

The European Children's Hospitals Organisation (ECHO) welcomes the Commission's initiative to revise legislation on medicines for children and rare diseases and to address some of the shortcomings with the current legal framework. Children's hospitals treat the majority of children and young people with rare diseases at a regional and national level and are also drivers of clinical and basic research in this field. Given our membership, we offer a unique perspective on the needs of children, their families, and the professionals and researchers working to treat and cure paediatric and rare diseases. ECHO urges the Commission to prioritize the following in their revision of the current legislation:

Centre children, young people, and families when defining unmet need

Unmet need is significant issue for children and young people with rare diseases, affecting both patients and families. To address this issue we recommend that:

- Unmet need should be defined by a multi-stakholer group, centred on and driven by patients and families with additional input from healthcare providers and researchers.
- Patients, families, and healthcare providers should be involved not only in defining unmet need but should be an integral part of any system to identify products for development. This system should be transparent and include periodic review and oversight to ensure that product development is in line with the needs of patients.
- The definition of unmet need should not be limited to ultra-rare conditions to the exclusion of other rare diseases.

Create public funding mechanisms to support rare disease research and the careers of rare disease researchers

Research funds targeting rare disease research are urgently needed to address unmet need. Given the broad public health emphasis of most European funding mechanisms, few funds are available for rare-disease research leaving most researchers to seek funding from philanthropic organisations.

Another barrier to conducting research on rare diseases is the potential limit on a researcher's career path. Publishing rare disease research in the highest impact journals is challenging given the broad scope and intended audience of these journals, limiting the capacity for rare-disease researchers to distinguish

themselves professionally. To address these issues ECHO recommends that the Commission should:

- Develop a targeted paediatric research plan as part of the strategic plan for Horizon Europe.
- Support research that addresses the broader impact of rare diseases treatments including the impact on families and the broader society.
- Clearly state the inclusion of paediatric health research across the Commission's health initiatives including the Beating Cancer Plan, EU4Health, and the Green Deal.
- Create funding mechanisms to support the long-term career development of rare-disease researchers to encourage entry and retention in this field.
- Data sets from clinical trials submitted to the EMA should be publicly available to further support research and innovation.

Embrace state of the art science and technology including data sharing systems

ECHO supports the adoption of a 'mechanism of action principle' to ensure that disease processes that are specific to children and young people, like cancer, are not left out of the drug development pipeline.

State of the art science will only be achieved with the support of a pan-European health data system that allows for the seamless sharing of research and clinical data. This will require addressing some challenges of the GDPR as well as issues around standardisation of coding of rare diseases. Support for these systems is essential for the success of any initiatives aimed at increasing access to paediatric and orphan medicines.

Increase access to reduce health inequities

Children and young people with rare diseases face differential access to treatment depending on where they live. The causes of these access issues are multifactorial and intersect with Member States' competence of organising, financing, and delivering healthcare. Therefore solutions need to be pan-European and include the full range of stakeholders, including patients, national and regional health authorities, and clinical healthcare leaders.

ECHO supports evaluating the impact of linking rewards and funding for SMEs and academia with requirements to place new products on the markets in most or all Member States as a way to increase access. These proposals should ensure that market entry happens within a similar timeframe in all Member States to prevent delays in access to care. They should also ensure that such requirements do not serve as a deterrent to market entry. ECHO further recommends that the Commission:

- Work with stakeholders, Member States, and health authorities to identify the root causes of access differences with subsequent implementation and monitoring of proposed solutions.
- Include health-services research as part of the research agenda to identify and address healthy system-level barriers to access.
- Provide support to the European Reference Networks (ERNs) and create mechanisms to support the provision of cross-border care when treatments are not available in a Member State.

ECHO would like to express our interest and willingness to work with the European Commission and other stakeholders on the next steps, including the upcoming public consultation and targeted consultations for stakeholders.

About ECHO

ECHO advocates for children's health and their access to the best quality care through the collaborative work of children's hospitals.

ECHO Members

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