

Raising the Standard in HEOR

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# Analysis Group Posters and Presentations

ISPOR EUROPE 2022 | NOVEMBER 6–9 | VIENNA, AUSTRIA

**Analysis Group's 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 and seven research posters. Please find details below.

# ISPOR Europe 2022 Analysis Group Educational Symposium

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## EDUCATIONAL SYMPOSIUM

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Tuesday, November 8 | 11:30 a.m.–12:30 p.m. | Austria Center Vienna, Hall E1

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### Unlock Real-World Data with Machine Learning

An increasing demand for real-world evidence (RWE) to support regulatory submissions and payer negotiation has accelerated the big-data revolution in health care. Major strides have been made in improving the availability and usability of real-world data (RWD), and the application of machine learning (ML) in RWD is an essential step forward to augment meaningful insights into the care pathway. This symposium will introduce several creative ML approaches to establish consistency across data sources, harness the research value of RWD, and discuss the application of transparent and interpretable ML methods.

First, a case study will illustrate how ML methods are used to improve the consistency, transparency, and traceability of RWD across hospitals in China, where RWE research has traditionally faced many challenges. In a large-scale multi-center hematology study, researchers established a disease model to incorporate both consensus-based decision logic and ML-based data-driven optimization to unify data standards and definitions. This tool was applied to mechanize information integration across top hematology centers in China to enable result synchronization, which is essential in the generation of high-quality evidence to support China's National Reimbursement Drug List's negotiation and post-launch activities.

The second presentation will demonstrate how ML tools help to empower data transformation while ensuring relevance and validity. The raw RWD captured by hospital information systems (HIS) are fragmentary and lack an integrated picture of the care pathway. To close this gap, researchers developed and validated progression algorithms, drawn from China's National Longitudinal Cohort of Hematological Diseases (NICHE), to reconstruct the complex patient journey through a hematologic condition. The progression algorithm was then incorporated into an automated data capture system to obtain, filter, and process traceable data, allowing researchers to supply important information that is not readily available from the HIS, including patients' treatment responses by line of therapy.

Lastly, the presenters will demonstrate new transparent and interpretable ML approaches using case studies. Generalized linear models (GLM) are often preferred over ML models because of concerns regarding the lack of interpretability of complex ML algorithms. State-of-the-art interpretable ML methodologies will be discussed, along with their applications to a wide-ranging set of ML models. The objective of these approaches is to describe both general model behavior and the logic behind individual data unit predictions. These methods provide an improved level of interpretability and transparency that is both informative and, to some extent, more granular than GLM.

We hope this symposium can introduce these recent creative examples in the intersection of ML and RWD to the audience and stimulate discussions to further advance RWE research and methodology.

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

**Panelists:** Jia Zhong, Sc.D., *Manager, Analysis Group*  
Xiaochen Zhang, M.S., *Project Director, Beijing Huashu Yihui Technology Co., Beijing, China*  
Max Leroux, M.Sc., *Director of Data Science, Analysis Group*

# ISPOR Europe 2022 Analysis Group Research Posters

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## POSTER SESSION 2

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Monday, November 7 | 3:00–6:15 p.m. | Presentation Time: 3:00–4:00 p.m.

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### Predicting Antimicrobial Resistance in Uncomplicated Urinary Tract Infections Using Machine Learning

**Objective:** Urinary tract infections (UTIs) are among the most common bacterial infections worldwide, with 80% classified as uncomplicated (uUTIs). Over 50% of patients with uUTI are prescribed non-guideline-based antimicrobial treatment, potentially contributing to antimicrobial resistance (AMR) and increased health care costs. Here, we present the methodology underlying our study, which used machine learning in the development and validation of robust models estimating the probability of resistance to commonly prescribed classes of antibiotics for uUTI.

**Conclusion:** This predictive algorithm for AMR among uUTI patients further improves upon existing models because Optum EHR data are larger than those used in other published models in the United States, enabling greater statistical power and generalizability.

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### Modelling Long-Term Clinical Outcomes of Patients with Sickle Cell Disease with Recurrent Vaso-Occlusive Crises in the United States

**Objective:** Sickle cell disease (SCD) is a rare hereditary blood disorder with severe symptom burden including vaso-occlusive crises (VOCs) and chronic end-organ damage. VOCs are associated with an increased risk of complications and death. A model was developed to predict long-term clinical outcomes for patients with SCD experiencing recurrent VOCs in the United States.

**Conclusion:** Model projections demonstrate that patients with SCD with recurrent VOCs have a reduced life expectancy and experience substantial disease burden. Treatments that can minimize the occurrence of VOCs and reduce disease complications could improve survival and long-term outcomes in this patient population.

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## POSTER SESSION 3

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Tuesday, November 8 | 10:00 a.m.–1:15 p.m. | VIRTUAL

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### Disease Management Costs for Patients with Intermediate-High or High-Risk Renal Cell Carcinoma (RCC) Following Nephrectomy

**Objective:** Disease management costs by health state, which are important in economic modeling, were not well studied in post-nephrectomy renal cell carcinoma (RCC). This study aims to assess the disease management costs by RCC health state post-nephrectomy, including disease-free (DF), locoregional recurrence (LR), distant metastasis (DM), and death.

**Conclusion:** Post-nephrectomy RCC at later stages has higher disease management costs than at earlier stages. Adjuvant therapies that could help in maintaining patients in a DF state may decrease disease management costs among this patient population. Additionally, future studies evaluating the economic impact of new treatment should account for the observed time-varying pattern of disease management costs within a state.

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### Country Differences in the Clinical Manifestations and Treatment Patterns of Patients with Eosinophilic Granulomatosis with Polyangiitis (EGPA): A European Perspective

**Objectives:** EGPA is a rare chronic disease characterized by the combination of small/medium vessel vasculitis, hypereosinophilia and asthma. Published literature on the burden of EGPA in Europe is limited. Using a large European cohort of patients with EGPA, we describe regional differences in the EGPA-related clinical and disease burden.

**Conclusions:** Regional variations in clinical manifestations, remission, and treatment patterns suggest differences in treatment approach for patients with EGPA across Europe. Strategies to optimize EGPA management are needed.

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## POSTER SESSION 4

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Tuesday, November 8 | 3:00–6:15 p.m. | Presentation Time: 3:00–4:00 p.m.

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### Modelling Long-Term Clinical Outcomes of Patients with Transfusion-Dependent Beta-Thalassemia in the United States

**Objective:** Transfusion-dependent  $\beta$ -thalassemia (TDT) is a rare hereditary blood disorder in which patients suffer from chronic anemia and the effects of iron overload due to chronic blood transfusions despite chelation therapy, resulting in significant morbidity and early mortality. The impact of transfusion dependence on chronic complications and their subsequent impact on long-term clinical outcomes is not fully characterized. A model was developed to predict long-term clinical outcomes for patients with TDT treated with standard of care in the United States.

**Conclusion:** Model projections demonstrate that patients with TDT receiving standard of care have a high risk of complications and reduced life expectancy. Innovative therapies that can remove the need for blood transfusions could improve long-term clinical outcomes in this patient population.

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## POSTER SESSION 5

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Wednesday, November 9 | 9:00 a.m–12:30 p.m. | VIRTUAL

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### Real-World Outcomes and Health Care Resource Utilization of Onasemnogene Abeparvovec for US Patients With Spinal Muscular Atrophy Type 2: Results of a Retrospective Chart Review Study

**Objective:** Data on disease-modifying therapy (DMT) use for patients with spinal muscular atrophy type 2 (SMA2) are limited. We sought to describe real-world outcomes and health care resource utilization (HCRU) for patients in the United States with SMA2 aged  $\geq 6$  months at time of treatment with onasemnogene abeparvovec (OA) monotherapy or nusinersen switching to OA.

**Conclusion:** Patients with SMA2 improved/maintained function across multiple outcomes after receiving OA, with rapid onset of therapeutic effect. Patients also experienced reductions in the rate of inpatient admissions, with no admissions reported after receiving OA as monotherapy or after nusinersen.

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### Healthcare Utilization of Patients with Eosinophilic Granulomatosis with Polyangiitis (EGPA): A European Perspective

**Objectives:** EGPA is a rare chronic disease characterized by the combination of vasculitis, hypereosinophilia, and asthma. Manifestations can affect any organ system. Treatment relies heavily on systemic corticosteroids and immunosuppressants. Published literature on the burden of EGPA in Europe is limited. We described EGPA-related health care resource use (HCRU) using a large real-world cohort of patients in Europe with a confirmed diagnosis of EGPA.

**Conclusions:** The HCRU burden for patients with EGPA in Europe was considerable. Patients had frequent outpatient visits, and many required ER visits and hospitalizations. Regional variability suggests differences in EGPA-related health care delivery and disease control.