1% ($P<0.05$), and 41.4% male, respectively. As expected, at-risk patients' baseline PDC was poor, ranging from 60.8% to 63.0%. Postperiod PDC rose to 68.0% for interventions but declined to 49.8% for controls and 54.9% for attempts. Age, sex, and baseline adherence-adjusted postperiod PDC increased 18.2% in completed interventions vs controls ($P<0.01$) and was 5.1% higher for attempts vs controls ($P<0.01$). Successful intervention converted 16.5% more patients to optimal PDC (>80%) compared with controls ($P<0.01$).

CONCLUSIONS: Motivational interviewing by trained telehealth clinicians showed meaningful 18.2% adherence increase for specialty patients scoring at risk of adherence decline. Without successful intervention, adherence continued to decline but was attenuated by clinical messaging, as demonstrated in the attempt population. Completed motivational coaching also improved attainment of optimal adherence within 6 months, compared with matched controls. Of note, controls became eligible for outreach after study completion.

SPONSORSHIP: Evernorth Health Services

Z2 Association between proteinuria, kidney function, and health care resource utilization among patients with primary immunoglobulin A nephropathy

Cannizzaro N[1], *Chen Q*[1], *Nunna S*[2], *Gandhi H*[3], *Mathur M*[4], *Sim J*[5]; *Sasikiran.nunna@otsuka-us.com*
[1]*Kaiser Permanente*; [2]*Otsuka Pharmaceuticals*; [3]*Otsuka Pharmaceutical Development & Commercialization, Inc.*; [4]*Visterra Inc*; [5]*Kaiser Permanente Los Angeles Medical Center*

BACKGROUND: Immunoglobulin A nephropathy (IgAN) is the most common primary glomerulonephropathy worldwide, with most patients progressing to kidney failure within their lifetime. The clinical severity of IgAN at diagnosis (based on proteinuria and estimated glomerular filtration rate [eGFR]) and subsequent clinical course could impact disease management and health care resource utilization (HCRU).

OBJECTIVE: To characterize patients with IgAN and determine associated HCRU within a single large integrated health care system.

METHODS: A retrospective cohort study (January 1, 2000, to December 31, 2021) was conducted within Kaiser Permanente Southern California of patients (aged ≥18 years) with biopsy-proven primary IgAN and eGFR greater than or equal to 15. The HCRU outcomes included emergency department visits, inpatient visits, outpatient visits, radiology, laboratory, and medication dispensations (reported as per patient per month [PPPM]) within 2 years after biopsy. Kruskal-Wallis tests compared differences in utilization PPPM by severity categories (proteinuria level and eGFR). Associations between baseline characteristics (proteinuria levels, eGFR, comorbidity, sex, and age) and HCRU (total number of visits per resource type per patient within 2 years) were assessed by negative binomial regression.

RESULTS: The study included 617 patients with biopsy-proven IgAN. The mean (SD) age was 45.5 (14.3) years with 52.5% male, Hispanic (39.9%), and Asian/Pacific Islander (30.6%) patients. Baseline median eGFR was 53.4 mL/min/1.73 m^2 (36.5-78.9) and median urine protein creatinine ratio (uPCR) was 1.7 g/day (0.9-3.3). The overall PPPM was 2.04, with higher PPPM among lower eGFR categories (1.8 to 2.9). A similar trend was observed for proteinuria, with PPPM of 2.39 (1.49), 1.89 (1.62), and 1.84 (1.54) for uPCR greater than 2, 1-2, and less than 0.5-1 g/g, respectively. Compared with those with baseline uPCR less than 1, those with uPCR greater than or equal to 1 had higher rates of HCRU: outpatient, 1.19 times as high (1.05-1.35); radiology, 1.43 times as high (1.21-1.69); laboratory visits, 1.60 times as high (1.39-1.85); and outpatient medication dispensing, 1.47 times as high (1.29-1.68).

CONCLUSIONS: Among a diverse IgAN population within a single integrated health system, we observed a relationship between lower baseline eGFR and higher levels of proteinuria with greater HCRU suggestive that HCRU may increase with higher clinical burden of IgAN. Proactive treatment of "low-risk" patients deemed (<1 g/day proteinuria) may minimize both subsequent clinical and health care resource burdens associated with IgAN.

SPONSORSHIP: Otsuka Pharmaceutical Development & Commercialization, Inc.

Z4 Building up social determinants of health data over time and space for linkage with real-world data and health equity enrichment

Ng C[1], *Zhang P*[2], *Kowal S*[3]; *ngc28@gene.com*
[1]*Genentech, Inc.*; [2]*Genentech*; [3]*Genentech, Inc*

BACKGROUND: Social determinants of health (SDOH) are key drivers that shape many health outcomes, and indices exist to evaluate areas' relative social deprivation. The Centers for Disease Control and Prevention (CDC) produces estimates of the Social Vulnerability Index (SVI) for counties biennially. However, datasets commonly used in real-world data (RWD) research, including claims and electronic health records, may not include a patient's county, making it challenging to incorporate SDOH. Although county is a useful administrative and political unit, choice of geography is limited by data availability. For example, risk of patient reidentification may require that analyses be conducted at higher geographic aggregations (eg, zip-3).

OBJECTIVE: To leverage CDC documentation, geographic crosswalks, and imputation to create county-, zip-5–, and zip-3–level datasets of SVI and its components for 2016-2021 to validate the methodology beyond county and explore SDOH trends over time and space.

METHODS: The SVI consolidates 16 SDOH variables from the American Community Survey, such as unemployment and proportion of seniors, and categorizes them into 4 themes: socioeconomic status, household characteristics, racial and ethnic minority status, and housing type and transportation. We extract American Community Survey estimates of the 16 variables for counties and zip code tabulation areas (which are crosswalked to zip-5 and aggregated to zip-3). Areas receive a percentile ranking (0-1) for each variable, composite theme, and SVI itself, with higher values indicating greater social vulnerability.

RESULTS: We derive SVI for up to 3,143 counties, 32,243 zip-5s, and 886 zip-3s. SDOH trends across the nation remain largely consistent, despite slight local changes over time. SVI varies drastically across regions, with generally higher vulnerability in the South and lower vulnerability in the North and Northeast. Individual components can indicate the SDOH domains driving specific areas' SVI outcomes. Derived estimates at the county level are validated against CDC reported values, confirming accuracy of the methodology.

CONCLUSIONS: We demonstrate that SVI can be created for other geographies, and thus linked with RWD, to provide area-level SDOH context, allowing for more robust health equity research. With SDOH enrichment, evidence can be expanded to inform the current state of health disparities, target areas of unmet need, and assess the benefit of health interventions, policies, or outreach programs to break down systemic barriers. The longitudinality further allows for monitoring these metrics and assessing structural changes over time.

SPONSORSHIP: None

Z5 Unlocking public health insights: Leveraging air quality data to develop social determinants of health metrics linked to asthma medication ratio

Brown-Gentry K, Hunter B, Scripture J, Santilli M, Prasla K;
kristin.browngentry@primetherapeutics.com
Prime Therapeutics

BACKGROUND: Environmental conditions such as air quality can impact lung health, making it a dire social determinant of health (SDoH) with exaggerated implications for chronic lung diseases, such as asthma. Air pollutants can increase inflammation of lung tissue, contribute to coughing, and exacerbate symptoms of asthma.

OBJECTIVE: To detail how publicly available data can be used to create measures of SDoH and assess the association of derived metrics with asthma medication ratio (AMR).

METHODS: A nationally representative commercial sample of continuously enrolled patients aged 18 years and older was analyzed. Patients with asthma were identified using claims from 2022 and the Healthcare Effectiveness Data and Information Set AMR inclusion criteria for dispensing events: at least 4 claims for asthma medications. Patients had at least 2 dates of service for a controller, with the first date of service being 91 days prior to year-end. AMR was calculated as the ratio of the quantity of controller to the total quantity of asthma medications. Patient data were joined to Environmental Protection Agency 2022 Air Quality Index (AQI) data using a Census Business Statistical Area to zip code crosswalk. EPA data for patients residing in multiple zip codes were averaged. Using the Environmental Protection Agency data, the following predictors were calculated: median AQI; proportion of good, moderate, and unhealthy days for sensitive groups; and the proportion of days in which each toxin was responsible for the highest AQI value (CO, NO_2, O_3, and particle matter 2.5 and 10). Regression models were used to assess outcomes individually ($\alpha = 0.05$). All models were controlled for demographic differences (sex, age, Chronic Disease Score, and adherence to controllers).

RESULTS: A total of 14,322 patients met inclusion criteria. The sample was 62% female, had a mean age of 47 (SD = 12) years, and had a mean Chronic Disease Score of 3.5 (SD = 2), with 54% of the sample adherent at an 80% threshold to their controllers. Mean AMR was 0.86 (SD = 0.2), with 94% of the sample compliant at an AMR threshold of 0.5 or greater. Significant associations were observed for the proportion of good ($\beta = 0.0002$, $P < 0.0001$), moderate ($\beta = -0.0003$, $P < 0.0001$), and unhealthy for sensitive group days ($\beta = -0.0007$, $P = 0.01$). Median AQI was a significant predictor of AMR ($\beta = -0.0003$, $P = 0.03$). Significant associations were observed for the following toxins: CO ($\beta = -0.0132$, $P = 0.02$), NO_2 ($\beta = -0.0005$, $P < 0.0001$), and O_3 ($\beta = -0.0001$, $P = 0.03$).

CONCLUSIONS: Publicly available AQI data can be combined with pharmacy claims data to create SDoH metrics specific to neighborhood and built environment. The created metrics were found to be associated with AMR within a commercially insured population.

SPONSORSHIP: Prime Therapeutics

Z6 Leveraging publicly available social determinants of health measures to segment members and quantify differences in diabetic medication adherence: A latent profile analysis

Scripture J, Brown-Gentry K, Hunter B, Prasla K;
jared.scripture@primetherapeutics.com
Prime Therapeutics

BACKGROUND: Social determinants of health (SDoH) measures have become increasingly necessary to implement in both prediction of health outcomes and targeting of interventions. The ability to use publicly sourced SDoH measures effectively is an area of active research, as sourcing data directly at the member level is costly and unstandardized.

OBJECTIVE: To identify latent profiles of US zip codes based on SDoH measures from American Community Survey data and to differentiate SDoH profiles by reporting medication adherence (MA) for patients with diabetes.

METHODS: Latent profile analysis (LPA) was conducted using the tidyLPA package in R for 31,923 zip codes using American Community Survey data from 2017 to 2021. Nine variables were used in the LPA including housing crowding, broadband Internet access, single-parent households, adults with no high school diploma, and persons living below 150% of the poverty level. A continuously enrolled commercial sample of patients with diabetes aged 18 years and older were identified as meeting Pharmacy Quality Alliance inclusion criteria for MA using pharmacy claims data from 2022 and 2023. The proportion of days covered (PDC) was calculated separately for each year. Patients were considered adherent if PDC was greater than or equal to 0.80. MA rates were determined for each latent profile for each year. Odds ratios (ORs) with CIs and Wald's test were used to compare adherence between profiles. ORs were also calculated for adherence year over year within each latent profile.

RESULTS: A 2-profile model requiring equal variances and covariances within classes was determined to be most appropriate for this analysis. LPA results revealed 2 types of zip codes: underserved zips (UZ; 16.6%) and advantaged zips (AZ; 83.5%). UZs had more severe metrics across all SDoH measures included in the LPA—in particular, UZs had

an average of 12.5% higher prevalence of persons living below 150% of poverty line and 9.7% higher rates of persons without a high school diploma. A total of 18,391 patients met study inclusion criteria; 4,978 members were in UZs, where the MA rate was 11% and 9% lower than for AZs for 2022 and 2023, respectively (2022 OR = 0.55, $P < 0.001$; 2023 OR = 0.60, $P < 0.001$), and 82% of members in UZs that were adherent in 2022 maintained MA in 2023 compared with 86% for members in AZs.

CONCLUSIONS: Using zip code–aggregated SDoH metrics to identify latent profiles of underserved communities could help focus support efforts where they are needed most to address health care disparities at a community level. Further efforts should be made to examine other conditions or health outcomes measures in relation to these groupings.

SPONSORSHIP: Prime Therapeutics

Z8 Social determinants of health burden in patients using CVS MinuteClinic health clinics

Dai D[1], *Fernandes J*[1], *Mikkilineni S*[1], *Kumar R*[1], *Berk A*[1], *Fairchild D*[2]; *Dingwei.Dai@cvshealth.com*
[1]*CVS Healthspire Life Sciences Solutions*; [2]*CVS Health*

BACKGROUND: Social determinants of health (SDOH) are a range of social, economic, and environmental factors that impact health and wellness. Health care systems are increasingly interested in addressing the SDOH to improve health outcomes and health equity and reduce healthcare costs. However, there are many barriers to collecting SDOH data to identify unmet health-related social needs. Geography-based neighborhood data may provide an accessible alternative to collecting individual SDOH data.

OBJECTIVE: To assess SDOH burden in patients using MinuteClinic (MC) health clinics.

METHODS: This is a retrospective analysis of MC's electronic health records (EHRs), a large US EHR database. Patients of all ages, care needs, and insurance who visited an MC from January 1, 2023, to December 31, 2023, were identified and included in the study. The Centers for Disease Control and Prevention Social Vulnerability Index (SVI), Agency for Healthcare Research and Quality SDOH database, and Social Vulnerability Metric (SVM) were appended to the MC EHR data using zip code. SVI ranges from 0 to 1 and SVM percentile ranges from 0 to 100, with a higher value indicating higher vulnerability for both scales. The individual-level and neighborhood-level SDOH were assessed.

RESULTS: Of 4,939,927 patients who received health care services in MC in 2023, 59.8% were female, mean (SD) age was 36.5 (19.3) years; 69.2% had commercial insurance and 13% had Medicaid or Medicare; and 58% were White, 13% Hispanic, 10.7% Black, 7.8% Asian, and 10.5% Other. Mean (SD) SVI was 0.38 (0.27) and SVM percentile was 27.1 (25.6). Comparing the least socially vulnerable (0 to 10 SVM percentile) with the most socially vulnerable (90 to 100 percentile) subpopulation, the prevalence of common chronic conditions was significantly increased: chronic obstructive pulmonary disease (4.3% vs 9%), diabetes (7.4% vs 14.2%), obesity (25.2% vs 37.2%), coronary heart disease (4.5% vs 7.6%), stroke (2.1% vs 4.3%), and mental health problems (9.9% vs 17%), all $P < 0.0001$. Similar patterns were observed using SVI.

CONCLUSIONS: Neighborhood-level SDOH measures based on 5-digit zip code can be incorporated into EHR data. The prevalence of common chronic conditions, such as chronic obstructive pulmonary disease, diabetes, obesity, coronary heart disease, stroke, and mental health problems, was higher in the subpopulation with higher SVI or SVM. Integrating validated publicly available SDOH data into EHRs provides an accessible option to overcome the issue of SDOH unavailability. Both SVI and SVM can be used to identify patients with high social vulnerability for additional outreach and support.

SPONSORSHIP: None

Student Poster Titles and Presenters

B2 Evaluation of attitudes, beliefs, and knowledge regarding furnishing preexposure prophylaxis among pharmacists who serve a minoritized community clinic population

Tumoyan H[1]; *htumoyan@hs.uci.edu*
[1]*UCI School of Pharmacy & Pharmaceutical Sciences*

B11 The economic burden of COVID-19 vaccine hesitancy: A systematic literature review

Wu A[1], *Ngo P*[1], *Ozawa S*[2], *Higgins C*[2]; *amywu089@gmail.com*; *paige.ngo@unc.edu*
[1]*UNC/AESARA*; [2]*University of North Carolina*

B16 Chimeric antigen receptor T-cell utilization patterns and associated outcomes

Phan C[1], *Wojcicki S*[1], *Patel A*[1], *Davis J*[2], *Wind H*[1], *Dharia, PharmD A*[1]; *phan33@purdue.edu*
[1]*Humana Inc.*; [2]*Humana, Inc.*

C31 The impact of time delays between breast cancer diagnosis and surgery on overall survival: A retrospective analysis in the Texas Cancer Registry

Mousavi P[1], *Yazdanfard S*[1], *Sujit S*[2], *Varisco T*[1]; *pmousavi@uh.edu*
[1]*University of Houston, College of Pharmacy, Department of Pharmaceutical Health Outcomes and Policy*; [2]*University of Houston*

C32 Utilizing artificial intelligence for enhanced diagnosis and treatment of breast cancer

Hughes A[1]; *ahughes@mail.lipscomb.edu*
[1]*Lipscomb University College of Pharmacy*

C35 Evaluating the impact of prior authorization on utilization of antineoplastic agents in Oregon Medicaid

Ho C[1]; *hocou@oregonstate.edu*
[1]*Oregon State University/Oregon Health and Science University*

C63 Documentation of cytogenetic testing in cancer registry data: A case example in multiple myeloma

Henderson G[1], *Onukwugha E*[2], *Ricci S*[3], *Dunn A*[2], *Qin X*[4], *Slejko J*[3]; *gabriel.henderson@umaryland.edu*; *jslejko@rx.umaryland.edu*
[1]*University of Maryland School of Pharmacy*; [2]*Department of Practice, Sciences, and Health Outcomes Research, University of Maryland School of Pharmacy*; [3]*University of Maryland School of Pharmacy*; [4]*Department of Health Policy and Management*

C64 Evaluating the impact of social determinants of health and clinical data in patients with breast cancer on CDK 4/6 inhibitor treatment adherence

Lebada L[1], *Roberts M*[1], *Boyd B*[2], *Brisco Fields M*[1], *Oster A*[3], *Coladonato C*[1]; *llebada@students.llu.edu*
[1]*MaxorPlus*; [2]*Oncology Medical Outcomes/Payers Team*; [3]*Maxor National Pharmacy*

D23 Optimizing the management of patients with hemophilia A: A descriptive analysis of patient demographics, sites of care, treatment strategies, and social determinants of health

Meng M[1], *Patel P*[2], *Lockhart C*[3]; *macymeng@buffalo.edu*
[1]*University at Buffalo School of Pharmacy and Pharmaceutical Sciences*; [2]*AMCP/AMCP Foundation Joint Research Committee*; [3]*AMCP*

E15 Analysis of the comparative efficacies between insulin glargine biosimilars

Liu A[1], *Fei A*[2]; *alexliu176@gmail.com*; *ayf12@scarletmail.rutgers.edu*
[1]*Rutgers University–New Brunswick*; [2]*Rutgers University–New Brunswick*

E16 Trends in chronic kidney disease stage–specific costs between individuals with and without diabetes

Kim M[1], *Kim K*[2]; *mkim379@uic.edu*
[1]*University of Illinois Chicago, College of Pharmacy, Department of Pharmacy Systems Outcomes & Policy / Takeda*; [2]*University of Illinois Chicago, College of Pharmacy, Department of Pharmacy Systems Outcomes & Policy*

E17 Ozempic (semaglutide): Safety, effectiveness, and advantages for weight management in type 2 diabetes

Boules J[1], *Dawod M*[1]; *jnb150@scarletmail.rutgers.edu*; *mrd251@scarletmail.rutgers.edu*
[1]*Ernest Mario School of Pharmacy*

E19 The association of glucagon-like peptide-1 receptor agonists and health-related quality of life in adults with diabetes: Insights from real-world data

Powell A[1], *Akpan N*[2], *Hearn E*[3], *Sambamoorthi U*[2]; *ardenpowell@my.unthsc.edu*
[1]*University of North Texas Health Science Center System College of Pharmacy*; [2]*UNTHSC*; [3]*University of North Texas Health Science Center System College of Pharmacy*

E39 Identifying discrepancies between actual body weight and self-perception of body weight among adults in the United States

Summers A[1], *Rodriguez de Bittner M*[1], *Shaya F*[1]; *aesummers@umaryland.edu*
[1]*University of Maryland School of Pharmacy*

E40 Two-year weight change in patients with obesity prescribed glucagon-like peptide-1 receptor antagonists in a large integrated delivery network

Ye W[1], *Reynolds T*[1], *Chen L*[1], *Godley P*[1]; *william.ye@bswhealth.org*
[1]*Baylor Scott & White Health*

E45 Rationale for coverage of atidarsagene autotemcel (Lenmeldy) for metachromatic leukodystrophy

Hsu E[1], *Johnson J*[1]; *ehsu@uams.edu*
[1]UAMS

E46 Associations between clinical and demographic characteristics and adherence to type 2 diabetes add-on therapy in metformin-experienced patients

Sinclair D[1], *Williams T*[2], *Canetto A*[3], *Fessler J*[3]; *daria.sinclair@modahealth.com*
[1]AMCP/*Moda Health/Novo Nordisk*; [2]*Moda Health*; [3]*Moda Health*

E47 Factors affecting the selection of type 2 diabetes add-on therapy in disadvantaged communities

Sinclair D[1], *Nhu Tran J*[2], *Patel P*[3], *Walker C*[2]; *daria.sinclair@bison.howard.edu*
[1]*Moda Health/Novo Nordisk*; [2]*Novo Nordisk*; [3]AMCP/AMCP *Foundation Joint Research Committee*

E48 The impact of the Inflation Reduction Act insulin cost-sharing cap on medication adherence for Medicare Advantage plan enrollees diagnosed with type 2 diabetes mellitus

Shan R[1], *Brent L*[2], *Schuster A*[3], *Frey A*[4], *Patel S*[3], *Hodge N*[5]; *ryan.shan@uconn.edu*
[1]*Cigna HealthSpring*; [2]*The Cigna Group*; [3]*Cigna Healthcare*; [4]*Cigna*; [5]*Cigna Group*

F6 Predictors of buprenorphine treatment retention among privately insured adults with opioid use disorder

Zalmai R[1], *Baffoe J*[1], *Hill L*[1], *Loera L*[1], *Johnsrud M*[1], *Avanceña A*[1]; *rana.zalmai@austin.utexas.edu*
[1]*The University of Texas at Austin College of Pharmacy*

F15 A 3-year utilization analysis of branded second-generation antipsychotics at a regional health plan

Saei K[1], *Button H*[2], *Thomas T*[3], *Miljkovic E*[2]; *ks03503@sjfc.edu*
[1]*St. John Fisher University*; [2]*CDPHP*; [3]*Capital District Physicians' Health Plan*

G15 Opioid prescribing disparities among Medicare enrollees with chronic pain and Alzheimer disease and related dementias

Decano J[1], *Calangian J*[1], *Langnas E*[2], *Chen C*[3], *Rodriguez-Monguio R*[4]; *jakob.decano@ucsf.edu*
[1]*Department of Clinical Pharmacy, School of Pharmacy, University of California San Francisco*; [2]*Department of Anesthesia and Perioperative Care, University of California San Francisco; Center for Health Equity in Surgery & Anesthesia, University of California San Francisco; Philip R. Lee Institute for Health Policy Studies, University of California San Francisco*; [3]*Department of Anesthesia and Perioperative Care, University of California San Francisco; Philip R. Lee Institute for Health Policy Studies, University of California San Francisco*; [4]*Department of Clinical Pharmacy, School of Pharmacy, University of California San Francisco; Philip R. Lee Institute for Health Policy Studies, University of California San Francisco; Medication Outcomes Center, University of California San Francisco*

G16 Evaluating the aducanumab treatment utilization: A real-world descriptive study

Dangpiaei S[1], Kamal K[1], Al-Mamun M[2]; sd00087@mix.wvu.edu
[1]*West Virginia University School of Pharmacy*; [2]*West Virginia University*

I11 Cost of congestive heart failure management by country, setting, and comorbidity: Managed care aspects and lesson learned

Nguyen C[1], White A[1], Ingram K[1], Wesling M[1], Truong T[1], Wagner T[2]; ChristinaNguyen4@my.unthsc.edu
[1]*University of North Texas Health Science Center College of Pharmacy*; [2]*University of North Texas Health Science Center, School of Public Health*

I17 Comparison of change in cardiac injury marker in glucagon-like peptide-1 receptor agonist, sodium-glucose cotransporter-2 inhibitor, and dipeptidyl peptidase IV inhibitor users in a real-world database

Walvekar M[1], Strand M[2], Kwon J[1], Watanabe J[1]; walvekam@uci.edu
[1]*Center for Data-Driven Drugs Research and Policy*; [2]*Center for Data-Driven Drugs Research and Policy*

I18 A retrospective observational analysis of tafamidis adherence and its influence on medical costs in Medicare Advantage enrollees diagnosed with transthyretin amyloid cardiomyopathy

Gaitor T[1], Sarodia A[2], Smith E[3]; tonisha.gaitor@uga.edu
[1]*United Health Group*; [2]*UnitedHealthcare*; [3]*UnitedHealthcare*

J10 Adherence in the cost-effectiveness analysis studies of Dupixent

Lee V[1], Poudel N[2], Ricci S[2], Lodise L[2], Oh J[2], Slejko J[2]; vhlee@umaryland.edu
[1]*University of Maryland School of Pharmacy - PAVE Center*; [2]*University of Maryland School of Pharmacy*

J11 Novel and patient-centered elements for chronic obstructive pulmonary disease value assessment

Lodise L[1], Poudel N[1], Oh J[1], Lee V[2], Ricci S[1], Slejko J[1]; leah.lodise@umaryland.edu
[1]*University of Maryland School of Pharmacy*; [2]*University of Maryland School of Pharmacy - PAVE Center*

J12 Applying an equity lens for chronic obstructive pulmonary disease value assessment

Oh J[1], Poudel N[1], Ricci S[1], Lodise L[1], Lee V[2], Slejko J[1]; joh2@umaryland.edu
[1]*University of Maryland School of Pharmacy*; [2]*University of Maryland School of Pharmacy - PAVE Center*

J18 Real-world health care resource utilization by older adults with respiratory syncytial virus: A retrospective claims analysis

Wildeman J[1], Diehl C[2], Tutino A[2], Lynch K[3], Heard T[4], Bender J[2], Stanton E[5]; jennamwildeman@gmail.com
[1]*Excellus BCBS NY*; [2]*Excellus BCBS*; [3]*Pfizer, Inc*; [4]*Pfizer Biopharmaceuticals Group*; [5]*University of Vermont*

K2 Impact of concomitant conventional synthetic disease-modifying antirheumatic drug use on biologic discontinuation rates in patients with Crohn disease: A retrospective claims database analysis

Diep E[1], Diehl C[2], Altieri D[2]; emilydie@buffalo.edu
[1]*University at Buffalo School of Pharmacy and Pharmaceutical Sciences*; [2]*Excellus BCBS*

L25 Genetic markers and isotretinoin response: Advancing personalized acne treatment

Nguyen M[1], Zheng K[1]; mtn67@scarletmail.rutgers.edu; kz261@scarletmail.rutgers.edu
[1]*Rutgers University*

M2 The global landscape of real-world utilization and clinical outcomes in patients treated with adalimumab: A scoping review

Imani K[1], Wenner C[2], Lockhart C[3]; kianai7@uw.edu
[1]*University of Washington*; [2]*Wilkes University*; [3]*AMCP*

M3 Racial and ethnic disparities associated with opioid treatment for chronic noncancer pain in the United States

Oliorah P[1], Sujit S[1]; pioliora@cougarnet.uh.edu
[1]*University of Houston*

M25 Barrier analysis of biologic rheumatoid arthritis outlier utilization in a New Jersey Medicare health plan

Gooden A[1], Jang H[2], Thompson T[3]; ayanna.gooden@ufl.edu
[1]*Horizon BCBS NJ*; [2]*Horizon Blue Cross Blue Shield of New Jersey*; [3]*Pfizer, Inc*

N11 Development of a preliminary conceptual disease model for overactive bladder

Duong A[1], Castro C[2], Shah D[3], Shirneshan E[4]; apduong@usc.edu
[1]AbbVie-Chicago; [2]Patient-Centered Outcomes Research at AbbVie; [3]HEOR Strategy-Toxins; [4]Patient-Centered Outcomes Research Neuroscience, Eyecare & Specialty

O3 Over-the-counter birth control pills: A comprehensive impact analysis

Kim S[1], Liang E[1], Ko S[1]; sk2445@scarletmail.rutgers.edu; ekl50@scarletmail.rutgers.edu; sk2282@scarletmail.rutgers.edu
[1]Rutgers Ernest Mario School of Pharmacy

U36 A review of methodologies proposed for evaluating the Inflation Reduction Act's Medicare Drug Pricing Negotiation Program

Newcomer S[1], Kamal K[1]; stn00001@mix.wvu.edu
[1]West Virginia University School of Pharmacy

U37 An enhanced survey tool exploring the utility and perspectives of the Academy of Managed Care Pharmacy Foundation Pharmacy & Therapeutics Competition

Ganas S[1], Pentela K[2], Gharib A[3], Balisalisa C[4], Lee D[4], Byun M[5]; sganas2@uic.edu; kpente4@uic.edu; aghari2@uic.edu; cjb5@uic.edu; dlee251@uic.edu; mbyun1@uic.edu
[1]University of Illinois Chicago College of Pharmacy; [2]University of Illinois Chicago; [3]University of Illinois Chicago; [4]University of Illinois Chicago College of Pharmacy; [5]University of Illinois - College of Pharmacy

U38 Research support in managed care pharmacy publications

Soon L[1], Moukoulis B[1], Oh S[1], Cooke C[1]; lsoon@umaryland.edu; bmoukoulis@umaryland.edu
[1]University of Maryland School of Pharmacy

U39 Real-world uptake of biosimilars among patients in specialty pharmacy

Kiener T[1], Tran T[1], Pham A[1], Jeong A[2]; takako.shibasaki@westernu.edu; tiffany.tran3@westernu.edu; andrew.pham1@westernu.edu
[1]Western University of Health Sciences; [2]California Specialty Pharmacy

U40 A retrospective chart review on the effect of therapeutic interchange of sodium-glucose cotransporter-2 inhibitors on medication errors, adherence, and readmission rates

Nguyen J[1], Gonzalez J[2]; julianguyen@ufl.edu
[1]University of Florida College of Pharmacy - Orlando; [2]Broward Health

U41 Optimizing economic and clinical outcomes for patients with cancer through pharmacy student–led medication reconciliation

Taghzout S[1], Brown B[2], Lagasse P[3]; salmataghzout@uri.edu
[1]University of Rhode Island - College of Pharmacy; [2]University of Rhode Island; [3]Westerly Hospital

U42 Cost-efficiency and expanded access modeling of conversion to adalimumab biosimilars from a US payer perspective

Jang J[1], Abraham I[1]; jangj@arizona.edu
[1]R. Ken Coit College of Pharmacy, University of Arizona

U43 The impact prior authorizations, step therapy, and quantity limits have on medication accessibility, time to treatment, and other patient outcomes in the United States: A scoping review

Clark J[1], Lockhart C[2], Johnson M[2], Galgalikar S[3]; Jclark86234@rx.lecom.edu
[1]Lake Erie College of Osteopathic Medicine; [2]AMCP; [3]RxBenefits

U44 Antimicrobial resistance and the global impact of overprescribing broad-spectrum antibiotics during the COVID-19 pandemic: A systematic literature review

Paul A[1], Balisalisa C[1], Jogan C[1], Chen J[1]; apaul35@uic.edu; cbalis3@uic.edu; cjogan2@uic.edu; jchen326@uic.edu
[1]University of Illinois Chicago

U45 Insights for enhancing educational experiences: Assessing pharmacist feedback and satisfaction with clinical workshops

McCluskey Wirtz M[1], Taddei-Allen P[2], Bernhardi C[2], Potter E[2]; mmccluskeywirtz@gmail.com; ptaddeiallen@clinicalcomm.com
[1]Rutgers University; [2]MJH Life Sciences

U47 How profitable are pharmacy benefit managers? A review of published financial data for major payers

Nguyen J[1], *Ben-Umeh K*[1], *Mattingly J*[1]; *jenni.nguyen@pharm.utah.edu*
[1]*University of Utah*

U48 Negative health outcomes of electronic cigarettes compared with combustible cigarettes: A systematic review

Elshazali A[1], *Reyes-Valiente E*[2], *Balisalisa C*[3]; *aelsha8@uic.edu*; *ereyes45@uic.edu*
[1]UIC College of Pharmacy; [2]University of Illinois Chicago; [3]University of Illinois Chicago College of Pharmacy

U49 Comparing member quality measures performance between clinical pharmacist practitioners and nonclinical pharmacist practitioners within a commercial and Medicare value-based care population

Mbami I[1], *O'Hara B*[2], *Smith S*[3], *Abolins N*[4], *Gums T*[4]; *ivanmbami@gmail.com*
[1]*BCBS NC*; [2]*Blue Cross NC*; [3]*Blue Cross and Blue Shield of North Carolina*; [4]*Pfizer*

Z3 Heterogeneity in patient preferences for use and adoption of digital health technology: A scoping review of discrete choice experiment studies

Wikina T[1], *dosReis S*[1], *Amill-Rosario A*[1]; *twikina@umaryland.edu*
[1]*University of Maryland School of Pharmacy*

Z9 A cost-benefit analysis of a social determinants of health program within a clinical integrated network of community pharmacies

Chan L[1], *Luppino F*[2], *Meng M*[3], *Daly C*[3], *Iqbal D*, *Chen E*[4], *Jacobs D*[5]; *lokhaych@buffalo.edu*; *macymeng@buffalo.edu*
[1]*University at Buffalo School of Pharmacy and Pharmaceutical Sciences*; [2]*University at Buffalo School of Pharmacy and Pharmaceutical Sciences*; [3]*University at Buffalo School of Pharmacy and Pharmaceutical Sciences*; [4]*Taipei Medical University*; [5]*University at Buffalo*

Z10 Opioid use in cancer survivorship

Owens A[1], *Elzein R*[2], *Akpokiro P*[3], *Nnadi C*[1]; *owensa3@vcu.edu*; *elzeinra2@vcu.edu*; *akpokirop@vcu.edu*; *nnadice@vcu.edu*
[1]*Virginia Commonwealth University School of Pharmacy*; [2]*Virginina Commonwealth University School of Pharmacy*; [3]*Virginia Commonwealth University*

Z11 Closing vaccine gaps: Analysis of adult vaccination rates and predictors of poor uptake

Salawu D[1], *Christensen A*[2], *blackner l*[3], *glover j*[4], *boyd j*; *dsalawu@regis.edu*
[1]*Regis University School Of Pharmacy*; [2]*Blue Cross and Blue Shield of Kansas City*; [3]*Pfizer Global Medical*; [4]*Pfizer Medical Affairs*

Encore Poster Titles and Presenters

A1 Longer use of amikacin liposome inhalation suspension during first 12 months associated with lower risk of hospitalizations and emergency department visits: A claims analysis

Winthrop K[1], *Waweru C*[2], *Wang P*[2], *Welch E*[3], *Markson L*[2]; *winthrop@ohsu.edu*
[1]*OHSU-PSU School of Public Health*; [2]*Insmed Incorporated*; [3]*Panalgo*

B3 Real-world effectiveness of dolutegravir + lamivudine in treatment-naive people with HIV-1 and low CD4+ cell count or high viral load at baseline: A systematic literature review

Letang E[1], *Barber T*[2], *Allavena C*[3], *Hocqueloux L*[4], *Casado J*[5], *Di Giambenedetto S*[6], *Cabello-Úbeda A*[7], *d'Arminio Monforte A*[8], *Kabra M*[1], *Priest J*[1], *Milinkovic A*[1], *Jones B*[1], *Walko S*[1]; *emilio.x.letang@viivhealthcare.com*; *shana.l.walko@viivhealthcare.com*
[1]*ViiV Healthcare*; [2]*Ian Charleson Day Centre, Royal Free London NHS Foundation Trust; Institute for Global Health, University College London*; [3]*CHU Hôtel-Dieu*; [4]*Centre Hospitalier Universitaire d'Orléans*; [5]*Hospital Universitario Ramón y Cajal*; [6]*Fondazione Policlinico Universitario Agostino Gemelli IRCCS and Università Cattolica del Sacro Cuore*; [7]*Fundación Jimenez Diaz University Hospital*; [8]*San Paolo' Hospital, University of Milan*

B4 Virological suppression in people with HIV-1 receiving dolutegravir/lamivudine was high and similar across age groups despite older people having increased rates of comorbidities and polypharmacy: TANDEM subgroup analysis

Brogan A[1], *Slim J*[2], *Verdier G*[1], *Harper G*[3], *Mycock K*[3], *Wallis H*[3], *Donovan C*[1], *Amato P*[1]; *andrew.p.brogan@viivhealthcare.com*; *paul.v.amato@viivhealthcare.com*
[1]*ViiV Healthcare*; [2]*New York Medical College*; [3]*Adelphi Real World*

B5 Real-world effectiveness and tolerability of the 2-drug regimen dolutegravir and lamivudine in people living with HIV-1: A systematic literature review and meta-analysis from clinical practice

Fraysse J[1], *Priest J*[1], *Turner M*[2], *Hill S*[2], *Jones B*[1], *Verdier G*[1], *Letang E*[1], *Nguyen K*[1]; *jeremy.r.fraysse@viivhealthcare.com*; *kaitlin.p.nguyen@viivhealthcare.com*
[1]*ViiV Healthcare*; [2]*HEOR Ltd*

B6 HIV following oral preexposure prophylaxis initiation

Metzner A[1], *Germain G*[2], *Laliberté F*[3], *Oglesby A*[1], *Swygard H*[1], *MacKnight S*[4], *Hilts A*[2], *Duh M*[5], *Herman G*[1]; *aimee.a.metzner@viivhealthcare.com*; *gabrielle.f.herman@viivhealthcare.com*
[1]*ViiV Healthcare*; [2]*Groupe d'analyse, Ltée.*; [3]*Groupe d'analyse, Ltée*; [4]*Groupe d'analyse, Ltée*; [5]*Analysis Group, Inc.*

B12 Budget impact analysis of VOWST oral spores (formerly SER-109) for prevention of recurrent *Clostridioides difficile* infection in the United States

Golan Y[1], *Amin A*[2], *Dubberke E*[3], *Wilson M*[4], *Nguyen D*[5], *Kvasz M*[5]; *ygolan@tuftsmedicalcenter.org*; *mwilson@rti.org*
[1]*Tufts Medical Center*; [2]*University of California*; [3]*Washington University*; [4]RTI; [5]*Nestlé Health Science*

B13 Clinical and economic outcomes of microbiome therapy for preventing recurrence in recurrent *Clostridioides difficile* infection in the United States: Comparison of fecal microbiota spores, live-brpk (formerly SER-109) vs fecal microbiota, live-jslm

Golan Y[1], *Amin A*[2], *Wilson M*[3], *Nguyen D*[4], *Kvasz M*[4]; *Yoav.Golan@tuftsmedicine.org*; *mwilson@rti.org*
[1]*Tufts Medical Center*; [2]*University of California*; [3]RTI; [4]*Nestlé Health Science*

C1 Real-world clinical effectiveness and treatment patterns in the early-stage esophageal/gastroesophageal junction cancer population treated with definitive chemoradiotherapy in the community oncology setting in the United States

Ramakrishnan K[1], *Beeks A*[2], *Valderrama A*[1], *Shi J*[2], *Patton G*[2], *Herms L*[2], *Bordia S*[1], *Nallapareddy S*[3]; *karthik.ramakrishnan1@merck.com*
[1]*Merck*; [2]*Ontada*; [3]*Rocky Mountain Cancer Centers*

C2 Comparing first-line atezolizumab plus bevacizumab with lenvatinib or sorafenib in patients with unresectable hepatocellular carcinoma: Findings from the national Veterans Health Administration database

Tan A[1], *Kaplan D*[2], *Xiang C*[3], *Mu F*[3], *Ogale S*[1], *Hernandez S*[1], *Li J*[3], *Lin Y*[4], *Shi L*[4], *Singal A*[5]; *tanr6@gene.com*; *dakaplan@pennmedicine.upenn.edu*
[1]*Genentech*; [2]*Division of Gastroenterology and Hepatology, Perelman School of Medicine, University of Pennsylvania*; [3]*Analysis Group, Inc.*; [4]*Tulane University*; [5]*University of Texas Southwestern Medical Center*

C3 Racial and ethnic differences in the effectiveness of atezolizumab plus bevacizumab vs tyrosine kinase inhibitors among veterans with unresectable hepatocellular carcinoma

Tan A[1], *Kaplan D*[2], *Xiang C*[3], *Mu F*[3], *Ogale S*[1], *Hernandez S*[1], *Sundar M*[3], *Lin Y*[4], *Shi L*[4], *Singal A*[5]; *tanr6@gene.com*; *dakaplan@pennmedicine.upenn.edu*
[1]*Genentech*; [2]*Division of Gastroenterology and Hepatology, Perelman School of Medicine, University of Pennsylvania*; [3]*Analysis Group, Inc.*; [4]*Tulane University*; [5]*University of Texas Southwestern Medical Center*

C11 Health-related quality of life with tepotinib in patients with MET exon 14 skipping non–small cell lung cancer with brain, liver, adrenal, or bone metastases in the phase 2 VISION trial

Yang M[1], *Reinmuth N*[2], *Mazieres J*[3], *Popat S*[4], *Paz-Ares L*[5], *Hook E*[6], *Hatswell A*[6], *Vlassak S*[7], *Johne A*[8], *Vioix H*[8], *Paik P*[9]; *mo.yang@emdserono.com*; *n.reinmuth@asklepios.com*
[1]*EMD Serono*; [2]*Thoracic Oncology Department, Asklepios Lung Clinics Munich-Gauting*; [3]*CHU de Toulouse, Université Paul Sabatier*; [4]*The Royal Marsden Hospital*; [5]*Hospital Universitario 12 de Octubre*; [6]*Delta Hat*; [7]*Merck N.V.-S.A., an affiliate of Merck KGaA*; [8]*The Healthcare Business of Merck KGaA*; [9]*Thoracic Oncology Service, Memorial Sloan-Kettering Cancer Center; Weill Cornell Medical College*

C12 TOGETHER: Pooled real-world datasets of MET exon 14 skipping non–small cell lung cancer and adjusted comparison of upfront (chemo-) immunotherapy with tepotinib from VISION

Yang M[1], *Christopoulos P*[2], *Ekman S*[3], *Guisier F*[4], *Ho C*[5], *Blasi M*[6], *Brunnstrom H*[7], *Cvetkovic J*[8], *Kazdal D*[8], *Kuon J*[9], *Haglund de Flon F*[3], *Stenzinger A*[8], *Wong S*[10], *Hatswell A*[11], *Mclean T*[12], *Bergman S*[13], *Orlowski K*[14], *Vioix H*[15], *Thomas M*[16]; *mo.yang@emdserono.com*; *Petros.Christopoulos@med.uni-heidelberg.de*
[1]*EMD Serono*; [2]*Thoraxklinik and National Center for Tumor Diseases, Heidelberg University Hospital; Translational Lung Research Center Heidelberg (TLRC-H), The German Center for Lung Research (DZL)*; [3]*Karolinska University Hospital/Department of Oncology-Pathology, Karolinska Institutet*; [4]*Univ Rouen Normandie, LITIS Lab QuantIF team EA4108, CHU Rouen, Department of Pneumology and Inserm CIC-CRB 1404*; [5]*University of British Columbia*; [6]*Thoraxklinik and National Center for Tumor Diseases at Heidelberg University Hospital*; [7]*University/Skåne University Hospital*; [8]*Institute of Pathology, University Hospital Heidelberg*; [9]*Thoracic Clinic of Heidelberg University Hospital*; [10]*Department of Medical Oncology, BC Cancer; University of British Columbia*; [11]*Delta Hat*; [12]*Merck Serono Ltd., Feltham, UK, an affiliate of Merck KGaA, Darmstadt, Germany*; [13]*Merck AB, an affiliate of Merck KGaA*; [14]*Merck Serono GmbH, an affiliate of Merck KGaA*; [15]*The Healthcare Business of Merck KGaA*; [16]*Department of Thoracic Oncology, Thoraxklinik and National Center for Tumor Diseases at Heidelberg University Hospital; Translational Lung Research Center Heidelberg (TLRC-H), Member of the German Center for Lung Research (DZL)*

C14 Epidemiological and genomic features of patients with advanced/metastatic non–small cell lung cancer with human epidermal growth factor receptor 2/ERBB2 mutations within and outside the tyrosine kinase domain

Maruti S[1], *Le X*[2], *Baik C*[3], *Nagasaka M*[4], *Patil T*[5], *Stanhope S*[1], *Kaya N*[6], *Herbertz S*[7], *Lovly C*[8]; *sonia.maruti@boehringer-ingelheim.com*; *XLe1@mdanderson.org*
[1]*Global Medical Affairs, Boehringer Ingelheim Pharmaceuticals, Inc.*; [2]*Department of Thoracic Head and Neck Medical Oncology, MD Anderson Cancer Center*; [3]*Fred Hutchinson Cancer Research Center, University of Washington*; [4]*Division of Hematology and Oncology, Department of Medicine, University of California Irvine Medical Center*; [5]*Department of Medicine, University of Colorado Cancer Center*; [6]*TMCP TA Oncology, Boehringer Ingelheim Pharma GmbH & Co. KG*; [7]*Global Medical Affairs Oncology, Boehringer Ingelheim International GmbH*; [8]*Department of Medicine, Division of Hematology and Oncology, Vanderbilt University Medical Center*

C15 Price landscape of non–small cell lung cancer

Cheng S[1], *Ambegaonkar A*[2]; *suyi@appertureheath.com*; *ambi@appertureheath.com*
[1]*Apperture*; [2]APPERTURE LLC

C16 Zongertinib (BI 1810631) in patients with human epidermal growth factor receptor 2–driven tumors: Final phase 1a and primary phase 1b analysis of Beamion LUNG-1

Ruiter G[1], *Tu H*[2], *Yoh K*[3], *Wu Y*[4], *von Wangenheim U*[5], *Rohrbacher M*[6], *Sadrolhefazi B*[7], *Heymach J*[8]; *g.ruiter@nki.nl*
[1]*Department of Clinical Pharmacology & Thoracic Oncology, The Netherlands Cancer Institute*; [2]*Guangdong Lung Cancer Institute, Guangdong General Hospital*; [3]*Department of Thoracic Oncology, National Cancer Center Hospital East*; [4]*Guangdong Lung Cancer Institute, Guangdong Provincial People's Hospital (Guangdong Academy of Medical Sciences), Southern Medical University*; [5]*Boehringer Ingelheim Pharma GmbH & Co. KG*; [6]*Boehringer Ingelheim International GmbH*; [7]*Boehringer Ingelheim Pharmaceuticals, Inc*; [8]*Department of Thoracic/Head and Neck Medical Oncology, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center*

C37 Economic burden of prostate cancer in the United States: A disease stage–based cost analysis

Kalayeh B[1], *Desmaras H*[2], *Sacco D*[2], *Curry L*[3], *Costa A*[2], *Singh R*[1], *Appukkuttan S*[4], *Jhaveri J*[1]; *bashir.kalayeh@bayer.com*
[1]*Bayer U.S. LLC*; [2]*Bayer AG*; [3]*Cencora*; [4]*Bayer US LLC*

C46 Long-term survival after elranatamab monotherapy in patients with relapsed or refractory multiple myeloma: MagnetisMM-3

Mohty M[1], *Iida S*[2], *Bahlis N*[3], *Sullivan S*[4], *Conte U*[4], *Leip E*[4], *Viqueira A*[5], *Lesokhin A*[6]; *mohamad.mohty@inserm.fr*
[1]*Sorbonne University, Hôpital Saint-Antoine, and INSERM UMRS938*; [2]*Department of Hematology and Oncology, Nagoya City University Graduate School of Medical Sciences*; [3]*Arnie Charbonneau Cancer Institute, University of Calgary School of Medical Sciences*; [4]*Pfizer Inc*; [5]*Pfizer SLU*; [6]*Memorial Sloan Kettering Cancer Center/Weill Cornell Medical College*

C47 Efficacy of imetelstat on red blood cell transfusion independence in the absence of platelet transfusions or myeloid growth factors in IMerge

Zeidan A[1], *Santini V*[2], *Platzbecker U*[3], *Sekeres M*[4], *Savona M*[5], *Fenaux P*[6], *Madanat Y*[7], *Raza A*[8], *Xia Q*[9], *Sun L*[10], *Riggs J*[11], *Shah S*[11], *Navada S*[11], *Berry T*[10], *Komrokji R*[12]; *amer.zeidan@yale.edu*; *snavada@geron.com*
[1]*Yale University School of Medicine/Yale Cancer Center*; [2]*University of Florence*; [3]*Department of Hematology, Cellular Therapy and Hemostaseology, Leipzig University Hospital*; [4]*Sylvester Cancer Center, University of Miami*; [5]*Vanderbilt-Ingram Cancer Center, Vanderbilt University Medical Center*; [6]*Département (DMU) d'hématologie et immunologie, APHP Nord Service d'hématologie séniors Hôpital St Louis/université de Paris*; [7]*Harold C. Simmons Comprehensive Cancer Center*; [8]*Department of Cancer Care and Internal Medicine, Herbert Irving Comprehensive Cancer Center, Columbia University*; [9]*Geron Corporate*; [10]*Geron*; [11]*Geron Corporation*; [12]*Moffitt Cancer Center*

C48 Overall survival, clinical benefit, and durable transfusion independence with imetelstat in the IMerge phase 3 trial of red blood cell transfusion–dependent lower-risk myelodysplastic syndromes

Santini V[1], *Komrokji R*[2], *Sekeres M*[3], *Savona M*[4], *Fenaux P*[5], *Madanat Y*[6], *Oliva E*[7], *Buckstein R*[8], *Jonášová A*[9], *Germing U*[10], *Mittelman M*[11], *Thepot S*[12], *Riggs J*[13], *Dougherty S*[13], *Navada S*[13], *Berry T*[14], *Xia Q*[15], *Sun L*[14], *Zeidan A*[16], *Platzbecker U*[17]; *valeria.santini@unifi.it*; *snavada@geron.com*
[1]*University of Florence*; [2]*Moffitt Cancer Center*; [3]*Sylvester Cancer Center, University of Miami*; [4]*Vanderbilt-Ingram Cancer Center, Vanderbilt University Medical Center*; [5]*Département (DMU) d'hématologie et immunologie, APHP Nord Service d'hématologie séniors Hôpital St Louis/ université de Paris*; [6]*Harold C. Simmons Comprehensive Cancer Center*; [7]*Grande Ospedale Metropolitano BMM*; [8]*Odette Cancer Center*; [9]*Charles University General Hospital, Hematology*; [10]*Universitätsklinikum Düsseldorf Hämatologie, Onkologie, Düsseldorf, Germany*; [11]*Tel Aviv Sourasky (Ichilov) Medical Center*; [12]*Centre Hospitalier Universitaire d'Angers*; [13]*Geron Corporation*; [14]*Geron*; [15]*Geron Corporate*; [16]*Yale University School of Medicine/ Yale Cancer Center*; [17]*Department of Hematology, Cellular Therapy and Hemostaseology, Leipzig University Hospital*

C49 Practice efficiency and total costs associated with treating relapsed or refractory diffuse large B-cell lymphoma with epcoritamab and glofitamab from an institutional perspective

Lei M[1], *Li Q*[2], *O'Day K*[2], *Meyer K*[2], *Wang A*[3], *Jun M*[4]; *mlei1@mgh.harvard.edu*
[1]*Massachusetts General Hospital*; [2]*Cencora*; [3]*AbbVie Inc.*; [4]*Genmab US, Inc*

C50 Clinical outcomes associated with anti-CD38 retreatment in relapsed/refractory multiple myeloma: A review of the literature

Gay F[1], *Zamagni E*[2], *Cole C*[3], *Chorazy J*[4], *Iheanacho I*[4], *Pandey A*[4], *Bitetti J*[5], *Boytsov N*[5], *Purser M*[5], *McNamara S*[5]; *Francesca.gay@unito.it*; *simon.j.mcnamara@gsk.com*
[1]*University of Torino; Azienda Ospedaliero-Universitaria Città della Salute e della Scienza di Torino*; [2]*University of Bologna*; [3]*Michigan State University; Karmanos Cancer Institute*; [4]*Evidera*; [5]*GSK*

C51 Efficacy of anti–B-cell maturation antigen antibody-drug conjugate belantamab mafodotin + bortezomib + dexamethasone vs alternative regimens in second-line-or-greater relapsed/refractory multiple myeloma: A network meta-analysis of randomized clinical trials

Richter J[1], *Nooka A*[2], *Rodriguez Otero P*[3], *Schjesvold F*[4], *Combe E*[5], *Cooper L*[5], *Scott M*[5], *Sly I*[5], *Ballew N*[6], *Bitetti J*[6], *Boytsov N*[6], *Purser M*[6], *McNamara S*[6]; *joshua.richter@mountsinai.org*; *simon.j.mcnamara@gsk.com*
[1]*Mount Sinai*; [2]*Winship Cancer Institute of Emory University*; [3]*Cancer Center Clinica Universidad de Navarra*; [4]*Oslo University Hospital*; [5]*FIECON*; [6]*GSK*

C52 Updated results of a matching-adjusted indirect comparison of elranatamab vs teclistamab in patients with triple-class exposed/ refractory multiple myeloma

Mol I[1], *Hu Y*[2], *LeBlanc T*[3], *Cappelleri J*[4], *Chu H*[4], *Nador G*[5], *Aydin D*[4], *Perez Cruz I*[4], *Hlavacek P*[4]; *isha.mol@cytel.com*
[1]*Ingress Health Nederland B.V. – A Cytel Company*; [2]*Cytel*; [3]*Duke University School of Medicine*; [4]*Pfizer Inc*; [5]*Pfizer Ltd*

D1 Real-world treatment patterns and outcomes in patients with myelofibrosis treated with pacritinib in the United States

Marrone M[1], *Geller R*[2], *Oladapo A*[1], *Suthar P*[1], *Vasudevan A*[2], *Vredenburg M*[1], *Zeng J*[2], *Rampal R*[3], *Mascarenhas J*[4]; *michael.marrone@sobi.com*; *john.mascarenhas@mssm.edu*
[1]*Sobi Inc.*; [2]*IntegraConnect, PrecisionQ*; [3]*Memorial Sloan Kettering Cancer Center*; [4]*Tisch Cancer Institute, Icahn School of Medicine at Mount Sinai*

D3 Evaluation of the health care system burden associated with intravenous treatment for paroxysmal nocturnal hemoglobinuria

Lee S[1], *Mulherin B*[2], *Winokur D*[3], *Yacoub A*[4], *Bilano V*[5], *Yen G*[1], *Geevarghese A*[1], *Paulose J*[1], *Guerin A*[6], *Latremouille-Viau D*[7], *Marathe G*[7], *Waheed A*[8]; *soyon.lee@novartis.com*; *Brian.Mulherin@aoncology.com*
[1]*Novartis Pharmaceutical Corp*; [2]*Hematology Oncology of Indiana*; [3]*UMass Memorial Health Care, University of Massachusetts Medical School*; [4]*KU Medical Center, The University of Kansas*; [5]*Novartis Pharmaceuticals UK Ltd*; [6]*Analysis Group, Inc.*; [7]*Analysis Group, Inc.*; [8]*Weill Cornell Medical Center*

D9 Long-term outcomes with efanesoctocog alfa prophylaxis for previously treated children with severe hemophilia A: An interim analysis of the phase 3 XTEND-ed study

Malec L[1], *Nolan B*[2], *Chan A*[3], *Albisetti M*[4], *Chou S*[5], *Zulfikar B*[6], *Simpson M*[7], *Feng L*[8], *Palmborg H*[9], *Neill G*[10], *Abad-Franch L*[11], *Gunawardena S*[12], *Fijnvandraat K*[13]; *manuela.albisetti@kispi.uzh.ch*
[1]*Versiti Blood Research Institute; Division of Hematology & Oncology, Departments of Medicine and Pediatrics, Medical College of Wisconsin*; [2]*Children's Health Ireland at Crumlin*; [3]*McMaster Children's Hospital, McMaster University*; [4]*University Children's Hospital Zurich*; [5]*Department of Internal Medicine, Division of Hematology, National Taiwan University Hospital, College of Medicine, National Taiwan University*; [6]*Department of Pediatric Hematology, Istanbul University Oncology Institute, Inherited Bleeding Disorders Center*; [7]*Rush University Medical Center, Rush Hemophilia and Thrombophilia Center*; [8]*Sanofi*; [9]*Sobi*; [10]*Sanofi*; [11]*Sobi*; [12]*Sanofi*; [13]*Emma Kinderziekenhuis/AMC, University of Amsterdam*

D10 Interim analysis of joint outcomes in patients with severe hemophilia A receiving efanesoctocog alfa during the phase 3 XTEND-ed long-term extension study

von Drygalski A[1], *Königs C*[2], *Konkle B*[3], *Hermans C*[4], *Khoo L*[5], *Fischer K*[6], *Suzuki N*[7], *Guo H*[8], *Khan U*[9], *Bystrická L*[10], *Fernandez A*[11], *Santagostino E*[10], *Mamikonian L*[9], *Klamroth R*[12]; *avondrygalski@health.ucsd.edu*
[1]*Division of Hematology/Oncology, Department of Medicine, University of California, San Diego*; [2]*Goethe University Frankfurt, University Hospital, Department of Pediatrics and Adolescent Medicine*; [3]*Washington Center for Bleeding Disorders and the University of Washington*; [4]*Division of Haematology, Haemostasis and Thrombosis Unit, Saint-Luc University Hospital, Université Catholique de Louvain*; [5]*Institute of Haematology, Royal Prince Alfred Hospital*; [6]*Centre for Benign Haematology, Thrombosis and Haemostasis, Van Creveldkliniek, University Medical Centre Utrecht, Utrecht University*; [7]*Department of Transfusion Medicine, Nagoya University Hospital*; [8]*Sanofi*; [9]*Sanofi*; [10]*Sobi*; [11]*Sanofi*; [12]*Vivantes Klinikum im Friedrichshain*

D11 First interim analysis of clinical outcomes in adults and adolescents with severe hemophilia A receiving efanesoctocog alfa prophylaxis in XTEND-ed, a phase 3 long-term extension study

Susen S[1], *P'ng S*[2], *Lissitchkov T*[3], *Matino D*[4], *Chowdary P*[5], *Weyand A*[6], *Klamroth R*[7], *Park Y*[8], *Alvarez-Román M*[9], *Feng L*[10], *Palmborg H*[11], *Dumont J*[12], *Santagostino E*[13], *Fetita L*[14], *Nogami K*[15]; *sophie.susen@chu-lille.fr*
[1]*Centre Hospitalier Universitaire de Lille, Université de Lille*; [2]*The Haemophilia and Haemostasis Centre, Fiona Stanley Hospital*; [3]*Specialized Hospital for Active Treatment of Hematological Diseases, Department of Chemotherapy, Hemotherapy and Hereditary Blood Diseases at Clinical Hematology Clinic*; [4]*Division of Hematology & Thromboembolism, Department of Medicine, McMaster University*; [5]*Katharine Dormandy Haemophilia and Thrombosis Centre, Royal Free Hospital*; [6]*Division of Hematology/Oncology, Department of Pediatrics, University of Michigan Medical School*; [7]*Vivantes Klinikum im Friedrichshain*; [8]*Department of Pediatrics, Kyung Hee University Hospital at Gangdong, School of Medicine, Kyung Hee University*; [9]*Department of Hematology, University Hospital La Paz, Autonoma University of Madrid*; [10]*Sanofi*; [11]*Sobi*; [12]*Sanofi*; [13]*Sobi*; [14]*Sanofi*; [15]*Nara Medical University*

D12 Perioperative management with efanesoctocog alfa in adults, adolescents, and children with severe hemophilia A in the phase 3 XTEND clinical program

Chan A[1], *Susen S*[2], *Khoo L*[3], *von Drygalski A*[4], *Oldenburg J*[5], *Shen M*[6], *Peyvandi F*[7], *Tarango C*[8], *Chowdary P*[9], *Mamikonian L*[10], *Palmborg H*[11], *Dumont J*[10], *Santagostino E*[12], *Hermans C*[13]; *akchan@mcmaster.ca*
[1]*McMaster Children's Hospital, McMaster University*; [2]*Centre Hospitalier Universitaire de Lille, Université de Lille*; [3]*Institute of Haematology, Royal Prince Alfred Hospital*; [4]*Division of Hematology/Oncology, Department of Medicine, University of California, San Diego*; [5]*University Hospital Bonn*; [6]*Division of Hematology-Oncology, Department of Internal Medicine, Changhua Christian Hospital; Department of Internal Medicine and Laboratory Medicine, National Taiwan University Hospital*; [7]*IRCCS Ca' Granda Maggiore Hospital Foundation, Angelo Bianchi Bonomi Hemophilia and Thrombosis Center; Università degli Studi di Milano, Department of Pathophysiology and Transplantation*; [8]*Department of Pediatrics, University of Cincinnati College of Medicine; Division of Hematology, Cincinnati Children's Hospital Medical Center*; [9]*Katharine Dormandy Haemophilia and Thrombosis Centre, Royal Free Hospital*; [10]*Sanofi*; [11]*Sobi*; [12]*Sobi*; [13]*Division of Haematology, Haemostasis and Thrombosis Unit, Saint-Luc University Hospital, Université Catholique de Louvain*

D13 Real-world treatment patterns and outcomes in patients with immune thrombocytopenia treated with avatrombopag in the United States: REAL-AVA 2.0 interim analysis results

Oladapo A[1], Bernheisel C[1], Swallow E[2], Goldschmidt D[2], Greatsinger A[2], Sarathy K[2], Ormenaj L[3], Vredenburg M[1], Kolodny S[1]; scott.kolodny@sobi.com
[1]*Sobi Inc.*; [2]*Analysis Group*; [3]*Analysis Group, Inc.*

E1 Evaluating need for larger insulin reservoir in patch pumps: Leveraging retrospective data for US adults with type 2 diabetes on multiple daily injections

Wright E[1], Shah V[2], Thach A[3], Javadi P[3], Davies S[4], Sieradzan R[3]; eewright51@gmail.com; avthach@gmail.com; Ray.Sieradzan@embecta.com
[1]*South Piedmont AHEC*; [2]*IU Center for Diabetes and Metabolic Diseases*; [3]*embecta*; [4]*PRECISIONheor*

E2 Health care utilization and cost with empagliflozin vs glucagon-like peptide-1 receptor agonists in patients with type 2 diabetes

Htoo P[1], Tesfaye H[2], Schneeweiss S[3], Wexler D[4], Glynn R[5], Schmedt N[6], Koeneman L[7], DiCesare E[8], Paik J[9], Patorno E[5]; phtoo@bwh.harvard.edu
[1]*Brigham & Women's Hospital, Harvard Medical School*; [2]*Brigham & Women's Hospital*; [3]*Brigham and Women's Hospital, Professor of Medicine, Harvard Medical School*; [4]*Massachusetts General Hospital*; [5]*Harvard Medical School, Brigham and Women's Hospital*; [6]*Boehringer Ingelheim International GmbH*; [7]*Lilly Diabetes Global Medical Affairs*; [8]*Brigham and Women's Hospital, Division of Pharmacoepidemiology,*; [9]*BWH and Harvard Medical School*

E4 Exploring real-world adherence and cost implications of continuous glucose monitoring in patients with diabetes: Impact of device sourcing

Allaire J[1], Masturzo A[2], Dennis C[2], Wittlin S[3]; jason.allaire@generativitysg.com
[1]*Generativity Health Outcomes Research*; [2]*CCS Medical*; [3]*University of Rochester*

E18 Development of a Markov model for assessing the cost-effectiveness of chronic weight management interventions

Richard E[1], Gangan N[1], Ketkar A[2], Grabner M[1], Gabler N[1], Head M[1], Smith J[1]; emma.richard@carelon.com
[1]*Carelon Research*; [2]*Carelon Research Inc.*

E25 Association of tirzepatide and weight reduction in people with obstructive sleep apnea and obesity: A post hoc analysis of the SURMOUNT trials

Kern S[1], Falcon B[1], Taylor R[1], Shinde S[1], Jouravskaya I[1], Dunn J[1], Bednarik J[1], Stefanski A[1], Liao B[1], Malhotra A[2]; kern_scott_a@lilly.com; falcon_beverly_l@lilly.com
[1]*Eli Lilly and Company*; [2]*University of California, Division of Pulmonary and Critical Care Medicine*

E26 Modeling long-term outcomes of tirzepatide compared with lifestyle management and other antiobesity medications as treatment for overweight and obesity

Wallem A[1], Hoog M[1], Kan H[1], Deger K[2], Sorensen S[2], Neff L[1], Patrick Bae J[1], Hankosky E[1], Mojdami D[1], White B[2], Harris M[2]; wallem_alexandra@lilly.com; meredith.hoog@lilly.com
[1]*Eli Lilly and Company*; [2]*Evidera*

E27 Synergistic effect of multimorbidity of obesity-related comorbidities on health care costs

Shepherd M[1], Pohl G[2], Ezendu K[3], Lee C[3], Wang H[4], Li X[1], Dunn J[1]; shepherd_michael_e@lilly.com; dunn_julia@lilly.com
[1]*Eli Lilly and Company*; [2]*Eli Lilly and Company (at the time of study)*; [3]*Eli Lilly and Company*; [4]*Techdata Service, Inc.*

E28 Obesity risk prediction through machine learning: A US administrative claims database study

Bentz B[1], Choong C[1], Brnabic A[1], Chinthammit C[1], Ravuri M[2], Terrell K[1], Kan H[3]; bobbi.bentz@lilly.com; choong_kar-chan@lilly.com
[1]*Eli Lilly and Company*; [2]*Eli Lilly and Company (at the time of the study)*; [3]*Eli Lilly and Company*

F1 Real-world Auvelity (AXS-05) patient characteristics in major depressive disorder

Muzyk A[1], Syed F[2], Zhou H[3], Chong J[3], Tabuteau H[4], Zhao Y[4]; yzhao@axsome.com
[1]*Department of Pharmacy Practice, Campbell University College of Pharmacy and Health Sciences and Duke University School of Medicine*; [2]*Duke University Division of General Internal Medicine*; [3]*KMK Consulting, Inc.*; [4]*Axsome Therapeutics Inc.*

F2 Clinical profile of AXS-05 (dextromethorphan-bupropion) in treating Alzheimer disease–related agitation: Results from the phase 2/3 development program

Cummings J[1], *Grossberg G*[2], *Porsteinsson A*[3], *Depue R*[4], *Streicher C*[4], *Tabuteau H*[4]; *rdepue@axsome.com*; *htabuteau@axsome.com*
[1]*University of Nevada*; [2]*Saint Louis University Hospital*; [3]*University of Rochester School of Medicine and Dentistry*; [4]*Axsome Therapeutics Inc.*

F4 Impact of telemedicine on medication for opioid use disorder retention during the SARS-CoV-2 pandemic period among patients with opioid use disorder

Flynn C[1], *Mullen W*[1], *Gaiazov S*[1], *Ogbonnaya A*[2], *Dhuliawala S*[2]; *courtney.flynn@Indivior.com*; *augustinao@gmail.com*
[1]*Indivior*; [2]*Cencora (previously, Xcenda)*

F5 An evaluation of the incidence of opioid use disorder among people with acute and chronic pain managed with prescription opioids and the associated economic burden in the United States

Jeyakumar S[1], *Morlando Geiger J*[1], *Princic N*[2], *Moynihan M*[2], *Varker H*[2], *Shi L*[1], *Stiegler M*[1], *Menzie A*[1], *Schoenfeld A*[3]; *sushanth_jeyakumar@vrtx.com*
[1]*Vertex Pharmaceuticals Incorporated*; [2]*Merative*; [3]*Harvard Medical School; Brigham and Women's Hospital*

F7 Costs and health care resource utilization associated with negative symptoms among patients with schizophrenia in the United States

Vaccaro J[1], *Nili M*[2], *Xiang P*[2], *Nelson J*[3], *Pack C*[3], *Thompson R*[4], *Mills C*[4], *Vasey J*[4], *Parks J*[5]; *jerryvaccaro@yahoo.com*; *mona.nili@boehringer-ingelheim.com*; *pin.xiang@boehringer-ingelheim.com*
[1]*JVV Consulting and RightPath HC*; [2]*Boehringer Ingelheim*; [3]*Veradigm*; [4]*Vearadigm*; [5]*National Council for Mental Wellbeing*

F8 Maintenance of efficacy of KarXT (xanomeline and trospium) in schizophrenia

Kaul I[1], *Cohen E*[2], *Boland P*[1], *Sidovar M*[1], *Claxton A*[1]; *ikaul@karunatx.com*; *Pboland@Karunatx.Com*
[1]*Bristol Myers Squibb*; [2]*CenExcel*

F9 Long-term safety of KarXT (xanomeline and trospium) in schizophrenia

Marcus R[1], *Kakar R*[2], *Claxton A*[1], *Jones J*[1], *Gillard K*[1], *Kaul I*[1]; *rmarcus@karunatx.com*; *jonathan.jones@karuatx.com*
[1]*Bristol Myers Squibb*; [2]*Segal Trials West Broward*

F10 Long-term metabolic outcomes associated with KarXT (xanomeline and trospium): Interim results from pooled, long-term safety studies EMERGENT-4 and EMERGENT-5

Claxton A[1], *Konis G*[2], *Kaul I*[1], *Henderson C*[1], *Cajigal A*[1], *Marcus R*[1]; *aclaxton@karunatx.com*; *chenderson@karunatx.com*
[1]*Bristol Myers Squibb*; [2]*Woodland International Research Group*

F16 Contemporary estimates of the direct medical cost burden of treatment-resistant depression

Wing V[1], *Lerner J*[1], *Teoh E*[1], *Orsini L*[1], *Gill J*[1], *O'Connor S*[1], *Taylor R*[1], *Young M*[2], *Levine S*[1]; *vicki.kwing@gmail.com*
[1]*Compass Pathways*; [2]*Compass Pathways, PLC*

F17 Patient characteristics and health care resource utilization of cognitive impairment among patients with schizophrenia in the Veterans Affairs Administration system

Nili M[1], *Xiang P*[1], *Magagnoli J*[2], *Cummings T*[2], *Sutton S*[2]; *mona.nili@boehringer-ingelheim.com*; *pin.xiang@boehringer-ingelheim.com*
[1]*Boehringer Ingelheim*; [2]*University of South Carolina College of Pharmacy*

F18 Auvelity (AXS-05) in major depressive disorder: Pooled data from two 6-week controlled trials (GEMINI and ASCEND)

Chepke C[1], *Iosifescu D*[2], *Zhao Y*[3], *Eglit G*[4], *Streicher C*[3], *Tabuteau H*[3]; *yzhao@axsome.com*; *htabuteau@axsome.com*
[1]*Excel Psychiatric Associates and Atrium Health*; [2]*Nathan Kline Institute and New York University School of Medicine*; [3]*Axsome Therapeutics Inc.*; [4]*Axsome Therapeutics Inc.*

F27 Baseline characteristics of pediatric and adult patients with attention-deficit/hyperactivity disorder prescribed viloxazine extended-release, stimulants, or atomoxetine, in open claims data

Hurwitz J[1], *Chorniy A*[2], *Grizzle A*[1], *Young J*[3], *Gopakumar S*[4], *Earnest J*[5]; *JasonT.Hurwitz@gmail.com*; *anna.chorniy@northwestern.edu*
[1]*Strategic Therapeutics*; [2]*Northwestern University*; [3]*Rochester Center for Behavioral Medicine*; [4]*Supernus Pharmaceuticals*; [5]*Supernus Pharmaceuticals, Inc.*

G6 Real-world retrospective safety analysis in patients treated with onabotulinumtoxinA for multiple therapeutic indications over repeat treatment periods

Forde G[1], *Patel A*[2], *Martinez K*[3], *Mayadev A*[4], *Brucker B*[5], *Brown T*[6], *Ayyoub Z*[7], *Singh R*[8], *Nelson M*[8], *Ukah A*[8], *Yushmanova I*[8], *Battucci S*[8], *Becker Infantides K*[8], *Rhyne C*[9]; *neuropain@aol.com*; *kim.becker@abbvie.com*
[1]*NeuroPain Care Center, NYU Langone*; [2]*Kansas City Bone & Joint Clinic*; [3]*Neurology & Pain Specialty Center*; [4]*Swedish Neuroscience Institute*; [5]*NYU Langone Health*; [6]*EvergreenHealth Kirkland*; [7]*Ranchos Los Amigos National Rehabilitation Center*; [8]*AbbVie*; [9]*Norton Neuroscience Institute*

G7 Real-world diagnosis of chronic migraine in patients with cervical dystonia analyzed by botulinum neurotoxin treatment exposure: An epidemiologic perspective

Rodriguez R[1], *Broner S*[2], *Forde G*[3], *Rhyne C*[4], *Sadeghi M*[5], *Yue E*[5], *Ukah A*[5], *Becker Infantides K*[5], *Sarva H*[6]; *rodriguez@neurologyone.com*; *marjan.sadeghi@abbvie.com*
[1]*Neurology One, University of Central Florida*; [2]*Weill Cornell Medicine,*; [3]*NeuroPain Care Center, NYU Langone*; [4]*Norton Neuroscience Institute*; [5]*AbbVie*; [6]*Weill Cornell Medicine*

G12 Efficacy and safety of donanemab, a novel amyloid-targeting therapy

Klein E[1], *Zimmer J*[1], *Evans C*[1], *Lu M*[1], *Sparks J*[1], *Brooks D*[1], *Sims J*[1]; *klein_eric_g@lilly.com*; *zimmer_jennifer_ann@lilly.com*
[1]*Eli Lilly and Company*

G17 Psychometric validation of PROMIS-Fatigue-MS-8a Questionnaire in patients with relapsing multiple sclerosis participating in a phase 2 study of frexalimab

Vermersch P[1], *Giovannoni G*[2], *Arnould B*[3], *Hakimi-Hawken N*[4], *Araujo L*[5], *Saubadu S*[6], *Truffinet P*[6], *Gourlain S*[7], *Msihid J*[6]; *patrick.vermersch@univ-lille.fr*; *lita.araujo@sanofi.com*
[1]*University of Lille, CHU of Lille*; [2]*Queen Mary University of London*; [3]*Sanofi*; [4]*Sanofi*; [5]*Sanofi, Health Economics & Value Assessment*; [6]*Sanofi*; [7]*Ividata*

G18 Real-world change in annualized relapse rate and health care resource utilization following initiation of ofatumumab in patients with multiple sclerosis

Tai M[1], *Brown B*[1], *Taiji R*[2], *Faucher A*[2], *Gadkari A*[1]; *mindy.tai@novartis.com*
[1]*Novartis Pharmaceuticals Corporation*; [2]*STATLOG Inc*

G19 Psychometric evaluation of multiple sclerosis impact scale 29 version 2 in adults with relapsing multiple sclerosis participating in a phase 2 trial of frexalimab

Giovannoni G[1], *Vermersch P*[2], *Gourlain S*[3], *Hakimi-Hawken N*[4], *Araujo L*[5], *Saubadu S*[6], *Truffinet P*[6], *Arnould B*[7], *Msihid J*[6]; *g.giovannoni@qmul.ac.uk*; *lita.araujo@sanofi.com*
[1]*Queen Mary University of London*; [2]*University of Lille, CHU of Lille*; [3]*Ividata*; [4]*Sanofi*; [5]*Sanofi, Health Economics & Value Assessment*; [6]*Sanofi*; [7]*Sanofi*

G20 Cost avoidance from health system specialty pharmacist interventions in patients with multiple sclerosis

Georgieva D[1], *Markley B*[2], *DeClercq J*[3], *Choi L*[3], *Zuckerman A*[2]; *georgiev@musc.edu*
[1]*Medical University of South Carolina Health*; [2]*Vanderbilt Specialty Pharmacy*; [3]*Vanderbilt University Medical Center*

G21 Evaluating association of chronic active lesions with disability in multiple sclerosis: A systematic literature review

Bagnato F[1], *Mordin M*[2], *Greene N*[3], *Mahida S*[2], *Higuchi K*[4], *Wingerden J*[5]; *francesca.r.bagnato@vumc.org*; *Nupur.Greene@sanofi.com*
[1]*Vanderbilt University Medical Center*; [2]*RTI Health Solutions*; [3]*Sanofi*; [4]*Sanofi*; [5]*Sanofi*

G22 Understanding the burden of illness in people with nonrelapsing secondary progressive multiple sclerosis in the United States: A matched-cohort study

Greene N[1], *Das A*[2], *Hemim I*[3], *Chang E*[2], *Tarbox M*[2], *Higuchi K*[4]; *Nupur.Greene@sanofi.com*
[1]*Sanofi*; [2]*PHAR*; [3]*Sanofi*; [4]*Sanofi*

G23 Clinical and economic burden in people with relapsing-remitting multiple sclerosis in the United States: A matched-cohort study

Greene N[1], *Das A*[2], *Hemim I*[3], *Chang E*[2], *Tarbox M*[2], *Higuchi K*[4]; *Nupur.Greene@sanofi.com*
[1]*Sanofi*; [2]*PHAR*; [3]*Sanofi*; [4]*Sanofi*

G29 Reduction in medical and pharmaceutical costs in US patients treated with eptinezumab for migraine prevention: A retrospective cohort study

Charleston IV L[1], *Nahas S*[2], *Rieckmann A*[3], *Regnier S*[4], *Fiore D*[4], *Sullivan C*[4], *Grossman S*[4], *Talon B*[4], *Graziadio A*[5]; *charles6@msu.edu*; *AMGZ@lundbeck.com*
[1]*Michigan State University College of Human Medicine*; [2]*Department of Neurology, Thomas Jefferson University, Jefferson Headache Center*; [3]*H Lundbeck A/S*; [4]*Lundbeck LLC*; [5]*Lundbeck LLC*

G30 Real-world effectiveness of intravenous eptinezumab in patients with chronic migraine and previous subcutaneous preventive migraine treatment

Argoff C[1], *Herzog S*[2], *Smith R*[3], *Soni-Brahmbhatt S*[4], *Awad S*[5], *Asher D*[4], *Khan F*[6], *Boyle L*[5]; *charles.argoff@gmail.com*; *LEBY@lundbeck.com*
[1]*Albany Medical Center*; [2]*Texas Neurology*; [3]*St. Luke's Clinic*; [4]*Lundbeck LLC*; [5]*Lundbeck LLC*; [6]*The McCasland Family Comprehensive Headache Center, Ochsner Neurosciences Institute*

G31 Number-needed-to-treat and cost-per-responder analysis of anti–calcitonin gene–related peptide monoclonal antibodies for migraine prevention

Mitsikostas D[1], *Lee X*[2], *Boserup L*[2], *Kongerslev R*[2], *Phul R*[2], *Sacco S*[3], *Graziadio A*[4]; *dmitsikostas@uoa.gr*; *AMGZ@lundbeck.com*
[1]*Aeginition Hospital, Medical School, National and Kapodistrian University of Athens*; [2]*H. Lundbeck A/S*; [3]*Università degli Studi dell'Aquila*; [4]*Lundbeck LLC*

G32 Long-term reductions in headache frequency, severity, and disability in patients with chronic migraine treated with eptinezumab: Post hoc analyses of the PREVAIL study

Blumenfeld A[1], *Starling A*[2], *Kudrow D*[1], *Kapur N*[3], *Grossman S*[4], *Awad S*[3], *Lipton R*[5], *Boyle L*[3]; *ablumenfeldmd@gmail.com*; *LEBY@lundbeck.com*
[1]*The Los Angeles Headache Center*; [2]*Mayo Clinic Arizona*; [3]*Lundbeck LLC*; [4]*Lundbeck LLC*; [5]*Department of Neurology, Albert Einstein College of Medicine*

G33 The economic burden of idiopathic hypersomnia in the United States: Analysis of the National Health and Wellness Survey

Drachenberg C[1], *Cambron-Mellott J*[2], *Yang L*[2], *Balkaran B*[2], *Whalen M*[1], *Plante D*[3]; *Caroleen.Drachenberg@jazzpharma.com*
[1]*Jazz Pharmaceuticals*; [2]*Oracle Life Sciences, Oracle Corporation*; [3]*School of Medicine and Public Health, University of Wisconsin–Madison*

G34 Combined efficacy and safety of AXS-07 (MoSEIC meloxicam and rizatriptan) in two phase 3 clinical trials

Tepper S[1], *Lipton R*[2], *Chhabra A*[3], *Streicher C*[4], *Tabuteau H*[4]; *sjtepper@gmail.com*
[1]*The New England Institute for Neurology and Headache*; [2]*Department of Neurology, Albert Einstein College of Medicine*; [3]*Axsome Therapeutics, Inc.*; [4]*Axsome Therapeutics Inc.*

G45 Real-world treatment patterns of patients with Dravet syndrome and Lennox-Gastaut syndrome in the United States

Kothare S[1], *Gibson G*[2], *Simontacchi K*[3], *Viswanathan H*[3], *Thomas S*[4], *Connolly H*[2], *Salpekar J*[5], *Lin J*[4]; *skothare@northwell.edu*; *Junji.Lin@jazzpharma.com*
[1]*Cohen Children's Medical Center & Zucker School of Medicine*; [2]*Adelphi Real World*; [3]*Jazz Pharmaceuticals, Inc*; [4]*Jazz Pharmaceuticals*; [5]*Kennedy Krieger Institute, Johns Hopkins University School of Medicine*

H8 Efficacy of cyclosporine ophthalmic solution 0.09% in patients with uncontrolled dry eye disease: An analysis by sex

Johnston J[1], *Adler R*[2], *Hessen M*[3], *Nichols K*[4], *Pflugfelder S*[5], *Truett K*[6], *Urbieta M*[7], *Mitchell B*[8]; *josh.johnston@gaeyepartners.com*; *Brittany.Mitchell@sunpharma.com*
[1]*Georgia Eye Partners*; [2]*Belcara Health*; [3]*Wilmer Eye Institute, Johns Hopkins University*; [4]*School of Optometry, University of Alabama at Birmingham*; [5]*Alkek Eye Center, Baylor College of Medicine*; [6]*KCT Data, Inc.*; [7]*Sun Pharmaceutical Industries, Inc.*; [8]*Sun Pharma*

I8 Differences in medical care across the left ventricular ejection fraction spectrum following first hospitalization for heart failure in a US health care system

Kraus B[1], *Qiao Q*[1], *Nichols G*[2]; *bettina.kraus@boehringer-ingelheim.com*
[1]*Boehringer Ingelheim International GmbH*; [2]*Kaiser Permanente Center for Health Research*

I9 Efficacy and safety of aficamten in the first cohort of patients with symptomatic obstructive hypertrophic cardiomyopathy completing 48-week follow-up: Findings from the FOREST-HCM study

Saberi S[1], *Abraham T*[2], *Choudhury L*[3], *Owens A*[4], *Tower-Rader A*[5], *Rader F*[6], *Garcia-Pavia P*[7], *Olivotto I*[8], *Coats C*[9], *Fifer M*[10], *Solomon S*[11], *Watkins H*[12], *Heitner S*[13], *Jacoby D*[13], *Kupfer S*[13], *Malik F*[13], *Melloni C*[13], *Richards A*[13], *Wei J*[13], *Maron M*[14], *Masri A*[15]; *saberis@med.umich.edu*; *arichards@cytokinetics.com*
[1]*University of Michigan Health Frankel Cardiovascular Center*; [2]*University of California, San Francisco*; [3]*Northwestern University Feinberg School of Medicine*; [4]*Perelman School of Medicine at the University of Pennsylvania*; [5]*Massachusetts General Hospital*; [6]*Cedars-Sinai Medical Center*; [7]*Hospital Universitario Puerta de Hierro Majadahonda, IDIPHISA, CIBERCV*; [8]*Meyer Children's Hospital, Istituto di Ricovero e Cura a Carattere Scientifico*; [9]*School of Cardiovascular and Metabolic Health, University of Glasgow*; [10]*Massachusetts General Hospital*; [11]*Brigham and Women's Hospital*; [12]*University of Oxford*; [13]*Cytokinetics*; [14]*Lahey Hospital and Medical Center*; [15]*OHSU Center for Hypertrophic Cardiomyopathy and Amyloidosis*

I12 Timing of proprotein convertase subtilisin/kexin type 9 inhibitor initiation and its relationship with clinical outcomes in US patients with prior cardiovascular events: A claims database analysis

Patel N[1], *Kalich B*[1], *Miglins M*[2], *Multani J*[3], *Sun K*[3], *Sidelnikov E*[4], *Baber U*[5]; *npatel05@amgen.com*; *bethanykalich@gmail.com*
[1]*Amgen*; [2]*BluePath Solutions, Inc.*; [3]IQVIA; [4]*Amgen (Europe) GmbH*; [5]*OU Health*

I14 Cardiac magnetic resonance imaging paralleled recurrent pericarditis clinical response to rilonacept treatment over 18 months: A RHAPSODY subgroup analysis

Khan A[1], *Cremer P*[2], *Brucato A*[3], *Insalaco A*[4], *Lin D*[5], *Luis S*[6], *Kwon D*[7], *Jellis C*[7], *Clair J*[1], *Curtis A*[1], *Wang S*[1], *Klein A*[7], *Imazio M*[8], *Paolini J*[1]; *akhan@kiniksa.com*; *paul.cremer@northwestern.edu*
[1]*Kiniksa Pharmaceuticals*; [2]*Northwestern University Feinberg School of Medicine*; [3]*University of Milan, Fatebenefratelli Hospital*; [4]*Ospedale Pediatrico Bambino Gesù*; [5]*Allina Health*; [6]*Mayo Clinic*; [7]*Cleveland Clinic*; [8]*University Hospital "Santa Maria della Misericordia", ASUFC*

J1 US patients with chronic rhinosinusitis with nasal polyps enrolled in the AROMA registry have a high burden of disease at baseline regardless of clinician specialty

Borish L[1], *Justice J*[2], *McWilliams L*[3], *Jang D*[4], *Xia C*[5], *Phalen T*[5], *De Prado Gomez L*[6], *Corbett M*[7], *Nash S*[5], *Jacob-Nara J*[7], *Sacks H*[5]; *lb4m@virginia.edu*; *timothy.phalen@regeneron.com*
[1]*University of Virginia Health System*; [2]*University of Florida College of Medicine*; [3]*Allergy Partners of the Triangle*; [4]*Duke University School of Medicine*; [5]*Regeneron Pharmaceuticals Inc.*; [6]*Sanofi*; [7]*Sanofi*

J2 Health care utilization and health disparities for patients with non–cystic fibrosis bronchiectasis in the United States

Zha B[1], *Jia S*[2], *Feliciano J*[3], *Mohanty M*[3], *Tkacz J*[4], *Moore-Schiltz L*[4], *Lauterio M*[3], *Weinberger J*[5]; *Shoshana.Zha@ucsf.edu*; *Joseph.Feliciano@insmed.com*
[1]*University of California San Francisco*; [2]*University of Michigan*; [3]*Insmed Incorporated*; [4]*Inovalon*; [5]*Tufts Medical Center*

J3 Chronic obstructive pulmonary disease–related health care resource utilization before and after initiating inhaled corticosteroid–containing maintenance regimens: Real-world claims data

Siddharthan T[1], *Lamprey C*[2], *Aggarwal K*[2], *Pan Y*[3], *Tejwani V*[4]; *tsiddhar@miami.edu*; *claudia.lamprey@veronapharma.com*
[1]*University of Miami*; [2]*Verona Pharma*; [3]*Stratevi*; [4]*Cleveland Clinic*

J4 Real-world evidence of the effectiveness of inhaled corticosteroid–containing therapies in reducing exacerbations and medical costs in chronic obstructive pulmonary disease

Siddharthan T[1], *Lamprey C*[2], *Aggarwal K*[2], *Pan Y*[3], *Tejwani V*[4]; *tsiddhar@miami.edu*; *claudia.lamprey@veronapharma.com*
[1]*University of Miami*; [2]*Verona Pharma*; [3]*Stratevi*; [4]*Cleveland Clinic*

J5 Progression to GOLD E in patients with chronic obstructive pulmonary disease results in substantial health care resource utilization and costs

Sethi S[1], Siddharthan T[2], Tejwani V[3], Lamprey C[4], Dixon A[4], Wan E[5]; ssethi@buffalo.edu; claudia.lamprey@veronapharma.com
[1]University at Buffalo; [2]University of Miami; [3]Cleveland Clinic; [4]Verona Pharma; [5]Brigham and Women's Hospital, Harvard Medical School

J6 Economic impact of inhaled chronic obstructive pulmonary disease maintenance medications: Costs and health care resource utilization from claims data before and after treatment initiation

Sethi S[1], Siddharthan T[2], Tejwani V[3], Lamprey C[4], Aggarwal K[4], Wan E[5]; ssethi@buffalo.edu; claudia.lamprey@veronapharma.com
[1]University at Buffalo; [2]University of Miami; [3]Cleveland Clinic; [4]Verona Pharma; [5]Brigham and Women's Hospital, Harvard Medical School

J7 Trends in treatment initiation of inhaled maintenance medications for chronic obstructive pulmonary disease over the last 7 years

Siddharthan T[1], Tejwani V[2], Wan E[3], Lamprey C[4], Aggarwal K[4], Sethi S[5]; tsiddhar@miami.edu; claudia.lamprey@veronapharma.com
[1]University of Miami; [2]Cleveland Clinic; [3]Brigham and Women's Hospital, Harvard Medical School; [4]Verona Pharma; [5]University at Buffalo

J8 Dupilumab safety and efficacy in a real-world clinical setting: The RAPID asthma registry

Peters A[1], Hilberg O[2], Lugogo N[3], Ramsey A[4], Shao L[5], de Prado Gómez L[6], Jacob-Nara J[7], Sacks H[5], J Rowe P[7], Deniz Y[5], Gall R[5]; anjupeters@northwestern.edu; Rebecca.Gall@regeneron.com
[1]Northwestern University Feinberg School of Medicine; [2]University of Southern Denmark; [3]University of Michigan; [4]Rochester Regional Health; [5]Regeneron Pharmaceuticals Inc.; [6]Sanofi; [7]Sanofi

J9 Dupilumab effect on exacerbations and lung function despite withdrawal of inhaled corticosteroids/long-acting β agonists

Pandit-Abid N[1], Wechsler M[2], J Jackson D[3], F Rabe K[4], D Pavord I[5], Virchow J[6], Katial R[7], Israel E[8], Xia C[9], Soliman M[10], J Rowe P[1], Deniz Y[9], Sacks H[9], Jacob-Nara J[1]; nami.pandit-abid@sanofi.com; wechslerm@njhealth.org
[1]Sanofi; [2]National Jewish Health; [3]Guy's Severe Asthma Centre, Guy's & St Thomas' NHS Trust; Asthma UK Centre, School of Immunology & Microbial Sciences, King's College London; [4]Airway Research Center North (ARCN), LungenClinic Grosshansdorf (member of the German Center for Lung Research [DZL]); Airway Research Center North (ARCN), Christian-Albrechts University (member of the German Center for Lung Research [DZL]); [5]National Institute for Health Research Oxford Biomedical Research Centre, and Nuffield Department of Medicine, University of Oxford; [6]Department of Pneumology, University of Rostock; [7]National Jewish Health, Division of Allergy and Clinical Immunology, Department of Medicine; [8]Division of Pulmonary and Critical Care Medicine and Allergy and Immunology, Brigham and Women's Hospital, Harvard Medical School; [9]Regeneron Pharmaceuticals Inc.; [10]Regeneron Pharmaceuticals, Inc

J15 A time and motion study of nebulized short-acting β agonists and muscarinic antagonists for chronic obstructive pulmonary disease in inpatient and long-term settings

Johnson K[1], Kuhn B[2], Jafari D[3], Gummadi R[4], De Cock E[5], Madaj K[6], Leung G[7], Craddock K[2], Johnson J[3]; karmonjohnson@outlook.com; btkuhn@ucdavis.edu
[1]Previously employed at Theravance Biopharma US; [2]UC Davis School of Medicine; [3]Northwell Health; [4]Barlow Respiratory Hospital; [5]Syneos Health Spain; [6]Syneos Health US; [7]Theravance Biopharma US

L1 Treatment efficacy in patients with moderate to severe atopic dermatitis who switched from dupilumab to abrocitinib in JADE EXTEND, a phase 3 long-term extension study

TBC T[1], Gooderham M[2], Weidinger S[3], Simpson E[4], Deleuran M[5], Stein Gold L[6], Farooqui S[7], Biswas P[8], Chan G[9], Güler E[10], Koppensteiner H[11]; TBC@tbc.com; mjgooderham@gmail.com
[1]Pfizer, Inc.; [2]SKiN Centre for Dermatology; [3]Univerity Hospital Schleswig-Holstein; [4]Oregon Health & Science University; [5]Aarhus University Hospital; [6]Henry Ford Health; [7]Pfizer R&D UK Ltd.; [8]Pfizer Inc.; [9]Pfizer Inc.; [10]Pfizer Inc.; [11]Pfizer Corporation Austria GmbH

L2 Real-world treatment patterns of patients with atopic dermatitis on tralokinumab by age: A claims-based analysis

Butler D[1], *Claxton A*[2], *Ng V*[3], *Guerin A*[4], *Gauthier-Loiselle M*[4], *Burne R*[4], *Balu S*[3]; *danielbutlermd@gmail.com*; *sabau@leo-pharma.com*
[1]*University of Arizona*; [2]*Ionis Pharmaceuticals, Inc.*; [3]*LEO Pharma Inc.*; [4]*Analysis Group, Inc.*

L3 Economic modeling of topical and systemic treatments for atopic dermatitis: A structured literature review

Silverberg J[1], *Stephenson B*[2], *Yim C*[3], *Lewis J*[3], *Almuallem L*[4], *Cohen L*[4], *Persaud E*[4], *Singh S*[4]; *jonathanisilverberg@gmail.com*; *bstephenson@arcutis.com*
[1]*George Washington University School of Medicine and Health Sciences*; [2]*Arcutis Biotherapeutics, Inc*; [3]*Arcutis Biotherapeutics, Inc*; [4]*EVERSANA*

L4 Efficacy comparison of targeted systemic monotherapies including lebrikizumab for moderate to severe atopic dermatitis: A network meta-analysis

Silverberg J[1], *Bieber T*[2], *Paller A*[3], *Beck L*[4], *Kamata M*[5], *Puig L*[6], *Wiseman M*[7], *Ezzedine K*[8], *Foley P*[9], *Johansson E*[10], *Dossenbach M*[10], *Akmaz B*[11], *Casillas M*[10], *Karlsson A*[12], *Chovatiya R*[13]; *jonathanisilverberg@gmail.com*; *johansson_erin@lilly.com*
[1]*George Washington University School of Medicine and Health Sciences*; [2]*Department of Dermatology and Allergy, University of Bonn and Christine Kühne-Center for Allergy Research and Education*; [3]*Department of Dermatology, Northwestern University*; [4]*University of Rochester Medical Center*; [5]*Department of Dermatology, Teikyo University School of Medicine*; [6]*Department of Dermatology, Hospital de la Santa Creu i Sant Pau*; [7]*Section of Dermatology, University of Manitoba and SKiNWISE Dermatology*; [8]*EpiDermE, Université Paris-Est*; [9]*Skin Health Institute*; [10]*Eli Lilly and Company*; [11]*Almirall S.A.*; [12]*Costello Medical*; [13]*Department of Dermatology, Northwestern University Feinberg School of Medicine*

L5 Real-world baseline characteristics and persistence in adult patients initiating tralokinumab in the CorEvitas Atopic Dermatitis Registry

Simpson E[1], *Balu S*[2], *Choi J*[3], *Li A*[3], *Pugach O*[3], *Schneider S*[2], *Silverberg J*[4]; *simpsone@ohsu.edu*; *sabau@leo-pharma.com*
[1]*Oregon Health & Science University*; [2]*LEO Pharma Inc.*; [3]*CorEvitas, LLC*; [4]*George Washington University School of Medicine and Health Sciences*

L6 Real-world effectiveness of persistent tralokinumab use on clinician- and patient-reported outcomes in patients with atopic dermatitis in the CorEvitas Atopic Dermatitis Registry

Silverberg J[1], *Balu S*[2], *Choi J*[3], *Li A*[3], *Pugach O*[3], *Schneider S*[2], *Simpson E*[4]; *jonathanisilverberg@gmail.com*; *sabau@leo-pharma.com*
[1]*George Washington University School of Medicine and Health Sciences*; [2]*LEO Pharma Inc.*; [3]*CorEvitas, LLC*; [4]*Oregon Health & Science University*

L7 Efficacy and safety of lebrikizumab in adult and adolescent patients with skin of color and moderate to severe atopic dermatitis: An interim analysis of the open-label phase 3b trial Admirable

Alexis A[1], *Moiin A*[2], *Waibel J*[3], *Wallace P*[4], *Cohen D*[5], *Kwong P*[6], *Atwater A*[7], *Harris C*[7], *Proper J*[7], *Pierce E*[7], *Moore A*[8], *Luy I*[7]; *drandrewalexis@gmail.com*; *irene.luy@lilly.com*
[1]*Weill Cornell Medicine*; [2]*Comprehensive Dermatology Center*; [3]*Miami Dermatology and Laser Institute*; [4]*Wallace Skin Research Center*; [5]*Skin Care Physicians of Georgia*; [6]*Suncoast Skin Solutions, Inc.*; [7]*Eli Lilly and Company*; [8]*Baylor University Medical Center; Arlington Research Center; Arlington Center for Dermatology*

L8 Pooled efficacy, patient-reported outcomes, and safety of roflumilast cream 0.15% from the INTEGUMENT-1 and INTEGUMENT-2 phase 3 trials of patients aged 6 years or older with atopic dermatitis

Simpson E[1], *Boguniewicz M*[2], *Eichenfield L*[3], *Gonzalez M*[4], *Hebert A*[5], *Prajapati V*[6], *Gooderham M*[7], *Krupa D*[8], *Chu D*[8], *Higham R*[8], *Berk D*[8]; *simpsone@ohsu.edu*; *dberk@arcutis.com*
[1]*Oregon Health & Science University*; [2]*National Jewish Health and University of Colorado School of Medicine*; [3]*Rady Children's Hospital-San Diego, University of California, San Diego*; [4]*Pediatric Skin Research, LLC*; [5]*UT Health McGovern Medical School*; [6]*Dermatology Research Institute, Probity Medical Research, Skin Health & Wellness Centre, and University of Calgary*; [7]*SkiN Centre for Dermatology, Probity Medical Research, and Queen's University*; [8]*Arcutis Biotherapeutics, Inc.*

L9 Long-term safety and efficacy of roflumilast cream 0.15% in patients aged 6 years or older with mild to moderate atopic dermatitis: A 52-week, phase 3, open-label extension trial

Simpson E[1], *Eichenfield L*[2], *Papp K*[3], *Forman S*[4], *Hebert A*[5], *Gonzalez M*[6], *Gooderham M*[7], *Hong H*[8], *Prajapati V*[9], *Guttman-Yassky E*[10], *Silverberg J*[11], *Seal M*[12], *Krupa D*[13], *Burnett P*[13], *Snyder S*[13], *Chu D*[13], *Higham R*[13], *Berk D*[13]; *simpsone@ohsu.edu*; *dberk@arcutis.com*
[1]*Oregon Health & Science University*; [2]*Rady Children's Hospital-San Diego, University of California, San Diego*; [3]*Probity Medical Research and Alliance Clinical Trials and University of Toronto*; [4]*ForCare Medical Center*; [5]*UT Health McGovern Medical School*; [6]*Pediatric Skin Research, LLC*; [7]*SkiN Centre for Dermatology, Probity Medical Research, and Queen's University*; [8]*Probity Medical Research and University of British Columbia, Department of Dermatology and Skin Science*; [9]*Dermatology Research Institute, Probity Medical Research, Skin Health & Wellness Centre, and University of Calgary*; [10]*Icahn School of Medicine at Mount Sinai*; [11]*George Washington University School of Medicine and Health Sciences*; [12]*Arcutis Biotherapeutics*; [13]*Arcutis Biotherapeutics, Inc.*

L10 Efficacy and safety of once-daily roflumilast cream 0.05% in pediatric patients aged 2-5 years with mild to moderate atopic dermatitis (INTEGUMENT PED): A phase 3 randomized controlled trial

Eichenfield L[1], *Browning J*[2], *Funk T*[3], *Gonzalez M*[4], *Hebert A*[5], *Lee M*[6], *Prajapati V*[7], *Serrao R*[8], *Swanson L*[9], *Higham R*[10], *Berk D*[10]; *leichenfield@gmail.com*; *dberk@arcutis.com*
[1]*Rady Children's Hospital-San Diego, University of California, San Diego*; [2]*Texas Dermatology and Laser Specialists*; [3]*Oregon Health and Science University*; [4]*Pediatric Skin Research, LLC*; [5]*UT Health McGovern Medical School*; [6]*Progressive Clinical Research*; [7]*Dermatology Research Institute, Probity Medical Research, Skin Health & Wellness Centre, and University of Calgary*; [8]*Dermatologists of Central States*; [9]*Ada West Dermatology*; [10]*Arcutis Biotherapeutics, Inc.*

L11 Tapinarof cream 1% once daily: Consistent efficacy across disease severity and age subgroups in the treatment of adults and children down to age 2 years with atopic dermatitis

Fonacier L[1], *Boguniewicz M*[2], *Ohayon J*[3], *Brown P*[4], *Rubenstein D*[4], *Tallman A*[4], *Silverberg J*[5]; *Luz.Fonacier@nyulangone.org*
[1]*NYU Long Island School of Medicine*; [2]*National Jewish Health, Denver, & University of Colorado School of Medicine*; [3]*Hamilton Allergy and McMaster University*; [4]*Dermavant Sciences, Inc.*; [5]*George Washington University School of Medicine and Health Sciences*

L17 A systematic literature review and meta-analysis of the real-world effectiveness, quality of life, and safety of tildrakizumab for moderate to severe plaque psoriasis

Gottlieb S[1], *Farberg A*[2], *Bhatia N*[3], *Fazeli M*[4], *Hofer K*[4], *Lam O*[4], *Barghout V*[5], *Mathew J*[6], *Ferro T*[7]; *scottgott@aol.com*; *Jacob.Mathew@sunpharma.com*
[1]*Schweiger Dermatology Group*; [2]*Section of Dermatology, Baylor Scott & White Health System and Bare Dermatology*; [3]*Therapeutics Clinical Research*; [4]*Evidinno Outcomes Research Inc.*; [5]*Viver Health LLC*; [6]*Sun Pharmaceutical Industries, Inc.*; [7]*Sun Pharmaceutical Industries, Inc.*

L20 Change in patient-reported hair satisfaction during deuruxolitinib treatment of severe alopecia areata: Pooled data from the phase 3 THRIVE-AA1 and THRIVE-AA2 trials

Mirmirani P[1], *Mesinkovska N*[2], *King B*[3], *Senna M*[4], *Mostaghimi A*[5], *Hamilton C*[6], *Cassella J*[6]; *Paradi.Mirmirani@kp.org*; *James.Cassella@sunpharma.com*
[1]*Deparment of Dermatology, The Permanente Medical Group*; [2]*Department of Dermatology, University of California Irvine*; [3]*Department of Dermatology, Yale School of Medicine*; [4]*Lahey Hair Loss Center of Excellence, Lahey Hospital and Medical Center*; [5]*Department of Dermatology, Brigham and Women's Hospital*; [6]*Sun Pharmaceutical Industries, Inc.*

L21 Improvement in anxiety and depression in adult patients with severe alopecia areata treated with deuruxolitinib: Pooled data from the THRIVE-AA1 and THRIVE-AA2 phase 3 trials

Mostaghimi A[1], King B[2], Senna M[3], Mesinkovska N[4], Mirmirani P[5], Hamilton C[6], Cassella J[6]; amostaghimi@bwh.harvard.edu; James.Cassella@sunpharma.com
[1]*Department of Dermatology, Brigham and Women's Hospital*; [2]*Department of Dermatology, Yale School of Medicine*; [3]*Lahey Hair Loss Center of Excellence, Lahey Hospital and Medical Center*; [4]*Department of Dermatology, University of California Irvine*; [5]*Deparment of Dermatology, The Permanente Medical Group*; [6]*Sun Pharmaceutical Industries, Inc.*

L22 Optimization of deuruxolitinib dosing in adult patients with alopecia areata: Results from a randomized, parallel-group, multicenter, phase 2 trial

King B[1], Kempers S[2], Mesinkovska N[3], Mirmirani P[4], McMichael A[5], Mackay-Wiggan J[6], Senna M[7], Roberts J[8], Mostaghimi A[9], Jarell A[10], Hamilton C[11], Cassella J[11]; brett.aking@gmail.com; James.Cassella@sunpharma.com
[1]*Department of Dermatology, Yale School of Medicine*; [2]*Minnesota Clinical Study Center*; [3]*Department of Dermatology, University of California Irvine*; [4]*Deparment of Dermatology, The Permanente Medical Group*; [5]*Department of Dermatology, Wake Forest School of Medicine*; [6]*Siperstein Dermatology Group*; [7]*Lahey Hair Loss Center of Excellence, Lahey Hospital and Medical Center*; [8]*Northwest Dermatology Institute*; [9]*Department of Dermatology, Brigham and Women's Hospital*; [10]*Department of Dermatology, Northeast Dermatology Associates*; [11]*Sun Pharmaceutical Industries, Inc.*

L23 Health care resource utilization among patients with generalized pustular psoriasis with and without documented flares

Lavasani L[1], Rasouliyan L[2], Noe M[3], Rhoads J[4], Laouri M[5]; layla.lavasani@boehringer-ingelheim.com
[1]*Boehringer Ingelheim Pharmaceuticals, Inc*; [2]*OMNY Health*; [3]*Brigham and Women's Hospital*; [4]*University of Utah*; [5]*Boehringer Ingelheim Pharmaceuticals Inc*

L24 Four-weekly dosing intervals with subcutaneous spesolimab appear to be required for optimal prevention of generalized pustular psoriasis flares: Data from the Effisayil 2 and Effisayil ON trials

Thaci D[1], Morita A[2], Strober B[3], Torres T[4], Pinter A[5], Marzano A[6], Krueger J[7], Tang M[8], Hofmann P[9], Thoma C[9], Lebwohl M[10]; diamant.thaci@uksh.de
[1]*University Luebeck*; [2]*Nagoya City University Graduate School of Medical Sciences*; [3]*Yale University*; [4]*University of Porto*; [5]*University Hospital Frankfurt am Main*; [6]*Foundation IRCCS Ca' Granda Ospedale Maggiore Policlinico*; [7]*The Rockefeller University*; [8]*Boehringer Ingelheim (China)*; [9]*Boehringer Ingelheim International GmbH*; [10]*Department of Dermatology, Icahn School of Medicine at Mount Sinai*

M1 Treatment patterns and outcomes in patients with macrophage activation syndrome secondary to Still disease treated with emapalumab: The REAL-HLH study

Allen C[1], Chandrakasan S[2], Jordan M[3], Leiding J[4], Oladapo A[5], Pednekar P[6], Walkovich K[7], Yee J[8]; ceallen@texaschildrens.org; abiola.oladapo@sobi.com
[1]*Division of Pediatric Hematology and Oncology, Baylor College of Medicine*; [2]*Division of Bone and Marrow Transplant Research, Aflac Cancer and Blood Disorders Center, Children's Healthcare of Atlanta*; [3]*Division of Bone Marrow Transplantation and Immune Deficiency, Cincinnati Children's Hospital Medical Center; Department of Pediatrics, University of Cincinnati College of Medicine*; [4]*Division of Allergy and Immunology, Department of Pediatrics, Johns Hopkins University; bluebird bio*; [5]*Sobi Inc.*; [6]*PRECISIONheor*; [7]*Division of Pediatric Hematology Oncology, Department of Pediatrics, University of Michigan Medical School*; [8]*Sobi Inc*

M6 Effectiveness of interleukin 6 receptor inhibitors vs conventional synthetic immunomodulatory therapy for treatment of frail patients with polymyalgia rheumatica

Patel S[1], Sattui S[2], Dejaco C[3], Ford K[4], Fiore S[5], Unizony S[6], Xie F[7], Curtis J[8]; shani.patel@sanofi.com; ssattui@pitt.edu
[1]*Sanofi*; [2]*Division of Rheumatology and Clinical Immunology, University of Pittsburgh*; [3]*Rheumatology, Hospital of Bruneck (ASAA-SABES), Teaching Hospital of the Paracelsius Medical University; Division of Rheumatology and Clinical Immunology, Department of Internal Medicine, Medical University*; [4]*Sanofi*; [5]*Sanofi, Medical Affairs*; [6]*Massachusetts General Hospital, Harvard Medical School*; [7]*Foundation for Advancing Science, Technology, Education and Research*; [8]*The University of Alabama at Birmingham; Foundation for Advancing Science, Technology, Education and Research*

M7 Clinical and economic burden of polymyalgia rheumatica in patients with an inadequate response to glucocorticoids or glucocorticoids taper in a real-world setting

Murawski J[1], *Curtis J*[2], *Araujo L*[3], *Fiore S*[4], *Sattui S*[5], *Stone J*[6], *Yip B*[7], *Ford K*[7], *Xie F*[8]; *jacob.murawski@sanofi.com*; *jrcurtis@uabmc.edu*
[1]*Sanofi*; [2]*The University of Alabama at Birmingham; Foundation for Advancing Science, Technology, Education and Research*; [3]*Sanofi, Health Economics & Value Assessment*; [4]*Sanofi, Medical Affairs*; [5]*Division of Rheumatology and Clinical Immunology, University of Pittsburgh*; [6]*Massachusetts General Hospital*; [7]*Sanofi*; [8]*Foundation for Advancing Science, Technology, Education and Research*

M8 Effectiveness of interleukin 6 receptor inhibitors vs methotrexate or any conventional immunomodulators in patients with steroid-refractory polymyalgia rheumatica

Reed C[1], *Curtis J*[2], *Ford K*[3], *Dua A*[4], *Spiera R*[5], *Fiore S*[6], *Isaman D*[3], *Araujo L*[7], *Petruski-Ivleva N*[3], *Xie F*[8]; *casey.reed@sanofi.com*; *jrcurtis@uabmc.edu*
[1]*Population Health Medical Engagement - Medical Value and Outcomes*; [2]*The University of Alabama at Birmingham; Foundation for Advancing Science, Technology, Education and Research*; [3]*Sanofi*; [4]*Division of Rheumatology, Northwestern University, Feinberg School of Medicine*; [5]*Sanofi*; [6]*Sanofi, Medical Affairs*; [7]*Sanofi, Health Economics & Value Assessment*; [8]*Foundation for Advancing Science, Technology, Education and Research*

M10 Micro-dystrophin expression and safety with delandistrogene moxeparvovec gene therapy for Duchenne muscular dystrophy in a broad population: Phase 1b trial (ENDEAVOR)

Proud C[1], *Zaidman C*[2], *McDonald C*[3], *Day J*[4], *Thrasher P*[5], *Asher D*[5], *Murphy A*[6], *Guridi M*[7], *Ding K*[5], *Reid C*[6], *Lewis S*[5], *Magistrado-Coxen P*[5], *Palatinsky E*[5], *Wandel C*[7], *Potter R*[5], *Rodino-Klapac L*[8], *Mendell J*[9], *Basoff D*[5]; *Crystal.Proud@chkd.org*; *DBasoff@sarepta.com*
[1]*Children's Hospital of the King's Daughters*; [2]*Department of Neurology, Washington University in St Louis*; [3]*UC Davis Health*; [4]*Department of Neurology, Stanford University*; [5]*Sarepta Therapeutics, Inc.*; [6]*Roche Products Ltd*; [7]*F. Hoffmann-La Roche Ltd*; [8]*Sarepta Therapeutics, Inc*; [9]*Center for Gene Therapy, Nationwide Children's Hospital and The Ohio State University*

M11 Five-year outcomes with delandistrogene moxeparvovec in patients with Duchenne muscular dystrophy: A phase 1/2a study

Mendell J[1], *Sahenk Z*[1], *Lowes L*[1], *Reash N*[2], *Iammarino M*[2], *Alfano L*[2], *Signorovitch J*[3], *Jin J*[4], *Gao P*[4], *Mason S*[4], *Elkins J*[4], *Rodino-Klapac L*[4], *Khachatourian A*[4]; *JMendell@sarepta.com*; *akhachatourian@sarepta.com*
[1]*Center for Gene Therapy, Nationwide Children's Hospital and The Ohio State University*; [2]*Center for Gene Therapy, Nationwide Children's Hospital*; [3]*Analysis Group*; [4]*Sarepta Therapeutics, Inc*

M12 Safety and efficacy of delandistrogene moxeparvovec vs placebo in Duchenne muscular dystrophy: Phase 3 EMBARK primary results

Mendell J[1], *Muntoni F*[2], *McDonald C*[3], *Mercuri E*[4], *Ciafaloni E*[5], *Komaki H*[6], *Leon-Astudillo C*[7], *Nascimento A*[8], *Proud C*[9], *Schara-Schmidt U*[10], *Veerapandiyan A*[11], *Zaidman C*[12], *Murphy A*[13], *Reid C*[13], *Asher D*[14], *Darton E*[15], *Mason S*[16], *Fontoura P*[17], *Elkins J*[16], *Rodino-Klapac L*[16], *Kennedy S*[15]; *JMendell@sarepta.com*; *SKennedy@sarepta.com*
[1]*Center for Gene Therapy, Nationwide Children's Hospital and The Ohio State University*; [2]*The Dubowitz Neuromuscular Centre*; [3]*School of Medicine, UC Davis Health*; [4]*Pediatric Neurology Institute*; [5]*University of Rochester Medical Center*; [6]*Translational Medical Center, Kodaira*; [7]*Department of Pediatrics, University of Florida*; [8]*Hospital Sant Joan de Déu*; [9]*Children's Hospital of the King's Daughters*; [10]*Center for Neuromuscular Disorders in Children and Adolescents*; [11]*Arkansas Children's Hospital*; [12]*Department of Neurology, Washington University in St Louis*; [13]*Roche Products Ltd*; [14]*Sarepta Therapeutics, Inc.*; [15]*Sarepta Therapeutics, Inc.*; [16]*Sarepta Therapeutics, Inc*; [17]*F. Hoffmann-La Roche Ltd.*

M13 Heterotopic ossification reduction in palovarotene-treated vs untreated (historical comparator) patients with fibrodysplasia ossificans progressiva: Sensitivity analyses from the phase 3 MOVE and NHS trials

Pignolo R[1], Baujat G[2], Hsiao E[3], Al Mukaddam M[4], Berglund S[5], Cheung A[6], De Cunto C[7], Delai P[8], Kannu P[9], Keen R[10], Mancilla E[11], Marino R[12], Strahs A[12], Kaplan F[4]; Pignolo.Robert@mayo.edu

[1]Department of Medicine, Mayo Clinic; [2]Département de Génétique, Hôpital Universitaire Necker-Enfants Malades, Institut Imagine, Université Paris Cité; [3]Division of Endocrinology and Metabolism, the UCSF Metabolic Bone Clinic, the Eli and Edyth Broad Institute for Regeneration Medicine, and the Institute of Human Genetics, Department of Medicine, and the UCSF Program in Craniofacial Biology, University of California San Francisco; [4]Departments of Orthopedic Surgery and Medicine, Center for Research in FOP and Related Disorders, Perelman School of Medicine, University of Pennsylvania; [5]Department of Clinical Sciences, Pediatrics, Umeå University; [6]Department of Medicine and Joint Department of Medical Imaging, University Health Network, University of Toronto; [7]Pediatric Rheumatology Section, Department of Pediatrics, Hospital Italiano de Buenos Aires; [8]Centro de Pesquisa Clinica, Hospital Israelita Albert Einstein; [9]Hospital for Sick Children; [10]Centre for Metabolic Bone Disease, Royal National Orthopedic Hospital; [11]Children's Hospital of Philadelphia, Perelman School of Medicine, University of Pennsylvania; [12]Ipsen

M14 Impact of palovarotene treatment status on heterotopic ossification in patients with fibrodysplasia ossificans progressiva: Long-term data from the phase 3 MOVE trial

Hsiao E[1], Pignolo R[2], Al Mukaddam M[3], Baujat G[4], Berglund S[5], Cheung A[6], De Cunto C[7], Delai P[8], Kannu P[9], Keen R[10], Mancilla E[11], Marino R[12], Strahs A[12], Kaplan F[3]; edward.hsiao@ucsf.edu

[1]Division of Endocrinology and Metabolism, the UCSF Metabolic Bone Clinic, the Eli and Edyth Broad Institute for Regeneration Medicine, and the Institute of Human Genetics, Department of Medicine, and the UCSF Program in Craniofacial Biology, University of California San Francisco; [2]Department of Medicine, Mayo Clinic; [3]Departments of Orthopedic Surgery and Medicine, Center for Research in FOP and Related Disorders, Perelman School of Medicine, University of Pennsylvania; [4]Département de Génétique, Hôpital Universitaire Necker-Enfants Malades, Institut Imagine, Université Paris Cité; [5]Department of Clinical Sciences, Pediatrics, Umeå University; [6]Department of Medicine and Joint Department of Medical Imaging, University Health Network, University of Toronto; [7]Pediatric Rheumatology Section, Department of Pediatrics, Hospital Italiano de Buenos Aires; [8]Centro de Pesquisa Clinica, Hospital Israelita Albert Einstein; [9]Hospital for Sick Children; [10]Centre for Metabolic Bone Disease, Royal National Orthopedic Hospital; [11]Children's Hospital of Philadelphia, Perelman School of Medicine, University of Pennsylvania; [12]Ipsen

M15 Comparative effectiveness of denosumab vs zoledronic acid among postmenopausal women with osteoporosis in the US Medicare program

Patel N[1], Curtis J[2], Arora T[3], Liu Y[3], Brunetti V[1], Lin T[1], Stad R[1], Spangler L[1], McDermott M[1], Bradbury B[4], Kim M[1]; npatel05@amgen.com; jrcurtis@uabmc.edu

[1]Amgen; [2]The University of Alabama at Birmingham; Foundation for Advancing Science, Technology, Education and Research; [3]School of Public Health; [4]Observational Research

M16 Comparative effectiveness of denosumab versus bisphosphonates among treatment-experienced postmenopausal women with osteoporosis in the US Medicare program

Patel N[1], Curtis J[2], Arora T[3], Liu Y[3], Brunetti V[1], Lin T[1], Stad R[1], Spangler L[1], McDermott M[1], Bradbury B[4], Kim M[1]; npatel05@amgen.com; jrcurtis@uabmc.edu

[1]Amgen; [2]The University of Alabama at Birmingham; Foundation for Advancing Science, Technology, Education and Research; [3]School of Public Health; [4]Observational Research

M18 Interim analysis of EVOLVE: Evaluating eteplirsen treatment in nonambulatory patients in routine clinical practice from a phase 4 observational study

Waldrop M[1], *Grabich S*[2], *Santra S*[2], *Hornibrook S*[2], *Sehinovych I*[2], *Scharf R*[3], *Mathews K*[4]; *megan.waldrop@nationwidechildrens.org*; *sgrabich@sarepta.com*
[1]*Center for Gene Therapy, Nationwide Children's Hospital and Ohio State University Wexner Medical Center*; [2]*Sarepta Therapeutics, Inc.*; [3]*UVA Children's Hospital*; [4]*The University of Iowa*

M19 Change in fracture rate among patients with hypophosphatasia following initiation of Strensiq (asfotase alfa): A retrospective US claims database analysis

Dahir K[1], *Bates T*[2], *Vlasnik J*[2], *Wakeford C*[2], *Donckels E*[3], *Chan P*[3], *Robinson S*[3], *Lyons G*[2]; *kathryn.dahir@vumc.org*; *jon.vlasnik@alexion.com*
[1]*Vanderbilt University Medical Center*; [2]*Alexion Pharmaceuticals, Inc.*; [3]*Inovalon*

M20 Efficacy and safety of corticosteroid-sparing treatments in patients with polymyalgia rheumatica: A systematic literature review

Cholasamudram S[1], *Fiore S*[2], *Steele K*[2], *Joseph J*[1], *Vepuri R*[1], *Voruganti S*[1], *Jasso-Mosqueda J*[3], *Araujo L*[4]; *sirish.cholasamudram@sanofi.com*; *lita.araujo@sanofi.com*
[1]*Sanofi, Health Economics & Value Assessment*; [2]*Sanofi, Medical Affairs*; [3]*Sanofi, Health Economics & Value Assessment*; [4]*Sanofi, Health Economics & Value Assessment*

N1 Characteristics of patients with complement 3 glomerulopathy in a US multicenter assessment

Ndife B[1], *Aldworth C*[1], *Nguyen J*[2], *Pivneva I*[3], *Edwards M*[3], *Anderson A*[3], *Signorovitch J*[4], *Canetta P*[5]; *briana.ndife@novartis.com*
[1]*Novartis Pharmaceuticals Corporation*; [2]*Novartis Pharmaceuticals Corporation*; [3]*Analysis Group*; [4]*Analysis Group*; [5]*Irving Medical Center, Columbia University*

N2 Chronic kidney disease progression in patients with complement 3 glomerulopathy in a US multicenter assessment

Ndife B[1], *Aldworth C*[1], *Nguyen J*[2], *Pivneva I*[3], *Edwards M*[3], *Anderson A*[3], *Signorovitch J*[4], *Canetta P*[5]; *briana.ndife@novartis.com*
[1]*Novartis Pharmaceuticals Corporation*; [2]*Novartis Pharmaceuticals Corporation*; [3]*Analysis Group*; [4]*Analysis Group*; [5]*Irving Medical Center, Columbia University*

N4 Effect of anticholinergic burden on health care costs in patients with overactive bladder: A retrospective database claims analysis

Nesheim J[1], *Richter H*[2], *Chastek B*[3], *Carrera A*[1], *Landis C*[3], *Snyder D*[1], *Abedinzadeh L*[1], *Bancroft T*[3], *Dmochowski R*[4], *Hijaz A*[5], *Frankel J*[6]; *jeffrey.nesheim@us.sumitomo-pharma.com*
[1]*Sumitomo Pharma America, Inc.*; [2]*Department of Obstetrics and Gynecology, University of Alabama at Birmingham*; [3]*Optum*; [4]*Department of Urologic Surgery, Vanderbilt University Medical Center*; [5]*University Hospitals Cleveland Medical Center*; [6]*Seattle Urology Research Center*

U2 Health care resource utilization following 6 months of treatment with olanzapine/samidorphan: Real-world assessment of patients with schizophrenia or bipolar I disorder

Cutler A[1], *Panchmatia H*[2], *Hughes A*[3], *Webb N*[3], *Doane M*[2], *Jain R*[4]; *acutler@ajcmd.com*; *hemangi.parekh@gmail.com*
[1]*SUNY Upstate Medical University*; [2]*Alkermes, Inc.*; [3]*Optum, Inc.*; [4]*Texas Tech University School of Medicine-Permian Basin*

U3 Impact of dose reductions on efficacy of adjuvant abemaciclib for patients with high-risk early breast cancer: Analyses from the monarchE study

O'Shaughnessy J[1], *Cicin I*[2], *Testa L*[3], *Tolaney S*[4], *Huober J*[5], *Guarneri V*[6], *Johnston S*[7], *Martin M*[8], *Rastogi P*[9], *Harbeck N*[10], *Rugo H*[11], *Wei R*[12], *Andre V*[12], *Shahir A*[13], *Goetz M*[14], *LeVoir A*[12]; *Joyce.OShaughnessy@usoncology.com*; *andrea.levoir@lilly.com*
[1]*Baylor University Medical Center, Texas Oncology, US Oncology*; [2]*Trakya University Faculty of Medicine*; [3]*Instituto D'Or de Pesquisa e Ensino (IDOR)*; [4]*Dana-Farber Cancer Institute*; [5]*Cantonal Hospital St. Gallen, Breast Centre St. Gallen and University of Ulm, Department of Gynecology*; [6]*Department of Surgery, Oncology and Gastroenterology, University of Padova; Oncology and Istituto Oncologico Veneto IRCCS*; [7]*Royal Marsden NHS Foundation Trust*; [8]*Hospital General Universitario Gregorio Marañon, Universidad Complutense, CIBERONC, GEICAM*; [9]*University of Pittsburgh/UPMC, NSABP Foundation*; [10]*Breast Centre, Department of Gynaecology and Obstetrics, Comprehensive Cancer Centre München, LMU University Hospital*; [11]*Department of Medicine, University of California San Francisco, Helen Diller Family Comprehensive Cancer Center*; [12]*Eli Lilly and Company*; [13]*Loxo@Lilly*; [14]*Department of Oncology, Mayo Clinic*

U4 Adjuvant abemaciclib plus endocrine therapy for hormone receptor–positive, human epidermal growth factor receptor 2–negative, high-risk early breast cancer: Results from a preplanned monarchE overall survival interim analysis, including 5-year efficacy outcomes

Harbeck N[1], *Rastogi P*[2], *O'Shaughnessy J*[3], *Boyle F*[4], *Cortes J*[5], *Rugo H*[6], *Goetz M*[7], *Hamilton E*[8], *Huang C*[9], *Senkus E*[10], *Tryakin A*[11], *Neven P*[12], *Huober J*[13], *Wei R*[14], *Andre V*[14], *Munoz M*[14], *Antonio B*[14], *Shahir A*[15], *Martin M*[16], *Johnston S*[17], *Suthar A*[14]; *munoz_fernandez_maria@lilly.com*; *ashish.suthar@lilly.com*

[1]*Breast Centre, Department of Gynaecology and Obstetrics, Comprehensive Cancer Centre München, LMU University Hospital*; [2]*University of Pittsburgh/UPMC, NSABP Foundation*; [3]*Baylor University Medical Center, Texas Oncology, US Oncology*; [4]*Sydney Medical School, University of Sydney*; [5]*International Breast Cancer Center (IBCC), Quironsalud Group*; [6]*Department of Medicine, University of California San Francisco, Helen Diller Family Comprehensive Cancer Center*; [7]*Department of Oncology, Mayo Clinic*; [8]*Sarah Cannon Research Institute/Tennessee Oncology*; [9]*Department of Surgery, National Taiwan University Hospital, National Taiwan University College of Medicine*; [10]*Medical University of Gdańsk*; [11]*N.N. Blokhin Russian Cancer Research Center*; [12]*Multidisciplinary Breast Center, University Hospitals Leuven*; [13]*Cantonal Hospital St. Gallen, Breast Centre St. Gallen and University of Ulm, Department of Gynecology*; [14]*Eli Lilly and Company*; [15]*Loxo@Lilly*; [16]*Hospital General Universitario Gregorio Marañon, Universidad Complutense, CIBERONC, GEICAM*; [17]*Royal Marsden NHS Foundation Trust*

U5 Real-world clinical outcomes in US patients with brain metastases secondary to hormone receptor–positive/human epidermal growth factor receptor–negative metastatic breast cancer treated with abemaciclib

Gathirua-Mwang W[1], *Martin H*[1], *He D*[2], *Zheng S*[3], *Sheffield K*[1], *John J*[1], *Rybowski S*[1], *Brastianos P*[4], *Lin A*[1]; *gathirua_mwangi_wambui_grace@lilly.com*; *alexandra.lin@lilly.com*

[1]*Eli Lilly and Company*; [2]*Syneos Health*; [3]*TechData Service Company LLC*; [4]*Harvard Medical School, Massachusetts General Hospital*

U6 Bimekizumab-treated patients with active psoriatic arthritis showed sustained improvements in health-related quality of life, physical function, and work productivity: Up to 2-year results from two phase 3 studies

Ndefo U[1], *Gossec L*[2], *Mease P*[3], *Nash P*[4], *Kristensen L*[5], *Tillett W*[6], *Ink B*[7], *Bajracharya R*[7], *Coarse J*[8], *Lambert J*[8], *Lyris N*[7], *Gladman D*[9]; *ciara.conway@costellomedical.com*; *laure.gossec@aphp.fr*

[1]*Employee and Shareholder of UCB Pharma*; [2]*Sorbonne Universite and Pitie-Salpetriere Hospital*; [3]*Providence-Swedish Medical Center and University of Washington*; [4]*School of Medicine, Griffith University*; [5]*The Parker Institute, Copenhagen University Hospital*; [6]*Royal National Hospital of Rheumatic Diseases; Department of Life Sciences, Centre for Therapeutic Innovation, University of Bath*; [7]*UCB Pharma*; [8]*UCB*; [9]*Schroeder Arthritis Institute, Krembil Research Institute, Toronto Western Hospital, University Health Network, University of Toronto*

SUPPLEMENT

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