

ePTRI

EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE



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European Paediatric Translational Research Infrastructure (EPTRI)

Donato Bonifazi – EPTRI Coordinator

EPTRI webinar: biotechnology to bring innovation in the paediatric drug development, 2nd October 2020

The event is part of the European Biotech Week 2020

PART OF

EUROPEAN
BIOTECH
WEEK



INNOVATION IS IN OUR GENES

The ID-EPTRI project



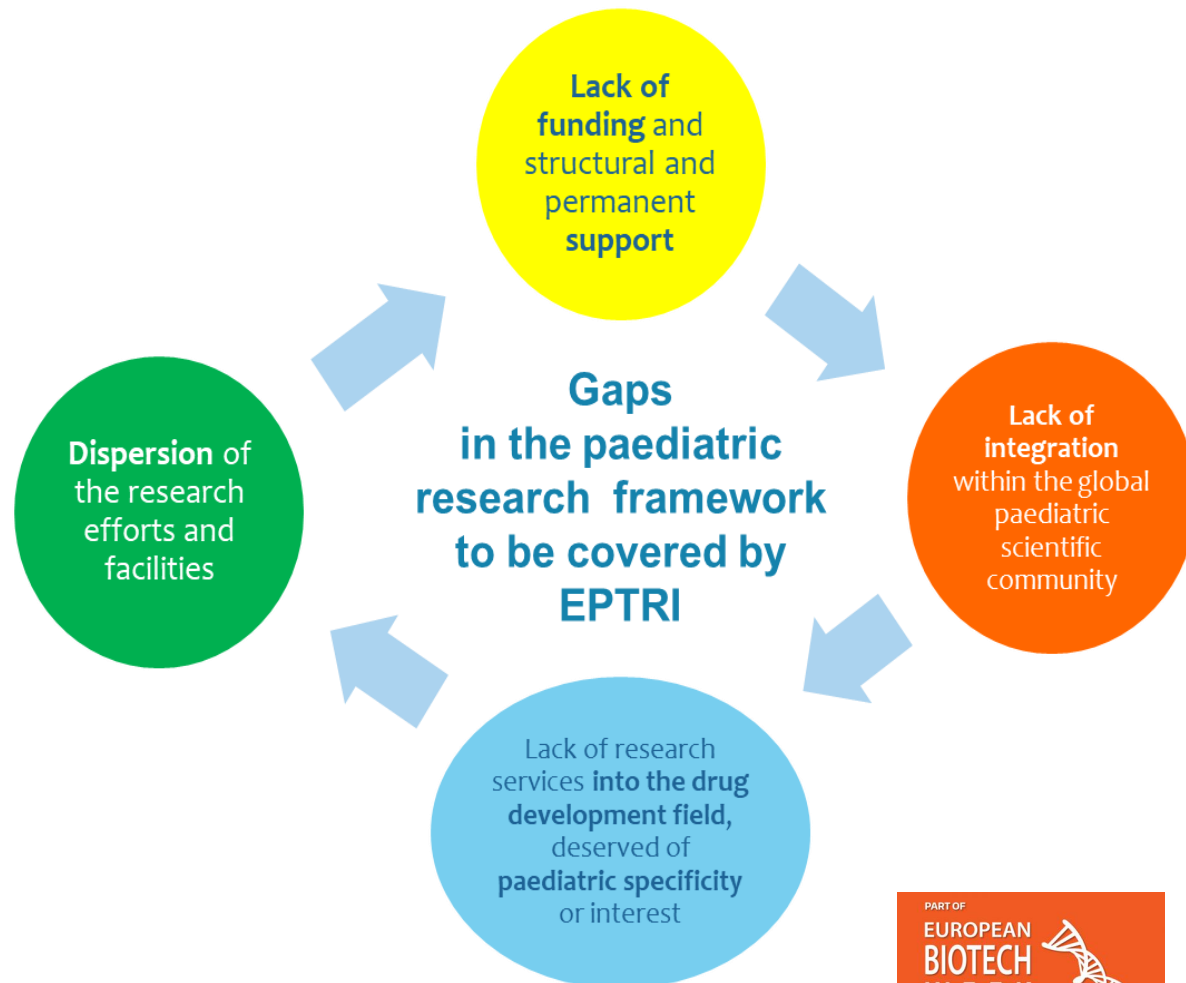
- ❖ **Funds:** Horizon 2020 EU Research and Innovation programme (INFRADEV-1-2017)
- ❖ **Coordinator:** Consorzio per Valutazioni Biologiche e Farmacologiche
- ❖ **Start date of the project:** 1 January 2018
- ❖ **29 partners** from 21 EU/non-EU countries
- ❖ **330 research units** from 259 Institutions candidate as EPTRI providers from 29 EU / non-EU countries

EPTRI - European Paediatric Translational Research Infrastructure

EPTRI is proposed as a new infrastructure, dedicated to paediatric research, aimed to cover some critical gaps using the instruments of the EU-RIs (ESFRI).

EPTRI aims to provide

- structural support** to researchers
- access to **specific paediatric research services**
- research platforms for **collaborative work**
- increased knowledge** on many scientific topics related to preclinical and translational paediatric research



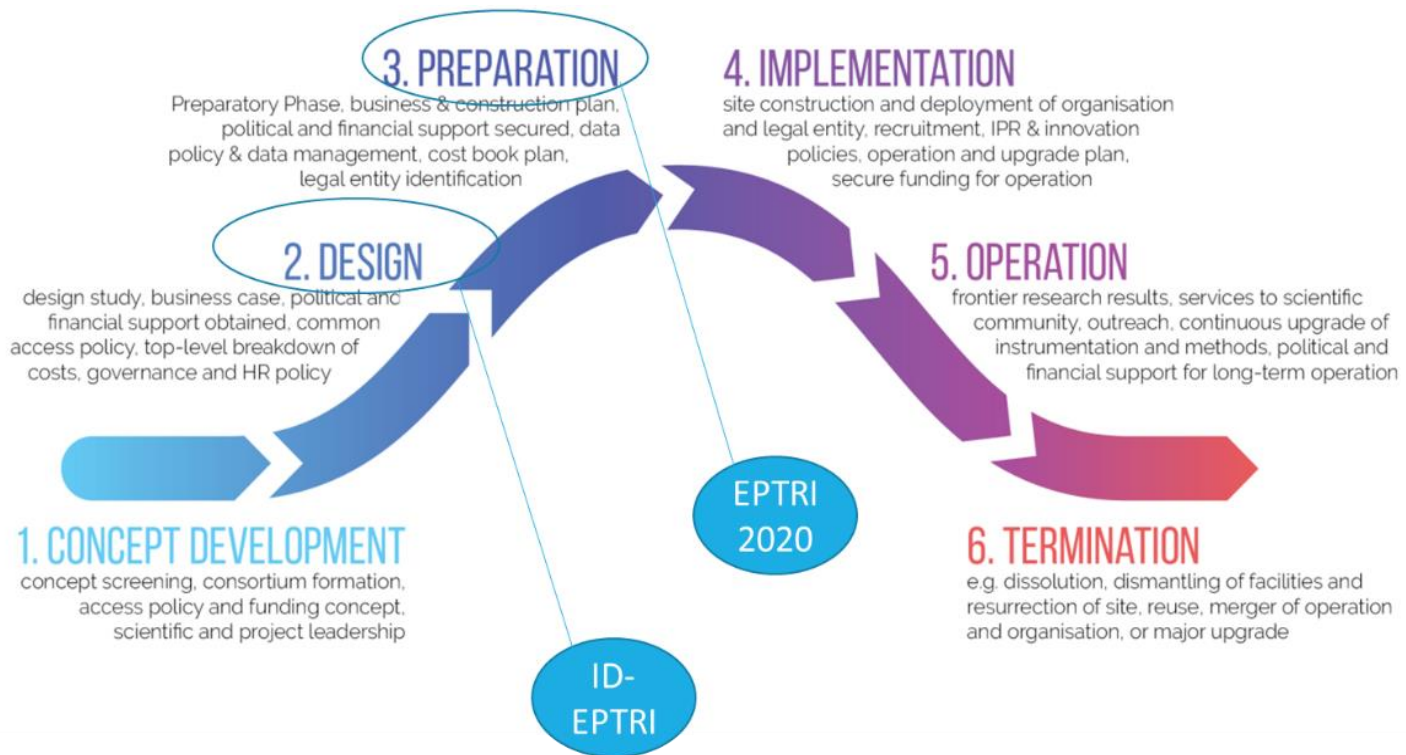
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The different phases of a research infrastructure

EPTRI has concluded the **DESIGN** phase and started the **PREPARATORY** phase to reach the ERIC status



EPTRI concept

To cover the wide range of needs for paediatric drug development, it is necessary to **aggregate all the available resources** and to **integrate them in a common effort**.



Aggregate a large research community focused on **preclinical and translational research** for paediatric medicines

EPTRI contribution

Promote an **integrated approach to paediatric drug development** by contributing to clinical studies implementation

Implement in the paediatric medicines discovery and development fields, **innovative methods and technologies currently not available or underused**

New target for paediatric/rare diseases
Pre-clinical package
MoA and effect size
Developmental
Pharmac/phase 0 study
Paediatric Biomarkers
Formulation



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Integration with ESFRI landmarks

In designing EPTRI, the relationships and possible overlapping with other existing ESFRI RIs have been carefully considered.

Services relevant for children needs, proposed in EPTRI, **have never been developed in other RIs** (e.g. ontogeny driven studies, developmental pharmacogenetics and related disease targets, micro dosing, placental platforms, palatability assessment, etc.)

Services provided by other RIs in research areas relevant for EPTRI (e.g. biomarkers, targets identification, animal models, cellular models, etc.) are **not tailored to children's needs**

Some **basic research activities**, developed in EPTRI, **have been declared not of interest for other RIs**: research on human development mechanisms relevant for paediatric diseases, human in vitro fertilization, safety of excipients



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EPTRI - CONCEPTUAL DESIGN REPORT

Context analysis results

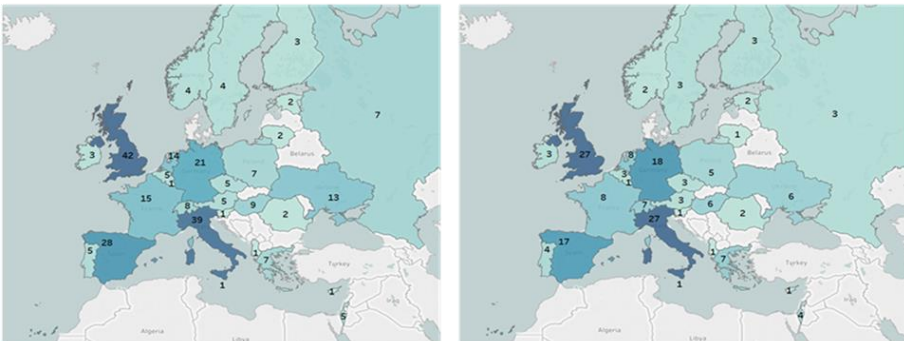
EPTRI scientific community, users and stakeholders identification

- 337 research groups** providing indication on scientific services possibly offered by EPTRI
- 259 research Institutions**, distributed across **29 countries** in the European- non European area
- 287** contribution received from research Institutions not associated with EPTRI (users' survey)
- Stable **relationship with research initiatives** having paediatric interest (c4c; EJP-RD; ERNs)
- Collaboration established with some **Biomedical Landscape RIs**
- 155** contribution from different stakeholders from **31 countries**

Map of Research Institutions and Units in EPTRI

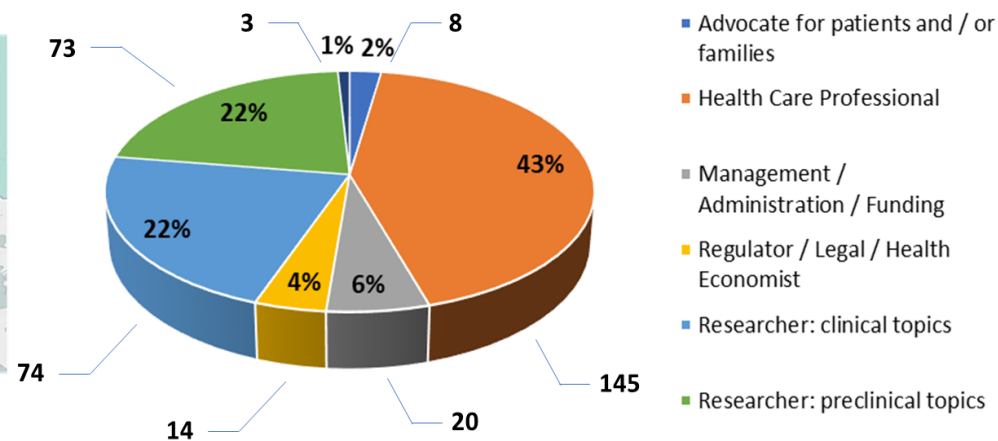
Research Units by country

Research Institutions by country



Notes: Update November 2019, RUs answering to more than one WP have been considered once in the maps, RUs referring to Italian CNR have been counted once.

Distribution of the targeted users divided by sort



EPTRI- CONCEPTUAL DESIGN REPORT

Architecture Design

EC, GOVERNMENTS, AGENCIES, PAEDIATRIC RESEARCH COMMUNITY, PAEDIATRIC NETWORKS AND INITIATIVES, ERNS, PATIENTS ORGANISATIONS, CHILDREN AND YOUNG PERSONS

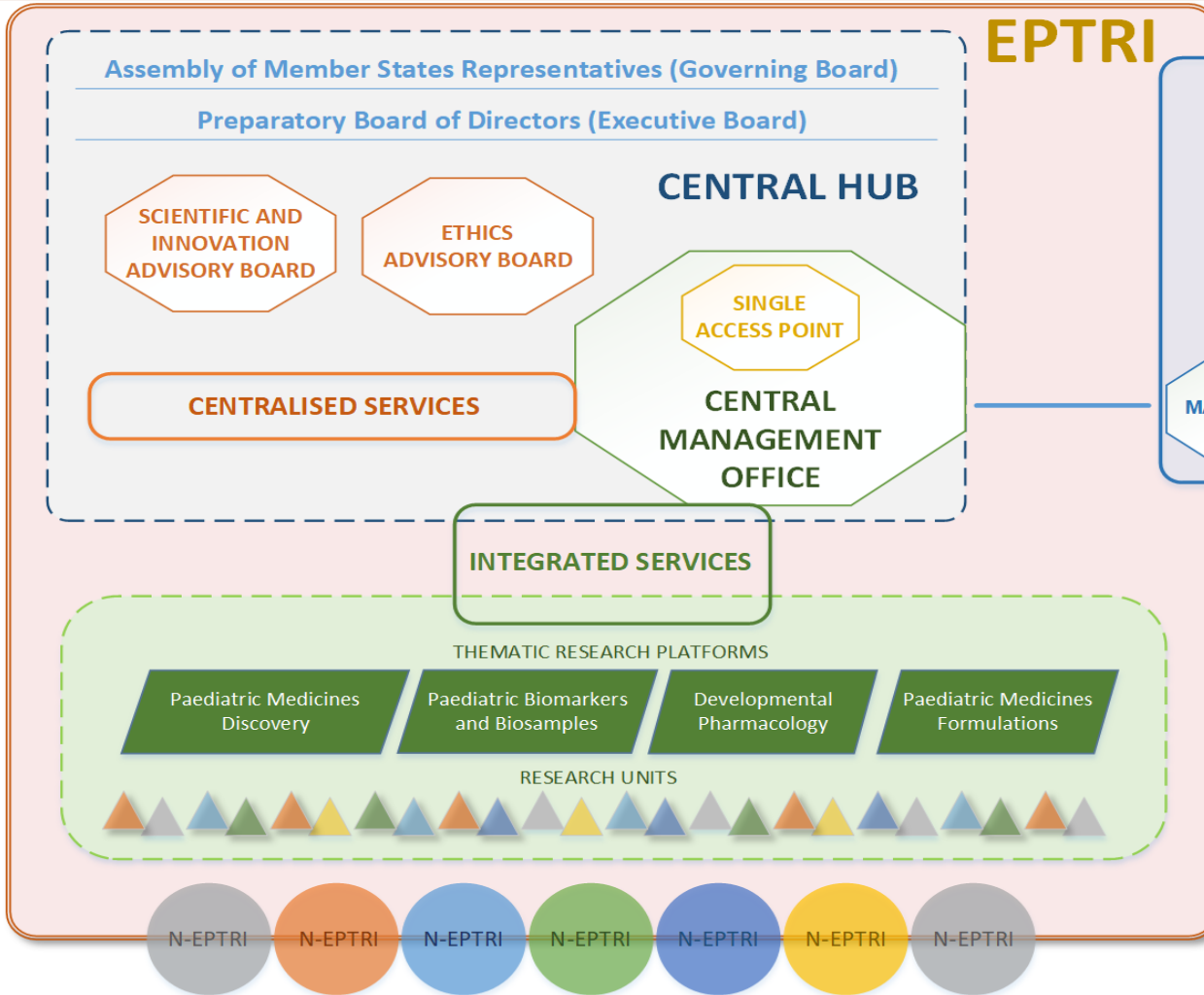


STAKEHOLDERS

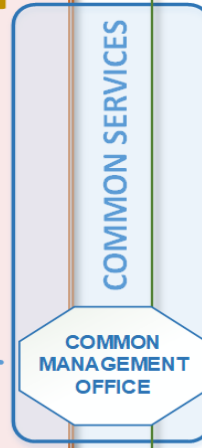
COMPANIES, LABORATORIES, SCIENTISTS, CHARITIES, RESEARCH ORGANIZATIONS, SPONSORS



USERS



NATIONAL EPTRI INFRASTRUCTURES



EPTRI- CONCEPTUAL DESIGN REPORT

SERVICES FROM EPTRI



- Providing services allowing **paediatric drug development** processes



Medicines discovery and preclinical research

Paediatric biomarkers

Developmental pharmacology

Paediatric formulations

Medical devices

- Centralised services including use results of basic and translational research to **underpin paediatric clinical trials and studies thanks to a strong collaboration with c4c and paediatric networks**

- Collaborative services with other **Biomed RIs**



EPTRI integrated services

<p>Paediatric Medicines Discovery N° of Countries: 19</p>	<p>Developmental Pharmacology N° of Countries: 13</p>	<p>Paediatric Biomarkers and Biosamples N° of Countries: 23</p>	<p>Paediatric Medicines Formulations N° of Countries: 12</p>
<p>In vitro screening of novel drugs using paediatric cellular targets</p>	<p>Microdosing to establish the “in vivo” PK profile of the new drug</p>	<p>Organisation and management of paediatric biosamples and related data for paediatric studies</p>	<p>Pre-formulation advice and Pre-formulation studies</p>
<p>In vitro pre-clinical studies (effect, efficacy, biomarkers, etc.) in paediatric cell models</p>	<p>In vitro models to study ontogeny of drug disposition (ADME)</p>	<p>Biomarker identification and levels’ measurement in paediatric sample set of appropriate ages</p>	<p>Formulation of drug for paediatric use for enteral routes of administration</p>
<p>Access to the neonatal and juvenile animal models to screen novel drug.</p>	<p>Placental studies</p>	<p>Bioinformatics for the analysis of the data</p>	<p>Formulation of drug for paediatric use for non-enteral routes</p>
<p>In silico screening of novel drugs for specific paediatric targets</p>	<p>In vivo toxicity juvenile animal studies</p>	<p>Validation of biomarkers for paediatric use</p>	<p>Assessment and design of drug delivery systems</p>
<p>In silico prediction of properties & toxicity for new molecular entity of paediatrics interest</p>	<p>Paediatric modelling and simulation.</p>		<p>Drug delivery design for enteral routes and for non-enteral routes</p>
	<p>Sensitive analytical methods adapted to paediatrics</p>		<p>Paediatric in vitro and in vivo palatability assessment</p>

EPTRI centralised services

The services planned to be provided by EPTRI are:

Advice on translation to clinical phases

Research project management

Document Repository and e-Library

Centralised services

Central Management Office

Advice on the design and requirements of not clinical specific studies/experiments

Scouting and application to funding opportunities

Access to e-learning and training

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EPTRI common services



**Common
services**



Collaboration with other
Research Infrastructures



ELSI paediatric service
(with BBMRI) also based on
previous TEDDY experiences



Paediatric data
interoperability service
(with ELIXIR)



Where we are now



Context analysis phase 2018

Design phase 2019

Feasibility phase 2020

Conceptual Design Report (Aug 2020)

Preparation phase ongoing-2023

ESFRI 2021 Roadmap (Sept 2020)

INFRAIA-02-2020 (May 2020)

eP TRI

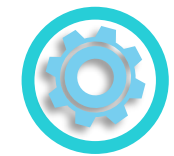
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The steps taken so far: INFRAIA

EPTRI participated to the INFRAIA-02-2020 call submitting a proposal on the 14th of May 2020 to fund the activities of the Preparation phase as:



Networking activities

★ To strengthen the coordination and collaboration between **scientific community and stakeholders**



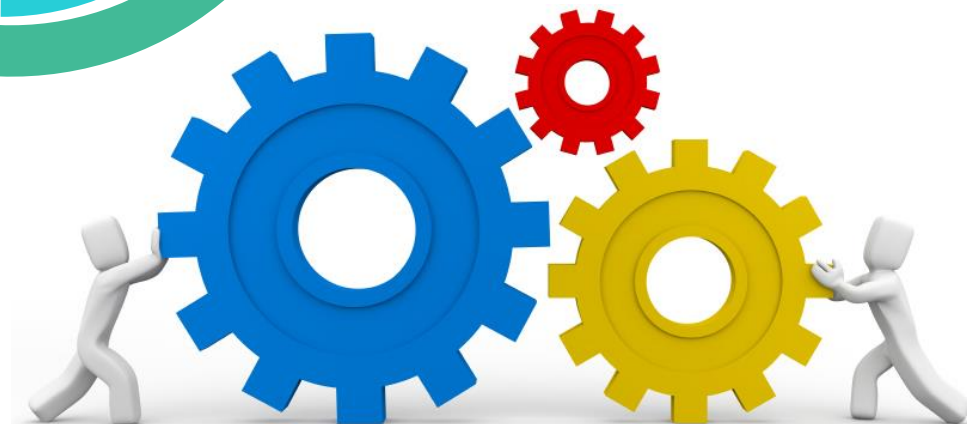
Trans-national access services

★ To provide efficient trans-national **access** to advanced **research services** provided by the research organizations participating in EPTRI



Joint research activities

★ To develop services enabling **basic, preclinical and translational paediatric research**



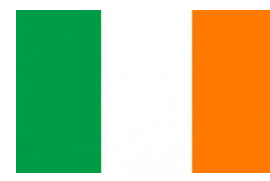
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The steps taken so far: the ESFRI Roadmap

EPTRI applied to the ESFRI Roadmap 2021 on September 9th 2020 to be included in the Roadmap and be officially recognised as a biomedical RI.

To this aim, EPTRI received letters of political support from 18 countries, 16 of which from the national authority relevant for RI.



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