ANALYSIS GROUP

Raising the Standard in HEOR

# Analysis Group Posters and Presentations

ISPOR 2022 | MAY 15–18 | NATIONAL HARBOR, MD

## Analysis Group health economics and outcomes 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, a podium presentation, and 15 research posters. We have included details on all of these below.

# ISPOR 2022 Analysis Group Educational Symposium and Podium Presentations

## EDUCATIONAL SYMPOSIUM

**Monday, May 16, 2022 | 11:45 a.m.–12:45 p.m. | Maryland Ballroom C**

### Advances in the Development and Application of Real-World Evidence: Lessons from the US and China

Major strides have been made to improve the availability and applicability of real-world data (RWD). However, generating reliable and timely real-world evidence (RWE) is a multifaceted process facing many challenges globally. This symposium will introduce several creative approaches to generating high-value RWD, showcase patient-centric data innovations, and discuss the application of RWE to support regulatory submissions and payer negotiations.

First, to augment patient-centric care, an increasing number of patient support programs (PSPs) have been implemented by pharmaceutical companies. The presenters will demonstrate how to generate high-quality RWE that leverages PSPs. Both early evidence and long-term RWE on patients' quality-of-life and clinical benefits post-product launch can be reliably developed through this approach to demonstrate the real-world value of innovative treatments.

Second, two case studies will be presented to illustrate how to generate high-quality RWE in China, where RWE research has traditionally faced many challenges. In one example, researchers developed and validated algorithms to generate high-quality RWD in order to study complex patient journeys for a hematologic condition based on China's National Longitudinal Cohort of Hematological Diseases (NICHE). In another example, researchers applied a creative design of a multi-center physician survey to generate reliable RWE to support China's National Reimbursement Drug List negotiation.

Lastly, the presenters will discuss considerations in the creation of a historical control arm to support regulatory approval. We hope this symposium can introduce these recent creative solutions for generating and applying RWD to the audience and stimulate discussions to further advance RWE research and methodologies.

**Moderator:**
Eric Q. Wu, Ph.D.; Managing Principal, Analysis Group

**Panelists:**
Lee-Jen Wei, Ph.D.; *Harvard T.H. Chan School of Public Health*
Min Yang, M.D., Ph.D.; *Vice President, Analysis Group*
Jia Zhong, Sc.D.; *Manager, Analysis Group*

## PODIUM PRESENTATION

On Demand

**APPLYING DISPARATE DATA SOURCES AND METHODS FOR EVALUATING REAL-WORLD EVIDENCE**

# Reasons for Treatment Changes in Children and Adolescents with Attention-Deficit/Hyperactivity Disorder (ADHD): A Chart Review Study

**Objective:** To examine the reasons underlying treatment changes among pediatric patients with ADHD.

**Methods:** Data were obtained through online medical chart abstraction (August–September, 2021). Eligible patients with ADHD had initiated a treatment regimen at ages 6–17 years and within 1–5 years of chart abstraction. Reasons contributing to treatment discontinuation were analyzed for a randomly selected treatment episode. The ADHD/treatment-related complication rate was also described. Physicians' perspectives on adherence to ADHD treatment were assessed through an online survey. Results were reported overall, and also reported separately for children (ages 6–12 years) and adolescents (ages 13–17 years).

**Results:** A total of 156 physicians abstracted 434 patient charts (235 children; 199 adolescents). Mean patient age was 11.3 years, and 68.7% of patients were male. Treatment regimens analyzed included stimulants (83.2%), nonstimulants (11.3%), and combination therapy (5.1%); average treatment duration was 23.3 months. Among patients who discontinued treatment (N=83), inadequate/suboptimal symptom management (60.2%) was the most common reason for discontinuing treatment, while 25.3% reported a treatment discontinuation due to ADHD/treatment-related complications. The most common ADHD/treatment-related complications leading to treatment discontinuation were anxiety (19.0%), insomnia/sleep disturbances (19.0%), and emotional impulsivity (19.0%). Overall, 42.4% of patients had ≥1 documented ADHD/treatment-related complication, and this proportion reached 54.5% among patients receiving combination therapy. Insomnia/sleep disturbance was the most common ADHD/treatment-related complication and occurred in 9.7% of patients. Notably, 75.5% of patients reported that the experience or fear of complications had a negative impact on their adherence to ADHD treatment. Physicians reported taking actions toward patients' non-adherence by further educating patients (81.0%), providing closer monitoring (59.9%), and changing the prescribed ADHD medication (38.1%). Results were similar among children and adolescents.

**Conclusion:** Lack of effectiveness and ADHD/treatment-related complications are important reasons for treatment changes among children and adolescents with ADHD, highlighting the need for more effective and tolerable treatments to mitigate the burden of ADHD.

# ISPOR 2022 Analysis Group Research Posters

## POSTER SESSION I

**Monday, May 16, 2022 | 9:45 a.m.–1:15 p.m. | Presentation Time: 12:15–1:15 p.m.**

### Development of an Administrative Claims and Electronic Medical Record-Based Algorithm to Classify Systemic Disease Severity Among Patients with Sjögren's Syndrome in an Integrated Delivery Network in the United States

**Objective:** Sjögren's syndrome (SjS) systematic disease activity cannot be ascertained in administrative claims and/or electronic medical records (EMR). Prediction algorithms to classify patients with moderate-to-severe systemic activity using claims/EMR were evaluated.

**Conclusion:** Overall, these prediction models, particularly including EMR data, were successful in ruling out patients with moderate-to-severe systemic disease activity. Given the heterogeneity of SjS, low sample size and sensitivity, and increased likelihood of model overfitting, random forests will be implemented to rank-order importance of predictors and develop a more parsimonious model.

### Economic Burden of Gastroesophageal Reflux Disease, Barrett's Esophagus, and Esophageal Neoplasia in the United States

**Objective:** Gastroesophageal reflux disease (GERD) is a risk factor for Barrett's esophagus (BE) and esophageal neoplasia (EN). The objective was to evaluate healthcare resource utilization (HRU) and costs associated with GERD, BE, and EN in the US.

**Conclusion:** Patients with GERD, BE, and EN had important HRU and costs, including inpatient admissions and office visits. As patients progressed from GERD to BE to EN, there were substantially higher disease-related resource utilization rates and associated costs.

### Economic Impact of Trilaciclib for Chemotherapy-Induced Myelosuppression (CIM) in Extensive-Stage Small Cell Lung Cancer (ES-SCLC): Economic Evaluation from the Provider and Patient-Caregiver Perspectives in the United States

**Objective:** Commonly manifested as neutropenia, anemia, and thrombocytopenia, CIM is a major complication of systemic chemotherapy. We evaluated the economic impact of trilaciclib, a first-in-class breakthrough therapy that protects multiple hematopoietic lineages simultaneously against CIM in adult patients with ES-SCLC receiving platinum/etoposide- or topotecan-containing chemotherapy from both US provider and patient-caregiver perspectives.

**Conclusion:** The findings suggest that the use of trilaciclib prior to chemotherapy in patients with ES-SCLC can be cost-beneficial owing to fewer myelosuppressive adverse events, less healthcare resource use, and lower costs from both US provider and patient-caregiver perspectives.

Monday, May 16, 2022 | 9:45 a.m.–1:15 p.m. | Presentation Time: 12:15–1:15 p.m.

## Indirect Treatment Comparison between Daprodustat and Roxadustat in Non-Dialysis Patients with Anemia Associated with Chronic Kidney Disease: An Analysis of Energy/Fatigue as Measured by the SF-36 Vitality Score

**Objective:** Prolyl hydroxylase inhibitors (PHIs) are oral agents that might increase availability of anemia treatment options for patients with chronic kidney disease (CKD). The 36-Item Short Form (SF-36) Vitality score is a patient-reported measure of energy/fatigue – common and important outcomes in patients with CKD. Direct comparative evidence on health-related quality of life from randomized controlled trials (RCTs) between PHIs has not been identified. We conducted an indirect treatment comparison (ITC) of changes in SF-36 Vitality score observed in placebo-controlled RCTs of daprodustat and roxadustat.

**Conclusion:** Our analyses suggest superiority of daprodustat compared with roxadustat for the SF-36 Vitality score. A potential limitation of this ITC is the difference in timepoints of SF-36 Vitality endpoints across studies; however, our analyses used pre-specified endpoints that considered dosing algorithms and expected hemoglobin rate of rise from PHIs.

## POSTER SESSION II

**Monday, May 16, 2022 | 3:00–6:30 p.m. | Presentation Time: 5:30–6:30 p.m.**

### Numbers Needed to Treat and Costs per Improved Outcome Among Treatments for Myasthenia Gravis

**Objective:** To assess the number needed to treat (NNT) and health care costs required to achieve improvements in symptoms and functional activities with targeted therapies for myasthenia gravis (MG).

**Conclusion:** NNT and cost per improved efficacy help inform comparative clinical efficacy and cost-effectiveness across MG treatments. Evidence indicates more favorable treatment benefit and economic value for EFG with fewer NNT and lower cost required to achieve improved outcomes compared to other treatments.

### Healthcare Resource Use Among Patients Treated with Lurasidone or Cariprazine for Depressive Episodes Associated with Bipolar I Disorder

**Objective:** Financial burden in the US. This retrospective chart review study compared healthcare resource utilization (HRU) among patients with depressive episodes associated with BPD-1 treated with lurasidone or cariprazine, two atypical antipsychotics approved for treatment of bipolar depression.

**Conclusion:** In this study, patients with depressive episodes associated with BPD-1 treated with lurasidone or cariprazine had low rates of inpatient and emergency room visits; HRU for BPD-1 related hospitalizations and number of outpatient visits were lower for patients taking lurasidone compared to patients on cariprazine.

### Cost Effectiveness of Pembrolizumab Combined with Chemotherapy vs. Chemotherapy as First-Line Treatment for Metastatic TNBC That Expresses PD-L1 in the United States:

**Objective:** This analysis aimed to evaluate the cost effectiveness of pembrolizumab in combination with chemotherapy (paclitaxel, nab-paclitaxel, or gemcitabine+carboplatin) versus chemotherapy as first-line treatment in patients with locally recurrent unresectable or metastatic triple-negative breast cancer (mTNBC) whose tumors express programmed death ligand-1 (PD-L1) (combined positive score (CPS) ≥10), from a US third-party public healthcare payer perspective.

**Conclusion:** Pembrolizumab combined with chemotherapy is projected to be a cost-effective option compared with chemotherapy alone as first-line treatment in mTNBC with PD-L1 CPS≥10 from a third-party US public payer perspective, based on the World Health Organization willingness-to-pay threshold of three times GDP per capita.

## POSTER SESSION III

**Tuesday, May 17, 2022 | 9:45 a.m. –1:45 p.m. | Presentation Time: 12:15–1:15 p.m.**

### Real-World Dosing Patterns of Eculizumab-Naive and Eculizumab-Experienced Patients with Paroxysmal Nocturnal Hemoglobinuria Receiving Ravulizumab in the US

**Objective:** Treatment of paroxysmal nocturnal hemoglobinuria (PNH) includes C5 complement inhibitors ravulizumab and eculizumab. Ravulizumab dosing varies by weight and includes a loading dose, followed by maintenance doses given every eight weeks via intravenous infusion. This study investigated real-world dosing patterns of eculizumab-naïve and eculizumab-experienced patients with PNH receiving ravulizumab.

**Conclusion:** Deviations from label-recommended ravulizumab dosages – notably, high loading dose irrespective of weight – may result from inadequate control of PNH in some patients.

## POSTER SESSION IV

**Tuesday, May 17, 2022 | 3:00–6:30 p.m. | Presentation Time: 5:30–6:30 p.m.**

### Screening and Surveillance Upper Endoscopy Utilization Patterns Among Patients with Gastroesophageal Reflux Disease, Barrett's Esophagus, and Esophageal Neoplasia in the United States

**Objective:** To assess screening and surveillance upper endoscopy (flexible transnasal/transoral esophagoscopy or esophagogastroduodenoscopy [EGD]) utilization patterns among patients with gastroesophageal reflux disease (GERD), Barrett's esophagus (BE), and esophageal neoplasia (EN) in the US.

**Conclusion:** One-fifth of the patients with a diagnosis of GERD underwent upper endoscopy. The number of upper endoscopies per patient increased as the stage of disease progressed from NDBE to BE to EAC, but was less than what would be expected from current clinical practice guideline recommendations.

## POSTER SESSION V

**Wednesday, May 18, 2022 | 9:00 a.m.–12:45 p.m. | Presentation Time: 9:00–10:00 a.m.**

### Patient Journey and Clinical Burden of Rett Syndrome in the United States

**Objective:** To describe the patient journey and clinical burden of Rett syndrome (RTT) among female patients in the United States.

**Conclusion:** Patients with RTT have substantial concomitant disease burden across their lifespan, as evidenced by a high prevalence of clinical manifestations and reliance on pharmacological and supportive therapy. These findings suggest an unmet need for effective therapies to treat RTT, with the potential to reduce overall burden and facilitate long-term clinical benefits.

## ON-DEMAND AND VIRTUAL POSTER PRESENTATIONS

### Comparative Efficacy and Safety of Ozanimod and Ponesimod for Relapsing Multiple Sclerosis: A Matching-Adjusted Indirect Comparison

*Identified as a semifinalist for a Research Presentation Award (Top 5%)*

**Objective:** Ozanimod and ponesimod are both sphingosine 1-phosphate receptor modulators approved by the US Food and Drug Administration (FDA) for treatment of relapsing forms of multiple sclerosis (MS). Without head-to-head trials between these two treatments, we performed a matching-adjusted indirect comparison (MAIC) of efficacy and safety outcomes between ozanimod and ponesimod for MS.

**Conclusion:** The MAIC showed that ozanimod is more efficacious in preserving brain volume compared with ponesimod but comparable in absolute risk reduction and has a favorable safety profile.

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### A Matching-Adjusted Indirect Comparison of Filgotinib Versus Tofacitinib for Moderately to Severely Active Ulcerative Colitis

**Objective:** In the absence of head-to-head trials, matching-adjusted indirect comparisons (MAICs) were conducted to compare efficacy and safety of filgotinib versus tofacitinib in patients with moderately to severely ulcerative colitis (UC) after adjusting for cross-trial differences.

**Conclusion:** Filgotinib showed similar efficacy and health-related quality of life compared with tofacitinib among patients with moderately to severely active UC, regardless of history of biologic treatment. Safety results were not conclusive due to the differences in placebo arms.

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### A USRDS Retrospective Cohort Study: Epidemiology, Treatment Modalities, and Burden of End-Stage Kidney Disease Attributed to Focal Segmental Glomerulosclerosis (FSGS)

**Objective:** FSGS is a histologic pattern of glomerular damage that results in proteinuria and may lead to end-stage kidney disease (ESKD). This study describes epidemiology, treatment modalities, resource burden, and mortality of ESKD attributed to FSGS in the United States.

**Conclusion:** Although rare, FSGS-attributed ESKD in the US is associated with a substantial burden to patients and the healthcare system. Safe, effective, and approved therapies for FSGS would significantly improve the lives of patients and substantially reduce the burden on the healthcare system.

## ON-DEMAND AND VIRTUAL POSTER PRESENTATIONS

### A USRDS Retrospective Cohort Study: Epidemiology, Treatment Modalities, and Burden of End-Stage Kidney Disease Attributed to Immunoglobulin A Nephropathy

**Objective:** Characterized by increased production of inflammatory cytokines that damage the glomerular filtration barrier resulting in proteinuria and hematuria, immunoglobulin A nephropathy (IgAN) leads to end-stage kidney disease (ESKD). This study describes epidemiology, treatment modalities, resource burden, and mortality of ESKD attributed to IgAN in the United States.

**Conclusion:** Although rare, IgAN-attributed ESKD in the US is associated with substantial burden to patients and the healthcare system. Safe, effective, and approved therapies for IgAN would significantly improve the lives of patients and substantially reduce the burden on the healthcare system.

### Burden of Invasive Extraintestinal Pathogenic E. coli Disease Among Older Adult Patients Treated in Hospitals in the US

**Objective:** To describe medical resource utilization and costs associated with invasive extraintestinal pathogenic E. coli disease (IED) in older adults in the US.

**Conclusion:** IED is associated with a substantial clinical and economic burden both during the initial encounter and over the following year, highlighting the need for and potential benefits of preventive management of IED.