

program content. The curriculum was designed to incorporate the heterogeneous medical and cultural backgrounds and varying levels of prior knowledge among participants, recognizing diverse experiences that individuals bring to the training. To enhance accessibility, a blended learning approach with offline, virtual, and in-person parts was adopted.

**Results:** The training comprised introductory (two hours), fast-track (eight hours), and extended (four days) training sessions. Training modules include competencies such as a profound understanding of HTA processes and key principles, domains, impact of HTA, outcome measurements, and study designs to raise awareness, promote, and enhance patient engagement as foreseen in the HTAR. The training modules focus on empowering patients to advocate for their rights and communities and to contribute meaningfully to comprehensive treatment evaluation. Specifically, modules on communication and personal skills, patient involvement, and engagement at both national and European levels underscore promotion of patient empowerment. Training sessions can be accessed online (EUCAPA.eu).

**Conclusions:** The training development within the EUCAPA project addresses the evolving landscape of joint HTA by emphasizing the significance of patient perspectives, fostering core competencies, and responding to the increasing demand for active patient involvement and advocacy at both national and supranational levels.

## PP61 A Decade Of Enhanced Patient And Citizen Involvement In Health Technology Appraisal In Scotland

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**Introduction:** Over the past 10 years, the Scottish Medicines Consortium (SMC) has strived to strengthen the involvement of patients and citizens in its work and improve public understanding of health technology appraisal (HTA). A series of innovations have been brought in since 2014 to increase levels of engagement and satisfaction of participants. This work describes these and their impact.

**Methods:** SMC has introduced numerous transformational changes since 2014. Innovations include a new Patient Group Partner (PGP) registration system, strengthened patient group submission process, participation of patient representatives at Patient and Clinician Engagement (PACE) and SMC Committee meetings, embargoed early release of SMC decisions to PGPs, provision of Summary Information for PGPs (from submitting company), and revised role of public representatives. The creation of the SMC Public Involvement Network Advisory Group has underpinned these achievements, as has the provision of comprehensive one-to-one support, information and education to PGPs. SMC has continually evaluated the satisfaction of participating PGPs using an online questionnaire.

**Results:** There has been a sustained increase over the past 10 years in both the number of patient groups engaging with SMC and the number of PGP submissions that SMC receives. Adopting a

continuous improvement approach, working in partnership with PGPs and public representatives, has helped SMC to ensure that stakeholders in the HTA process are effectively engaged and informed about the HTA of new medicines in Scotland. Surveys of public involvement in SMC consistently show an extremely high level of satisfaction from PGPs who work with SMC, with most PGPs consistently rating their experience of working with SMC as excellent.

**Conclusions:** Over the past 10 years, SMC has strived to strengthen how it involves citizens and patient representatives in HTA. Various innovations and a continuous improvement approach have helped to ensure that there are high levels of satisfaction and understanding of the HTA process from patient groups who engage with SMC. This is underpinned by a partnership approach to working.

## PP62 Improving Patients' Access To Medicines Through Health Technology Assessment In Ukraine: Measuring The Impact Of The National HTA Roadmap

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**Introduction:** In 2020, health technology assessment (HTA) became mandatory for any new medicine covered by public funds. The aim of the review is to present the impact of HTA implementation despite Russia's full-scale war against Ukraine. Thanks to the introduced reforms, including HTA introduction, the coverage from the public payer in providing patients with medicines increased from 11 percent in 2013 to 34 percent in 2023.

**Methods:** This review summarizes all Department of HTA conclusions with recommendations made during 2023, decisions made by the Ministry of Health (MOH) based on these recommendations and their impact on patient access, and the steps made to further institutionalize HTA in Ukraine. In 2023, the HTA guidelines for medicines were revised based on the lessons learned and global experience along with knowledge exchange with the United Kingdom HTA agency, NICE International.

**Results:** In 2023, the HTA Department prepared 19 HTA conclusions. The majority concerned treatment of oncologic diseases (32%), orphan diseases (21%), and multiple sclerosis (16%). HTA assessments resulted in recommendations for listing (84% cases). Fifty-three percent of medicines were recommended for managed entry agreement, and the MOH decided to forward 10 medicines for negotiations in 2023. Thirty-two percent of recommendations considered inclusion into the National Essential Medicines List (NEML); 16% with further access through the reimbursement program. Almost 70 percent of total need in NEML is currently covered by

public funds, compared to 40 percent in 2020 (according to the Support in Market Development (SMD) analytical database).

**Conclusions:** The HTA function in Ukraine has evolved since 2020 with a visible impact on patients' access. Introduction of new procedures, such as HTA for medical devices, may improve patient access to health technologies and provide evidence for rational and transparent decision-making processes, with gradual transformation of the Department of HTA at the State Expert Centre into an independent HTA agency by 2026.

## PP63 Perspectives Of Physical And Organic Disability Organizations On Health Technology Assessment Processes

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**Introduction:** Effective participation of individuals with disabilities in health technology assessment (HTA) processes is paramount. Aware of the reality of people with physical and organic disabilities, COGAMI (a not-for-profit umbrella organization of disability associations) conducted an internal study to gather perspectives on the participation of people with disabilities in HTA processes.

**Methods:** An ad hoc questionnaire of four open-ended questions was designed and distributed via email to COGAMI's socio-health commission, representing 23 entities and 4,000 people in Galicia. A thematic analysis of the responses obtained was carried out.

**Results:** Consensus underscores the fundamental role of individuals with disabilities and their representative organizations in HTA processes, though currently, only those with greater resources actively participate. The participants found that insufficient information reaching patient organizations hinders participation (e.g., lack of awareness in proposal submission), complicating their involvement. Additional challenges include accessibility and the digital divide. Proposed solutions involve enhancing communication channels and information accessibility, establishing collaborative frameworks nationally, and actively considering the disability condition to ensure a fair and equitable implementation.

**Conclusions:** This study suggests the need for concrete actions to enhance the participation of individuals with disabilities in HTA processes. Recommendations include improving communication channels, capacity building, and recognizing disability as a key element in HTA.

## PP64 Impact Of The Cost-Effectiveness Threshold On Drug Funding Recommendations: The Case Of Rare Diseases In Brazil

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**Introduction:** In 2022, the Brazilian public health system has adopted an explicit cost-effectiveness threshold of USD24,232.91 per quality-adjusted life years (QALY) for evaluating technologies intended for rare diseases treatment. Although regarded as a strategy for increasing efficiency, the National Committee for Health Technology Incorporation (Conitec) has recommended that the threshold should not be used as a knockout parameter.

**Methods:** A retrospective analysis of Conitec's recommendations regarding technologies for rare diseases issued between January and October 2023 was conducted. The following data were extracted from Conitec's reports: (i) disease (rare or ultra-rare); (ii) health technology evaluated; (iii) scientific evidence regarding efficacy, effectiveness, and safety of the technology; (iv) incremental cost-effectiveness ratio (ICER); (v) recommendation issued; and (vi) rationale for recommendation. Identified technologies will be divided into two groups according to the recommendation issued (positive or negative). The rationale for each recommendation, along with other information, will be reviewed to look for explicit threshold utilization and its impact on decision-making.

**Results:** Twelve technologies for nine rare diseases evaluated in 2023 were retrieved and eleven were included in the analysis. Six drugs have received a positive recommendation for funding. The ICERs estimated varied between -USD5,278,362.06 for emicizumab for hemophilia A and USD116,407.47 for elexacaftor/tezacaftor/ivacaftor for cystic fibrosis. Two of them have received a positive recommendation despite their associated ICER exceeding the explicit threshold. All negative recommendations were associated with ICERs higher than the threshold, which varied between USD52,456.32 and USD847,942.97. For two, the threshold was specifically mentioned as the rationale for the negative.

**Conclusions:** After the adoption of an explicit cost-effectiveness threshold in 2023, Conitec has issued two positive funding recommendations for rare diseases technologies despite their associated ICER exceeding the threshold: agalsidase alpha for Fabry disease and elexacaftor/tezacaftor/ivacaftor for cystic fibrosis. For both recommendations, the drug's favorable impact on the natural history of the disease was considered decisive.