Methods: All enzyme replacement therapies for rare diseases evaluated by the National Committee for Health Technology Incorporation in the Brazilian Public Health System (Conitec) and with at least one year of use were included. For each technology, the following were identified: number of patients, median patient weight, annual quantity of medication, unit price, and budget impact. The attributes were compared between previous estimates and real-world observation after use. The data sources were publicly accessible administrative databases and Conitec technical reports.
Results: Five technologies were selected: elosulfase alfa, alglucosidase alfa, idursulfase, laronidase, and galsulfase. In the first year, the difference between the estimated and the observed number of patients treated was up to 15 percent lower or higher for four technologies, but with monthly fluctuation throughout the year. The median weight of users was between 23 percent and 468 percent higher for three technologies. The observed price was as expected, with variations between three percent lower and 14 percent higher. The quantity of medicines used was lower (between 39% and 46%) than expected for all technologies. The observed budget impact was 37 percent to 47 percent lower than estimated.
Conclusions: Real-world budget impact was lower than expected for all technologies. The main cause of discrepancies was the estimate of the annual amount of medication, which did not consider gradual adherence and discontinuation of treatment. This highlights the need to review the budget impact methodology for rare diseases, forecasting monthly market share and treatment discontinuation rate.

OP32 Identification Of Factors Alongside Costs And Effectiveness For The Technology Assessment Of Comprehensive Genomic Profiling: A Systematic Review

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Introduction: Comprehensive genomic profiling (CGP) identifies many targets at once. However, it is challenging for reimbursement decision-makers to incorporate all potential effects in their assessment. The aim of this study is twofold: first, to identify which factors, besides effectiveness and costs, might influence the choice for CGP in advanced cancer patients, and second, to identify the available evidence for these factors.
Methods: We performed a systematic literature review in MEDLINE, Embase, and Scopus with a two-step design. First, a scoping search was performed to identify relevant factors. Extracted factors were grouped with domains of the EUnetHTA core model and ISPOR (Professional Society for Health Economics and Outcomes Research) "value flower." Two expert sessions were held to validate factors and construct definitions. Second, a systematic search was conducted to identify the available empirical evidence for these factors. Eligibility criteria for the systematic search were the use of CGP (≥200 genes), advanced cancer patients, and the presentation of empirical evidence on one of the factors.
Results: Five factors were identified in the scoping search: "feasibility" (adopting tests in the health care system), "test journey" (pathway from requesting tests until reporting of results), "wider implications of diagnostic results" (impact of test beyond identifying on-label treatments), "organization of laboratories" (organization of tests and access to tests), and "scientific spillover" (learnings of testing). Eighty-three articles were included following the systematic search, and empirical evidence was identified for the factors "test journey" and "wider implications of diagnostic results". Few studies had adequate comparative study designs. Heterogeneity was observed among studies in the definitions of outcomes and the reported evidence.
Conclusions: Comprehensive reimbursement decision-making for CGP can be supported by including the five identified factors. However, quantifiable evidence was only identified for the "patient test journey" and "wider implications of diagnostic results". Current literature provides limited high-quality evidence to establish the added benefit of CGP, as adequately designed comparisons are lacking. For evidence-based decision-making, uniform outcome measurements are recommended.

OP33 Advancing Patient Experience Data Implementation In Reimbursement Decision-Making: Insights On Challenges And Opportunities From Multistakeholder Interviews

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Introduction: Patient experience data (PED), encompassing patient preferences (PP), patient-reported outcomes (PROs), and patient input, play a pivotal role in understanding patient needs and informing healthcare decision-making, including reimbursement decisions. This study aimed to assess the current barriers hindering the integration of PED into practice and its particular challenges, opportunities, and concrete policy actions for the systematic implementation of PED.
Methods: Semistructured interviews (n=38) were conducted with industry (n=12), non-profit organizations and academia (n=4), regulatory authorities (n=6), health technology assessment (HTA) bodies and reimbursement agencies (n=6), and patient organizations (n=10) in Europe. A thematic analysis was conducted to explore stakeholders' perspectives and to gain a comprehensive understanding of challenges and opportunities related to the systematic implementation of PED. Interview transcripts were analyzed using the thematic

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