

# EPTRI on the scene – what will be EPTRI's impact in the future?

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## What is EPTRI?

- EPTRI is a Research Infrastructure (RI) aimed to increase knowledge on children development and ontogeny, as well as on their impact on drug effects and toxicity, and to implement and provide services on basic, preclinical and translational paediatric research.
- Research organizations from the academic sector whose interest is to develop drugs and devices for the paediatric population by providing basic, pre-clinical and translational research are invited to join.

# Role of EPTRI



- EPTRI will be an open science space, allowing researchers to collaborate without geographical, institutional or financial boundaries, and a system with many interconnected research areas, which will contribute to bringing new paediatric medicines, therapies and medical devices to be developed and approved for the benefit of children.
- EPTRI will also promote stakeholders' engagement and knowledge translation processes by integrating the perspectives of young patients and families, policymakers and payers, regulators and researchers, agencies and charities, academies and industries.

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# MANIFESTO

## ON PAEDIATRIC RESEARCH

A supportive document for policy makers and other stakeholders to promote more paediatric and orphan medicines and the inclusion of paediatric research topics in the EU Agenda.

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## **CHARTER OF FUNDAMENTAL RIGHTS OF THE EUROPEAN UNION** **CHAPTER III - EQUALITY - Article 24** **The rights of the child**

Children shall have the right to such protection and care as is necessary for their well-being. They may express their views freely. Such views shall be taken into consideration on matters which concern them in accordance with their age and maturity. In all actions relating to children, whether taken by public authorities or private institutions, the child's best interests must be a primary consideration.

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## *Paediatric Drugs Research – existing needs*

### CHARTER OF FUNDAMENTAL RIGHTS OF THE EUROPEAN UNION (2000/C 364/01) SOLIDARITY Article 35 - Health care

Everyone has the right of access to preventive health care and the right to benefit from medical treatment under the conditions established by national laws and practices. A high level of human health protection shall be ensured in the definition and implementation of all Union policies and activities.

**20%** of the EU population are **children**.

However, paediatric drugs research is neglected due to low industrial interest in such a limited market.

**Only 30-50% of medicines** are available for children at different ages/diseases.

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## Current obstacles in paediatric drug research – **lack of:**



**Sufficient pre-clinical information** based on **innovative tools** such as molecular targets, paediatric cellular lines models, biomarkers variability, animal juvenile models



**Paediatric clinical studies covering all the paediatric ages**, also considering the **influence of physiological maturation of biological functions** on study conduct and results



**Innovative study design applications** like modeling, use of existing data, Artificial Intelligence applications



**Tools to translate R&D** into specific **paediatric and orphan therapeutic approaches**



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## Current obstacles in paediatric drug research



**Fragmentation of expertise**, scattered knowledge, and intradisciplinary technologies



**Low capacity of data/information sharing** among Institutions



**Limited participation of patients and caregivers** in drug and therapies development as well as clinical phases



**Poor interest on children's fundamental rights** on health care and safe medicines



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## *Innovative paediatric drug development processes*



Basic and preclinical research allowing increase of knowledge on human development and discover of new treatments for children



New paediatric biomarkers targeting the most common paediatric diseases as well as the rarest and still undiagnosed ones



Age-tailored paediatric medicines formulations and medical devices appropriate for children's body



Translational research to underpin paediatric clinical studies



Access to key technologies and standardized models and analytical tools in the paediatric setting

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„Vision without action is a dream.  
Action without vision is simply passing  
the time.  
Action with vision is making a positive  
difference“.

Ruí Moreno,  
Andrew Rhodes



EPTRI AISBL- General Assembly, July 18, 2024