OP36 Real-World Data From Early Access Programs In France: A Two-Year Review

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Introduction: The 2021 reform of early access (EA) for medicinal products in France includes a mandatory data collection for each patient. The objective of this work was to perform a two-year review of the protocols for therapeutic use and data collection (PUT-RD) validated by the health technology assessment body (Haute Autorité de Santé [HAS]) in collaboration with the national regulatory body where applicable.
Methods: The minimum dataset defined in the PUT-RD varies depending on the regulatory status of the product and may include patients' characteristics, conditions of use, efficacy, and safety data. A descriptive review of the PUT-RDs approved between 1 July 2021 and 30 June 2023 was carried out by HAS. The following information was retrieved in each published PUT-RD: type of EA (pre- or post-marketing authorization); collection of efficacy data (yes or no) (if yes, inclusion of a patient-reported outcome measure [PROM] or no); type of data source (registry or electronic platform provided by a contract research organization [CRO]).
Results: During the review period, a total of 98 PUT-RDs were validated, corresponding to 98 EA favorable decisions. EAs were authorized prior to marketing authorization in 40 percent of the cases. A collection of follow-up efficacy data was planned in 52 PUT-RDs, including integration of patients' perspectives through a PROM in 32 PUT-RDs. Data collection through a registry was planned in six PUT-RDs (using the DESCAR-T registry), otherwise data collection was mainly planned through private electronic platforms operated by CROs (80% of the PUT-RDs).
Conclusions: To optimize the generation of relevant clinical data to be used in the regular HTA process, EAs should be requested early, prior to marketing authorization. Additionally, the use of existing data sources, in particular disease registries, is to be developed to avoid duplicate data collection and alleviate the burden for healthcare professionals.

OP37 The Spanish Health Technology Assessment Network Methodological Approach For The Utilization Of Real-World Data In The Preadoption Phase

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Introduction: Preadoption assessments are the most frequent type of evaluation conducted by the Spanish Network of Agencies for Health Technology Assessment and Services of the National Health System (RedETS). RedETS aimed to develop a framework to utilize real-world data (RWD) to better adjust its assessments to the Spanish population's context and provide live assessments throughout the lifecycle of health technologies.
Methods: A working group within RedETS was set up, which held several meetings to define the primary uses of RWD in the short term. Next, a manual review was conducted on national and international initiatives that provided guidance on the use of RWD in HTA. Common pathways for utilizing RWD in HTA were identified. The working group agreed to outline and explain the key overarching steps and provide general guidelines for working with RWD, developing as illustration a use case for an interventional technology. The Big Data project of Aragon (BIGAN) was chosen as the data source for the use case.
Results: We formulated a case for leveraging RWD in the assessment of implantable cardiac defibrillators (ICDs) for the prevention of sudden cardiac death (SCD). Based on this scenario, we developed a methodological framework outlining a workflow consistent with RedETS practices. RWD complemented the usual process of systematic review of a technology. Crucial steps comprised the definition of data requirements through a data model specification, an exploratory data analysis, and the construction of a decision model. We presented solutions for dealing with unavailable data on essential variables and unstructured records. We discussed the main limitations to account for when working with RWD.
Conclusions: The task ahead holds great hope but requires overcoming some challenges to fully deploy RWD-driven methods. This entails fostering collaboration with health authorities and designated data holders to address data access challenges. In the short term, it is essential to include data scientists in assessment teams and provide appropriate capacity-building to encompass RWD tools and modeling techniques.

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