PP38 Assessment Outcome By Type Of Available Evidence: Retrospective Analysis Of Oncological Early Benefit Assessment Reports

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Introduction: For early benefit assessment in Germany, drugs must be assessed at market entry as to whether they provide an added benefit versus the current standard therapy. This retrospective analysis focuses on assessment reports prepared by the independent Institute for Quality and Efficiency in Health Care (IQWiG). The objective was to describe the relationship between available evidence and assessment outcome.
Methods: Data were retrospectively extracted from IQWiG's early benefit assessment reports of oncological drugs that entered the market between June 2021 and December 2021. The underlying evidence for the benefit assessments was divided into three groups: direct comparison (based on a randomized controlled trial [RCT]), indirect comparison, and other sources (e. g., only a single-arm trial was submitted). Afterwards, the outcome of the assessment was described by type of available evidence.
Results: In the period under review, IQWiG worked on 27 oncology projects addressing 40 different oncological research questions resulting in 46 conclusions. About half of the available evidence in the benefit assessments was a direct comparison with standard therapy based on an RCT (n=22/46). From this level of evidence, an added benefit was derived for the drug under assessment in 64 percent of cases. Indirect comparisons (n=7/46) mostly led to an added benefit not proven (86%). If only other sources were submitted (n=17/46), the added benefit was assessed as not proven in all projects examined.
Conclusions: This retrospective analysis highlights the pivotal role of RCTs in providing strong evidence for health technology assessment. Conversely, single-arm and observational studies fail to yield substantial evidence. In the future, RCTs should be made simpler, faster, and cheaper; for example, by integrating them into existing infrastructures in routine practice (e.g., registry-based RCTs) or by conducting randomized adaptive platform trials.

PP39 Co-Creation Of A European Digital Health Technology Assessment Framework: The EDiHTA EU-Funded Project

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Introduction: The widespread adoption of digital health technologies (DHTs) brings new methodological challenges to health technology assessment (HTA). Different tools, guidelines, and methods have been defined by different stakeholders at national/international levels. In this scenario, the real thread for HTA is the lack of coordination and harmonization in the efforts to finalize the definition of an HTA framework for DHTs.
Methods: The European Digital Health Technology Assessment (EDiHTA) project aims to provide the first framework co-created by all stakeholders along the value chain for DHTs. The consortium is comprised of 15 partners from nine countries (Italy, the Netherlands, Denmark, Spain, Germany, Norway, UK, Belgium, and Poland). Stakeholders included are HTA agencies, regulators, hospitals, patient/citizen representatives, academics/universities, notified bodies, and developers. The project covers all types of DHTs and Technology Readiness Levels (TRLs). A multistakeholder, multidomain, and modular approach is adopted with the final goal to provide a validated, ready-to-use digital HTA framework for DHTs for the European ecosystem.
Results: The EDiHTA is articulated in eight working packages and covers four years. Starting from the mapping of the current scenario, after the definition of a common taxonomy, EDiHTA will develop a DHT-dedicated HTA framework. A common digital platform to support the assessment will be developed. Both the HTA framework and platform will be tested, refined, and validated through a piloting process involving disparate DHTs (mApp, telemedicine, artificial intelligence, and others) with different TRLs, available in different countries, with the participation of all stakeholders. As a consequence, evidence requirements will be defined and made more transparent; this will support health technology.
Conclusions: The EDiHTA project will deliver the first European digital HTA framework able to support and inform decision-making (at the macro [policy], meso [management providers], and micro [clinicians] level) towards faster and safer access to DHTs of added value, taking European regulations and legal requirements into account and assessing all HTA domains.

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