

## PP131 European Market Access Landscape Analysis Of Reimbursement Drivers In Pompe Disease: Results From 26 Payer Interviews

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**Introduction.** Pompe disease (PD) is a rare, progressive neuromuscular disease that severely affects motor and respiratory functions. Late onset PD (LOPD) is the most common phenotype. Current treatment involves enzyme replacement therapy (ERT) with alglucosidase alfa, which was first approved in 2006. In Europe, the treatment landscape is changing as two new ERTs have been filed for regulatory approval: avalglucosidase alfa and cipaglucosidase alfa plus miglustat. We analyzed how health technology assessment (HTA) and reimbursement criteria may be applied to ERTs in key countries for patients with PD.

**Methods.** Eighteen different factors were identified from the pivotal trials (LOTS, COMET, and PROPEL) for the three recombinant enzymes. These covered the categories of trial design, endpoints, quality of life, and other product characteristics. Twenty-six HTA experts and health economists from Denmark, England, France, Germany, Italy, the Netherlands, Spain, and Sweden with rare disease experience were interviewed during the period from July to September 2021. In structured discussions, each participant was asked to rate (from one to seven) the factors in terms of their importance and impact on the HTA evaluation and reimbursement of treatments for adults with PD.

**Results.** The following factors were highly rated: a well-defined PD trial population; use of an active trial comparator; efficacy in both treatment naïve and experienced subpopulations; a superiority study design; and payer-relevant endpoints and quality of life improvements. The five lowest rated factors were open-label data, biomarkers, innovation, ease of administration, and mode of action. While the results were mostly consistent across countries, the HTA expert viewpoints varied depending on the country. For example, HTA experts in Italy, the Netherlands, and Sweden rated innovation and biomarkers more highly than German experts.

**Conclusions.** As new ERTs become licensed, achieving reimbursement and successful HTA of them will require a clear exposition of payer-relevant evidence for the LOPD population in the target country, including comparative randomized controlled trial data, benefits for experienced and treatment naïve subgroups, and payer-relevant endpoints and quality of life gains.

## PP132 Disinvestment Initiatives In Health Care: A Scoping Review Of Systematic Reviews

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**Introduction.** Disinvestment of ineffective, low value technologies is growing as a priority for international health policy in order to improve quality and maximize value in health care. Different strategies have been implemented at the international and national level using various methods of evidence gathering and technical assessment. However, the success of these initiatives is mixed, with fewer than half of the empirical studies reporting reductions in the use of low value services.

This review explored the role of stakeholders in the disinvestment process by describing the initiatives and analyzing the methods used for reassessment. We also identified the facilitators and barriers related to disinvestment implementation.

**Methods.** This scoping review was guided by the JBI Manual for Evidence Synthesis and the PRISMA statement for scoping reviews. Strategic literature searches were performed to identify published reviews on disinvestment in health care using the MEDLINE, Web of Science, and Scopus databases. Data were extracted using a predesigned form and then synthesized narratively to identify similarities and differences across the approaches according to prespecified domains.

**Results.** Sixteen reviews were included. We identified various disinvestment initiatives across 16 countries, with a minimum of 34 initiatives at different levels of implementation and with various agencies responsible for the activities. Two of the most used methods for facilitating disinvestment decisions were program budgeting and marginal analysis (PBMA) and health technology assessment (HTA). Stakeholder involvement was the most important aspect to be addressed since it acts as both a facilitator and a barrier in implementing disinvestment initiatives. Meaningful engagement may be strengthened with continuous stakeholder participation, transparency in methods and processes, and ongoing knowledge transfer.

**Conclusions.** This scoping review highlights the role of stakeholder involvement in disinvestment, which is a double-edged sword because it both facilitates and hinders disinvestment implementation. The most common methods for assessing candidates for disinvestment are PBMA and HTA, but there is a lack of clarity on which HTA dimension is suitable for a disinvestment process.

## PP134 Health Technology Assessment Life Cycle Approach In Asthma Care

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**Introduction.** Within Dutch health care, Zorginstituut Nederland regularly selects topics for reassessment of diagnostic and therapeutic interventions. Until now, about 15 topics have been investigated, such as hip/knee arthrosis, stable angina, low back pain, urinary incontinence, palliative care, retinopathy and asthma.

**Methods.** For each topic, stakeholders were asked to report what could be improved. Also a working group was created to analyze: what do guidelines recommend, what does actual care look like seen from administrative data, is there a gap between guidelines and delivered care, what can be improved? A report with recommendations was written with a calculation of the impact. Agreements were made on implementation. This report focused on findings relating to asthma.

**Results.** We identified the multiple issues in the management of asthma: (i) Only 11 percent of patients had their diagnosis confirmed (with spirometry and reversibility or visit to pulmonologist). (ii) 60,000 patients had intensive short acting broncho-adrenergic agents (SABA) without inhaled corticosteroids (ICS) representing overuse of SABA. (iii) 200,000 patients use inhalers that can be empty, without the patient knowing this, as there is no indicator showing the number of actuation/puffs left, which leads to under treatment. (iv) 60,000 patients have aerosol and powder inhalers together, each requiring a different technique, leading to mistakes. (v) 67 percent of asthma adults get a chest x-ray on referral to hospital, which is a high proportion. (vi) 49 percent of patients use inhalers with propellant, which is needlessly high, given their undesirable impact on climate change. This causes 36 million kg co<sub>2</sub> equivalent, the same as 36000 extra gasoline cars. (vii) Only 37 percent of patients receive yearly monitoring. We calculated that EUR 14 million annually can be saved as a result of better diagnosis leading to less overdiagnosis and overtreatment and less spacers.

**Conclusions.** Despite that 'HTA (Health Technology Assessment) at the doorstep' has been applied for asthma interventions, we noticed considerable room for improvement. We consider this method important for real HTA life cycle approach.

## PP140 Economic Evaluation Of Several Vaccination Strategies Against Rotavirus In Spain

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**Introduction.** The Spanish Ministry of Health asked us about the efficiency of extending the current rotavirus vaccination strategy to all newborns. The current strategy is to vaccinate only to high-risk newborns (premature and those qualified as high-risk by a pediatrician). The objective of this research was to compare three strategies: no-vaccination, vaccination of high-risk newborns and universal vaccination, considering the two vaccines available in Spain: RotaTeq® and Rotarix®.

**Methods.** A cost-utility analysis, based on a de novo Markov model, was carried out both from a societal and a healthcare system perspective. The model follows a cohort of newborns during their life-course. The cycle length is annual and a half-cycle correction was applied. A discount rate of 3 percent was applied in the base case both to costs and utilities. Most of the incidence, probabilities and costs data were Spanish. The Quality Adjusted Life Year (QALY) data were taken from international literature. We assumed a willingness to pay threshold of EUR 25,000 per QALY gained. We performed deterministic one-way sensitivity analysis.

**Results.** Compared to no-vaccination, the high-risk vaccination strategy is cost-effective assuming the above-mentioned threshold only with Rotarix® from a societal perspective (RotaTeq® EUR 32,008 per QALY; Rotarix® EUR 23,368 per QALY). Universal vaccination is not cost-effective either compared to no-vaccination or compared to the high-risk vaccination strategy and with both perspectives. Vaccine prices and efficacy data are highly sensitive variables. We find that universal vaccination would be cost-effective with a discount of 44.6 and 36.9 percent of the current price of RotaTeq® and Rotarix®, respectively.

**Conclusions.** Universal vaccination would not be a cost-effective strategy for Spain with either of the two vaccines at current prices. Vaccination of high-risk newborns would be cost-effective at current prices and from a societal perspective only with Rotarix®. Substantial vaccines price reductions could make the universal vaccination a cost-effective option in Spain.

## PP142 Time-Driven Activity-Based Costing (TDABC) Of Brazilian Public Healthcare System (SUS): Preliminary Results For Osteogenesis Imperfecta (OI)

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**Introduction.** Improving the value of healthcare delivered requires accurate cost information, which can be challenging for rare diseases. We report direct costs of patients with OI using the TDABC methodology.

**Methods.** This research is part of a nationwide observational study to assess the "Value of Healthcare Journey for Patients With Rare Diseases" in SUS. Patient journey and economic data was collected for the actual clinical practice in each medical center enrolled in the project. We set the starting point of disease and map a patient's path in the healthcare journey, including treatment, exams, procedures and appointments needed. Unit cost, time and amount of resources to perform each activity, such as human resources, materials and medicines, was assessed, disregarding indirect costs. Here we present