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Empowering Patients in HTA: Joint Initiative by OPEN Health, EPF, and EAMDA to Optimise PICO Scoping

OPEN Health, the European Patients' Forum (EPF), and the European Alliance of Neuromuscular Disorders Associations (EAMDA) join forces on a Delphi consensus panel project for patients focusing on Population, Intervention, Comparator, and Outcomes (PICO) scoping.

From January 12th, 2025, a European Union (EU) regulation 2021/2282 (HTAR) will apply to evaluate the relative clinical effectiveness and safety of health technologies, starting from cancer medications and ATMP therapies. The regulation aims to foster joint work on Health Technology Assessments (HTAs) across the EU by establishing the creation of the Joint Clinical Assessment (JCA) that will be developed in parallel to EMA regulatory procedures.

The JCA relies on the PICO (population, intervention, comparator, and outcomes) framework which provide the base for the relative clinical effectiveness and safety assessment. Currently, the extensive number of PICOs driven by the heterogeneity between healthcare systems and stakeholders' perspectives represents a major challenge.

OPEN Health, the European Patients' Forum (EPF), and the European Alliance of Neuromuscular Disorders Associations (EAMDA) have partnered to design and conduct a Modified Delphi panel to generate patient expert consensus on PICO scoping, across the European Union. In particular, OPEN Health, EPF and EAMDA would like to test the modified Delphi panel method to obtain patient experts' consensus on the PICO questions and to minimise heterogeneity across inputs from European patients with Spinal muscular atrophy (SMA).

"Consensus research methods, such as Modified Delphi Panels, have a great potential to reduce heterogeneity across experts' inputs by identifying areas of consensus structure, while enhancing stakeholder engagement and fostering transparency. This is particularly relevant in the context of the EU HTA PICOS scoping challenge. This methodology also allows for early and structured engagement of patients, and this is an opportunity not to be missed for patient engagement in HTA decision-making. Patients can and should be the drivers of change, also from a methodological approach point of view! We as OPEN Health, are excited to partner with EPF and EAMDA to test this method and share our learnings."
Emanuele Arcá, Open Health

"The workability of the HTAR is one of EPF main concerns. Contributing to address it is one of our priorities. The potential fragmentation of PICOs risks neutralising the expected benefits coming with the HTAR for faster and more equal access to innovative technologies for all patients across Europe. With this exercise, we wish to identify and - if successful - promote a method to minimize divergence of views and increase consensus around what



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matters the most to patients from a specific disease area. We are grateful to have partners such as Open Health and our member EAMDA, who have accepted with enthusiasm to collaborate on this matter". Valentina Strammello, Director of Strategic Initiatives, EPF.

"This partnership with EPF and OPEN Health highlights our commitment to shaping the European HTA framework and refining the PICO model to better address the challenges faced by patients with neuromuscular disorders. SMA, a severe condition with significant impact on quality of life and complex treatment needs, is central to this effort. With recent advances in SMA therapies and numerous clinical trials underway, it's crucial that HTA processes accurately evaluate the efficacy and real-world outcomes of these innovative treatments, ensuring that patients receive the most effective care possible." Arabela Acălinei, President, EAMDA

About OPEN Health

OPEN Health brings together deep scientific knowledge, global understanding, and broad specialist expertise to support our clients in improving health outcomes and patient well-being. We are united as one flexible organization, harnessing the power of the collective to solve complex challenges. Our global team of experts—many with PhD or PharmD degrees—work strategically alongside our client partners in Medical Affairs, HEOR, Market Access, and Commercial teams across a wide range of therapy areas.

About the European Patients' Forum

The European Patients' Forum (EPF) is an independent non-profit, non-governmental umbrella organisation of patient organisations across Europe and across disease areas. EPF's nearly 80 members include disease-specific patient groups, active at EU level and national coalitions of patients.

EPF leads the collective patient advocacy in Europe, providing a cross-disease perspective from a wide European patient community to the policy-making process. With their members, EPF brings together people, knowledge and expertise working to drive profound and positive change for patients within the European healthcare landscape.

About the European Alliance of Neuromuscular Disorders Associations

Founded in 1974, EAMDA brings together neuromuscular associations across Europe, sharing resources and knowledge to provide a unified voice for individuals living with neuromuscular disorders (NMDs).

The organisation's activities span a broad spectrum, including providing support and information to people with NMD and their families, advocating for their rights and interests at the European level, and facilitating collaboration and networking among member organisations. EAMDA also plays a pivotal role in bringing together researchers and medical professionals to accelerate advancements in the NMD field and enhancing communication among all stakeholders connected to neuromuscular care.

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