



ANALYSIS GROUP

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Analysis Group Posters and Presentations

ISPOR 2023 | MAY 7-10 | BOSTON, MA

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

This year, we are pleased to present a Forum session, two podium presentations, and 16 research posters. Please find details below.

If time permits, please stop by and say hello to our team at Booth #1017

ISPOR 2023 Analysis Group Forum Session and Podium Presentations

FORUM

Monday, May 8 | 11:45 a.m.–12:45 p.m.

Generalized Cost Effectiveness Analysis – From Theory to Practice

Considerable attention has been given to the ISPOR “value flower” and potential ways to expand traditional cost-effectiveness analyses (CEAs). Most of these discussions have remained at the theoretical level, however. This forum will provide an overview of recent theoretical and practical extensions to traditional CEAs and their implications for value assessment and also discuss challenges and opportunities in the implementation of generalized cost-effectiveness analysis (GCEA) methods in practice, including the generalized risk-adjusted cost-effectiveness (GRACE) framework, real option value, life cycle pricing, and various societal dimensions of value.

Dr. Kirson will briefly survey the research landscape and moderate the discussion. Dr. Garrison will discuss the degree to which recent efforts provide tools to reliably implement elements of the “value flower,” key areas of progress, and remaining gaps. Ms. Synnott will speak about trends in the literature they are seeing through CEVR’s CEA Registry and HTA work. Dr. Campbell will address the topic of implementation of GCEA methods in formal HTAs, and the key challenges that arise in the policy context. Dr. Leibman will provide an industry perspective and discuss the impact of expanded value assessment on future innovation. All forum participants will discuss how future research efforts could further improve value assessment.

In addition, the relationship between GCEA, societal value, and use of value assessment as guidance for pricing will also be examined. Live polling and the Q&A session will be used to engage the audience.

Moderator: [Noam Kirson](#), Ph.D.; *Managing Principal, Analysis Group*

Panelists: [Lou Garrison](#), Ph.D.; *The Comparative Health Outcomes, Policy, and Economics (CHOICE) Institute, School of Pharmacy at the University of Washington*

Patricia Synnott, M.S.; Senior Manager, Cost-Effectiveness Analysis Registry and Global Health Initiatives, Center for the Evaluation of Value and Risk in Health (CEVR) at the Tufts Medical Center Institute for Clinical Research and Health Policy Studies

Jon Campbell, Ph.D.; Senior Vice President for Health Economics, Institute for Clinical and Economic Review (ICER)

Chris Leibman, Pharm.D.; Senior Vice President, Value, Access, Public Policy and Government Affairs, Biogen

PODIUM PRESENTATION

Monday, May 8 | 11:00–11:15 a.m.

Economic Evaluation of Exagamglogene Autotemcel (EXA-CEL) Gene-Edited Therapy in Patients with Transfusion-Dependent Beta-Thalassemia

Objectives: Exagamglogene autotemcel (exa-cel) is a one-time potentially curative gene-edited therapy being evaluated for patients with transfusion-dependent β -thalassemia (TDT). The standard of care (SOC) for these patients includes red blood cell transfusions and iron chelation therapies. In this study, we assessed the potential cost-effectiveness of exa-cel versus SOC in the United States for patients with TDT.

Methods: A Markov cohort model was developed to compare clinical and cost outcomes of exa-cel versus SOC over a lifetime horizon. The base-case modeled population had a mean age of 21.3 years with 17 transfusions/year. Modeled outcomes included proportion of patients developing chronic complications, number of transfusions, life years (LYs), quality-adjusted life years (QALYs), costs, and cost-effectiveness using a \$200,000/QALY willingness-to-pay (WTP) threshold (ultra-rare disease modifier). Clinical efficacy was informed from published exa-cel clinical trial data; SOC inputs were derived from published literature. Complication risks, utility, mortality, and cost inputs were also derived from published literature. Costs and outcomes were discounted 3% annually. Mean age and number of transfusions were varied, from both a payer and societal perspective, to provide a range of outcomes.

Results: Compared to SOC, exa-cel is projected to improve patient survival (undiscounted) by 16.1–18.7 years and lower the number of transfusions by 215–519 over a lifetime. Exa-cel results in improved discounted LYs (+5.4–5.6) and discounted QALYs (+8.5–8.8), as well as reduced undiscounted disease management costs (\$2,120,000–\$2,850,000) versus SOC. At a \$200,000/QALY WTP threshold, exa-cel would be cost-effective at prices ranging from \$2,820,000 to \$3,550,000.

Conclusions: Model projections suggest that exa-cel could considerably improve survival and quality-of-life and reduce disease management costs and the incidence of complications in patients with TDT compared to SOC.

Authors: Vice President [Hongbo Yang](#), Managers Honghao Fang and [Xiaoyu Nie](#), and researchers from Vertex Pharmaceuticals and Memorial Regional Hospital

PODIUM PRESENTATION

Tuesday, May 9 | 10:45–11:00 a.m.

Psychometric Evaluation of Two Novel Hyperphagia Questionnaires for Patients with Bardet-Biedl Syndrome (BBS)

Objectives: Rare genetic disorders of obesity including Bardet-Biedl Syndrome (BBS) are characterized by early onset obesity, hyperphagia, and multi-systemic complications. Symptoms of Hyperphagia (SoH) and Impacts of Hyperphagia (IoH) are novel questionnaires designed to assess severity of hyperphagia symptoms and their impact on patients and caregivers. As part of the CARE-BBS study, we evaluated the performance of these scales.

Methods: CARE-BBS was a multi-country cross-sectional survey study of adult caregivers of patients with BBS experiencing obesity and hyperphagia. In addition to the SOH and IOH questionnaires, other instruments included Impact of Weight on Quality of Life (IWQOL)-Kids Parent Proxy, PROMIS Scale Global Health of Caregiver, Revised Impact on Family Scale (RIOFS), and Work Productivity and Activity Impairment (WPAI): BBS-Caregiver.

Results: 242 eligible caregivers from Canada, Germany, UK, and US completed the survey. Exploratory factor analysis identified 1 factor each for SoH-Proxy, IoH-Proxy, and IoH-Caregiver (weighted eigenvalues: 0.83, 2.03, 2.79, respectively). Strong correlations were observed across SoH/IoH scales ($r=0.558-0.798$) (all $p<.001$). Strong internal consistency was observed for IoH-Proxy (Cronbach's α coefficient=0.66) and IoH-Caregiver ($\alpha=0.72$) and moderate for SoH-Proxy ($\alpha=0.40$). All three scales correlated moderately to strongly with school days missed and all domains of IWQOL-Kids except Physical Comfort (range=0.315-0.573, all $p<.001$). Both Impact scales also correlated moderately with both WPAI-Caregiver total productivity and total activity impairments (range=0.417-0.448, all $p<.001$). Known-groups analyses indicated significantly worse SoH-proxy scores for caregivers applying more weight management approaches (6-10 vs. 5, or >10). Worse IoH scores were found when caregivers reported greater strain on RIOFS items and worse mental health on PROMIS.

Conclusions: Caregiver versions of the SoH and IoH scales demonstrated validity, reliability, and consistency for use in a real-world setting. Additional studies to further validate psychometric properties of the scales, including the IOH and SOH patient versions across other health care settings, are recommended.

Authors: Vice President [Min Yang](#), Associate Ali Greatsinger, Senior Analyst Ella Hagopian, and researchers from Rhythm Pharmaceuticals, the Marshfield Clinic Research Institute, and the Division of Pediatric Endocrinology at the University of Alberta.

ISPOR 2023 Analysis Group Research Posters

POSTER SESSION 1

Monday, May 8 | 9:45 a.m.–1:15 p.m.

Characterization of Patients with Duchenne Muscular Dystrophy across Previously Developed Health States

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

Objectives: The natural history of disease progression in Duchenne muscular dystrophy (DMD) has been categorized into eight health states based on the input from clinicians, patients, and caregivers by Project HERCULES. The current study uses natural history data to further characterize patients with DMD by health state.

Conclusions: Health states proposed for DMD disease progression showed concordantly worsened function for later health states across different domains, including ambulatory, pulmonary, upper-limb, and cardiac function. These findings further characterize health states and their interpretation in economic modeling and decision-making.

Authors: Managing Principal [James Signorovitch](#), Vice President [Michaela Johnson](#), Associate Andres Gomez-Lievano, and researchers from University College London, University Hospitals Leuven, Pfizer, Chiesi, the Collaborative Trajectory Analysis Project (cTAP), the University of California, Sacramento, the University of Florida, and Fondazione Policlinico Universitario "Agostino Gemelli"

Real-World Treatment Response and Persistence Among Black and Non-Black Patients with Metastatic Castration-Sensitive Prostate Cancer Treated with Apalutamide in a Urology Setting

Objectives: In the United States, real-world evidence (RWE) on apalutamide (APA) for treatment of patients with metastatic castration-sensitive prostate cancer (mCSPC) is limited. This study describes prostate-specific antigen (PSA) response and treatment persistence among patients with mCSPC initiated on APA, with stratifications for Black/non-Black patients.

Conclusions: Patients with mCSPC initiated on APA exhibited high real-world PSA response rates, and few patients had gaps in therapy. Robust real-world PSA response rates and treatment persistence were also demonstrated for both Black and non-Black patients.

Authors: Managing Principal [Patrick Lefebvre](#), Vice President [Dominic Pilon](#), Manager [Carmine Rossi](#), Senior Research Professional Frederic Kinkead, Research Professional Bronwyn Moore, and researchers from Chesapeake Urology and Janssen

A Retrospective Cohort Study of Veterans Affairs Data: Epidemiology, Treatments, Clinical Outcomes, and Burden of Immunoglobulin A Nephropathy

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

Objectives: Immunoglobulin A nephropathy (IgAN), the glomerular accumulation of IgA-containing immune complexes in the kidneys, may lead to damage of the glomerular filtration barrier which results in proteinuria, hematuria, and often kidney failure (KF). This study describes the epidemiology, treatments, clinical outcomes, and resource burden of patients with IgAN using United States Veterans Affairs (VA) data.

Conclusions: Among the VA population, IgAN is associated with substantial clinical and resource burden. Safe and effective therapies approved for IgAN would significantly improve the lives of patients and reduce patient and healthcare system burden.

Authors: Vice Presidents [Debbie Goldschmidt](#) and [Jenny Zhou](#), HEOR Data Analyst Specialist Sherry Shi, and researchers from Travele Therapeutics and Tulane University

A Retrospective Cohort Study of Veterans Affairs Data: Epidemiology, Treatments, Clinical Outcomes, and Burden of Focal Segmental Glomerulosclerosis

Objectives: Focal segmental glomerulosclerosis (FSGS) is a histologic pattern of glomerular injury reported in patients who present with proteinuria, chronic kidney disease, and sometimes nephrotic syndrome, and may lead to kidney failure (KF). This study describes epidemiology, treatments, clinical outcomes, and burden of patients with FSGS using United States Veterans Affairs (VA) data.

Conclusions: FSGS is associated with substantial clinical and resource burden among patients in the VA. Safe and effective therapies approved for FSGS would significantly improve the lives of patients and reduce patient and healthcare system burden.

Authors: Vice Presidents [Debbie Goldschmidt](#) and [Jenny Zhou](#), HEOR Data Analyst Specialist Sherry Shi, and researchers from Travele Therapeutics and Tulane University

POSTER SESSION 2

Monday, May 8 | 3:15–6:45 p.m.

Economic Evaluation of Exagamglogene Autotemcel (EXA-CEL) Gene-Edited Therapy in Patients with Sickle Cell Disease with Recurrent Vaso-Occlusive Crises

Objectives: Exagamglogene autotemcel (exa-cel) is a one-time potentially curative gene-edited therapy being evaluated for patients with sickle cell disease (SCD) with recurrent vaso-occlusive crises (VOCs). The standard of care (SOC) for these patients includes hydroxyurea and red blood cell transfusions. In this study, we assessed the potential cost-effectiveness of exa-cel versus SOC in the United States (US) for patients with SCD with recurrent VOCs.

Conclusions: Model projections suggest that exa-cel could considerably improve survival and quality of life and reduce disease management costs and the incidence of VOCs and complications in patients with SCD with recurrent VOCs compared to SOC.

Authors: Vice President [Hongbo Yang](#), Associate Yanwen Xie, Senior Analyst Xin Chen, and researchers from Vertex Pharmaceuticals and Memorial Regional Hospital

Healthcare Resource Utilization and Costs Associated with Psychiatric Comorbidities in Adult Patients with Attention-Deficit/Hyperactivity Disorder

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

Objectives: To assess the impact of psychiatric comorbidities, specifically anxiety and depression, on healthcare resource utilization (HRU) and costs in adults with attention-deficit/hyperactivity disorder (ADHD) in the United States.

Conclusions: Coexisting anxiety and depression is associated with additional HRU and costs burden in patients with ADHD. Co-management of these conditions is important and has the potential to alleviate the burden experienced by patients and the healthcare system.

Authors: Managing Principal [Annie Guérin](#), Vice Presidents [Martin Cloutier](#) and [Marjolaine Gauthier-Loiselle](#), Associate Rebecca Bungay, Senior Research Professionals Kathleen Chen and Deborah Chan, and researchers from Otsuka and the Center for Psychiatry and Behavioral Medicine, Las Vegas

Risk Factors Associated with Newly Diagnosed Attention-Deficit/Hyperactivity Disorder in Adults

Objectives: To identify risk factors associated with newly diagnosed attention-deficit/hyperactivity disorder (ADHD) in adults in the United States (US).

Conclusions: Psychiatric comorbidities and associated treatments are key predictors of newly diagnosed ADHD in US adults. Screening for patients with risk factors for ADHD may allow early diagnosis and appropriate management.

Authors: Managing Principal [Annie Guérin](#), Vice Presidents [Martin Cloutier](#) and [Marjolaine Gauthier-Loiselle](#), Associate Rebecca Bungay, and researchers from Otsuka and the Center for Psychiatry and Behavioral Medicine, Las Vegas

POSTER SESSION 3

Tuesday, May 9 | 9:45 a.m.–1:15 p.m.

Cost Burden of Geographic Atrophy and Visual Impairment/Blindness in US Elderly Patients

Objectives: Geographic atrophy (GA) is an advanced form of age-related macular degeneration that can lead to irreversible visual impairment (VI) and blindness. This study quantified the cost burden of GA and GA progression to VI or blindness.

Conclusions: The cost burden of GA is substantial and increases significantly as GA patients further develop VI or blindness. Early treatment may mitigate healthcare costs related to disease progression.

Authors: Managing Principal [Mei Sheng Duh](#), Vice President [Wendy Cheng](#), Manager [Guillaume Germain](#), Associates Malena Mahendran and Jacob Klimek, and researchers from Apellis

Real-World Economic Burden Pre- and Post-Progression to Metastatic Castration-Resistant Prostate Cancer (mCRPC) and after First-Line mCRPC Therapy Initiation

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

Objectives: Patients with advanced prostate cancer (PC) may develop resistance to androgen deprivation therapy and progress to metastatic castration-resistant prostate cancer (mCRPC). Limited evidence exists characterizing real-world healthcare costs of patients with mCRPC. This study described healthcare costs of patients pre- and post-progression to mCRPC as well as after first-line (1L) mCRPC therapy initiation.

Conclusions: Prior to 1L mCRPC therapy initiation, PC-related total costs were more than 2 times higher post-relative to pre-progression to mCRPC. Relative to before 1L mCRPC therapy initiation, PC-related medical costs more than tripled, and PC-related total costs were more than 4 times higher during 1L mCRPC therapy. Clinical interventions aiming to delay costly progression in patients with advanced PC are warranted.

Authors: Managing Principal [Patrick Lefebvre](#), Vice President [Dominic Pilon](#), Associate Laura Morrison, Senior Research Professional Frederic Kinkead, and researchers from Janssen and Duke University Cancer Center

Real-world Effectiveness of Sotrovimab for the Early Treatment of COVID-19: Evidence from the National COVID Cohort Collaborative (N3C)

Objectives: To describe and compare real-world outcomes for patients with mild-to-moderate COVID-19 at high risk for progression to severe COVID-19, treated with sotrovimab versus untreated.

Conclusions: Sotrovimab demonstrated clinical effectiveness in preventing severe outcomes (hospitalization, mortality) between 26 May 2021 and 30 April 2022, which included the Delta variant and early surge of Omicron BA.1/BA.2.

Authors: Managing Principal [Mei Sheng Duh](#), Vice Presidents [Priyanka Bobbili](#) and [Maral DerSarkissian](#), Associate Raj Desai, and Senior Analyst Adina Zhang, and researchers from GSK

Real-World Treatment Outcomes Among Relapsed/Refractory Patients with Mantle Cell Lymphoma Treated with Ibrutinib or Acalabrutinib

Objectives: Evaluate treatment outcomes among patients with mantle cell lymphoma (MCL) treated with the Bruton's tyrosine kinase inhibitors (BTKis) ibrutinib or acalabrutinib in second- or later-line therapy (2L+).

Conclusions: Treatment persistence was similar in patients with MCL treated with ibrutinib or acalabrutinib in 2L+, with slightly longer median time to discontinuation observed among patients treated with ibrutinib.

Authors: Vice Presidents [Bruno Émond](#) and [Marie-Hélène Lafeuille](#) and researchers from Pharmacocyclics (an AbbVie company)

A Retrospective Analysis of Real-World Treatment Patterns in Patients Over Age 64 with Dry Eye Disease Receiving OTX-101 Ophthalmic Solution 0.09%, Cyclosporine Ophthalmic Emulsion 0.05%, or Lifitegrast Ophthalmic Solution 5%

Objectives: Dry eye disease (DED) causes chronic ocular surface inflammation and damage. DED prevalence increases with age. Cyclosporine ophthalmic solution 0.09% (CEQUA®; OTX-101), cyclosporine ophthalmic emulsion 0.05% (Restasis®; CsA), and lifitegrast ophthalmic solution 5% (Xiidra®; LFT) are anti-inflammatory agents indicated for DED treatment. We compared treatment patterns in patients >64 years with DED receiving OTX-101, CsA, or LFT.

Conclusions: OTX-101 and CsA patients >64 years were significantly more likely to discontinue treatment than those ≤64 years. Patients >64 years receiving OTX-101 remained on treatment significantly longer than those on CsA; time on treatment was similar for OTX-101 and LFT.

Authors: Managing Principal [Mihran Yenikomshian](#), Vice President [Lynn Huynh](#), Manager [Enrico Zanardo](#), Senior Analyst Anamika Khanal, and researchers from the Kentucky Eye Institute, VEB HealthCare, Sun Pharmaceutical Industries, and CM Associates

Temporal Trends in Adverse Event Costs with Nivolumab + Relatlimab Combination Therapy or Nivolumab Monotherapy for Patients with Unresectable or Metastatic Melanoma

Objectives: This study described hospitalization costs of grade 3/4 all-cause adverse events (AEs) and treatment-related AEs (TRAEs) of nivolumab plus relatlimab fixed-dose combination therapy (Nivo+Rela) and nivolumab monotherapy (Nivo) for unresectable or metastatic melanoma.

Conclusions: The AE cost trends over the study period were similar for the Nivo+Rela and Nivo arms, with slightly higher cost associated with Nivo+Rela. This analysis suggests minimal incremental economic burden associated with AE management for Nivo+Rela.

Authors: Vice Presidents [Viviana García-Horton](#) and [Jenny Zhou](#), Associate Yiqiao Xin, Analyst Dana Christensen, and researchers from Bristol Myers Squibb

POSTER SESSION 4

Tuesday, May 9 | 3:15–6:45 p.m

Characterizing Progressive Disease Burden amid Heterogeneity: Data Visualizations for Ambulatory Motor Function in Duchenne Muscular Dystrophy (DMD)

Objectives: DMD is an X-linked recessive disorder that causes progressive loss of muscle function in affected patients. Given variation in ages at symptom onset and rates of motor function decline, we aimed to develop data visualizations characterizing typical patterns of disease progression amid heterogeneity.

Conclusions: When patterns of disease progression are heterogeneous, as in DMD, the population average trajectory obscures the more rapid pace of decline experienced by the most representative individuals. As the pace of progression can have profound impacts on patients and caregivers, visualizations characterizing representative individual experiences amid population heterogeneity are important to understanding and communicating disease burden.

Authors: Managing Principal [James Signorovitch](#), Vice President [Michaela Johnson](#), Associate Molly Frean, and researchers from The John Walton Muscular Dystrophy Research Centre, the Collaborative Trajectory Analysis Project (cTAP), and the Dubowitz Neuromuscular Centre UCL, Great Ormond Street Institute of Child Health & Great Ormond Street Hospital

A Systematic Literature Review (SLR) of Economic Burden of Patients With Hormone Receptor-Positive/Human Epidermal Growth Factor Receptor 2-Negative (HR+/HER2-) Metastatic Breast Cancer (mBC) With at Least One Prior Therapy

Objectives: With the HR+/HER2- mBC treatment landscape evolving between endocrine therapy (ET), chemotherapy, and novel therapies, economic burden may impact treatment decisions, especially in later-line settings. This SLR summarizes the current evidence of economic evaluations, costs, and resource use among adults with HR+/HER2- mBC and ≥ 1 prior therapy in the metastatic setting.

Conclusions: Patients with HR+/HER2- mBC receiving chemotherapy versus other therapies incurred higher direct healthcare costs driven by IP costs and higher indirect costs driven by lower productivity. This SLR also highlights the lack of recent studies evaluating economic burden in later-line settings and studies examining indirect costs in the US.

Authors: Manager [David Proudman](#), Senior Analyst Travis Wang, and researchers from Gilead Sciences

POSTER SESSION 5

Wednesday, May 10 | 8:30–11:30 a.m.

A Real-World Assessment of Healthcare Costs Related to Agitation Associated with Alzheimer's Dementia

Objectives: To assess the incremental economic burden of agitation in patients with Alzheimer's dementia (AD) in the United States.

Conclusions: Among patients with AD, agitation was associated with increased healthcare costs, highlighting the additional burden that agitation poses. Strategies focusing on improving agitation management have the potential to mitigate the burden associated with AD for payers and the healthcare system.

Authors: Managing Principal [Annie Guérin](#), Vice Presidents [Martin Cloutier](#) and [Marjolaine Gauthier-Loiselle](#), Associate Rebecca Bungay, Senior Research Professional Deborah Chan, and researchers from SSM Health St. Mary's Hospital – Saint Louis, Lundbeck, and Otsuka