

ECHO response to roadmap: EU strategy on Pharmaceuticals – safe and affordable medicines

The European Children's Hospitals Organisation (ECHO) welcomes the Commission's objective of ensuring Europe's supply of safe and affordable medicines. ECHO represents leading children's hospitals caring for children with complex, chronic, and rare diseases. We offer a unique perspective on the needs of children, their families, and the professionals who care for them. We also represent hospital administrators who are charged with ensuring that all children have access to the medications they need to stay healthy.

Although the EU Paediatric Regulation has helped increase the availability of medicines for paediatric patients, many children still face significant barriers to accessing the medicines they need. These barriers include:

- A limited availability of medicines for conditions specific to children, such as childhood cancer or other rare diseases
- A lack of paediatric formulations
- The high cost of new therapies for hospitals and health systems, and
- Ongoing drug shortages.

Patient centred care

To ensure that the strategy is "holistic" and "patient-centred," any new drug development strategies should **involve the full range of stakeholders** including patients and patient advocates, regulators/payers, academics, and clinicians. Many children's hospitals already involve youth in clinical trials. These or similar networks could be utilized to inform the Pharmaceutical Strategy to ensure patient needs are centred.

Research and Innovation

We agree with the Commission that innovation efforts are not always aligned to public health and health system needs. This is especially true in the case of paediatrics where, since 2007, only 9 paediatric specific anti-cancer medicines were authorized compared to over 150 for adult cancers. To address this gap we recommend that:

- New drug development must be **driven by scientific data** and the **mechanism of action of drugs**, not by the adult market potential.
- Additional mechanisms to incentivise the pharmaceutical industry to conduct **dedicated paediatric pharmaceutical research** are also needed.

Pricing and Accessibility

The high cost of new therapies can be a barrier at the health system level, but can also pose a challenge for hospitals charged with providing care to patients with complex diseases. This barrier to access can contribute to geographic disparities in health outcomes for children across Europe. We need joint strategies to ensure all health systems and hospitals can afford to provide new therapies once they come to market. Potential strategies include:

- Supporting negotiations between member states and the pharmaceutical industry to regulate pricing.
- Linking the price of treatment to favourable health outcomes.

Supply Chain

Paediatric patients are at particular risk of harm due to drug shortages. One reason is the decreased amount of quality paediatric data to guide the selection of alternative medications. Furthermore, alternative products may also carry a higher risk of paediatric-specific adverse effects. To address supply chain issues affecting children in hospital we recommend that the strategy actively involve paediatric pharmacy specialists to help identify solutions. We also recommend that the strategy facilitate communication and collaboration between children's hospitals to promote information exchange to share best practices in addressing critical drug shortages.

Sustainability

ECHO also agrees with and strongly support the Commission's aim of addressing the environmental risks associated with medicines. The climate crisis is one of the greatest threats to child health and children's hospitals are committed to making sure children are healthy in and out of hospital. Pharmaceuticals can be a significant part of a health system's environmental footprint. Standards and tools are needed to support hospitals in implementing sustainable procurement initiatives to ensure that the medicines we use to make children healthy are not hurting the world they live in.

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

Barcelona: Sant Joan de Déu Barcelona Children's Hospital – **Copenhagen:** Rigshospitalet – **Dublin:** Children's Hospital Group – **Florence:** Meyer Children's Hospital – **Helsinki:** HUS New Children's Hospital – **London:** Great Ormond Street Hospital for Children – **Munich:** Dr. von Hauner Children's Hospital – **Oslo:** Oslo University Hospital – **Paris:** Necker-Enfants Universitary Malades Hospital – **Riga:** Children's Clinical University Hospital – **Rotterdam:** Erasmus – MC Sophia Children's Hospital – **Petach-Tikva:** Schneider Children's Hospital of Israel – **Warsaw:** Children's Memorial Health Institute