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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