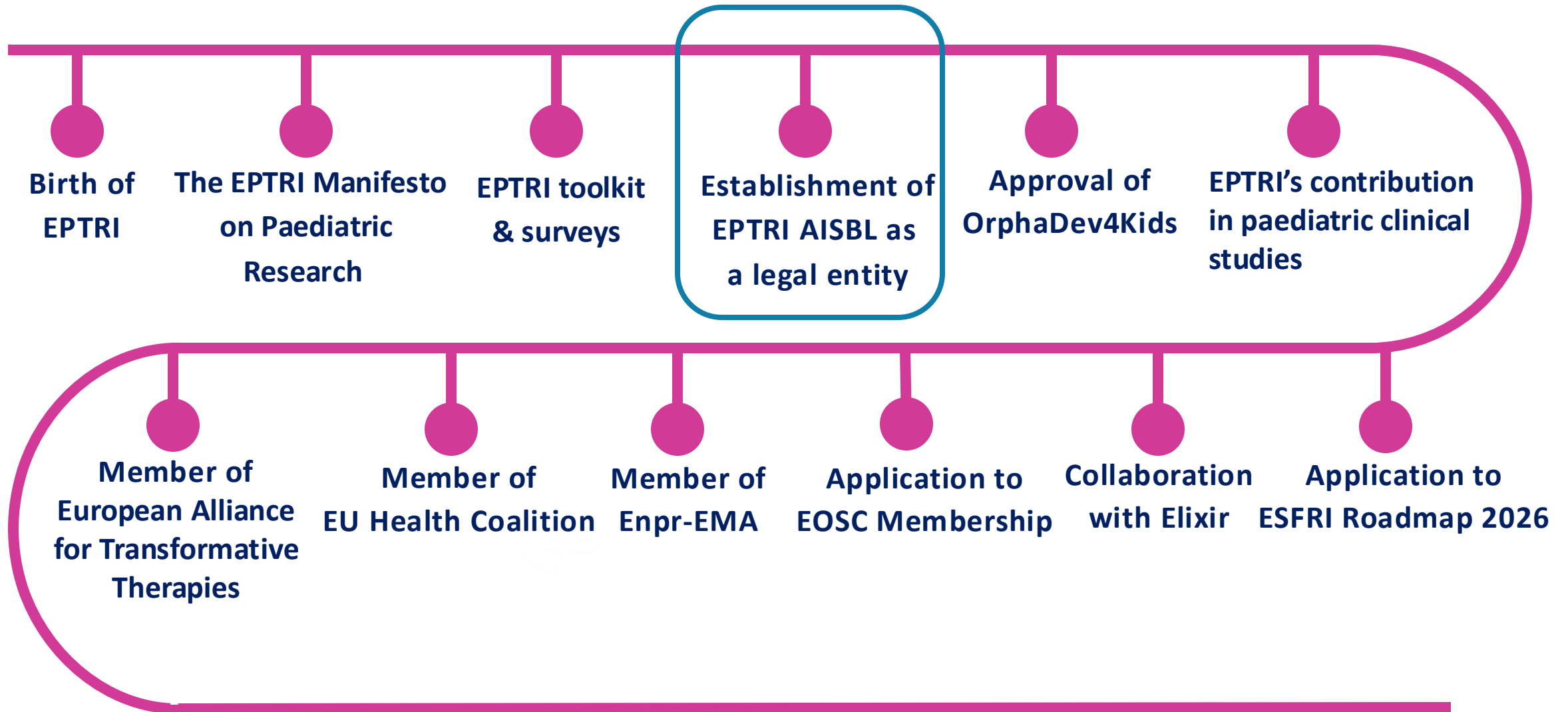


# The Birthday of EPTRI AISBL

*Marek Migdal*

The Challenge of Paediatric Research – Bologna – 13/03/2025





# EPTRI: The Journey Behind It's Creation

The European Paediatric Translational Research Infrastructure (EPTRI) started as a project coordinated by **Consorzio per Valutazioni Biologiche e Farmacologiche (CVBF)** and funded within the **H2020-INFRADEV-01-2017** programme, arising from the need to find answers to the serious lack of medicines for children in EU and worldwide, which aims to propose developmental models for a future research infrastructure focused on paediatric medicines, integrating technology-driven aspects with clinical trials.



It aimed to **design the framework for the new research infrastructure (RI)** providing a **conceptual design report (CDR)** of EPTRI describing the scientific and technical requirements as well as the key components of the new RI.

**Five technical and scientific domains have been identified:**

1. Human Development and Paediatric Medicines Discovery
2. Paediatric Biomarkers and Biosamples
3. Developmental Pharmacology
4. Paediatric Medicines Formulations and Medical Devices
5. Underpinning Medicines Development to Paediatric Clinical Studies

The project involves **29 Partners from 21 EU/ non EU countries and 114 research institutions as signatories of the MoU**

# PUBLICATIONS AND EDUCATIONAL PROGRAMS



The **EPTRI Manifesto on Paediatric Research** is a call to action for policy makers and other stakeholders to promote the inclusion of *paediatric research* and *orphan medicines development for children* in the EU Agenda.

- to **support children health needs**
- to **overcome the current obstacles** that are slowing down **paediatric drug research**
- to **secure a new European political framework** focused on children and their right to proper health treatments

**EPTRI special issues** aiming to contribute to the international progress in paediatric research by supporting and facilitating the publication of high-quality studies on a variety of aspects of drug development that can have an impact on children's therapeutics and health



## EPTRI Special Issue

EPTRI has launched the research topic collection "Supporting the Pediatric Drug Development: From Basic Research to Clinical Studies and Technological Advancements"



## EPTRI Toolkit

On Basic and Translational Paediatric Research for Patients and Families



Animal testing



Patient and Public Involvement (PPI)



Fundraising



Donation of patients' samples



Ethics

**EPTRI toolkit and surveys** aiming to gather the patient's preferences and to promote transfer of knowledge and science communication to the lay community



# PUBLICATIONS AND EDUCATIONAL PROGRAMS

The **EPTRI Medical Device Research Platform** organized a one-year **online educational program** on child health technologies, covering regulation, compliance, commercialization, and funding.



Webinar on *Robotics for child health*



Webinar on *Neonatal Technologies*



Webinar on *Child Health Data and Artificial Intelligence*



Webinar on *Patient and Public Involvement and Engagement in Child Health Tech development*



Webinar on *Co-design in Child Health Technology*

## New research paper

The research paper entitled: “*Innovative research methodologies in the EU regulatory framework: an analysis of EMA qualification procedures from a paediatric perspective*”, published on **Frontiers in Medicine**

Innovative research methodologies in the EU regulatory framework: an analysis of EMA qualification procedures from a paediatric perspective

Viviana Giannuzzi<sup>1\*</sup>, Arianna Bertolani<sup>2,3</sup>, Silvia Torretta<sup>1</sup>, Giorgio Reggiardo<sup>4</sup>, Eleonora Toich<sup>2</sup>, Donato Bonifazi<sup>2,3</sup> and Adriana Ceci<sup>3,5</sup> on behalf of the European Paediatric Translational Research Infrastructure (EPTRI)

<sup>1</sup>Department of Research, Fondazione per la Ricerca Farmacologica Gianni Berio Oriani, Bari, Italy; <sup>2</sup>Department of Project Development, Consorzio per Valutazioni Biologiche e Farmacologiche (CVBF), Pavia, Italy; <sup>3</sup>ESOP, European Network of Excellence for Paediatric Research, Pavia, Italy



# March 2024: A Major Milestone - Establishment of EPTRI AISBL as a legal entity

On March 4<sup>th</sup>, 2024, EPTRI (European Paediatric Translational Research Infrastructure) has been established as a **non-profit research organization** incorporated in the form of an Association Internationale Sans But Lucrative (**AISBL**) governed by Belgian law, **based at Leuven University**.

## OUR MEMBERS

EPTRI consists of **18 members** representing **9 different countries**, bringing together a diverse range of expertise and institutions dedicated to paediatric research and innovation.





## Our Vision

To increase knowledge on children growth and ontogeny characteristics relevant for the identification of novel therapies dedicated to children and to provide services for their development.



## Our Mission

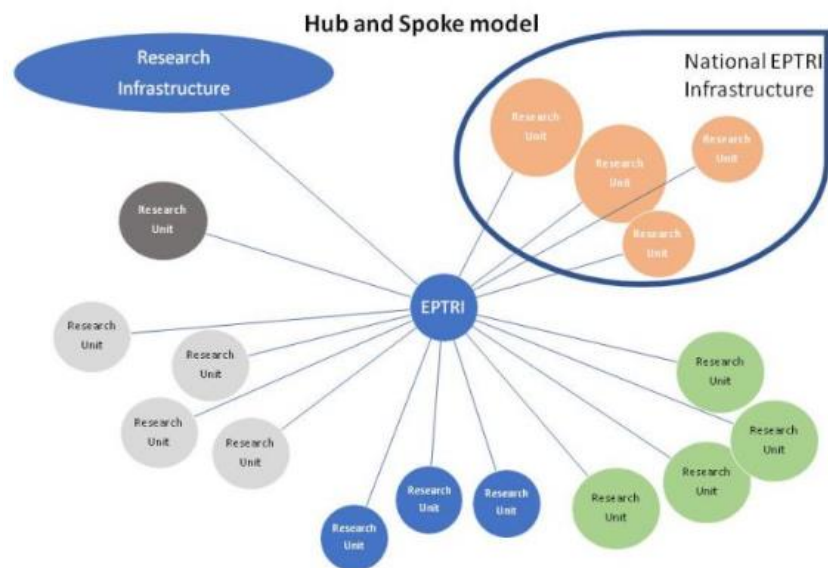
To implement competences, research strategies, access to key technologies, standardized models, and analytical tools, to offer to public and private stakeholders qualified basic, preclinical and translational research services covering paediatric medicines discovery and development.

EPTRI is a pan-European initiative that unites research units to strengthen the paediatric research ecosystem and offer services aimed at developing high-quality, safe medicines, technologies, and devices for children.

EPTRI fosters stakeholder engagement and knowledge translation by incorporating the perspectives of young patients and families, policymakers and payers, regulators and researchers, agencies and charities, as well as academia and industry.

# GOVERNANCE

EPTRI is organized as a distributed **Research Infrastructure (RI)** according to a **Hub and Spoke model**, with a Central Management Office (CMO), interconnected with the distributed research units, grouped within **Thematic Research Platforms – TRPs** (according to the field of expertise) and **National Nodes – NNs** (according to their location).

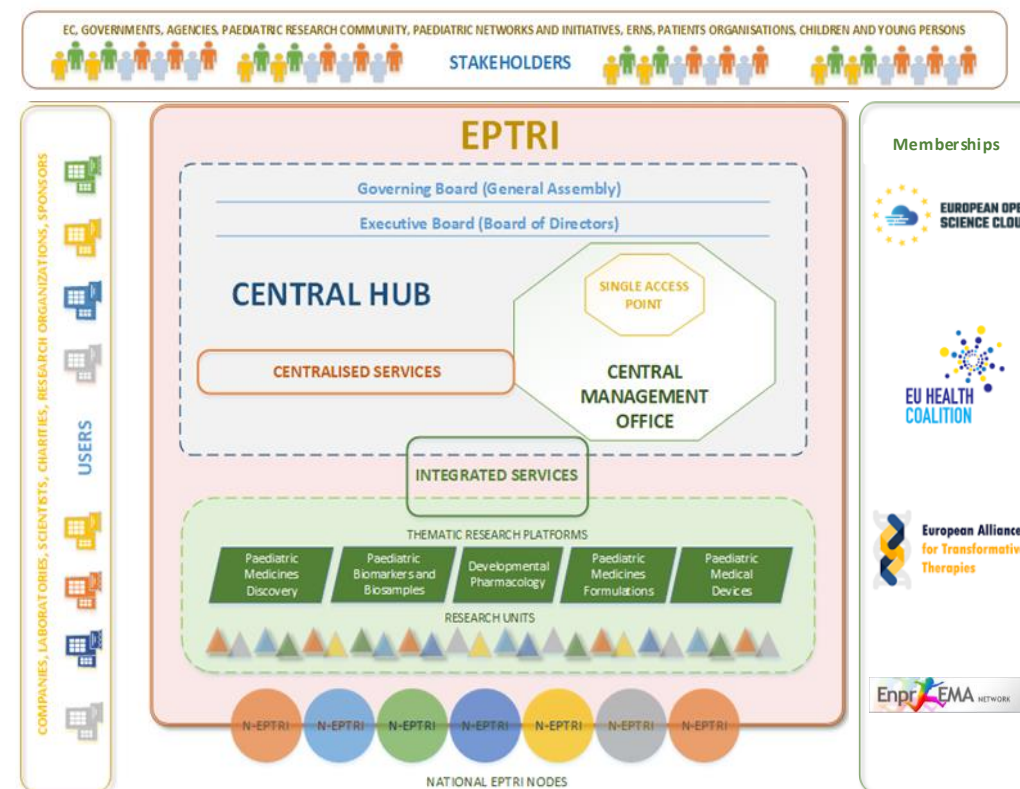


The EPTRI governance is organised through its statutes and internal procedures, and its management involves different bodies includes **General Assembly and Board of Directors** with precise roles and responsibilities.

## UPCOMING TRP's

Advanced therapy medicinal products

Digital Health Technology





# EPTRI PAEDIATRIC RESEARCH SERVICES

**1. INTEGRATED SERVICES** provided through the **TRPs** according to their specific research area of expertise



## **Paediatric Medicines Discovery**

These services integrate basic and applied research across interdisciplinary fields such as medicinal chemistry, structural biology, developmental science, and juvenile animal models to advance paediatric drug development without relying on adult study extrapolation.



## **Paediatric Biomarkers and Biosamples**

These services can be integrated with research infrastructures to enhance networking and create a unified platform, with a special focus on paediatric data and the identification of significant biomarkers in child development.



## **Developmental Pharmacology**

These services can be integrated with screening facilities to evaluate drug effects considering the developmental processes in children



## **Paediatric Medicines Formulations**

This is a unique and specialized service offered by EPTRI. It provides the necessary support to companies that wish to develop paediatric-specific products, ensuring that the formulations are accessible and usable for children.

# EPTRI PAEDIATRIC RESEARCH SERVICES



## Paediatric Medical Devices

EPTRI addresses the lack of dedicated support for paediatric medical device development by assisting academia and small industries with regulatory guidance, ethical considerations, preclinical expertise, and access to developmental physiology insights.



## Advanced therapy medicinal products

**EPTRI is launching** a dedicated Thematic Research Platform (TRP) aimed to support the development of gene therapies by addressing their complexity, cutting-edge nature, regulatory challenges, long-term safety and efficacy concerns, and economic risks due to their small market size.



## Digital Health Technology

**EPTRI is launching** a dedicated Thematic Research Platform (TRP) to significantly improve paediatric data management, address the challenges of data management, promote open science, and improve data accessibility within the life sciences domain, ultimately leading to better research outcomes and better health outcomes for children.

# EPTRI PAEDIATRIC RESEARCH SERVICES

**2. THE CENTRALISED SERVICES** are advice or hands-on activity transversal to the TRPs and intended to support any step of the paediatric research conducted in the basic, pre-clinical and translational settings. These services may be proposed to be conducted in collaboration with other RIs, paediatric networks and initiatives.

- Providing access to paediatric patients' cohorts.
- Providing access to paediatric samples and related data.
- Advice on in-vivo / in-silico nonclinical study design.
- Advice on in-vivo / in-silico clinical study design.
- Advice and Document preparation for regulatory and ethic procedures.
- Advice on preparing HTA reports for paediatric medicines, diagnostic and MDs.
- Advice on Data and sample sharing preparation.
- Supporting application to (public and private) funding opportunities.





# EPTRI IN THE EUROPEAN RESEARCH LANDSCAPE

- **Enpr-EMA** (European Network of Paediatric Research at the European Medicines Agency) - **EPTRI become** a member of **under Category 3**, which includes networks that are specialized in clinical trial methodology and support to **paediatric drug development** which significantly impact the conduct of **high-quality trials**.
- **European Open Science Cloud (EOSC)** - The inclusion of **EPTRI in EOSC as a Member** to enhance collaboration, improve **data sharing**, and **leverage open science resources** to advance paediatric research and **innovative digital services**.
- **EU Health Coalition**- EPTRI is a partner of the EU Health Coalition and contributes to actions aimed at the inclusion of paediatric research in health policies and initiatives
- **European Alliance for Transformative Therapies (TRANSFORM)**- TRANSFORM is involved in lobbying efforts to improve the regulatory environment. EPTRI is a member and actively supports TRANSFORM

# EPTRI IN ACTION



- **EPTRI is a partner in the OrphaDev4Kids project** which implement a complex innovation ecosystem in the **orphan and paediatric Medical Devices** field to **maximise the value-for-patients** by supporting academy, research centres, and SMEs **that invest on paediatric devices development.**

## With specific objectives:

- Grant access to a **paediatric MDs Platform** for Users in need to receive dedicated research and development services
- Implement a **complex ecosystem** including tools, facilities, and supportive services
- Develop **three orphan and paediatric case studies**, one dealing with **osteogenesis imperfecta** and two concerning **Cyanotic Congenital Cardiac Diseases.**







## FUTURE DIRECTIONS

- **EPTRI application for ESFRI (European Strategy Forum on Research Infrastructures) 2026 Roadmap**

Under the polish leadership, the inclusion of EPTRI in the ESFRI 2026 Roadmap will **represent the recognition of EPTRI's efforts to boost paediatric therapy development** and will strengthen EPTRI's mission of implementing competences, research strategies, access to key technologies, standardized models, and analytical tools and to **offer to public and private stakeholders qualified basic, preclinical and translational research services covering paediatric therapy discovery and development**, as well as the role of **digital health technologies** in paediatric research.

**The application will focus on the following topics:**

- Focus on ATMPs,
- Focus on MDs not only for diagnostics, but for other purposes, as long as they are targeting the paediatric population,
- Focus on digital health applications
- Development of an innovative clinical research platform to support cutting-edge methodological approaches.



## WE ARE HAPPY TO WELCOME YOU TO JOIN EPTRI AISBL

EPTRI members aim to increase knowledge on human development and ontogeny and to provide high quality services to support the discovery of new therapies and devices for children

To join EPTRI AISBL, please complete the [Association Form](#), sign it and send it back via email to [coordinator@eptri.eu](mailto:coordinator@eptri.eu).

EPTRI continuously maps the paediatric expertise and competences available all over EU and non-EU countries through online surveys to identify expert groups and organisation suitable to become EPTRI service providers

Through the following [link](#) you can provide information about the nature of your organization, its legal status and the services it can provide.

If you have any additional questions before committing to a rewarding membership with EPTRI AISBL contact us at [coordinator@eptri.eu](mailto:coordinator@eptri.eu)

A group of diverse children are playing outdoors with colorful water balloons. In the foreground, a boy in a green plaid shirt is pouring water from a yellow balloon into a blue cup. Next to him, a girl in a grey tank top is holding a green balloon. Other children in the background are also playing with balloons. The scene is set in a park-like area with trees and a wooden fence.

LET'S KEEP WORKING TOGETHER TO  
ADVANCE PAEDIATRIC RESEARCH !  
THANK YOU



## FUTURE DIRECTIONS



*Nothing in life is to be feared, it is only to  
be understood.  
Now it is the time to understood more, so  
we may fear less.*

*Maria Curie-Skłodowska*