technology assessment (HTA) methods in addition to traditional HTA domains. In 2018, the Finnish Ministry of Social Affairs and Health recognized the need for new HTA methods for DHTs in Finland and commissioned method development.
Methods: The development work of the new HTA method for DHTs and the findings related to it were based on three substudies:
(i) The new HTA method was developed through a literature review, expert interviews, and four multiprofessional workshops.
(ii) Feedback about new HTA recommendations was collected from healthcare decision-makers through a web-based survey (n=24). Feedback on the developed HTA framework was collected through a web-based survey for companies offering DHT products (n=8).
(iii) Initial experiences about the state of data security and protection of assessed products were gathered through the assessment process.
Results: A new Digi-HTA method that supports a wide range of DHTs, such as health apps, AgeTech, artificial intelligence, and robotic solutions, was published in 2019. According to the healthcare decision-makers participating in the study, although the Digi-HTA recommendations included clear and beneficial information, their integration into healthcare decision-making processes should be improved. Responses from companies offering different DHTs indicated that the Digi-HTA framework would be an appropriate tool for performing assessments for their products. During the assessments, deficiencies in compliance with the best practices of data security and protection as well as data security problems were found.
Conclusions: The rapid development of DHTs requires that the HTA methods also adapt to the development so that no new and innovative products are excluded from the assessments. In addition to the value of DHTs, their quality, such as data security and protection, should be assessed so that decision-makers can be supported in the best possible ways.

OP29 Determinants Of The Financial Impact Of Rare Disease Drugs In Italy: Differences Between Expected And Observed Pharmaceutical Expenditure

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Introduction: In Italy, a fixed proportion of health spending is allocated to pharmaceutical expenditure. While the main objective of setting a budget for pharmaceuticals is to control spending, the effectiveness of this ceiling is questionable. This study aims to investigate the determinants of pharmaceutical expenditure for orphan drugs and gather information for effective planning and programming of pharmaceutical spending.
Methods: Data analysis relied on pharmaceutical companies' pricing and reimbursement (P&R) dossiers submitted to the Italian Medicines Agency (AIFA) for drug-reimbursement approval, along with AIFA's internal procedural documents. The study encompassed all rare disease drugs reimbursed from January 2013 to January 2019. For each drug, a comparison was made between the expected post-negotiation expenditure and the actual spending observed over the three years following reimbursement approval. Potential determinants of the normalized ratio between observed and expected spending were identified using univariate and multivariate beta regression models. The same methodology was replicated to identify potential determinants of the difference between expected spending before and after negotiation.
Results: Fifty-two rare disease drugs admitted for reimbursement during the study period were analyzed. The median expenditure in the first three commercialization years was 7.6 percent lower than the expected post-negotiation spending. Beta regression analysis indicated a significantly lower reduction for innovative drugs (β 0.736, p-value 0.011 univariate, β 0.585, p-value 0.045 multivariate). Similar effects were observed for P&R procedures (β 0.902, p-value 0.007) and the number of indications presented (β 0.754, p-value 0.021), but only in univariate model. Beta regression analysis for the expected expenditure ratio before/after negotiation revealed a significant effect only for the payment-by-result variable (β 1.485, p-value 0.001).
Conclusions: Observed expenditure for orphan drugs aligns with the expected spending post-negotiation. However, in the subgroup of innovative orphan drugs, the observed pharmaceutical spending was higher than estimated. This could be attributed to prescriber preferences and to a prevalent patient pool awaiting innovative treatment. It appears that the recognition of innovativeness favors orphan drugs that are rewarded with faster market access.

OP31 Monitoring Of The Budget Impact Determinants Of Incorporated Technologies For Rare Diseases In The Brazilian Health System

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Introduction: Budget impact analyses for the treatment of rare diseases are especially important for the sustainability of health systems due to high treatment costs and uncertainties in target population estimates. The objective of this work is to analyze the elements that influence discrepancies between predicted and observed budget impacts for enzyme replacement therapies for rare diseases in Brazil's public health system.

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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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