LRs, with no additional information. IQWiG, in its general methods, recommends the use of ML-validated classifiers for identifying randomized controlled trials (RCTs) within bibliographic searches.
Conclusions: Our research indicates that there is scarce guidance available for the use of AI in LRs for HTA submissions. However, considering the rapidly evolving nature of this field, it is anticipated that guidance documents and manuals will be updated in the near future.

OP70 Implementation Of An Online Consultation Hub To Facilitate Consumer Engagement In Health Technology Assessment Processes

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Introduction: In 2021, the Australian Government Department of Health and Aged Care's Consumer Evidence and Engagement Unit (CEEU) launched an online consultation hub to provide a centralized pathway for consumer engagement in health technology assessment (HTA) processes. The hub enables consumers (patients, carers, health professionals, and citizens) to provide commentary on items being considered by HTA committees for subsidization.
Methods: A survey was developed by the CEEU, committee members, and consumer representatives to facilitate consultation on applications assessed by the Pharmaceutical Benefits Advisory Committee (PBAC)—a principal Australian HTA committee. Questions were designed as simple and engaging, including guidance on the type of information required. Responses are summarized thematically by efficacy, safety, accessibility, and quality-of-life impacts of the proposed health technology. New surveys are launched to coincide with each PBAC meeting agenda publication and allow a ten-week consultation period. Awareness of consultations is supported by the CEEU's HTA Engage e-newsletter, which alerts the public and targeted stakeholders.
Results: The hub surveys have enabled streamlined consumer commentary to be provided for PBAC considerations. It has also allowed increased time for quality consultation with stakeholders. The success of the hub is further demonstrated in the current development of a similar survey for another principal committee, the Medical Services Advisory Committee (MSAC), to transition its consultation processes to the hub. Of note, while consumers' feedback on the hub is positive, there remains a desire for educational resources and face-to-face interactions to support consumer engagement in HTA processes. The CEEU are developing materials to address and further support this need.
Conclusions: In a time when technological communication can be optimized to complement face-to-face conversations, it is vital consumer engagement in HTA processes follows suit. To facilitate continued engagement that is sustainable for both present and future Australians, the CEEU continues to evolve a strategy regarding virtual consultations to increase consumer awareness and education and promote effective participation in Australian HTA.

OP71 Patient Disease Strategy: A New Operational Framework For Collecting And Applying Patient Experience Data Into Clinical Development Programs

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Introduction: Understanding patient experience and needs is crucial to develop high-value therapies. Patient experience data (PED) inform trial design and evidence generation plans. The U.S. Food and Drug Administration's roadmap to patient-focused outcome measurement advocates integrating PED into product development. We adapted this theoretical framework to include the health technology assessment (HTA) perspective and operationalized it as a patient disease strategy (PDS) framework.
Methods: The PDS framework is a methodology that systematically integrates patient-informed activities to reflect the patient health value of a new treatment. A PDS is developed per indication, initiated in the preclinical phase, applied in clinical development, and continuously adapted throughout the product development lifecycle. The three PDS phases include: (i) development of patient profile, including epidemiology, demographics, patient journey, disease, and treatment burden for patients and caregivers; (ii) PED gap analysis, focusing on identification of patient priorities, unmet needs, preferences, and expectations for new therapies; and (iii) translation into actions, such as diversity and inclusion (D&I) plans and outcomes strategy.
Results: Out of 58 indications, 31 percent have endorsed PDS and 67 percent are in progress. Patient-relevant label opportunities increased by over 50 percent. Each indication was informed on average by patients from three different countries. The PDS framework helped to identify factors that impacted health outcomes for integration into trial designs and D&I plans. Early understanding of heterogeneous patient populations, unmet needs, benefit/risk trade-offs, and patient experiences ensured development programs measured the most meaningful outcomes while also addressing evidence gaps. Early understanding of patient priorities and barriers to participation optimized the studies by reducing burden and identifying proactive support needed to complete the trial.
Conclusions: The PDS framework systematically identified health value opportunities for a target population and integrated the patient needs into the overall development plan. PED informs clinical trial design and endpoint strategy optimization, including factors that

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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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