PP26 Systematic Literature Review Of Funding Models For The Access To Drugs For Patients With Rare Diseases

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Introduction: There is no comprehensive framework that considers the various features of current funding models on drug accessibility for rare diseases; such a framework would assist policymakers to more effectively meet the challenges of these patients. This article reviews the funding models implemented worldwide to facilitate this access.
Methods: The PRISMA guidelines were used to conduct a systematic literature review. The following databases were searched: Ovid (Embase/MEDLINE), Cochrane database, Web of Science, EconLit, the National Institute for Health and Care Research (NIHR), Centre for Review and Dissemination (CRD), and International Network of Agencies for Health Technology Assessment (INAHTA). Two independent reviewers screened all titles and abstracts, and one reviewer did the full-text review and data extraction. Data were collected on general study characteristics, general aspects of rare diseases, source of funding, allocation of resources, and pricing strategies.
Results: A total of 3,815 unique citations were screened, and 148 were included for data extraction. Each funding model was characterized based on its unique features specific to rare diseases, focusing on process, methods applied, and consideration of attributes. Sixty funding models were identified in 41 countries, categorized as separate processes (42%), exceptions to standard processes (32%), standard processes with no changes (23%), and alternative pathways (3%). More than one funding model was available for 29 percent of countries. Funding models varied in their approach to HTA, source of funding, consideration of uncertainty, and pricing strategies.
Conclusions: The diversity of funding models highlights the complexity of addressing access to treatments for rare diseases. Special considerations towards rare diseases generally targeted the greater uncertainty in the clinical evidence. Despite the existing platform that enables access for drugs for rare diseases, only 10 percent of rare diseases have an available treatment and fewer patients can access these technologies.

PP27 Evidence Quality and HTA outcomes in Reappraisals For Drugs For Rare Diseases In Germany

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Introduction: Evidence on re-appraisals of health technologies in Germany is limited, and for rare disease treatments (RDTs) the Federal Joint Committee (GBA) uses different processes depending on whether the annual revenue threshold has been exceeded. We analyze RDTs for which an initial appraisal and a reappraisal were conducted to better understand processes used to determine the clinical benefit rating and their outcomes.
Methods: We review appraisal documents for 55 RDT indications published between January 2011 and September 2023. We extract information for the change in the maturity of survival data, the type of evidence, the risk of bias, and the availability of additional evidence as proxies for evidence quality. Specifically, we review the reasons for conducting reappraisals; examine how evidence quality and the clinical benefit rating differed between initial appraisals and re-appraisals; and explore the association between evidence quality and the clinical benefit rating following reappraisal.
Results: Most reappraisals were conducted because of exceeding the revenue threshold of EUR50 million (USD54 million) per year or reaching the review date when an initial decision was time limited. Almost all initial appraisals used the limited process, while the majority of reappraisals used the regular process. While nine out of 55 reappraisals achieved a higher benefit rating in reappraisals compared to initial appraisals, in 21 out of 55 reappraisals the benefit rating decreased. There was some evidence that reappraisals with accepted randomized controlled trial evidence were significantly more likely to achieve a higher clinical benefit rating.
Conclusions: Our findings confirm that reasons and processes for completing reappraisals of RDTs in Germany differ. Moreover, the quality of the evidence submitted for both initial appraisals and reappraisals of RDTs was limited and achieving a high clinical benefit rating in reappraisals was rare.

PP28 The Final Arguments To Incorporate Or Not Incorporate Technologies For Ultra-Rare Diseases Into The Brazilian Public Healthcare System

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Introduction: The decision-making process for health technology assessment (HTA) in ultra-rare diseases is a global challenge.

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