Raising the Standard in HEOR

Analysis Group Posters and Presentations

ISPOR 2024 | MAY 5-8 | ATLANTA, GA

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 and 26 research posters. Please find details below.

ISPOR 2024 Analysis Group Presentation

EDUCATIONAL SYMPOSIUM

Tuesday, May 7 | 11:45 a.m.–12:45 p.m. | Georgia World Congress Center, Room A311–312

Advancing HEOR and RWE Research with Generative AI (GenAI)

GenAl is revolutionizing HEOR and real-world evidence (RWE) by offering innovative methods to process and analyze vast datasets, model complex health economics phenomena, and enhance decision-making processes. GenAl 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, including electronic health records, claims data, and scientific literature. By harnessing the power of GenAl, 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 GenAl, discuss key developments and challenges, and present examples of GenAl's applications in HEOR and real-world research. Among these examples is Analysis Group's own proprietary GenAl platform, which excels in text classification, research summarization, and automated data analysis. The introduction of this platform and similar GenAl 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.

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

Panelists: Jimmy Royer, Ph.D.; Principal, Analysis Group

Rajeev Ayyagari, Ph.D.; Vice President, Analysis Group

Hua Xu, Ph.D.; Robert T. McCluskey Professor of Biomedical Informatics and Data Science; Vice Chair for Research and Development, Section of Biomedical Informatics and Data Science; Assistant

Dean for Biomedical Informatics, Yale School of Medicine

ISPOR 2024 Analysis Group Research Posters

POSTER SESSION 1

Monday, May 6 | 10:30 a.m.-1:30 p.m.

Cost Per Recurrence-Free Survivor (CPRS) and Cost Per Recurrence-Free Life Month (CPRLM) of Adjuvant Nivolumab (NIVO) Versus Pembrolizumab (PEMBRO) Among Patients with Resected Stage IIB/IIE Melanoma

Objectives: Both NIVO and PEMBRO have been approved by the US Food and Drug Administration (FDA) as adjuvant treatments for patients with resected stage IIB/IIC melanoma in the US. It is important to understand the cost per outcome of the two treatments to inform payers and decision makers. This study compared the CPRS and CPRLM of NIVO vs. PEMBRO from a US payer perspective.

Conclusions: Adjuvant NIVO had consistently lower incremental CPRS and CPRLM (relative to PBO) compared with adjuvant PEMBRO. This suggests the potential for long-term cost savings associated with the use of adjuvant NIVO vs. PEMBRO in resected stage IIB/IIC melanoma in the US.

Authors: Vice President <u>Keith Betts</u>, Manager <u>Ella X. Du</u>, Senior Analyst Churong Xu, and researchers from Atrium Health Levine Cancer and Bristol Myers Squibb

Funding for this study was provided by Bristol Myers Squibb.

Healthcare Costs Among Commercially-Insured Patients with Hereditary Angioedema Managed with Long-Term Prophylaxis: A Retrospective US Claims Database

Objectives: Management of hereditary angioedema (HAE) consists of on-demand treatment and, for appropriate patients, the addition of long-term prophylaxis (LTP). Given the increasing number of patients receiving non-androgen LTP and limited data on related real-world healthcare costs in the US, we estimated such costs based on a large retrospective insurance claims database.

Conclusions: While LTP therapies have been shown to reduce HAE attack rates, analyses revealed that HAE-related non-pharmacy resource utilization costs were substantial in the current study. Future insights related to HAE attack management and cost drivers are needed among LTP users.

Authors: Vice Presidents Maral DerSarkissian and Rose Chang, Senior Analysts Megan Pinaire and Manasi Mohan, and researchers from the David Geffen School of Medicine at UCLA, Asthma & Allergy Associates, and KalVista Pharmaceuticals

Funding for this study was provided by KalVista Pharmaceuticals.

The Impact and Burden of Visual Impairment in Patients with Dry Age-Related Macular Degeneration Relative to Older Adults Without Major Eye Conditions: A Qualitative Interview Study

Objectives: Dry age-related macular degeneration (AMD) leads to progressive vision loss. This qualitative study assessed functional impairment and humanistic burden associated with dry AMD vision loss, for patients relative to older adults without dry AMD (controls).

Conclusions: Patients with dry AMD had greater functional impairment due to vision loss, and worse QoL and emotional well-being, relative to similar aged adults without dry AMD.

Authors: Vice President <u>Yan Song</u>, Manager Marie Louise Edwards, Associate Annika Anderson, Senior Analysts Kirthana Sarathy and Dana Christensen, and researchers from Astellas Pharma

Funding for this study was provided by Astellas Pharma.

Impact of Attention-Deficit/Hyperactivity Disorder Treatment Adverse Effects on Quality of Life and Functioning of Pediatric Patients and Their Parents/Caregivers

Objectives: To assess the impact of attention-deficit/hyperactivity disorder (ADHD) treatment adverse effects (AEs) on quality of life (QoL) and functioning of pediatric patients and their parents/caregivers.

Conclusions: The presence of ADHD treatment AEs was associated with worse QoL, mental health, and impairment in ADHD patients and their parents/caregivers. Therapies with better safety profiles may help improve QoL in this population and improve parent/caregiver outcomes.

Authors: Vice Presidents Martin Cloutier and Marjolaine Gauthier-Loiselle, Manager Maryaline Catillon, Associate Louise Yu, Research Professionals Beatrice Libchaber and Yuxi Wang, 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.

Prevalence and Characteristics of Urinary Tract Infections in Office-Based Physician Practices in the United States, 2016–2019

Objectives: There are few contemporary data on urinary tract infection (UTI) rates in the United States (US). Historically, complicated UTIs (cUTIs) were considered to be encountered in inpatient or emergency department (ED) settings. This study aimed to describe the prevalence and burden of cUTI and uncomplicated UTI (uUTI) in the US ambulatory setting using nationally representative data of office-based physician practices.

Conclusions: This study utilizing a nationally representative data source highlights UTI as a highly prevalent diagnosis in US office-based physician practices. Additionally, cUTI accounts for a significant portion of outpatient UTI encounters, primarily in men. Additional investigation is needed to better understand the burden of UTIs and associated healthcare utilization in inpatient, ED, and hospital-based ambulatory settings.

Authors: Managing Principal Mei Sheng Duh, Vice President Rose Chang, Associate Raj Desai, Senior Analyst Megan Pinaire, Analyst Daisy Liu, and researchers from Duke University School of Medicine and GSK

Funding for this study was provided by GSK.

Real-DMD: Baseline Findings From an Electronic Observer-Reported Outcome (OBSRO) Survey of Long-Term Real-World Experiences of Patients with Duchenne Muscular Dystrophy (DMD)

Objectives: DMD is a rare, debilitating neuromuscular disease leading to muscle weakness and functional decline, primarily affecting young males. Due to early onset and progressive nature, caregivers play a crucial role in assessing and relaying information about patients' health, including their clinical status and functional ability. REAL-DMD, an observational, prospective, longitudinal cohort study involving caregivers of ambulatory DMD patients, aims to generate real-world data on caregiver-reported patient function and experiences. Patient baseline characteristics and functional ability are reported.

Conclusions: REAL-DMD baseline data provide insights into real-world characteristics and functional ability of ambulatory DMD patients. Findings indicate moderate to severe functional impairments among this patient population. Follow-up surveys will longitudinally characterize functional changes as disease progresses.

Authors: Managing Principal <u>Edward Tuttle</u>, Vice President <u>Min Yang</u>, Manager <u>Bruno Martins</u>, Associate Fan Yang, and researchers from Sarepta Therapeutics

Funding for this study was provided by Sarepta Therapeutics.

Real-World Clinical and Economic Burden of Primary Biliary Cholangitis in the United States

Objectives: Primary biliary cholangitis (PBC) is a chronic cholestatic liver disease that can progress to cirrhosis and end-stage liver disease. This study assessed the real-world clinical/economic burden of PBC in the US by line of treatment.

Conclusions: This claims data analysis suggests that many patients with PBC remain untreated years after diagnosis despite approved treatments and experience increased comorbidity burden as lines of therapy increase, likely due to disease progression. These findings indicate an unmet need among all patients with PBC, especially in the untreated and 2L+ settings.

Authors: Managing Principal <u>Hongbo Yang</u>, Manager <u>Su Zhang</u>, Associate Dongni Ye, Senior Analyst Shravanthi Seshasayee, and researchers from Ipsen, Weill Cornell Medical College, and Liver Institute Northwest

Funding for this study was provided by Ipsen.

POSTER SESSION 2

Monday, May 6 | 3:30-6:30 p.m.

Evaluation of the Healthcare System Burden Associated with Intravenous Treatment for Paroxysmal Nocturnal Hemoglobinuria

Objectives: To delineate steps in the intravenous (IV) treatment workflow with ravulizumab for paroxysmal nocturnal hemoglobinuria (PNH) from a healthcare system perspective and identify steps potentially simplified with an oral therapy.

Conclusions: The study revealed complexities associated with IV treatment with ravulizumab for PNH from a healthcare system perspective. Given IV treatment is resource- and time-intensive for both patients and providers and that infusion centers are operating at full capacity, adopting an oral treatment could potentially enhance infusion center capacity and improve scheduling flexibility for other competing conditions. Further studies are warranted to understand the IV treatment burden from additional healthcare personnel (nurses, pharmacists) and the patient perspective.

Authors: Managing Principal Annie Guérin; Vice President Dominick Latrémouille-Viau; Associate Gayatri Marathe; and researchers from Hematology Oncology of Indiana; UMass Memorial Health Care, University of Massachusetts Medical School; KU Medical Center; The University of Kansas; Novartis Pharmaceuticals; Novartis Pharmaceuticals UK; Massachusetts General Hospital; and Harvard Medical School

Funding for this study was provided by Novartis Pharmaceuticals.

Health State Transition Probabilities for Duchenne Muscular Dystrophy: Estimation from Published Data

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

Objectives: To characterize the progression of Duchenne muscular dystrophy (DMD) for healthcare decision makers, a natural history model (NHM) of disease progression across eight health states has been developed by Project HERCULES. Estimation of transition probabilities for this model, using traditional approaches, has been challenging because of limited longitudinal data. We applied an advanced estimation approach to refine, derive, and calibrate transition probabilities based on real-world DMD data.

Conclusions: Using the cumulant method to bridge data gaps yielded estimated health state transitions that accorded with multiple published sources. This congruency will be helpful to inform and enhance the use of the HERCULES model for decision making involving cost-effectiveness analyses and health technology appraisals.

Authors: Managing Principal <u>James Signorovitch</u>, Vice President <u>Michaela Johnson</u>, Manager Andres Gomez-Lievano, Senior Analyst Hana Akbarnejad, Analyst Eden Ma, and researchers from Pfizer

Funding for this study was provided by Pfizer.

Real-World Clinical Burden of Friedreich Ataxia in the United States

Objectives: Friedreich ataxia (FA) is a rare, inherited neurodegenerative, progressive disorder that leads to loss of ambulation, multisystem involvement, poor quality of life, and ultimately, premature death. This study assessed real-world clinical burden of FA in the United States.

Conclusions: Real-world data suggested patients with FA were associated with substantial and incremental clinical burden compared to matched controls and will benefit in advances in treatment for FA.

Authors: Managing Principal Hongbo Yang, Manager Su Zhang, Associate Anya Jiang, Senior Analyst Qi Hua, and researchers from the University of Pennsylvania, Reata Pharmaceuticals, and Ronald Reagan UCLA Medical Center Funding for this study was provided by Reata Pharmaceuticals.

Treatment Patterns and Healthcare Resource Use Among Patients with Axial Spondyloarthritis and Comorbid Inflammatory Bowel Disease

Objectives: Biologics approved for both axial spondyloarthritis (axSpA) and inflammatory bowel disease (IBD) may optimize outcomes among axSpA/IBD patients. This research describes real-world characteristics and treatment patterns of newly diagnosed axSpA patients (stratified by comorbid IBD).

Conclusions: Understanding treatment journey for axSpA/IBD patients may inform treatment decisions. axSpA/IBD patients had greater baseline disease burden versus patients without IBD. Most axSpA/IBD patients used axSpA/IBD indicated biologics during baseline and maintained through follow-up.

Authors: Vice Presidents <u>Elyse Swallow</u> and <u>Jess Marden</u>, Senior Analyst Kirthana Sarathy, and researchers from UCB Pharma

Funding for this study was provided by UCB Pharma.

Treatment Preferences of Adult Patients with Attention-Deficit/Hyperactivity Disorder in Canada: A Discrete Choice Experiment

Objectives: To quantify patient preferences for attention-deficit/hyperactivity disorder (ADHD) treatment attributes among adults in Canada.

Conclusions: While efficacy was highly valued by patients when making treatment decisions, safety attributes together had a greater relative importance than efficacy alone. Accordingly, ADHD treatments with favorable safety profiles may be preferred by patients. A better understanding of attributes of ADHD treatments valued by patients may help improve shared decision making, enhance treatment satisfaction, and foster adherence.

Authors: Vice Presidents Martin Cloutier and Marjolaine Gauthier-Loiselle, Manager Maryaline Catillon, Associate Yan Meng, Research Professionals Beatrice Libchaber and Yuxi Wang, 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.

POSTER SESSION 3

Tuesday, May 7 | 10:30 a.m.–1:30 p.m.

Economic Burden of Disease Recurrence in Elderly Patients with High-Risk Endometrial Cancer (HR-EC) Receiving Adjuvant Therapy (AT)

Objectives: The economic burden of disease recurrence among patients with HR-EC is not well understood. This study quantified healthcare resource utilization (HRU) and healthcare costs by recurrence status in elderly patients with HR-EC receiving AT.

Conclusions: Findings from this descriptive study suggest that a substantial economic burden is incurred by patients with recurrent HR-EC despite treatment with existing AT. Future multivariable research that adjusts for confounders can help validate the findings.

Authors: Vice President <u>Yan Song</u>, Manager <u>Kalé Kponee-Shovein</u>, Associate Mu Cheng, Senior Analyst Qi Hua, Senior Research Professional Annalise Hilts, and researchers from Merck, MSD (UK), and the University of Virginia *Funding for this study was provided by Merck*.

Public Sector Replacement of Privately Funded Pharmaceutical R&D: Cost and Efficiency Considerations

Objectives: Economic research shows that sizable investment in basic medical research provides important societal benefits. In turn, the costs of developing medicines from molecules in pre-clinical research to FDA approvals are currently overwhelmingly borne by industry. However, calls have grown to replace private sector activity with government funding. We developed a model to estimate these replacement costs and to investigate the economic implications of funding changes.

Conclusions: Relative to the total NIH budget, the estimated replacement cost of private sector drug R&D is substantially larger. We found no empirical evidence for efficiency improvement associated with a shift from private to public funding. Public and private roles are both important and not likely substitutable without some unknown degree of welfare change. Economic literature points to several concerns, including adverse selection, yardstick competition, hold-up, political rent seeking and under-rewarding of incremental innovation.

Authors: Manager <u>David Proudman</u> and researchers from Johnson & Johnson and Duke University Funding for this study was provided by Janssen Scientific Affairs, a subsidiary of Johnson & Johnson.

Symptom Burden and Healthcare Resource Use in Claudin 18.2+ (CLDN18.2+), HER2– Patients with Locally Advanced (LA) Unresectable or Metastatic Gastric or Gastroesophageal Junction (mG/GEJ) Adenocarcinoma

Objectives: This retrospective study described the real-world symptoms of patients with CLDN18.2+, HER2–, LA unresectable or mG/GEJ adenocarcinoma and the healthcare resource utilization (HRU) in this population.

Conclusions: This analysis of patients with CLDN18.2+, HER2–, LAmG/GEJ adenocarcinoma showed a substantial symptom burden and high HRU.

Authors: Managing Principals <u>Eric Q. Wu</u> and <u>Hongbo Yang</u>, Manager <u>Todor Totev</u>, and researchers from the University of Texas MD Anderson Cancer Center and Astellas Pharma

Funding for this study was provided by Astellas Pharma.

Treatment Patterns, Healthcare Resource Utilization and Costs, and Clinical Outcomes Among Elderly Patients with Advanced HER2-Positive Gastric or Gastroesophageal Junction Adenocarcinoma

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

Objectives: This study evaluated treatment patterns, healthcare resource utilization (HCRU) and costs, and clinical outcomes following first-line (1L) trastuzumab-contained therapy among advanced HER2-postive gastric or gastroesophageal junction (GEJ) adenocarcinoma patients.

Conclusions: These analyses characterized real-world treatment patterns and clinical outcomes in elderly patients with advanced HER2+ gastric/GEJ adenocarcinoma before the introduction of immuno-oncology therapy. This study highlights the substantial costs of care especially at the end of life and the importance of offering life-prolonging therapies to this population.

Authors: Vice President <u>Jenny Zhou</u>, Manager <u>Aolin Wang</u>, Associate Travis Wang, Senior Analyst Matthew Mattera, and researchers from Merck and the Icahn School of Medicine at Mount Sinai

Funding for this study was provided by Merck

POSTER SESSION 4

Tuesday, May 7 | 3:30-6:30 p.m.

Economic Burden of Diagnosed Congenital Cytomegalovirus in the United States and Japan

Objectives: Congenital cytomegalovirus (cCMV) is the leading infectious cause of birth defects in high-income countries. We examined healthcare resource utilization (HCRU) and cost burden among cCMV patients in the US and Japan using insurance claims data.

Conclusions: In the US and Japan, infants with cCMV had substantial resource burden during their first-year post-diagnosis, with 6–8 times greater incremental costs versus non-CMV controls. Future studies should continue evaluating the economic burden of diagnosed cCMV patients in countries where data are available.

Authors: Managing Principal Noam Kirson; Vice Presidents <u>Debbie Goldschmidt</u>, <u>Urvi Desai</u>, and <u>Jess Marden</u>; Associate Annika Anderson; Senior Analyst Kirthana Sarathy; and researchers from Moderna, Baylor College of Medicine, and Texas Children's Hospital

Funding for this study was provided by Moderna.

Evaluation Of Real-World Treatment Patterns in Patients With Uncontrolled Gout in the USA

Objectives: There is limited real-world evidence on the treatment patterns of patients with uncontrolled gout in the USA. This study assessed the treatment patterns associated with management of uncontrolled gout among US patients treated in routine clinical practice.

Conclusions: In this population of patients with uncontrolled gout, recorded sUA levels were <6.0 mg/dL at some point during the follow-up period in less than half of patients, representing a high unmet need for new effective treatments.

Authors: Vice President <u>Elyse Swallow</u>, Manager <u>Emily Gao</u>, Associate Sakshi Sethi, and researchers from Sobi and M-Spective

Funding for this study was provided by Sobi.

Impact of Advanced/Metastatic Non–Small Cell Lung Cancer (A/MNSCLC) on Patients in the US and UK: Survey Results

Objectives: As existing data are limited, this online survey assessed cancer-related burden in a/mNSCLC.

Conclusions: This survey revealed a/mNSCLC to be associated with high real-world burden. Notably, one-quarter of patients had brain metastases, which may have contributed to the impairment reported. Novel treatments, particularly those that could treat or prevent brain metastases, are needed to mitigate this burden.

Authors: Managing Principal Hongbo Yang, Manager Yao Wang, Associate Yan Meng, Senior Analyst Mingchen Ye, and researchers from the Boston University Chobanian & Avedisian School of Medicine and GSK

Funding for this study was provided by GSK.

Real-World Healthcare Resource Utilization and Costs of Patients with Alzheimer's Disease By Stage: A Retrospective Observational Study

Objectives: This study aimed to compare the Alzheimer's disease (AD)-related healthcare resource utilization (HCRU) and costs by disease stage and identify the predictors of high AD-related healthcare costs.

Conclusions: Patients with AAD incurred higher AD-related HCRU and costs compared to those with EAD. Further research is necessary to determine whether interventions earlier in disease progression can mitigate these costs for AAD patients.

Authors: Vice President Marianne Cunnington; Associates Raj Desai, Christopher W. Yee, and Chia-Lun Liu; and researchers from Novo Nordisk and the University of Southern California Center for Economic and Social Research Funding for this study was provided by Novo Nordisk.

POSTER SESSION 5

Wednesday, May 8 | 9:00–11:30 a.m.

A Matching-Adjusted Indirect Comparison of Centanafadine Vs Lisdexamfetamine, Methylphenidate, and Atomoxetine in Adults with Attention-Deficit/Hyperactivity Disorder: Long-Term Safety and Efficacy Outcomes

Objectives: To compare long-term safety and efficacy outcomes of centanafadine vs. lisdexamfetamine, methylphenidate, and atomoxetine, respectively, in adults with attention-deficit/hyperactivity disorder (ADHD) using matching-adjusted indirect comparison (MAIC).

Conclusions: At up to 52 weeks, centanafadine showed significantly lower incidence of several AEs than lisdexamfetamine, methylphenidate, and atomoxetine; efficacy was lower than lisdexamfetamine and non-different from methylphenidate and atomoxetine. Future studies should evaluate patients' and physicians' valuations on treatment attributes and how tradeoff between safety and efficacy affects ADHD management.

Authors: Vice Presidents Martin Cloutier and Marjolaine Gauthier-Loiselle, Manager Maryaline Catillon, Senior Analyst Chunyi Xu, 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.

Burden Of Disease in Patients With Uncontrolled Gout in the USA

Objectives: Limited real-world evidence exists on the healthcare burden of patients with uncontrolled gout in the USA. This study assessed the burden of uncontrolled gout among patients treated in routine clinical practice.

Conclusions: Uncontrolled gout confers a high burden on patients and the US healthcare system; ~1 in 6 patients made ≥1 ER visit, and all patients made gout-related outpatient visits.

Authors: Vice President <u>Elyse Swallow</u>, Manager <u>Emily Gao</u>, Associate Sakshi Sethi, and researchers from Sobi and M-Spective

Funding for this study was provided by Sobi.

The Caregiver Perspective on Dry Age-Related Macular Degeneration: A Qualitative Interview Study

Objectives: Dry age-related macular degeneration (AMD) causes progressive vision loss. This qualitative study assessed impairment in ability to perform daily activities and humanistic burden associated with dry AMD for patients and caregivers. Patient-reported outcomes are presented elsewhere; here, caregiver perspective is reported.

Conclusions: These interview data emphasize the broad impact of dry AMD. Caregiving for patients with dry AMD imposes burdens on work productivity, personal relationships, and mental health.

Authors: Vice President <u>Yan Song</u>, Manager Marie Louise Edwards, Associate Annika Anderson, Senior Analysts Kirthana Sarathy and Dana Christensen, and researchers from Astellas Pharma

Funding for this study was provided by Astellas Pharma.

Economic Burden of Disease Recurrence in Elderly Patients with Human Epidermal Growth Factor Receptor 2-Negative (HER2–) Early Breast Cancer (EBC)

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

Objectives: Many patients with HER2– eBC treated with standard of care experience recurrent disease. To advance the understanding of the economic burden of recurrence, this study quantified the impact of recurrence on healthcare resource utilization (HRU) and costs in elderly patients with HER2– eBC.

Conclusions: Our findings shed light on the significant economic burden of recurrent disease in HER2–eBC and underscore the unmet need for early identification of high-risk patients and optimization of therapies that mitigate recurrence in this population.

Authors: Vice President <u>Yan Song</u>, Manager <u>Kalé Kponee-Shovein</u>, Associate Malena Mahendran, Senior Analyst Qi Hua, Senior Research Professional Annalise Hilts, and researchers from Merck and Stanford University

Funding for this study was provided by Merck.

Prevalence, Real-World Healthcare Resource Utilization, and Costs of Patients with Paroxysmal Nocturnal Hemoglobinuria in the US: A Retrospective Claims Database Analysis

Objectives: To estimate the 5-year prevalence of paroxysmal nocturnal hemoglobinuria (PNH) and describe the healthcare resource utilization (HRU) and direct healthcare costs associated with C5 inhibitors (C5i; eculizumab or ravulizumab) among commercially insured patients in the US.

Conclusions: Findings of this study indicated the 5-year prevalence of PNH to be 2.4 per 100,000 persons in commercial claims. Despite treatment with C5i associated with high annual PNH-related costs, patients with PNH still exhibited BTH and required blood transfusions, potentially indicating an unmet clinical need for more effective treatments.

Authors: Managing Principal <u>Annie Guérin</u>, Vice President <u>Dominick Latrémouille-Viau</u>, Associate Raj Desai, Senior Analyst Shravanthi Seshasayee, and researchers from the Huntsman Cancer Institute and Novartis Pharmaceuticals

Funding for this study was provided by Novartis Pharmaceuticals.

Reductions in Real-World Healthcare Resource Utilization Among United States Hereditary Angioedema (HAE) Patients Following Berotralstat Initiation

Objectives: To evaluate angioedema-related healthcare resource utilization (HRU) before and after initiation of berotralstat for long-term prophylaxis (LTP) of hereditary angioedema (HAE) in the US.

Conclusions: Results from this real-world study of patients with HAE demonstrate that berotralstat was associated with significant reductions in angioedema-related HRU.

Authors: Principal <u>François Laliberté</u>; Manager <u>Sean MacKnight</u>; Research Professional Colleen Spencer; and researchers from the University of California, San Diego; Veterans Administration Healthcare; and BioCryst Pharmaceuticals

Funding for this study was provided by BioCryst Pharmaceuticals.