

The Birth of EPTRI

Promoting paediatric research and providing optimal support to facilitate the accruing of knowledge in the many research fields necessary to guarantee the access to high quality and safe paediatric medicines, technologies, and devices

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Paediatric Research

specific answers for specific needs

“EPTRI arises from the need to find answers to the serious lack of medicines for children in EU and worldwide”

Adriana Ceci

The European Paediatric Translational Research Infrastructure (EPTRI) started as a project coordinated by Consorzio per Valutazioni Biologiche e Farmacologiche (CVBF – Clinical Validation from Biopharmaceutical Findings) 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.



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

26 Partners from 19 EU/ non EU countries



Romanian Angel Appeal



swiss
clinical
trial
organisation



THE PROJECT PHASES

CONTEXT ANALYSIS PHASE

Performed in all the five technical and scientific domains identified. The strategic approach and possible gaps to be covered were defined by enquiring the scientific Communities, the concerned national Authorities and many other Stakeholders

OPERATIONAL PHASE

The different components of the new RI were planned, including governance model, strategies for interaction with national Authorities and the existing RIs, the IT-architecture model, services to be provided and the business plan

FEASIBILITY PHASE

Virtual exercises simulated the operations of EPTRI to work with:

- Scientists
- Regulators
- Companies
- Patients (YPAGs)
- Other RIs and Networks

EPTRI at present



EPTRI (European Paediatric Translational Research Infrastructure) is a **non-profit research organisation** incorporated in the form of an Association Internationale Sans But Lucrative (AISBL) governed by Belgian law, **based at Leuven University**.

EPTRI is a **pan-European initiative** involving hundreds of research units gathered to **boost the paediatric research ecosystem** and **provide services for the development of medicines for children**.

EPTRI promotes processes allowing stakeholders' engagement and knowledge translation by integrating the perspectives of young patients and families, policymakers and payers, regulators and researchers, agencies and charities, academia and industries.

Our Vision



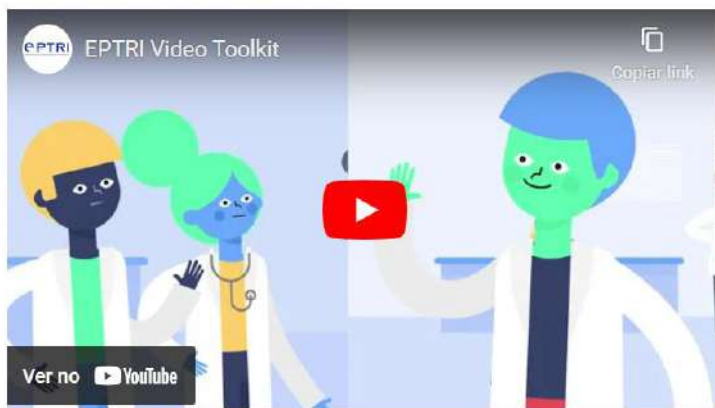
To increase knowledge on children growth and ontogeny characteristics relevant for the identification of novel therapies dedicated to children and to provide services for their development.

Our Mission



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

Projects / Initiatives



EPTRI Toolkit
On Basic and Translational Paediatric Research for Patients and Families

Surveys



Paediatric Manifesto



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

TRP MD webinars



Projects / Initiatives

OrphaDev4Kids



The development of orphan paediatric medical devices (MDs) faces unique challenges. **The OrphaDev4Kids aims to address these unmet needs.**

General objective: implement a complex innovation ecosystem in the orphan and paediatric MDs field to maximise the value-for-patients of the research and development projects by supporting academy, research centres and SMEs that invest on paediatric devices development.

Specific objectives:

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



Governance

The EPTRI governance is organised through its statutes and internal procedures, and its management involves different bodies with precise roles and responsibilities.

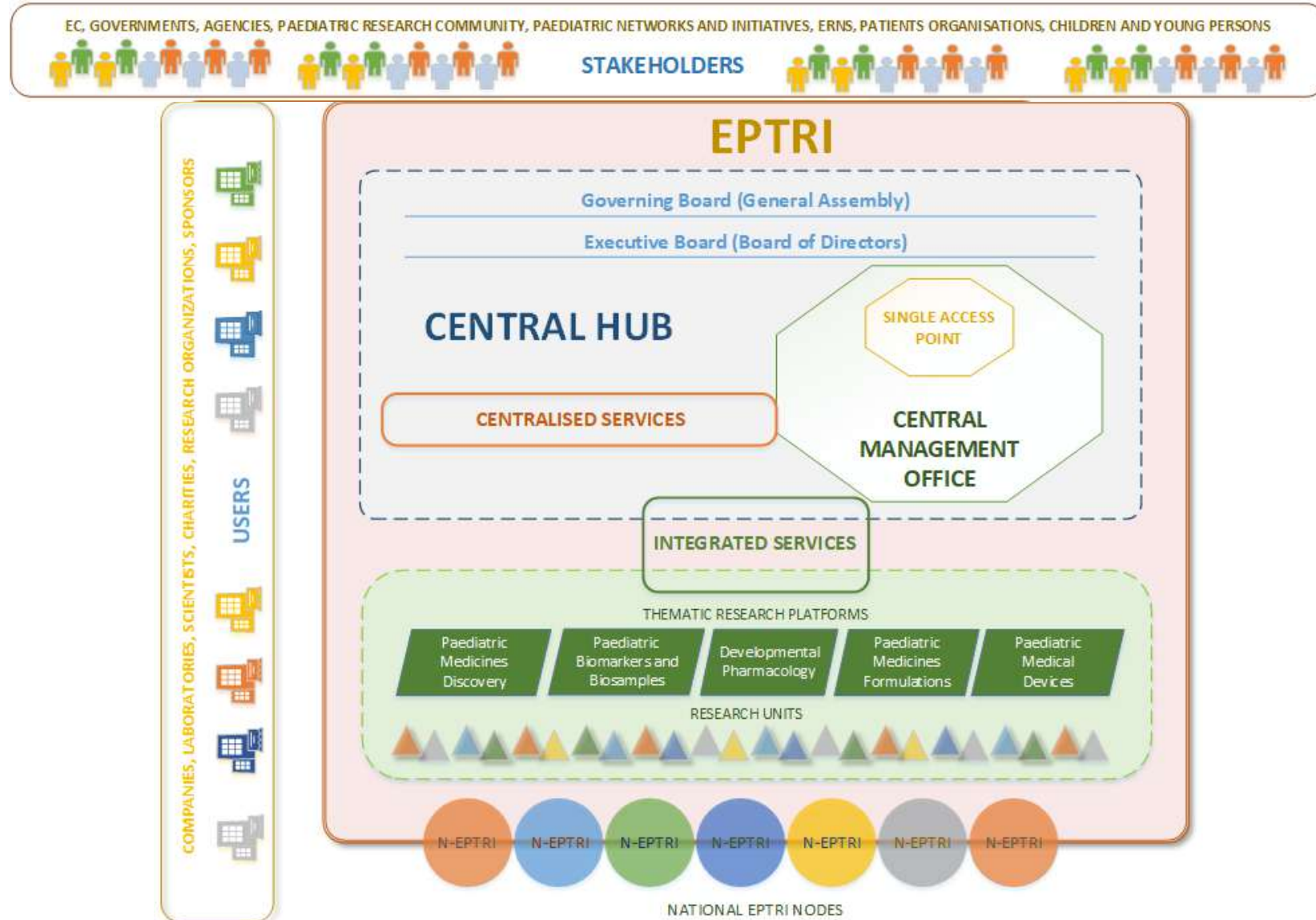
CENTRAL HUB AND CENTRAL MANAGEMENT OFFICE

GENERAL ASSEMBLY

BOARD OF DIRECTORS

THEMATIC RESEARCH PLATFORMS (TRPs)

NATIONAL NODES



Paediatric Research Services



The EPTRI 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.**

Centralised services are directly provided by the EPTRI Central Management Office (CMO) with dedicated experts from EPTRI Members. These services may be conducted in collaboration with other RIs, paediatric networks and innovative projects.

Centralised services

- 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.

Paediatric Research Services



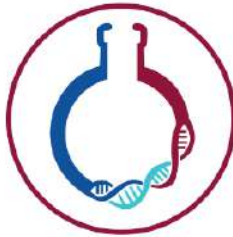
Integrated services are provided by five **Thematic Research Platforms**

<https://eptri.eu/paediatric-research-services/research-units-services-catalogue/>

Integrated services



Paediatric Medicines
Discovery



Paediatric
Biomarkers &
Biosamples



Developmental
Pharmacology



Paediatric Medicines
Formulations



Paediatric Medical
Devices

Become a Service Provider

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. If your Institution is interested to join the EPTRI AISBL as a service provider, please go to our website or contact us.

Website: <https://eptri.eu/>

<https://eptri.eu/join-eptri-as-a-service-provider/>

Thank you!

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