



ANALYSIS GROUP
ECONOMIC, FINANCIAL and STRATEGY CONSULTANTS

Raising the Standard in HEOR

Analysis Group Posters and Presentations

ISPOR EUROPE 2019 | NOVEMBER 2-6 | COPENHAGEN, DENMARK

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 five research posters. We have included details on all of these below.

If time permits, please stop by and say hello to our team at Booth #C2-002.

ISPOR Europe 2019 Analysis Group Educational Symposium and Podium Presentations

EDUCATIONAL SYMPOSIUM

Monday, November 4, 2019 | 7:15–8:15 a.m. | Hall C1 M2-3 (Ground Floor)

Gene Therapy – Value and Access in Europe

Recent advances in gene therapies are rapidly evolving to care for many life-threatening conditions with high unmet need. The introduction of durable and potentially curative new treatment options is changing the prospects for providers, patients, and their families. Regulatory agencies are accounting for these innovative treatments with new programs and guidelines. The European Medicines Agency (EMA) has developed a number of scientific guidelines specific to gene therapy products to keep pace with clinical and scientific innovations, and introduced the priority medicines (PRIME) scheme in 2016. As one type of advanced therapy medicinal products (ATMPs), gene therapy can receive the PRIME designation, which expedites the process for new medicine to reach the market and patients.

As regulators put policies in place to prepare for the coming wave of gene therapies, there are significant challenges in using conventional metrics to assess long-term value. A lack of effective comparators and the heterogeneity of the patient population in clinical trials introduce uncertainties when assessing the value of these medicines. High upfront costs and limited evidence of long-term outcomes also highlight the need for new reimbursement models. A new value assessment framework is needed to incorporate broader elements of value into the decision making. In light of this situation, presenters will discuss well-known considerations and potential alternative approaches to assessing value-based pricing for gene therapies. They will facilitate a dialogue on how to prepare the healthcare system for the advancement of gene therapies, from the perspectives of manufacturers, policy makers, and payers.

Moderator:

Eric Q. Wu, Ph.D., *Managing Principal, Analysis Group, Boston, Massachusetts, USA*

Panelists:

Michael F. Drummond, M.Com., D.Phil., *Centre for Health Economics, University of York, York, England, UK*

Omar Dabbous, M.D., M.P.H., *AveXis, Inc., Bannockburn, Illinois, USA*

Jenny Zhou, Ph.D., *Vice President, Analysis Group, London, England, UK*

PODIUM PRESENTATION – BREAKOUT SESSION I

Monday, November 4, 2019 | 11:00 a.m.–12:00 p.m.

P1: ARTIFICIAL INTELLIGENCE STUDIES

AI1: Predictors of High Healthcare Utilisers Among Patients with Chronic Hypoparathyroidism – Application of Machine Learning Methodology

Objectives: To identify patient characteristics that predict high healthcare utilisers among adult patients with chronic hypoparathyroidism using machine learning methodology.

Conclusions: Recent studies showed that chronic hypoparathyroidism is associated with increased risk of infections, renal, or cardiovascular complications, and diabetes. Improved treatment for patients with chronic hypoparathyroidism may reduce risks of these complications, which are predictive of healthcare utilization that may lead to high medical care expenditure in the following year.

Analysis Group Authors:

Elyse Swallow, *Vice President*, and Wei Gao, *Manager*

ISPOR Europe 2019 Analysis Group Research Posters

SESSION II

Monday, November 4, 2019 | 3:30–7:00 p.m. ■ Poster Discussion Hour: 6:00–7:00 p.m.

PCN168: CANCER

Economic Burden of Relapse After Hematopoietic Stem Cell Transplantation (HSCT) in Patients with Acute Myeloid Leukemia (AML): A Retrospective Claims-Based Analysis

Objectives: The economic impact of relapse post-HSCT has not been evaluated in AML. A claims-based study was performed to assess healthcare resource utilization (HRU) and costs in patients with AML following HSCT in the US.

Conclusions: Significantly increased HRU and healthcare costs associated with relapse post-HSCT highlight the tremendous unmet need for new treatments that reduce post-HSCT relapse risk.

SESSION III

Tuesday, November 5, 2019 | 10:30 a.m.–2:00 p.m. ■ Poster Discussion Hour: 1:00–2:00 p.m.

PBI18: BIOLOGICS/BIOSIMILARS/REGENERATIVE MEDICINE

Estimation of the Healthcare Resource Utilization (HCRU) Costs in Patients with Relapsed or Refractory Diffuse Large B-Cell Lymphoma Receiving Tisagenlecleucel: A Micro-Consulting Study in the UK and France

Objectives: To estimate the cost of healthcare resource utilization (HCRU) in relapsed or refractory (r/r) diffuse large B-cell lymphoma (DLBCL) patients treated with tisagenlecleucel.

Conclusions: The HCRU costs within two months of tisagenlecleucel administration equated to £24,009 in the UK and €30,172 in France. Further research with estimates based on real-world clinical use of tisagenlecleucel is warranted.

SESSION V

Wednesday, November 6, 2019 | 9:30 a.m.–2:00 p.m. ■ Poster Discussion Hour: 12:45–1:45 p.m.

PRO4: RARE & ORPHAN DISEASES

Impact of Enzyme Replacement Therapy on the Risk of Developing Gaucher Disease-Related Complications

Objectives: Enzyme replacement therapy (ERT) prevents or delays the onset of clinical manifestations of type 1 Gaucher disease (GD). This study assessed the impact of ERT initiation timing on the risk of developing new GD-related complications.

Conclusions: Earlier treatment with ERT was associated with a decreased risk of developing a new GD-related complication.

PRO125: RARE & ORPHAN DISEASES

Suitability of Natural History Data for External Controls in Duchenne Muscular Dystrophy

Objectives: Use of natural history (NH) controls in Duchenne Muscular Dystrophy (DMD) drug evaluations is of high interest; however, the heterogeneity among patients or outcome assessments could potentially bias comparisons between NH and clinical trials, especially for performance-based outcomes. As a follow-up to our previous work using the six-minute walk distance (6MWD), we aimed to assess this concern by comparing outcomes between NH data sources and clinical trial placebo arms using the North Star Ambulatory Assessment (NSAA), a commonly used primary or secondary outcome measure.

Conclusions: These findings align with our previous research using 6MWD, and further demonstrate the potential of NH controls to augment or replace placebo arms in DMD drug evaluations.

PRO126: RARE & ORPHAN DISEASES

A Composite Prognostic Score for Time to Loss of Walking Ability in Duchenne Muscular Dystrophy (DMD)

Objectives: Prediction of loss of ambulation (LoA) is a critical milestone in DMD. We assessed the extent to which combinations of patient characteristics help predict time to LoA across different data sources.

Conclusions: A composite prognostic score incorporating multiple measures of ambulatory function can improve prediction of time to LoA. Once validated, such a score can inform clinical practice and trial designs, and enable adjusted comparisons between patients receiving newer therapies (e.g., in extension trials) and natural history controls.
