ANALYSIS GROUP

Raising the Standard in HEOR

Analysis Group Posters and Presentations

ISPOR 2025 | MAY 13–16 | MONTREAL, QC, CANADA

Analysis Group's health economics and outcomes research (HEOR) professionals have extensive experience helping clients quantify product value in a dynamic and rapidly changing marketplace.

This year, we are pleased to present an educational symposium, podium presentation, and 32 research posters. Please find details below.

If time permits, please say hello to our team at Booth #1001.

ISPOR 2025 Analysis Group Symposium and Podium Presentation

EDUCATIONAL SYMPOSIUM

Wednesday, May 14 | 11:45 a.m.–12:45 p.m. | Montreal Convention Center, Room 518

Driving Evidence-Based Medicine Forward With Generative AI (GenAI)

GenAI is revolutionizing HEOR and RWE by offering innovative methods to process and analyze vast datasets, model complex health economics phenomena, and enhance decision-making processes. GenAI tools powered by large language models (LLMs) facilitate the development of a deeper and more comprehensive understanding of diseases and treatment outcomes, and significantly improve the extraction of insights from diverse data sources such as electronic health records, claims data, and scientific literature. By harnessing the power of GenAI, researchers and practitioners in the field are unlocking new possibilities for advancing health care innovation and improving patient care. Participants in this symposium will explore the frontiers in GenAI, discuss key developments and challenges, and present examples of GenAI's applications in HEOR and RWE research. Among these examples is Analysis Group's own proprietary GenAI platform, AGHealth.ai, which excels in text classification, research summarization, and rapid data analysis. GenAI can also streamline the automated screening of research published in various languages by providing accurate translations and summarizations, thereby informing decision making with comprehensive insights across diverse sources. These GenAI-powered capabilities have greatly improved the efficiency of HEOR and RWE research and offered powerful and creative insights into health care data and literature. The introduction of this and similar GenAI platforms is set to further empower the health care sector, offering more effective and efficient tools to researchers for streamlining complex analyses, enhancing research accuracy, facilitating evidence-based decision making, and deepening overall understanding of complex diseases and treatments, ultimately leading to more effective health care solutions and policymaking.

Presenters:
Eric Q. Wu, Ph.D.; *Managing Principal, Analysis Group*
Jimmy Royer, Ph.D.; *Principal, Analysis Group*
Rajeev Ayyagari, Ph.D.; *Vice President, Analysis Group*
Guo Li, M.B.A.; *Senior Director, Global Real-World Evidence, Johnson & Johnson Innovative Medicine*
Song Wang, Ph.D.; *EUCAN/GEM Lead in Innovation, Global Medical Evidence, Takeda*

PODIUM PRESENTATION

Wednesday, May 14 | 10:15–11:15 a.m.

Economic Burden of Recurrence among Patients with High-Risk Non-Muscle-Invasive Bladder Cancer who Received Bacillus Calmette-Guérin in the United States: A SEER-Medicare Study

Identified as a Top 5% Finalist for the ISPOR 2025 Research Presentation Awards

High-risk non-muscle invasive bladder cancer (HR-NMIBC) is primarily managed with Bacillus Calmette-Guérin (BCG) therapy, yet up to 80% of patients experience disease recurrence within 15 years. While the clinical implications of recurrence are well established, the economic burden of HR-NMIBC remains poorly characterized. In the study being presented, we utilized the SEER-Medicare linkage data to assess health care resource utilization (HCRU) and health care costs among patients with HR-NMIBC who were treated with BCG. We also developed a novel algorithm based on diagnosis and treatment records to identify recurrence events and stratify patients into two cohorts: those with recurrence and those without. Comparative analysis between the two cohorts revealed that patients who experienced recurrence incurred significantly higher HCRU and health care costs, underscoring the unmet need for more effective therapies to reduce economic burden.

Presenting Author: Yizhen Lai, M.Sc.; *Principal Scientist, Merck & Co., Inc.*

Moderator: Xiao Xu, Ph.D.; *Associate Professor, Columbia University Irving Medical Center*

Associated Experts & Consultants:
Wei Gao, Ph.D.; *Vice President, Analysis Group*
Yan Song, Ph.D.; *Vice President, Analysis Group*
Honghao Fang, M.Sc.; *Manager, Analysis Group*
Jiayang Li, Ph.D.; Associate, *Analysis Group*
Pangsibo Shen, M.Sc.; *Senior Analyst, Analysis Group*

Funding for this study was provided by Merck.

ISPOR 2025 Analysis Group Research Posters

POSTER SESSION 1

Wednesday, May 14 | 10:30 a.m.–1:30 p.m.

Temporal Trends in Healthcare Costs Associated with First-Line (1L) Nivolumab + Ipilimumab (N+I) and Pembrolizumab + Lenvatinib (P+L) in Advanced or Metastatic Renal Cell Carcinoma (aRCC)

Objectives: While immuno-oncology combinations have become the standard of care for aRCC, real-world cost comparisons remain limited. This study aims to assess and compare all-cause and RCC-related healthcare costs for patients receiving 1L N+I vs. P+L treatment for aRCC.

Conclusions: N+I is a first-line treatment option for aRCC that offers long-term healthcare-related cost savings and sustainable economic value, largely driven by drug cost differences. Further research with a larger sample and longer follow-up is warranted.

Authors: Managing Principal Keith Betts, Vice President Ella X. Du, Associate Travis Wang, Senior Analyst Sydney Ng, and researchers from Bristol Myers Squibb

Funding for this study was provided by Bristol Myers Squibb.

Changes in HbA1c and Body Weight in US Adults With Type 2 Diabetes and Chronic Kidney Disease Who Received Oral Semaglutide

Objectives: To assess changes in HbA1c and body weight in US adults with type 2 diabetes (T2D) and chronic kidney disease (CKD) treated with oral semaglutide.

Conclusions: Improved glycemic control and reduced body weight were observed among real-world patients with T2D and CKD treated with oral semaglutide.

Authors: Vice Presidents Yan Song and Yan Wang, Senior Analyst Manasvi Sundar, and researchers from Novo Nordisk and the University of Maryland School of Medicine

Funding for this study was provided by Novo Nordisk.

Health Utilities Associated With Pneumococcal Diseases in Children and Adults in the United States: A Targeted Literature Review and Meta-Analysis

Objectives: Pneumococcal disease (PD) and post-meningitis sequelae (PMS) significantly affect health-related quality of life (HRQoL). This study presents meta-analyses of utility estimates for PD and PMS in children and adults in the United States (US).

Conclusions: PD and PMS significantly impair HRQoL in children and adults. However, few studies estimated utility values for these health states in the US, and none have been published within the past 15 years. More recent data is needed to better understand the current burden in the US.

Authors: Vice President Yan Song; Manager Hela Romdhani; Senior Analyst Daisy Liu; and researchers from Merck; XL Source; Sheffield Centre for Health and Related Research, University of Sheffield; and the Division of Pediatric Infectious Diseases at the Duke University School of Medicine

Funding for this study was provided by Merck.

Real-World Cancer Recurrence and Overall Survival Outcomes in Muscle-Invasive Bladder Cancer (MIBC) Patients Treated With Radical Cystectomy (RC): A Retrospective Surveillance, Epidemiology, and End Results-Medicare Study

Objectives: Real-world quantification of cancer recurrence and overall survival (OS) in radically resected MIBC patients is lacking, particularly outcomes by disease stage or treatment received. This study describes the overall clinical burden of MIBC patients undergoing RC and further examines the association between cancer recurrence and OS.

Conclusions: MIBC patients post-RC had high recurrence rates and poor overall survival, regardless of disease stage at diagnosis and treatment received. These findings underscore the substantial clinical burden for these patients and the need for effective therapies that may prevent recurrence and improve survival.

Authors: Vice President Yan Song, Manager Erin Cook, Associate Anya Jiang, Senior Analysts Adina Zhang and Shravanthi Seshasayee, and researchers from Merck and the Penn Presbyterian Medical Center

Funding for this study was provided by Merck.

Characterization of Economic Burden in Individuals With Narcolepsy or Idiopathic Hypersomnia at Higher Risk of Sodium-Associated Negative Clinical Outcomes in the United States

Objectives: Quantify healthcare resource utilization (HCRU) and costs for individuals with narcolepsy or idiopathic hypersomnia (IH) at higher risk of sodium-associated negative clinical outcomes.

Conclusions: Findings highlight an elevated economic burden for individuals with narcolepsy or IH at higher risk of sodium-associated negative clinical outcomes, predominantly driven by sodium-associated negative clinical outcomes and risk factors, emphasizing the need for careful risk management to mitigate avoidable HCRU and costs.

Authors: Managing Principal Annie Guérin, Vice President Patrick S. Gagnon, Associate Kaitlyn Easson, Senior Research Professionals Remi Bellefleur and Mohira Levesque-Leroux, and researchers from Jazz Pharmaceuticals, CardioSleep Diagnostics, Indiana University School of Medicine, and the University of California San Diego

Funding for this study was provided by Jazz Pharmaceuticals.

Progression-Free Survival (PFS) as a Surrogate for Overall Survival (OS) in the Front-Line Maintenance Setting for Advanced Ovarian Cancer (OC): A Trial-Level Meta-Analysis

Objectives: High unmet need persists in the front-line setting for advanced OC, and no approved therapy to date has demonstrated an OS benefit. With OS data requiring years to mature in front-line OC trials, intermediate endpoints like PFS are crucial for accelerating clinical development and informing decision-making. Robust PFS-OS surrogacy can reduce uncertainty around treatment benefits and support stakeholder evaluations. This study assessed PFS-OS surrogacy in the front-line maintenance setting for advanced OC.

Conclusions: This study provides compelling and up-to-date evidence supporting PFS as a surrogate for OS in the front-line maintenance setting for advanced OC, underscoring its utility in enabling earlier assessments of treatment efficacy, facilitating regulatory approvals, strengthening reimbursement submissions, and providing timely access to innovative therapies.

Authors: Vice Presidents Yan Song and Kalé Kponee-Shovein, Associate Jessie Liu, Senior Analyst Qi Hua, Senior Research Professional Jingyi Liu, and researchers from Merck

Funding for this study was provided by Merck.

POSTER SESSION 2

Wednesday, May 14 | 4:00–7:00 p.m.

Real-world Infusion and Post-Infusion Health Care Resource Use (HCRU) and Costs for CAR T Cell Treatment in Large B-Cell Lymphoma (LBCL): a Comparative Study of Liso-cel Versus Axi-cel

Objectives: This retrospective study compares infusion and post-infusion healthcare resource utilization and associated costs for patients with LBCL treated with liso-cel versus axi-cel.

Conclusions: Liso-cel cumulative (infusion and 90-day follow-up) costs were significantly lower than axi-cel. Results were robust to alternative cost inputs.

Authors: Managing Principal Mihran Yenikomshian, Vice Presidents Enrico Zanardo and Lynn Huynh, Associate Jacob Klimek, and researchers from Bristol Myers Squibb

Funding for this study was provided by Bristol Myers Squibb.

Real-World Healthcare Resource Utilization Patterns Among Patients With Non-Small Cell Lung Cancer Treated With Amivantamab Monotherapy

Objectives: Among patients with non-small cell lung cancer (NSCLC), ~19-24% have epidermal growth factor receptor (EGFR) mutations. Amivantamab received United States Food and Drug Administration approvals in advanced NSCLC on 05/21/2021 for patients with EGFR exon 20 insertions (Exon20Ins) who progressed after platinum-based chemotherapy (PBC), on 03/01/2024 for first-line (1L) EGFR Exon20Ins, and for 1L (08/20/2024) and second-line (2L) (09/19/2024) EGFR exon 19 deletion and L858R. This study describes real-world healthcare resource utilization (HRU) among patients with advanced NSCLC receiving amivantamab monotherapy.

Conclusions: Among real-world patients with advanced NSCLC receiving amivantamab monotherapy following PBC, HRU was similar before and during treatment with amivantamab, suggesting that amivantamab does not contribute to an increase in medical services compared to treatment regimens used in earlier lines of therapy.

Authors: Managing Principal Patrick Lefebvre, Vice President Bruno Émond, Manager Laura Morrison, Senior Research Professional Lilian Diaz, Research Professional Yuxi Wang, and researchers from Oncology Hematology Care and Johnson & Johnson Innovative Medicine

Funding for this study was provided by Johnson & Johnson Innovative Medicine.

Healthcare Costs Associated With Adverse Events During Treatment Episodes for Pediatric Attention-Deficit/Hyperactivity Disorder

Objectives: Although adverse events (AEs) are common in pediatric patients treated for attention-deficit/hyperactivity disorder (ADHD), there is limited real-world evidence on their costs from a payer's perspective. Therefore, this study assessed the healthcare costs associated with selected AEs among pediatric patients treated for ADHD in the United States (US).

Conclusions: AEs were common during ADHD treatment episodes in pediatric patients and were associated with significant healthcare costs. Findings highlight the importance of ADHD treatments with a favorable safety profile to help reduce the considerable burden of AEs experienced by patients and corresponding healthcare costs.

Authors: Vice Presidents Martin Cloutier and Marjolaine Gauthier-Loiselle, Manager Maryaline Catillon, Associates Frédéric Kinkead and Anaïs Lemyre , Senior Analyst Alice Qu, and researchers from Otsuka Pharmaceutical Development & Commercialization and the Center for Psychiatry and Behavioral Medicine

Funding for this study was provided by Otsuka Pharmaceutical Development & Commercialization.

Direct Costs of Pneumococcal Disease in the United States - A Targeted Literature Review

Objectives: Understanding the costs associated with pneumococcal disease (PD) is essential for assessing its economic burden and the cost effectiveness of pneumococcal vaccines. This study aims to summarize the direct medical costs of PD in the US.

Conclusions: PD imposes substantial economic burdens on the healthcare system, particularly IPD and IP. Adults appear to incur higher costs per pneumonia episode than children. The considerable variability in estimated costs across studies highlights the importance of considering the methodologies of source studies, including health state definitions and model perspectives, to ensure alignment with those applied in economic evaluations.

Authors: Vice President Yan Song, Manager Hela Romdhani, and researchers from Merck, XL Source, the Emory University School of Medicine, and the Duke University School of Medicine

Funding for this study was provided by Merck.

Treatment Patterns and Healthcare Resource Use (HRU) in Patients With Metastatic Melanoma (mMEL) Treated With BRAF-MEK Inhibitor Targeted Therapies (TTs)

Objectives: To describe treatment patterns and HRU in patients with mMEL receiving encorafenib+binimetinib (E+B) or dabrafenib+trametinib (D+T).

Conclusions: E+B was associated with lower treatment discontinuation and lower IP admission rates in both 1L and 2L, but higher OP visit rates in 2L. Future research should explore reasons for these differences to understand their clinical and economic implications.

Authors: Managing Principal James Signorovitch, Vice President Gautam Sajeev, Manager Mu Cheng, Senior Analysts Jessie Lan and Jingyi Chen, Research Professional Joanne Chukwueke, and researchers from the Keck School of Medicine of USC and Pfizer

Funding for this study was provided by Pfizer.

Economic and Clinical Burden of Recurrence among Elderly Patients with Resected Locoregionally Advanced Head and Neck Squamous Cell Carcinoma (LA HNSCC) in the US

Objectives: Real-world evidence on the economic and clinical burden of recurrence following primary surgery in LA HNSCC is limited. This study evaluated the impact of recurrence on overall survival (OS), healthcare resource utilization (HRU), and healthcare costs among elderly patients treated in the US.

Conclusions: Recurrence following primary surgery was associated with significantly worse OS and increased HRU and healthcare costs among elderly patients with surgically treated LA HNSCC. The findings highlight a patient population where more effective therapies are needed.

Authors: Managing Principal James Signorovitch, Vice President Yan Song, Manager Su Zhang, Associate Jiayang Li, Analyst Anyu Zhu, and researchers from Merck and the Dana-Farber Cancer Institute

Funding for this study was provided by Merck.

POSTER SESSION 3

Thursday, May 15 | 10:30 a.m.–1:30 p.m.

Medical Costs in Patients with Pulmonary Arterial Hypertension (PAH) on Oral Triple Therapy including Selexipag in the United States (US): A retrospective Claims-Based Study

Identified as a Top 5% Finalist for the ISPOR 2025 Research Presentation Awards

Objectives: The 2022 ESC/ERS guidelines for the treatment of pulmonary arterial hypertension (PAH) recommend treating patients to achieve a low-risk status. If low-risk status is not achieved by dual combination therapy, selexipag is recommended as add-on treatment to reduce morbidity and mortality. Studies have examined the real-world effectiveness of selexipag on clinical outcomes, but very few have examined costs. Therefore, this study assessed medical costs among PAH patients on triple therapy with macitentan+phosphodiesterase type 5 inhibitors (PDE5i)+selexipag in the United States (US).

Conclusions: In this retrospective claims-based analysis, our findings suggest that patients with PAH on this triple oral therapy incur high medical costs. However, they remain medically complex patients as their all-cause costs were nearly three times higher than their PAH-related costs.

Authors: Vice President Marjolaine Gauthier-Loiselle, Manager Ambika Satija, Associate Louise Yu, and researchers from Johnson & Johnson Innovative Medicine

Funding for this study was provided by Johnson & Johnson Innovative Medicine.

Impact of Post-Discharge Rifaximin Use Following an Overt Hepatic Encephalopathy (OHE) Hospitalization on Annual Rates of OHE Rehospitalization in the United States

Objectives: Practice guidelines support rifaximin as an add-on to lactulose following breakthrough OHE for improved quality of care (QoC). We assessed the impact of post-OHE-hospitalization-discharge use of rifaximin on OHE rehospitalization rates, accounting for pre-index OHE medication use.

Conclusions: Rifaximin use post-OHE-hospitalization-discharge was associated with a significant reduction in OHE rehospitalization rates, regardless of pre-index OHE medication use. These findings highlight the importance of high-quality post-discharge care with rifaximin irrespective of patient treatment history.

Authors: Managing Principal Annie Guérin, Vice President Patrick S. Gagnon, Associates Rebecca Bungay and Kaitlyn Easson, Senior Research Professional Kana Yokoji, and researchers from Weill Cornell Medicine and Bausch Health

Funding for this study was provided by Bausch Health.

Unmet Needs and Evidence Gaps Among Patients with DLBCL treated with CAR-T Therapy: A Systematic Literature Review

Objectives: This study aimed to review current literature on the real-world (RW) efficacy, safety, healthcare resource use (HRU), and costs of innovative treatments for diffuse large B-cell lymphoma (DLBCL).

Conclusions: In this SLR, formal comparisons across CAR-T therapies and in different settings (i.e., inpatient vs. outpatient) in DLBCL are scarce. More research is needed to identify the most appropriate bridging therapy and treatments post-CAR-T failure to optimize outcomes for patients with different clinical profiles. Additional RW evidence is also needed to better understand how CAR-T administration can be further optimized to improve patient outcomes.

Authors: Vice President Bruno Émond, Associate Priyanka Gogna, and researchers from the Medical College of Wisconsin and Johnson & Johnson Innovative Medicine

Funding for this study was provided by Johnson & Johnson Innovative Medicine.

Bridging the Gap between the NAPOLI 3 Trial and Real-World Practice: Real-World Overall Survival (OS) of First-Line (1L) FOLFIRINOX in Metastatic Pancreatic Ductal Adenocarcinoma (mPDAC)

Objectives: NALIRIFOX (liposomal irinotecan + 5-fluorouracil/leucovorin + oxaliplatin) showed significantly improved OS compared to 1L gemcitabine + nab-paclitaxel in the phase 3 NAPOLI 3 trial (NCT04083235). The median OS (95% CI) for NALIRIFOX was 11.1 (10.0, 12.1) months. However, its efficacy relative to FFX (irinotecan + 5-fluorouracil/leucovorin + oxaliplatin), another standard-of-care regimen for 1L mPDAC, has not been evaluated. To contextualize findings from NAPOLI 3 trial, this study examined OS among patients treated with 1L FFX in the real-world setting.

Conclusions: NALIRIFOX in the NAPOLI 3 trial had numerically higher OS compared to FFX, including mFFX, in real-world practice. Further analyses adjusting for baseline characteristics are warranted to evaluate the relative efficacy of these regimens.

Authors: Chief Epidemiologist and Managing Principal Mei Sheng Duh, Vice President Rose Chang, Associate Louise Yu, Senior Analyst Chunyi Xu, and researchers from Ipsen and the University of Florida Health Cancer Center

Funding for this study was provided by Ipsen.

Economic Burden of Erythropoietic Protoporphyria (EPP) and X-linked Protoporphyria (XLP): A Large United States (US) Nationwide Claims Analysis

Objectives: EPP and XLP are rare genetic disorders that cause severe pain and phototoxicity upon exposure to sunlight. This retrospective study evaluated real-world healthcare utilization (HRU) and costs among patients with EPP (inclusive of XLP) vs. controls in the US.

Conclusions: EPP imposes a substantial economic burden, underscoring significant unmet needs in this population.

Authors: Chief Epidemiologist and Managing Principal Mei Sheng Duh, Managing Principal Maral DerSarkissian, Vice President Priyanka Bobbili, Manager Hela Romdhani, Associate Aruna Muthukumar, Analyst Becca Liu, Research Professional Ly Trinh, and researchers from Disc Medicine

Funding for this study was provided by Disc Medicine.

Economic Impact of Recurrence in High-Risk Early-Stage Estrogen-Receptor Positive (ER+)/Human Epidermal Growth Factor Receptor 2-Negative (HER2-) Breast Cancer (BC)

Objectives: Patients with high-risk early-stage ER+/HER2– BC face a three-fold higher recurrence risk than those without high-risk features. Real-world evidence on the economic burden of recurrence in this population is scarce, limiting comprehensive assessment of unmet need. This study quantified the incremental healthcare resource utilization (HRU) and associated costs of recurrence in high-risk early-stage ER+/HER2– BC.

Conclusions: Recurrence in high-risk early-stage ER+/HER2– BC imposes a substantial economic burden. Therapeutic advancements that reduce recurrence risk are essential to addressing unmet need and mitigating financial burden on patients and the healthcare system.

Authors: Vice Presidents Kalé Kponee-Shovein and Yan Song; Manager Malena Mahendran; Senior Analyst Qi Hua; Senior Research Professional Annalise Hilts; and researchers from Merck and the University of California, San Francisco

Funding for this study was provided by Merck.

Real-World Disease Management Costs for U.S. Patients With Muscle Invasive Bladder Cancer (MIBC) Following Radical Cystectomy (RC) in Contemporary Practice

Objectives: Disease management costs (i.e., non-drug costs) constitute a significant portion of overall cost burden for MIBC patients. However, the pattern of these costs is not well understood. This study aims to assess disease management costs by disease state post-RC: disease free (DF), locoregional recurrence (LR), distant metastasis (DM), and death.

Conclusions: For MIBC patients post-RC, disease management costs decrease as patients remain longer in the DF state. Recurrence, particularly metastatic recurrence, is associated with increased disease management costs. These results highlight the potential economic benefit of novel therapies which can maintain patients in the DF state or prevent recurrence in MIBC patients following RC.

Authors: Vice President Yan Song, Manager Erin Cook, Senior Analyst Shravanthi Seshasayee, and researchers from Merck and the Hospitals of the University of Pennsylvania-Penn Presbyterian

Funding for this study was provided by Merck.

Assessment of Real-World Adverse Events Associated with Ozanimod in Relapsing Remitting Multiple Sclerosis (RRMS)

Objectives: This study evaluated whether there was disproportionate reporting of adverse events (AEs) for ozanimod relative to other disease-modifying therapies (DMTs) indicated for RRMS.

Conclusions: Based on this descriptive analysis of the FAERS data, ozanimod has a lower proportion of AEs linked to serious outcomes than the other DMTs. Ozanimod generally had a larger share of the ten labeled AEs compared with the other DMTs. However, these labeled AEs made up a small percentage of all the AEs reported for ozanimod and the other DMTs.

Authors: Managing Principal Elyse Swallow, Vice President Oscar Patterson-Lomba, Associate Arshya Feizi, Senior Analyst Hana Akbarnejad, and researchers from Bristol Myers Squibb

Funding for this study was provided by Bristol Myers Squibb.

Real-World Hereditary Angioedema Attack Rates Before and After Berotralstat Initiation Among Adolescents

Objectives: This study evaluated hereditary angioedema (HAE) attacks before and after initiation of berotralstat among adolescent patients with HAE. Berotralstat, a once-daily, oral long-term prophylaxis for HAE, may be preferred by younger patients over parenteral treatment options. However, limited real-world evidence is available regarding its impact on HAE attack rates among adolescents.

Conclusions: Adolescents initiating berotralstat reported statistically significant and sustained reductions in HAE attack rates through 18 months of follow-up.

Authors: Principal François Laliberté, Manager Sean MacKnight, Senior Research Professional Cristina Martinez, and researchers from UCLA Health's Department of Pediatrics, ENT and Allergy Associates, BioCryst Pharmaceuticals, and Allergy and Asthma Research Associates

Funding for this study was provided by BioCryst Pharmaceuticals.

POSTER SESSION 4

Thursday, May 15 | 4:00–7:00 p.m.

Real-World Evidence of Setmelanotide in Patients with Rare Melanocortin-4 Receptor Pathway Diseases: Baseline Findings from the RESTORE Longitudinal Survey Study

Identified as a Top 5% Finalist for the ISPOR 2025 Research Presentation Awards

Objectives: Setmelanotide is approved for weight reduction long-term in patients aged ≥2 years with obesity due to certain rare melanocortin-4 receptor (MC4R) pathway diseases. RESTORE, a prospective, observational, longitudinal survey study, assesses setmelanotide's real-world effectiveness on hyperphagia, clinical burden, weight management, and quality of life. We report participants' characteristics at baseline before setmelanotide initiation.

Conclusions: RESTORE study baseline data highlight substantial unmet needs in hyperphagia and its impacts, weight management and clinical burden. Findings from follow-up surveys will longitudinally assess the effectiveness of setmelanotide in this patient population.

Authors: Managing Principal Min Yang, Manager Su Zhang, Senior Research Professional Jingyi Liu, and researchers from Rhythm Pharmaceuticals, Children's Mercy, the University of Alberta, and Marshfield Clinic Research Institute

Funding for this study was provided by Rhythm Pharmaceuticals.

Synthetic Patient Trajectories Using Generative AI on Electronic Health Records

Objectives: To provide a comprehensive understanding of obesity patients' long-term disease progression and impact of weight loss interventions using a generative AI (GenAI) disease model.

Conclusions: A robust GenAI-based obesity disease model was developed using EHR data based on a large US cohort of obese patients. The model was validated to accurately represent patient journeys and distributions of multiple clinical outcomes simultaneously and has been successfully used to predict the long-term cardiovascular benefits of weight loss.

Authors: Managing Principal Eric Q. Wu, Principal Jimmy Royer, Vice President Jinlin Song, Director of Data Science Max Leroux, Senior Data Scientist Intekhab Hossain, and researchers from McGill University

Economic Burden of Opioid-Induced Constipation Among Patients With or Without Cancer in the United States

Objectives: To compare healthcare costs between commercially insured continuous opioid users with or without opioid-induced constipation (OIC) in the United States (US), separately among patients with or without cancer.

Conclusions: In this real-world analysis of continuous opioid users in the US, OIC was associated with a significant economic burden in the management of both cancer-related and non-cancer related pain. Targeted treatment for OIC among long-term opioids users could help alleviate this burden.

Authors: Managing Principal Annie Guérin, Vice President Patrick S. Gagnon, Associate Rebecca Bungay, Senior Research Professional Remi Bellefleur, Research Professional Nathan Gobeil, and researchers from Salix Pharmaceuticals and Bausch Health

Funding for this study was provided by Bausch Health.

Targeted Literature Review and Qualitative Synthesis of the Burden of Oral Corticosteroid-Related Adverse Events in Autoimmune Conditions

Objectives: To understand the types and rates of adverse events (AEs) associated with long-term OCS use in autoimmune conditions.

Conclusions: OCS use is linked to a range of AEs including severe and life-threatening conditions, with substantial clinical and economic burden. Further research is needed to quantify the cost and quality-of-life impact of long-term OCS-related AEs among patients with autoimmune conditions.

Authors: Managing Principal Noam Kirson, Manager David Proudman, Associate Arshya Feizi, Senior Analysts Sydney Ng and Adrienne Kwok, and researchers from Massachusetts General Hospital and Argenx

Funding for this study was provided by Argenx.

Real-World Hereditary Angioedema Attack Rates Before and After Berotralstat Initiation Among Patients With C1 Inhibitor Deficiency (Type I/II) and ≥8 Attacks Per Month

Objectives: This study evaluated hereditary angioedema (HAE) attacks before and after initiation of berotralstat, an oral long-term prophylaxis, among patients with C1-inhibitor deficiency (Type I/II; HAE-C1-INH) and ≥8 attacks per month. Frequent HAE attack rates before berotralstat initiation likely reflect a patient population with more severe underlying disease activity.

Conclusions: Patients with ≥8 monthly baseline attacks and C1-inhibitor deficiency reported significantly lower HAE attack rates after berotralstat initiation. Treatment effectiveness was consistent and sustained through 18 months of follow-up.

Authors: Principal François Laliberté, Manager Sean MacKnight, Associate Ramya Ramasubramanian, and researchers from UCLA Health's Department of Pediatrics, ENT and Allergy Associates, BioCryst Pharmaceuticals, and Allergy and Asthma Research Associates

Funding for this study was provided by BioCryst Pharmaceuticals.

Impact of Increased Patients' Out-of-Pocket Costs on Oral Anticoagulant Discontinuation among Medicare Beneficiaries with Atrial Fibrillation Treated with Apixaban

Objectives: To describe patients' out-of-pocket costs (OOPC) for apixaban before and after a formulary tier increase and association between increased OOPC and treatment discontinuation among Medicare beneficiaries with atrial fibrillation (AF).

Conclusions: Following formulary tier increase among Medicare beneficiaries with AF, OOPC for 30-day apixaban supply rose by $61 on average, and almost one-third subsequently discontinued treatment. Patients with increased OOPC were associated with a higher probability of OAC discontinuation.

Authors: Vice President Dominick Latrémouille-Viau, Manager Aolin Wang, Senior Analysts Abigail Zion and Grace Chen, and researchers from Bristol Myers Squibb and Pfizer

Funding for this study was provided by Bristol Myers Squibb and Pfizer.

Real-World Adherence, Healthcare Resource Utilization, And Costs Among Patients With Schizophrenia Utilizing Once-Monthly, Once-Every-Three-Months, And Once-Every-Six-Months Paliperidone Palmitate in the United States

Objectives: Once-every-6-months paliperidone palmitate (PP6M) is the longest dosing interval long-acting injectable (LAI) antipsychotic, followed by once-every-three-months paliperidone palmitate (PP3M). Patients are eligible for PP6M after 1 dose of PP3M or adequate treatment with once-monthly paliperidone palmitate (PP1M) per label. This study described outcomes among patients using PP6M, PP3M, and PP1M.

Conclusions: In this real-world descriptive study, patients with schizophrenia using PP3M or PP6M were more adherent, incurred lower medical costs, and a lower proportion had a schizophrenia-related inpatient admission relative to patients using PP1M, suggesting LAIs with longer dosing intervals may improve clinical outcomes and decrease medical costs.

Authors: Vice President Dominic Pilon, Managers Laura Morrison and Arthur Voegel, Senior Research Professionals Lilian Diaz and Kana Yokoji, and researchers from Johnson & Johnson Innovative Medicine

Funding for this study was provided by Johnson & Johnson Innovative Medicine.

Treatment Patterns, Clinical Outcomes, Healthcare Resource Utilization, and Healthcare Costs Among Patients With Metastatic Gastric/Gastroesophageal Junction Cancer in Medicare Beneficiaries

Objectives: This study describes real-world patient characteristics, treatment patterns, overall survival (OS), healthcare resource utilization (HCRU), and costs among Medicare beneficiaries with metastatic gastric and gastroesophageal junction cancer (G/GEJC) during the first-line (1L) therapy.

Conclusions: Our study describes recent treatment patterns in elderly patients and highlights the economic burden of the disease. Despite the approval and increased use of novel immunotherapies, patients with metastatic G/GEJC have poor prognosis, suggesting significant remaining unmet need.

Authors: Managing Principal Hongbo Yang, Manager Wei Song, Senior Analysts Adina Zhang and Grace Chen, and researchers from Amgen

Funding for this study was provided by Amgen.

Budget Impact Analysis of Venetoclax Combination Therapies for the Treatment of Newly Diagnosed Acute Myeloid Leukemia Patients Who Are Aged 75 Years or Older, or Who Have Comorbidities That Preclude Use of Intensive Induction Chemotherapy

Objectives: Venetoclax, combined with azacitidine or decitabine, received FDA approval for treating newly diagnosed (ND) acute myeloid leukemia (AML) patients who are aged 75 years or older or have comorbidities precluding use of intensive chemotherapy. This study evaluated the budget impact of adopting venetoclax combinations for this population from a US third-party payer perspective.

Conclusions: Use of venetoclax combinations for the treatment of FDA-approved indication of ND AML reduced the budget impact and provides potential financial benefit for US payers.

Authors: Vice President Xinglei Chai, Senior Analyst Xin Chen, and researchers from AbbVie, Genentech, and the Duke Cancer Institute

Funding for this study was provided by AbbVie.

Healthcare Cost Comparison Between 1L Ibrutinib and Acalabrutinib in Chronic Lymphocytic Leukemia Patients in the Veterans Affairs Population

Objectives: Ibrutinib and acalabrutinib are Bruton's tyrosine kinase inhibitors (BTKis) recommended as first-line treatments for chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL). This study aimed to compare total cost of care during the initial 12 months of BTKi treatment in CLL/SLL patients receiving 1L monotherapy ibrutinib or acalabrutinib.

Conclusions: To our knowledge, this is the first study to evaluate costs of 1L BTKis among CLL/SLL patients in the VA. Costs during the first year of treatment were similar between patients initiating BTKis, with ibrutinib showing numerically lower total healthcare costs. While costs in the VA may be different than those outside the VA, these results can serve as a benchmark for future research on cost of care with BTKis following implementation of inflation reduction act policies.

Authors: Vice President Fan Mu, Manager Angela Lax, and researchers from Huntsman Cancer Institute, the University of Utah School of Medicine, Johnson & Johnson Innovative Medicine, Tulane University School of Public Health and Tropical Medicine, and the University of Washington School of Medicine

Funding for this study was provided by Johnson & Johnson Innovative Medicine.

POSTER SESSION 5

Friday, May 16 | 9:00–11:30 a.m.

Evaluation of the Impact of Fezolinetant vs Placebo on Indirect Costs Among Women With VMS Associated With Menopause

Objectives: Menopause-associated vasomotor symptoms (VMS; hot flashes and night sweats) are common and bothersome, affecting up to 80% of US women. VMS contribute to sleep disturbances, anxiety, mood changes, fatigue, and cognitive impairment, impacting quality of life and work productivity. Fezolinetant is an oral, nonhormonal, neurokinin 3 receptor antagonist treatment option for moderate to severe VMS due to menopause and is approved in the US, Europe, and Australia at a dose of 45 mg once daily. We evaluated the impact of fezolinetant versus placebo on work productivity and indirect costs among women with moderate to severe VMS.

Conclusions: Fezolinetant, which alleviates VMS frequency and severity, offers meaningful work productivity benefits and reduced indirect costs for women with moderate to severe VMS, underscoring a potential economic value in managing menopause-related symptoms.

Authors: Managing Principal Hongbo Yang, Manager Wei Song, Associate Yechu Hua, Senior Analyst Qi Hua, and researchers from Astellas Pharma

Funding for this study was provided by Astellas Pharma.