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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framework analysis to extract and elucidate the insights provided by the diverse stakeholders.
Results: HTA and reimbursement interviewees agreed on the value of including quality-of-life data, particularly when assessed using validated PRO measures. Despite acknowledging the potential of PP, there remained reluctance to integrate PP into reimbursement decision-making. Participants expressed divergent opinions regarding who should collect PED, with some regulators favoring industry, while HTA and reimbursement agencies emphasized transparency and independent PED collection. Limited experience in assessing PED also contributed to hesitancy, underscoring the need for more guidelines, especially at the national reimbursement level. Stakeholders endorsed collaboration through joint scientific consultations, expressing optimism about the impact of the Regulation (EU) 2021/2282 on health technology assessment (HTAR).
Conclusions: This study emphasizes the high potential of PED in informing reimbursement decision-making, fostering a more patient-centered approach. Stakeholder disparities highlight the complexity, necessitating more guidance, scientific robustness, transparency, and collaboration. In light of these stakeholder considerations, the upcoming HTAR holds promise to enhance the systematic implementation of PED, aligning healthcare decision-making with patients' needs and preferences.

OP34 Implications Of The New EU Regulation For Orphan Drug Health Technology Assessments In Ireland

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Introduction: Time to reimbursement has been described as a hurdle to availability of new medicines to European patients, with assessment and decision-making processes frequently quoted as taking the majority of time. In light of the upcoming Regulation (EU) 2021/2282 on health technology assessment (HTAR), the aim was to examine timelines and health technology assessment (HTA) recommendations for orphan drugs in Ireland.
Methods: The study reviewed all orphan drug submissions to the National Centre for Pharmacoeconomics (NCPE) from January 2020 to December 2023 inclusive. The number of days from marketing authorization to rapid review (RR) commissioning was calculated. The RR and HTA recommendations were identified for all medicines. The timelines for the RR and HTA process were evaluated.
Results: Of the 66 submissions identified, 38 percent were made prior to marketing authorization (MA), eight percent were made within 30 days of MA, and 79 percent were made 30 days post MA. RRs were completed within 32 days (mean). Full HTA was recommended in 62 percent (n=41). Price negotiations were recommended in 38 percent (50% of which have been reimbursed to date). Where a full HTA was recommended (n=41), 20 have been completed to date (price negotiations were recommended in 90%). For those 20 HTAs completed, 11 have been reimbursed to date; a decision is pending for the remainder. HTAs were completed within 200 days (mean).
Conclusions: Data shows that the majority of submissions were made 30 days post MA. A pragmatic approach may have to be taken nationally to accommodate the HTAR post 2024 and those submissions that are made prior to publication of a joint clinical assessment. The majority of orphan drug HTA recommendations lead to reimbursement recommendations.

OP35 Early Access To Medicinal Products In France: A Positive Evaluation Two Years Into The New Framework

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Introduction: Introduced in July 2021, early access to pharmaceuticals allows French patients to have rapid and reimbursed access to presumed innovative medicines prior to marketing authorization or regular reimbursement registration. The French National Authority for Health (Haute Autorité de Santé [HAS]) is responsible for granting early access authorizations and for health technology assessment upstream of regular reimbursement. This analysis evaluates the outcomes of the 2021 reform.
Methods: In collaboration with the national regulatory body (ANSM) and the Ministry of Health, a descriptive and retrospective analysis has been conducted to assess the impact of this reform. The data were extracted from published HAS decisions and from the HAS internal information system (EVAMED). The period considered is the first two years of the reform, from 1 July 2021 to 30 June 2023. Different information has been analyzed, such as conclusions of decisions returned, therapeutic areas concerned, and decision processing time. An additional analysis focused on HAS opinions on regular reimbursement for the medicines for which early access was granted.
Results: In two years, over 250 applications (including first applications, modifications, and renewals) have been submitted, increasing over time, and 180 decisions were issued by HAS within this period. Eighty percent of HAS decisions were positive for early access. Among the medicines for which early access was granted, 86 were also assessed for regular reimbursement, of which 78 percent obtained a positive clinical added value score (at least ASMR IV). Overall, the early access program is estimated to have facilitated coverage approximately nine months before regular reimbursement and benefited over 100,000 patients.
Conclusions: Manufacturers make extensive use of early access applications. A positive assessment of the reform outcomes emerges from this analysis, emphasizing the role of HAS in accelerating patient access to presumed innovative treatments. In light of US experience and of the uncertainties surrounding fast-track authorization procedures, monitoring of the French early access system will continue.

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