ISPOR Europe 2023

12 - 15 November

Poster Tour Guide Packet

Poster Session:	**Poster Session 4**
Tour Name:	**Rare Disease**
Tour Date/Time:	Tuesday, 14 November 2023, 15:30 - 16:15
Tour Location:	Area A, Poster and Exhibit Hall, Hall C

Acceptance Code:	PT37
Board Number:	1A
Abstract Title:	A Systematic Review of Modelling Approaches in Economic Evaluations of Treatments for Inherited Bleeding Disorders
Presenting Author:	Diaz Prameyllawati

Abstract Body:

OBJECTIVES: The aim of this review was to identify and assess published model-based economic evaluations of treatment for patients with inherited bleeding disorders (IBDs) to help inform the development of future models by adopting common approaches to enhance treatment comparison.

METHODS: A comprehensive search was performed on seven major databases from the database inception until the latest search date of 26 March 2023. Eligible studies had to report economic evaluations (e.g., cost-effectiveness or cost-utility analyses) using a decision-analytic model. The included models were grouped based on the type of IBDs, and the commonalities and differences in their modelling approaches were analyzed.

RESULTS: A total of 1644 articles were identified through the search, of which 48 articles fulfilled the predetermined eligibility criteria. All included studies focused on evaluating treatments for two types of IBD, namely hemophilia A and B. The most commonly adopted model was a Markov model (25/48), followed by a decision tree (17/48), microsimulation (3/48), and combination models (2/48), such as Markov decision tree and Markov microsimulation model. Irrespective of the model type, bleeding events such as joint bleeds were frequently selected as health states or disease pathways of the model (21/48). Half of the included studies (24/48) implicitly justified their approaches, with the adaptation of the prior models (12) and the author's perception of model suitability (9) being typical rationales for their selected model.

CONCLUSIONS: There are a considerable number of model-based economic evaluations for IBDs published. These evaluations predominantly focus on hemophilia patients, despite the existence of several other types of IBDs. Commonalities observed in modelling approaches indicate the potential to develop a standardized hemophilia model, which can shape future research. Further areas of investigation involve evaluating the transferability of hemophilia models to other IBDs and establishing a standardized model applicable to all IBDs.

Tour Guide's Questions for Starting Q&A (Each poster will have ~5 minutes for Q&A with attendees/Tour Guide)

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Acceptance Code:	PT38
Board Number:	2A
Abstract Title:	Budget Impact Analysis of rFVIII Glycopegylated-Exei for the Treatment of Haemophilia A in Colombia
Presenting Author:	Daniel Casas-Ramirez

Abstract Body:

OBJECTIVES: To calculate the budget impact of introducing rFVIII glycopegylated-exei for the management of previously treated patients with haemophilia A in the Colombian health system

METHODS: The model was developed using the best practice guidelines of ISPOR and populated using the Colombian institutional health market for the prophylactic treatment of haemophilia A. There are two scenarios of comparison: 1) versus standard half-life (SHL) and extended half-life (EHL) rFVIII available in the Colombian market (750 patients) and, 2) Only EHL rFVIII (84 patients). Both scenarios used local data from The National Administrative Department of Statistics (DANE) and epidemiological data from the public and official records of the High-Cost Account (CAC). Annual bleeding rates and dosage schemes were obtained from the Colombian regulatory agency. Prices were extracted from the Medicine Price Information System (SISMED) and expressed in US dollars (USD) for a 3-year horizon and the perspective was from the Colombian health system. Probabilistic and deterministic sensitivity analysis were implemented

RESULTS: For both scenarios, the inclusion of rFVIII glycopegylated-exei resulted in savings. For the first scenario (SHL and EHL), the savings resulted in USD 30,120, USD 57,208, and USD 183,217 for the first, second, and third year, respectively, and for the second scenario (Only EHL), the savings are USD 16,819, USD 138,638 and USD 233,120 for the first, second and third year. As an extra analysis, the number of additional patients that could be treated using the first scenario during the three years due to savings are 3.6 patients

CONCLUSIONS: The inclusion of rFVIII glycopegylated-exei in the Colombian health system can result in savings of up to USD 270,545 over a 3-year horizon used for the treatment of patients with haemophilia A in prophylaxis

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Acceptance Code:	PT40
Board Number:	3A
Abstract Title:	Epidemiology and Burden of Illness of Lupus in Children by Form of Lupus: A Retrospective Longitudinal National Hospital Claims Study in France
Presenting Author:	Nicoleta Petrica

Abstract Body:

OBJECTIVES: Lupus is an autoimmune disease that can affect children, with usually an onset during teen years. In children, symptoms are similar to those in adults, but can be more severe and with a greater frequency of renal manifestations. The objective of this study was to describe the characteristics and burden of lupus with child-onset in the hospital in France.

METHODS: A retrospective analysis was conducted using the French national claims hospitals database (PMSI). Eligible pediatric patients (<18 years at first hospitalization for lupus) were included at first hospitalization for lupus (ICD-10 for Systemic Lupus Erythematosus (SLE, ICD-10 M32.X) and/or Lupus Erythematosus (LE, ICD-10 L93.X)) between January 2018 and December 2021 and were followed until last hospitalization or up to December 2022. Burden of disease was evaluated with the number of hospitalizations and average length of stay (aLOS).

RESULTS: 595 patients were identified, including 484 (81.3%) with SLE and 111 (18.7%) with LE. At inclusion, mean age were 13.8 (± 3.3) years with 85.6% of female in SLE patients and 12.2 (± 4.5) years, with 79.3% of females in LE patients. SLE patients presented more severe profile with more comorbidities than LE patients (cardiovascular diseases 17.1% vs 9.9% ; kidney disorders 13.0% vs 9.9% ; metabolic diseases disorders 7.9% vs 3,6% and psychiatric disorder 6.6% vs 0%). Hospital burden was higher in SLE patients with an annual hospitalization rate of 6.4 (±10.3) per patient and an aLOS of 2.2 (±7.0) days vs annual hospitalization rate of 3.9 (±5.0) per patient and an aLOS of 1.9 (±9.2) days in LE patients.

CONCLUSIONS: This study confirms the severity of pediatric lupus, in particular for SLE form that requires early multidisciplinary disease management to minimize associated lifetime burden and morbidity.

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Acceptance Code:	PT41
Board Number:	4A
Abstract Title:	Real-World Evidence in Rare Diseases in France: Treatment Patterns, Healthcare Resource Use, and Related Costs in Von Willebrand Disease (FORvWARD Study)
Presenting Author:	Cinira Lefevre

Abstract Body:

OBJECTIVES: Von Willebrand disease (VWD) is a rare disease caused by missing/defective von Willebrand factor (VWF). VWD-patients have an increased bleeding risk and can receive replacement therapies (RT) as long-term prophylaxis (LTP-RT) and/or on-demand (OD-RT) as part of their disease management. FORvWARD is a national real-world study aiming to describe RT-treatment patterns, healthcare resource use (HRU) and related-costs in VWD-treated patients in France.

METHODS: Using a national healthcare claims database (SNDS), patients with ≥1 RT reimbursement between 2017/01/01 and 2021/09/30 were included and followed from 1st RT evidence until 2021/12/31, loss to follow-up, or death. Based on a previously published algorithm to distinguish LTP-RT and OD-RT (using RT frequencies, delivery types, and percentage of RT days covered), HRU/related-costs (VWD-related [e.g., RTs, VWD co-treatments, VWD-hospitalizations/specialist visits/procedures], and VWD-unrelated) were analyzed for LTP-RT and OD-RT users. Descriptive statistical analyses were conducted considering all RT exposure periods (EPs) within each LTP-RT/OD-RT population.

RESULTS: From 3,354 RT-users identified, 63 were LTP-RT patients, with 116 EPs (mean: 1.8 EPs/patient, incl. switches), mean follow-up of 4.4-y, mean age of 39.8-y, 58.7% females. In means/year per patient-period, LTP-RT patient-periods received 17.8 out-hospital RT-dispensings, 349,193 RT-IUs dispensed in- and out-hospital a RT-related cost of 347,953€. Other 3,291 patients were OD-RT users, with 4,908 EPs (mean: 1.6 EPs/patient), mean follow-up of 2.6-y, mean age of 44.1-y, 55.0% females. In means/30-days patient-period, OD-RT patient-periods had a length of hospital stay of 5.9-days, 11,810 RT-IUs dispensed in- and out-hospital, with RT-related costs of 11,701€.

CONCLUSIONS: This is the first large-scale real-world study on RT patterns of use, HRU/costs by type of RT-users in a VWD-treated population in France. These findings allow the quantification of specific disease burden and associated management costs related to each RT-user type. Future comparative analyses across RTs might identify potential treatment differences.

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Acceptance Code:	PT42
Board Number:	5A
Abstract Title:	The Impact of Spinal Muscular Atrophy Type 2 on Caregivers in Argentina: Results of a Global Survey
Presenting Author:	Anish Patel

Abstract Body:

OBJECTIVES: Spinal muscular atrophy type 2 (SMA2) is an intermediate form of spinal muscular atrophy, characterized by progressive muscle weakness and atrophy. Evidence on the impact of disease management and support on caregivers of patients with SMA2 is limited, and therefore we sought to describe health care resource use, time costs, and out-of-pocket expenditures for the families/caregivers of these patients in Argentina.

METHODS: An online survey was disseminated through patient advocacy group FAME from February 2022 through May 2022. Eligible respondents were voluntary non–health care provider (HCP) caregivers.

RESULTS: Forty-nine caregivers (mean age, 43.6 years) managing 50 total patients with SMA2 participated in the survey. In 30/50 cases (60%), the patient's mother was the primary caregiver. Mean patient care time was 79.7 hours/week. Of the 50 cases, 8 (16%) had a caregiver who stopped working to provide care and 17 (34%) had a caregiver who reduced their working hours (mean reduction, 13.8 hours/week). For caregivers experiencing income changes, the average income reduction per month was 42.2%. Twenty-five patients had caregivers take days off from work for caregiving in the last 6 months (mean, 22 days). Caregivers experienced out-of-pocket expenditures, including home adaptations (mean, 356,198.50 Argentine pesos). In addition, most caregivers consulted at least one HCP for their patient(s) and 11 patients (22.0%) had at least one overnight hospitalization, excluding SMA-related surgeries, in the last 6 months.

CONCLUSIONS: Caregivers of patients with SMA2 in Argentina reported a substantial impact on their time, employment status, and out-of-pocket costs, affecting their own well-being. Early identification and treatment of SMA2 may reduce associated costs and resulting impact on caregivers and patients.

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