



EPTRI is a new infrastructure proposed to accelerate and expand the paediatric drug discovery and development processes by focusing on basic and preclinical research methods and taking into account: 1) the continuous ontogeny induced variability in drug disposition, disease targets and biomarkers; 2) the natural condition of the child growth requiring size adaptation of medicine forms and tests (small volumes, microtracing, microdosing, etc.); 3) the advancements in life science, technology and data science to be implemented in the paediatric setting.

EPTRI is organized as a Distributed Research Infrastructure that allows to cover some critical gaps in the paediatric research.

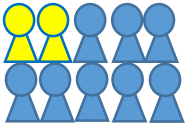
Shortage of medicines in Europe has increased the risk of undercured patients affected by COVID-19 and its complications and should be strongly reduced. However, in case of children the situation has proven to be more and more challenging, due to the chronic shortage of appropriate treatments, even when the treatment exists but has been tested and produced for adult's use (currently 34% of medicines approved centrally in EU are available for children). Moreover, medicines are often not available in a pharmaceutical form suitable for all the children's age groups and some age categories, like neonates and infants, are quite completely orphan of appropriate and innovative treatments.

During the COVID-19 pandemic, EPTRI at policy level to promote the inclusion of the themes of paediatric research in the EU Agenda and **has launched a Paediatric manifesto** advocate to improve the lives of children who are often orphans of treatments and to create a new EU political framework focused on children and their health.

EPTRI is promoting a paediatric dedicated approach to fill the most relevant unmet medical needs, taking into consideration the best interest of child and his/her evolving capacities as well as the protection of children as 'future generation' that has the "right to an open future".

We need to protect the paediatric population in time and put in place adequate measures aimed at guarantying the implementation of fundamental/children rights (including the principle of the equality of treatment of all EU patients).

MANIFESTO ON PAEDIATRIC RESEARCH



Paediatric drugs research is **neglected** due to **low industrial interest** in a limited market. A percentage of only **30-50%** of medicines in different ages/diseases is available for children.¹

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



Barriers

- Lack of **sufficient pre-clinical information** on paediatric medicines based on **innovative discovery tools** (molecular targets, cellular paediatric lines models, biomarkers variability, animal juvenile models, etc.)
- Lack of **paediatric clinical studies covering all the paediatric ages**, also considering the **influence of the physiological maturation of biological functions** on study conduct and results
- Lack of **innovative study design applications** (modelling, use of existing data, AI applications, etc.)
- **Dispersion of the expertise, knowledge and technologies** and lack of **instruments for channeling R&D** into specific **paediatric and orphan therapeutic areas**
- **Low sharing of data and information** among Institutions
- Scarce **participation of patients and caregivers** in drug development and clinical phases leading to therapies that not properly fit with the needs of paediatric patients, families and society at large
- **Poor consideration** of the **children fundamental rights** to receive a high level of **health protection and care** as well as to **high quality and safe medicines** (according to art.24 and 35 of the EU Charter of Fundamental Rights and the art.168 of the Treaty on European Union) in policies and funds allocation, notwithstanding the numerous unmet therapeutic needs connected to all the paediatric diseases.
- Too many unsolved and **uncovered ethical and legal issues related to children peculiarities hampering research** from preclinical phases to access policy and education.

To foster and support research and innovation in the paediatric field, **EP TRI** proposes a **Paediatric Research Plan** that:

- 1) Builds on the **most relevant publicly funded paediatric research initiatives** supporting the paediatric drug development processes (from new target paediatric disease identification, basic, preclinical and translational research to clinical phases and access for patients).
- 2) Is clearly defined as an integral part of the **European Commission Strategic Plan for Horizon Europe**.



In accordance to fundamental and children rights, a Paediatric Research Plan should be developed and cited as integral part of the 'first Strategic Plan for Horizon Europe' to **provide children with high quality and safe medicines** as announced in the European Strategic Plan for Research



Recommendations


for the inclusion of Paediatric Research in the HORIZON EUROPE strategic plan

Poor is the attention on the paediatric research among European initiatives even if the right to healthcare and safe medicine is recognised in many legal texts such as the EU Charter of Fundamental Rights and the Treaty on European Union. In addition, in 2015 the European Strategy Forum on Research Infrastructures (ESFRI) stated that **'children have been poorly served by research even though they have specific emotional and physical needs that must be met'**.





Recommendation 1. It is necessary to **secure public funding for paediatric research** by including the paediatric medicines research issues **in the framework of the upcoming Horizon Europe framework programme**. In particular, European Initiatives promoting and serving paediatric research should be strongly supported across Horizon Europe.

Paediatric Research Partnership represents an innovative tool foreseen in the Orientations towards the first Strategic Plan for Horizon Europe, but on a total of 8 proposed European Partnerships related to Health **none mentions paediatric issues**.


Recommendation 2. A **paediatric Research Partnership**, dealing with Cluster 1 and relevant for Pillar 1- 2 and 3, should be explored by the EC. The paediatric research groups already existing in Europe should be involved in some of the already planned Partnerships (particularly the Innovative Health Initiative and the Rare Disease) with a **structured paediatric group to provide 'solutions to the global challenge' represented by children health protection**.

The current version of the EU4Health program seems to be not **paediatric oriented** and in particular, it does not include any topic specifically devoted to implement research and **development of paediatric health products** (Pharmaceuticals, Diagnostics, Devices, etc).

Recommendation 3. State for a visible and powered inclusion of paediatric research activities and funds in the **Horizon Work Programme Annex 4 Health**, by **defining paediatric topics or specify provisions** securing **paediatric successful participation** within topics of general interest that are also relevant for children health in terms of translational research from pre-clinical phases to access policy and education.

Medicines' availability and access for the paediatric and rare diseases populations have been not sufficiently considered in the **Pharmaceutical Strategy** and the measures foreseen in the **Paediatric Regulation** are insufficient to foster paediatric research.

Recommendation 4. **Prioritise the paediatric unmet needs in the EC Pharmaceutical Strategy** to direct investments in research and development of paediatric innovative medicines that are not already in industries' pipelines or R&D activities and **propose effective rewards** through a full revision of the Paediatric Regulation **to encourage industries to develop medicines in line with children's most pressing needs and rights**.



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