



# 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.

## #childrenfirst

Children and young patients are still left behind an acceptable level of available treatments. Most of the drugs used today have not been specifically developed and tested for them. Children and young people have different anatomy and metabolism, and their drugs reactions change radically with growth and development.

EPTRI's mission is to contribute to solving this gap by supporting paediatric research and creating a new EU political framework focused on children and their health.

It is time to change!

*Donato Bonifazi, EPTRI Coordinator*





**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.

**CONVENTION ON THE RIGHTS OF THE CHILD - Article 5**  
**Parental guidance and the child's evolving capacity**

States Parties shall respect the responsibilities, rights, and duties of parents or, where applicable, the members of the extended family or community as provided for by local custom, legal guardians, or other persons legally responsible for the child, to provide, in a manner consistent with the evolving capacities of the child, appropriate direction and guidance in the exercise by the child of the rights recognized in the present Convention.

# WHY do we need a Manifesto?

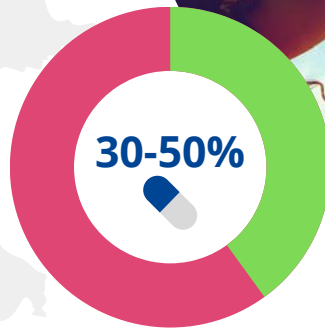
**20%** of the EU population are **children**.

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.

## 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.





# HOW can we overcome the gap to help our paediatric population?

Numerous **unmet therapeutic needs** connected to several paediatric diseases are still on the ground.

**Policy makers** must be the **change** by promoting targeted **rules** and allocating the necessary **funds**.



# WHAT are the current obstacles that are slowing down 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**





**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



**Unsolved and uncovered ethical as well as legal issues on paediatric research** from preclinical phases to access policy and education



# Join EPTRI PAEDIATRIC RESEARCH PLAN to foster and support paediatric R&D

## BE THE CHANGE!

**Support innovative paediatric drug development processes including:**



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



# It is time to change!

## #PaediatricResearchPlan

In accordance with fundamental children's rights, a **Paediatric Research Plan** is proposed as an integral part of the '*Strategic Plan for Horizon Europe*' to provide our children with high quality and safe medicines as announced in the European Strategic Plan for Research.

You will tangibly "promote health and empowering citizens to manage their health, including children and adolescents" as stated in the new European Commission Strategic Plan - Horizon Europe (cluster 1, Health).



# EPTRI's recommendations for the inclusion of Paediatric Research in the Horizon Europe Strategic Plan

Poor attention to paediatric research among European initiatives despite the fact the right to healthcare and safe medicines have been recognized in many legal documents as crucial, like the *EU Charter of Fundamental Rights* and the *Treaty on European Union*.

In 2015, the European Strategy Forum on Research Infrastructures (ESFRI) stated:

***"children have been poorly served by research even though they have specific emotional and physical needs that must be met".***



# Recommendation 1

Secure public funding for paediatric research.

The actual Horizon Europe strategic plan should be revised to include paediatric research. European Initiatives promoting and serving **paediatric medicines research** should be strongly supported across **Horizon Europe**.

To this aim, specific paediatric research topics relevant to children's health should be placed in the core actions of Horizon Europe, as part of Missions, Clusters and Partnerships.

In particular, **research funds** in Horizon Europe should be reserved to study **new paediatric drugs** and other treatments to cover relevant paediatric conditions.



# Recommendation 2

Set up a European Paediatric Research Partnership.

A **Paediatric Research Partnership** maybe a very innovative tool but unfortunately it is not considered in the first Strategic Partnership Plan, and in the premises of all the proposed Health European Partnerships **there is no mention of paediatric issues**. This Paediatric Research Partnership should be explored and the **paediatric research groups** already established in Europe **should be actively involved** in the planned Partnerships, especially in the **Innovative Health Initiative and in the Rare Diseases Partnership**, as well as in the International Consortium for Personalized Medicine.

## Recommendation 3

Include paediatric research topics at large in the Horizon Europe Programmes

Clearly state paediatric research topics and funds covering the full development of health products.

Currently, the **Horizon Europe research initiatives seem not to be paediatric oriented** since **any topics targeting paediatric** research and paediatric products development such as pharmaceuticals, diagnostics, and medical devices, are specifically mentioned. To cover the existing gaps, paediatric research topics spanning **from translational to preclinical and clinical phases** should be planned in the Horizon Europe initiatives such as **EU4Health, Horizon-INFRA**.



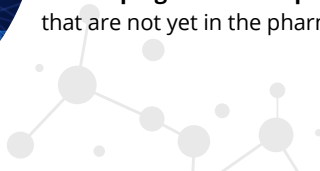
## Recommendation 4

Prioritize paediatric unmet needs in the EC Pharmaceutical Strategy

Focusing efforts and incentives to develop medicines in line with children's most pressing needs and rights. Medicines' availability and access for the paediatric and rare diseases populations have to be specifically considered in the **new EU Pharmaceutical Strategy**.

The actions foreseen in the Paediatric Regulation revision should include initiatives to **foster paediatric research in the area of uncovered paediatric needs**.

Direct proper investments in research aimed at **developing innovative paediatric medicines** that are not yet in the pharmaceutical pipelines or R&D activities.



# INSTITUTIONS SUPPORTING ePTRI PAEDIATRIC RESEARCH PLAN



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CNR-IN



KU LEUVEN  
CHILD & YOUTH  
INSTITUTE





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Sheffield Children's  
NHS Foundation Trust



Universitätsklinikum  
Erlangen



univerzitetni klinični center ljubljana  
*University Medical Centre Ljubljana*



UNIVERSITÀ DI PARMA



UNIVERSITÄT ZU LÜBECK



USTANOVA ZA OTROŠKO NEVROLOGIJO  
Foundation of Child Neurology, Ljubljana, SLOVENIA



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