influence diversity and data integrity. We anticipate that the PDS will enable HTA decisions to reflect patients' health value and ultimately improve access to innovative therapies.

OP72 Patient Organization Submissions Made To A National Health Technology Assessment Agency In Ireland

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Introduction: The National Centre for Pharmacoeconomics (NCPE) has a Patient Organisation Submission Process (POSP) enabling patients to communicate their experiences to the drug-reimbursement decision-maker. The NCPE proactively invites and supports patient organizations to make submissions to the decision-maker for ongoing health technology assessments (HTAs). We evaluate uptake of the POSP and determine whether submission trends differ by drug type.
Methods: We reviewed all HTAs completed by the NCPE since 2016 (when the POSP was first introduced) to present (data cut-off date 22 November 2023). Time trends in the proportions of HTAs for which a patient organization submission had been made to the NCPE were analyzed descriptively. We also compared the proportions of HTAs for which a patient organization submission had been made for (i) orphan versus non-orphan drugs and (ii) oncology versus non-oncology drugs.
Results: The number of patient organization submissions made to the NCPE has increased over time. In 2016, 24 percent (6/25) of completed HTAs were associated with a patient organization submission compared with 50 percent (11/22) in 2023. The proportions of completed HTAs associated with a patient organization submission are comparable between orphan (38%; 24/64) and non-orphan drugs (29%; 34/117) (Chi^2; p=0.245). The proportion of completed HTAs for which a patient organization submission had been made was lower for oncology drugs (14%; 14/97) versus non-oncology drugs (52%; 44/84) (Chi^2; p<0.001).
Conclusions: Patient organization submissions to the NCPE have increased over time. The proportions of HTAs for which patient organization submissions have been made are comparable between orphan and non-orphan drugs. However, the proportion of patient organization submissions for oncology drugs is lower than the proportion for non-oncology drugs. The NCPE will continue to liaise with patient organizations to increase engagement with the POSP.

OP73 Navigating The Advanced Therapy Medicinal Products (ATMPs) Conundrum: Insights From ATMPs Withdrawn In The European Market

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Introduction: Advanced therapy medicinal product (ATMP) is a classification used by the European Medicines Agency (EMA) for cell-, gene-, and tissue-engineered therapies. Since the first ATMP received market authorization in 2009, a substantial proportion of these highly innovative therapies have been withdrawn from the European market. This research investigates the key reasons underlying these withdrawals.
Methods: ATMPs with withdrawn EMA marketing authorizations were identified from the EMA website. A targeted review of relevant company press releases was undertaken and key information extracted. Health technology assessment (HTA) outcomes in France, Germany, and England were extracted from their respective websites (1 Sep 2023).
Results: Thirty-two ATMPs have received EMA marketing authorizations, with 22 percent (7/32) withdrawn. One of these seven withdrawals was driven by unfavorable clinical results versus six out of seven due to commercial reasons. Of the six withdrawals driven by commercial reasons, four were associated with negative HTA and/or reimbursement issues (Chondro-Celect, Glybera, and Zalmoxis's Service Médical Rendu (SMR, actual medical benefit) insufficient, driven by data-related issues in France; Glybera's challenge to obtain reimbursement by insurance funds with only one sale reported, and Zynteglo's price agreement not reached in Germany). One of the six withdrawals (Chondro-Celect) was also associated with hospital exemption (continued production without marketing authorization). One of six (Provenge) was also associated with chemistry-, manufacturing-, and controls (CMC)-related issues.
Conclusions: The ATMP landscape has rapidly evolved in the past decade. While ATMPs offer the promise of long-term benefits, the unique manufacturing, clinical, and especially the reimbursement challenges they face can lead to commercial failure. To ensure ATMP patient access and commercial success, manufacturers should engage early with payers, understand potential reimbursement challenges, and proactively plan to mitigate these.

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