ANALYSIS GROUP
ECONOMIC, FINANCIAL and STRATEGY CONSULTANTS

Raising the Standard in HEOR

Analysis Group Posters and Presentations

ISPOR 2019 | MAY 18–22 | NEW ORLEANS, LA

Analysis Group health 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 19 research posters. In addition, one of our experts will be moderating a podium presentation. We have included details on all of these below.

If time permits, please stop by and say hello to our team at Booth #405.

ISPOR 2019 Analysis Group Educational Symposium and Podium Presentations

EDUCATIONAL SYMPOSIUM

Tuesday, May 21, 2019 | 12:30–1:30 p.m. | Room 278-282 (Second Floor)

Cell and Gene Therapy – Value and Access

Cell and gene therapy is an emerging field in healthcare that demonstrates promising results to treat and potentially cure serious, life-threatening conditions. Under the FDA's 21st Century Cures Act, a regenerative medicine therapy, including cell and gene therapy, can receive a regenerative medicine advanced therapy (RMAT) designation, which accelerates its FDA approval timeline.

However, while there is excitement about the potential approval of more cell and gene therapies within the next few years, stakeholders are concerned about the high upfront costs of these therapies and extremely limited evidence of their long-term value. In addition, many cell and gene therapies will be launched based on the results of single-arm Phase I or II trials with small sample sizes targeting rare diseases. This may introduce additional uncertainties for policymakers and payers when assessing the value of these therapies and making reimbursement decisions.

In light of this landscape, this education symposium aims to introduce attendees to common considerations when valuing and pricing cell and gene therapies, as well as those concerning reimbursement access. The presenters will discuss potential approaches to assess the value of cell and gene therapies and facilitate a discussion of how to prepare the healthcare system for the coming wave of potentially curative medicine from the perspectives of policymakers, clinicians, and payers.

Moderator:
Michael Kuehn, R.Ph.; *Vice President, Analysis Group*

Panelists:
Michael Sherman, M.D.; *Chief Medical Officer & Senior Vice President, Harvard Pilgrim Health Care*
Rick Chapman, Ph.D.; *Director of Health Economics, Institute for Clinical and Economic Review (ICER)*
Eric Q. Wu, Ph.D.; *Managing Principal, Analysis Group*
Hongbo Yang, Ph.D.; *Vice President, Analysis Group*

PODIUM PRESENTATION

Monday, May 20, 2019 | 3:45–4:45 p.m

P4: RARE & ORPHAN DISEASE STUDIES

RO2: Assessing the Relationships Between Lifelong Value and Pricing for Orphan Drugs in Ultra-Rare Diseases

Objectives: As an increased number of orphan drugs have been developed and marketed, the pricing of treatments for rare diseases has attracted more payer attention and scrutiny. This study assessed the relationship between incremental quality-adjusted life-year (QALY), a measure of the value of health outcomes, and drug prices for orphan drugs recently approved for ultra-rare diseases; and shed light on the determination of reasonable pricing for new orphan drugs based on estimated incremental QALY gained.

Conclusions: Innovative therapies in ultra-rare diseases that offer enhanced value are rewarded with a greater market price. Such analysis can be used to estimate the prices of new orphan drugs under development.

Presenter:
Jenny Zhou, Ph.D.; *Vice President, Analysis Group*

PODIUM PRESENTATION

Wednesday, May 22, 2019 | 8:30–9:30 a.m.

P17: Conceptual Papers

Moderator:
Mei Sheng Duh, M.P.H., Sc.D; *Managing Principal, Analysis Group*

ISPOR 2019 Analysis Group Research Posters

SESSION I

Monday, May 20, 2019 | 10:30 a.m.–2:00 p.m. ▪ Poster Author Discussion Hour: 1:00–2:00 p.m.

PCN22: CANCER

Clinical Heterogeneity Among First-Line Sunitinib Patients with Metastatic Renal Cell Carcinoma (MRCC) Categorized as Intermediate Risk (IR) Group According to the International MRCC Database Consortium (IMDC) Risk Model

Objectives: The IMDC risk model, a well-established prognostic model for mRCC, provides benchmarks for trial design, patient counseling, and risk-specific treatments. Based on six risk factors, the IMDC model categorizes patients as favorable (no factors), intermediate (1 or 2 factors), or poor (≥3 factors). To date, limited studies have examined heterogeneity in the IR group. This study assessed patient characteristics and clinical heterogeneity among mRCC patients in the IMDC IR group who received 1L SUN therapy in the real-world setting.

Conclusions: This real-world study shows that mRCC patients in the IR group are heterogeneous in characteristics and clinical outcomes, with longer OS and TTD in patients with 1 versus 2 risk factors. These findings are consistent with previous analysis of clinical trial data, and should be considered when counseling IMDC IR patients.

PCN48: CANCER

Economic Burden Associated with Severe Adverse Events in Patients with Metastatic Non-Small Cell Lung Cancer Treated with First-Line Epidermal Growth Factor Receptor Tyrosine Kinase Inhibitors

Objectives: To estimate incremental economic burden associated with severe adverse events (SAEs) in patients with mNSCLC treated with first-line (1L) EGFR-TKIs.

Conclusions: SAEs are associated with high HRU, contributing to higher costs in patients with mNSCLC treated with 1/2G EGFR-TKIs in 1L. EGFR-TKI treatments with better safety profiles may help alleviate this burden.

PCN109: CANCER

Real-World Adherence, Costs, and Treatment Dosage of Patients with Neuroendocrine Tumors (NET) Treated with Octreotide or Lanreotide

Objectives: There is limited research assessing real-world use and costs of somatostatin analogs and outcomes in NET patients (pts). This study examined adherence, costs, and treatment dosage among NET pts receiving long-acting octreotide or lanreotide.

Conclusions: Octreotide and lanreotide appear to have similar treatment adherence, and the majority of patients used their respective labeled dosage in this study. Octreotide pts were seen associated with significantly lower total costs, both overall and in pts with CS.

PCN130: CANCER

Real-World Demographics and Clinical Characteristics of Patients Diagnosed with Primary Mediastinal B-Cell Lymphoma (PMBCL)

Objectives: PMBCL is a rare mature B-cell neoplasm (2-4% of non-Hodgkin lymphomas), with sparse data about healthcare resource utilization (HRU) and treatment costs. In October 2015, PMBCL was administratively differentiated from diffuse large B-cell lymphoma (DLBCL) with the advent of ICD-10-CM PMBCL-specific codes. This retrospective study characterized these patients.

Conclusions: This analysis showed patients at diagnosis of PMBCL in the US had substantial clinical and economic burden.

PGI2: GASTROINTESTINAL DISORDERS

Use of Different Statistical Approaches to Compare Real-World Outcomes Associated with Vedolizumab Versus Infliximab in Biologic-Naïve Patients with Inflammatory Bowel Disease

Objectives: To compare real-world outcomes related to treatment effectiveness in biologic-naïve patients with inflammatory bowel disease (IBD) initiated on vedolizumuab versus infliximab using different statistical approaches to account for potential unbalanced confounding factors.

Conclusions: Regardless of the statistical approach, vedolizumab was associated with lower rates of treatment discontinuation and HRU composite endpoint versus infliximab in IBD patients. E-balance may be the preferred approach since it achieved better balanced patient characteristics, compared to other statistical approaches.

SESSION II

Monday, May 20, 2019 | 3:30 –7:00 p.m. ▪ Poster Discussion Hour: 6:00–7:00 p.m.

PMH14: MENTAL HEALTH

Cost Efficiency of Esketamine Nasal Spray Versus Standard of Care for Treatment-Resistant Depression

Objectives: To compare the annual per-patient direct and indirect costs associated with achieving remission with esketamine nasal spray plus oral antidepressants (ESK+OAD) versus oral antidepressant plus placebo (OAD+PBO) among patients with treatment-resistant depression (TRD) and determine the break-even net price per ESK device.

Conclusions: These findings suggest that esketamine nasal spray in conjunction with oral antidepressants is a cost-efficient alternative compared with antidepressant alone for TRD below the net price per device of $325–470, depending on the plan type. The benefits associated with ESK+OAD treatment are particularly pronounced when considering the costs associated with lost productivity.

PMH22: MENTAL HEALTH

Is Increased Severity Status Associated with Incremental Economic Burden in Privately Insured US Patients with Treatment Resistant Depression?

Identified as a semifinalist for a Research Presentation Award (Top 10%)

Objectives: Among patients with major depressive disorder (MDD), those with treatment-resistant depression (TRD) have a higher economic burden. However, healthcare resource utilization (HRU) and costs may vary by MDD severity in patients with TRD. This study compares treatment patterns, HRU, and costs of TRD by MDD severity.

Conclusions: Increased MDD severity is associated with incremental economic burden in patients with TRD.

PIH15: INDIVIDUAL'S HEALTH

Comorbidity and Economic Burden of Peanut Allergy in Privately Insured Pediatric Patients in the United States

Objectives: This study assessed the cost of care of peanut allergy (PA) among privately insured pediatric patients in the US.

Conclusions: In pediatric PA patients, comorbidities, emergency department (ED) visits, inpatient admissions, days with outpatient services, and annual direct healthcare costs, regardless of asthma-related costs, were consistently higher versus matched PA-free patients in the privately insured population.

PIH25: INDIVIDUAL'S HEALTH

Healthcare Resource Utilization and Direct Healthcare Costs Related to Peanut Allergy in Pediatric Medicaid Patients

Objectives: Evaluation of the cost of care of peanut allergy (PA) among pediatric Medicaid-insured patients.

Conclusions: In pediatric PA patients, comorbidities, emergency department (ED) visits, inpatient admissions, days with outpatient services, and annual direct healthcare costs, regardless of asthma-related costs, were consistently higher versus matched PA-free patients in the Medicaid-insured population.

SESSION III

Tuesday, May 21, 2019 | 10:30 a.m.–2:00 p.m. ▪ Poster Discussion Hour: 1:00–2:00 p.m.

PIN17: INFECTIOUS DISEASES

Rapid Initiation of Antiretroviral Treatment (ART) Following Diagnosis of Human Immunodeficiency Virus (HIV) Among Medicaid Beneficiaries: A Real-World Evaluation

Identified as a semifinalist for a Research Presentation Award (Top 10%)

Objectives: This study aims to assess real-world time to ART initiation and describe outcomes in Medicaid patients based on timeliness of ART initiation.

Conclusions: This study revealed that only 20% of Medicaid patients initiated ART within 14 days post-diagnosis, and that patients with delayed initiation accumulated more healthcare costs than those with rapid initiation, highlighting the long-term benefits of rapid ART initiation.

SESSION IV

Tuesday, May 21, 2019 | 3:30–7:00 p.m. ▪ Poster Discussion Hour: 6:00–7:00 p.m.

PMU62: MULTIPLE DISEASES

US Care Pathways: Continued Focus on Oncology and Outstanding Challenges

Objectives: The use of care pathways has proliferated in the US. This study assessed current methods used in care pathway development, implementation, and evaluation, and examined the emerging relationship between care pathways and other components of value-based care.

Conclusions: This study identified growing use of high standards of evidence and adoption of other best practices in the development, implementation, and evaluation of care pathways. Increasingly driven by providers and provider networks, and as the influence of care pathways on patient care continues to expand, additional efforts are needed to increase transparency, disclose conflicts of interest, and engage with patients. Furthermore, awareness of care pathway use and associated financial incentives remains lacking. While pathways can be an effective tool in the delivery of value-based care and promise to improve value, integration with other value-based care initiatives remains limited.

PMU85: MULTIPLE DISEASES

Adjustment for Effect Modifiers and Prognostic Factors in Matching-Adjusted Indirect Comparisons

Objectives: Indirect comparisons incorporating population adjustment, such as matching-adjusted indirect comparisons (MAICs), can be used to adjust for differences in population characteristics across the trials being compared. When many baseline characteristics are available for adjustment, the selection of an appropriate subset of variables to match on may be considered. A common approach is to include all possible baseline covariates; two of the most common alternatives are to include effect modifiers and prognostic factors. However, the feasibility and impact of selecting these variables is not well studied.

Conclusions: Variable selection methods can have significant impact on the bias and precision of MAICs. It is important to examine the assumptions made when selecting the variables.

PMU86: MULTIPLE DISEASES

Arm-to-Arm Weighting and Variance Adjustment in Matching-Adjusted Indirect Comparisons

Objectives: Matching adjusted indirect comparison (MAIC) is a useful tool to compare the relative effectiveness of treatments across clinical trials and inform health technology assessments. Despite its increasing popularity, there is a need to further understand the appropriate ways to apply this method. Prior studies indicate that matching individual patient data (IPD) and aggregate-level data (AD) at the treatment arm level, rather than at the trial level, produced more precise estimates, and that simultaneously matching on mean and variance of baseline characteristics rendered similar results than when matching on the mean only. Here we investigate how these observations hold when 1) the sample size of the IPD changes and 2) the relationship between baseline characteristics and outcomes is non-linear.

Conclusions: This study characterizes how the accuracy of MAICs depends on the properties of the population sample, the outcome type, and the operational specifications of the method.

PMU87: MULTIPLE DISEASES

Matching-Adjusted Indirect Comparisons: Recommendation for the Selection of Baseline Characteristics Used for Matching

Objectives: Matching-adjusted indirect comparisons (MAICs) offer an alternative in situations where Bucher comparisons are likely to be biased due to differences in trial populations. MAIC applies weights to the individual patient data (IPD) from one trial, matching a set of baseline characteristics to those in the other trial(s). The selection of the baseline characteristics used for adjustment can impact the reliability of an MAIC.

Conclusions: The analyses show that improvements to standard error may be possible without an increase in bias, and suggest that statistical tools to identify such situations may be valuable.

PUK10: URINARY/KIDNEY DISORDERS

Societal Burden of Autosomal Dominant Polycystic Kidney Disease (ADPKD) in the United States: Beyond Healthcare Costs

Objectives: ADPKD is one of the most common life-threatening inherited kidney diseases characterized by cysts in kidneys, eventually leading to kidney failure. Given the life-long progressive nature of the disease, it is expected to carry a substantial economic burden; however, there is limited evidence of its societal economic impact. The study objective was to estimate the direct and indirect costs associated with ADPKD in the United States.

Conclusions: ADPKD is associated with a substantial economic burden, attributed principally to direct healthcare costs, the majority of which are incurred by payers. Interventions to slow down the progression of ADPKD have the potential to reduce the economic burden of ADPKD.

PRO22: RARE & ORPHAN DISEASES

Burden of Illness of Spinal Muscular Atrophy: An Update

Objectives: Spinal muscular atrophy type 1 (SMA1) is a rare, debilitating, genetic neuromuscular disease. The first treatment for SMA1, nusinersen, was recently FDA approved (12/23/2016). As the treatment landscape evolves, an updated assessment of the burden of SMA1 is warranted. This study assessed the healthcare resource use (HRU) and costs among patients with SMA1 in real-world practice.

Conclusions: HRU and costs associated with SMA1 are substantial, even among patients treated with nusinersen.

SESSION V

Wednesday, May 22, 2019 | 9:30 a.m.–2:00 p.m. ▪ Poster Discussion Hour: 12:45–1:45 p.m.

PSY10: SYSTEMIC DISORDERS/CONDITIONS

Opioid and Healthcare Resource Use Among Privately Insured Moderate-to-Severe Psoriasis Patients in the US

Objectives: To evaluate opioid and resource use among moderate-to-severe psoriasis (PsO) patients stratified by number of PsO-related comorbidities.

Conclusions: Opioid use in moderate-to-severe PsO patients is substantial and increases with the number of PsO-related comorbidities. Relative to all moderate-to-severe PsO patients, healthcare resource use was higher among those treated with opioids.

PSY24: SYSTEMIC DISORDERS/CONDITIONS

Early Predictors of Sjögren's Syndrome: A Machine Learning Approach

Objectives: To develop a predictive model to identify patients at high risk of Sjögren's syndrome (SjS) and early indicators of SjS.

Conclusions: Advanced machine learning methods can be used to inform clinicians about early indicators to facilitate timely diagnosis of SjS.

PNS196: NO SPECIFIC DISEASE

Use of Machine Learning in Health Economics and Outcomes Research (HEOR): Optimizing Predictive Models

Objectives: Predictive modeling has a wide range of applications in HEOR. When the number of predictors is large in comparison to number of observations, conventional generalized linear regression models can yield poor predictions. Machine learning (ML) methods are increasingly being used in HEOR as an alternative framework to increase prediction accuracy. Commonly used ML models include Logistic-LASSO, tree-based models (CART, Random Forest), and Neural Networks. This research aimed to explore the value of ML for predictive modeling in HEOR, using high-dimensional datasets, such as genomic or claims data, and the trade-offs between model performance and interpretability. Although ML models often outperform regression-based approaches in prediction quality, questions remain about their interpretability.

Conclusions: ML models exhibited better predictive performance measured by ROC-AUC and bias than conventional logistic regression models, but did so at the expense of interpretability. Conventional regression models can provide a better framework if interpretability is central to the prediction analysis.