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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