Conclusions: The study shows a decade long rise in global orphan drug approvals, underpinned by regulatory flexibility, particularly by the FDA and the PMDA. Identified divergences in decision frameworks among regulatory and HTA agencies, as well as HTA agencies themselves, call for increased stakeholder alignment. This necessitates synchronizing evidence generation during development and improving decision frameworks for streamlined review and reimbursement processes.

PD202 Reimbursement Success For Pharmaceutical Products With Noncomparative Data: Are Health Technology Assessment Bodies Well Suited To Embrace Innovations?

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Introduction: Randomized controlled trials are ideal for securing marketing authorization (MA) for pharmaceuticals. However, ethical and feasibility constraints have led to the use of noncomparative trials by regulatory bodies to balance evidence uncertainty and patient access, especially for medicines addressing rare diseases or unmet healthcare needs. This research focused on pharmaceuticals approved with noncomparative data and how these translate into reimbursement outcomes.
Methods: Indications approved by the European Medicines Agency (EMA) between January 2018 and October 2023 based on noncomparative data were identified. Generics and biosimilars were excluded. Approvals based on non-comparative trial data were identified from European public assessment reports and from "Procedural steps taken and scientific information after authorization" documents. The latter were also used to identify changes in MAs after submission of new data. Missing trial information was extracted from ClinicalTrials.gov. Health technology assessment (HTA) outcomes from the EU4Health programme and the UK were extracted from agency websites.
Results: The EMA approved 46 indications based on noncomparative data: 18 of the 46 (39%) received full MA, 23 (50%) received conditional MA (CMA), and five (11%) had a CMA converted to full MA with additional data. The results for HTAs conducted in various countries were as follows:

- England: 29 of 46 (63%) indications assessed, (23/29 [79%] recommended);
- Scotland: 28 of 46 (61%) indications assessed, (20/28 [71%] recommended);
- France: 33 of 46 (72%) indications assessed, (7/33 [21%] received Amélioration du Service Médical Rendu level I to III);
- Germany: 37 of 46 (80%) indications assessed, (2/37 [5%] achieved additional benefit);
- Italy: 37 of 46 (80%) products assessed, (33/37 [89%] reimbursed); and
- Spain: 34 of 46 (74%) products assessed, (23/34 [68%] reimbursed).

Conclusions: Indications approved by the EMA with noncomparative data achieved mixed HTA outcomes in the EU4Health programme and the UK. More negative outcomes were seen in markets with clinical-benefit payer archetypes (France and Germany). All conversions from CMA to full MA occurred within the last 24 months.

PD203 Comparison Of Health Technology Assessment Methodologies Across Australia, Canada, New Zealand And The United Kingdom: Implications For Future Collaboration

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Introduction: In 2022, a group of health technology assessment (HTA) bodies from Australia, Canada, and the UK announced a collaboration to identify solutions to common challenges. This collaboration was later expanded to include agencies from New Zealand and Quebec, Canada. Since one possible activity of the consortium is joint assessments, we compared the methodologies of the agencies on 11 topics to assess the feasibility of this.
Methods: We reviewed the methodological guidelines of the Canadian Agency for Drugs and Technologies in Health (CADTH), L'Institut national d'excellence en santé et services sociaux (INESSS), the National Institute for Health and Care Excellence (NICE), the Pharmaceutical Benefits Advisory Committee (PBAC), the Pharmaceutical Management Agency (Pharmac), and the Scottish Medicines Consortium (SMC). The topics considered were real-world evidence, consideration of health effects, economic reference case, survival analysis, surrogate endpoints, patient involvement, uncertainty, orphan pathways, clinical evidence requirements, carer perspective, and decision modifiers. We analyzed the level of alignment across the collaborating agencies using information from the guidelines, supplemented by published literature where necessary.
Results: Three topics exhibited high alignment: consideration of health effects, clinical evidence requirements and surrogate endpoints. The topics of orphan pathways and carer perspective had low alignment. The remaining topics had moderate alignment. Regarding orphan pathways, NICE and the SMC had separate processes for ultra-orphan drugs, CADTH and INESSS implicitly consider rarity, and PBAC and Pharmac do not appear to consider rarity.

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Since carer perspective is not commonly accepted in HTA, NICE was the only agency with relevant guidance on this topic. INESSS required the societal perspective as standard, while the PBAC and Pharmac explicitly excluded it. CADTH may consider carer perspective in some circumstances, whereas the SMC guidance was ambiguous.
Conclusions: While there is good alignment on most topics, there are several areas where agencies would need to resolve divergences in preferred methodology if joint assessments are going to be carried out in the future. All relevant stakeholders should be part of this process, including patient groups and industry.

PD204 Comparison Of Reimbursement Systems In France Denmark, Norway, And The United Kingdom - Possibilities Of Implementation In Poland

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Introduction: Reimbursement schemes should be regularly updated to maintain a trade-off between costs for the system and access to medicines. The aim of this study was to review reimbursement systems in several European countries in terms of solutions that could have a positive impact on the health technology assessment (HTA) and reimbursement processes in Poland (e.g., increasing patient access to medicines while maintaining payers' spending).
Methods: Secondary publications and the websites of key institutions responsible for drug policy (the Ministry of Health, HTA agencies, and payers) in selected European countries were searched to find unique solutions that could have a positive impact on drug policies in Poland. The keywords used were "drug reimbursement" and "HTA". A more specific search was conducted as needed.
Results: The following solutions considered worthy of further consideration:

- central organization of tenders for hospital drugs and conducting price negotiations with regional financial responsibility;
- determining the maximum annual copayment per patient for reimbursed drugs;
- complementary private health insurance that reduces patient copayments;
- creating a separate path for hybrid drugs in the HTA and pricing processes;
- increasing the number of consultations with the market authorization holder, clinical experts, and the public during drug evaluation; and
- not accounting for the costs of lost productivity during HTA due to the discrimination of seniors and children.

Conclusions: Minor changes in the HTA process, such as increasing the role of consultations, as well as major systemic changes (e.g., introducing complementary private insurance, creating a separate path for hybrid drugs, and introducing a maximum annual copayment for reimbursed drugs) could improve patients' access to drugs. Implementing these solutions requires significant adaptation of the local legal framework.

PD205 "Medical Fund" – A Novel Approach To Granting Individuals Access To Cutting-Edge Therapies

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Introduction: Access to innovative and expensive medicines is a significant challenge for Poland's healthcare system. These therapies often do not meet the reimbursement criterion that is currently set at three times the gross domestic product per capita. Nevertheless, there are ongoing efforts to identify funds that can cover the cost of innovative and expensive medicines.
Methods: A new legal act established the Medical Fund, which is an addition to the regular National Health Fund. The Medical Fund finances medical technologies recognized by the Polish healthcare system as highly innovative or of high clinical value. Lists of such therapies are prepared by the health technology assessment agency, in consultation with clinical experts, and then approved by the Ministry of Health. Simplified marketing authorization applications based on a budget impact analysis, a more straightforward assessment process, and a separate budget may allow patients to access these therapies faster.
Results: Since January 2022, ten highly innovative therapies have been funded for patients with conditions such as spinal muscular atrophy, acute hepatic porphyria, and primary hyperoxaluria. The reimbursement decisions were issued for a two-year period, during which data on treatment efficacy were collected. If the data collected after two years is insufficient to assess the treatment's efficacy, the decision can be extended without an additional procedure for another two years. After two or four years, the marketing authorization holder must submit a reimbursement application based on a full health technology assessment report. Risk-sharing schemes based on clinical outcomes are mandatory.
Conclusions: The Medical Fund has granted early access to modern therapies. The decision to continue funding for a particular drug depends on whether registry results confirm data from experimental studies.

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