EPTRI

EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE

European Paediatric Translational Research Infrastructure (EPTRI)

Donato Bonifazi – EPTRI Coordinator

Enpr-EMA Meeting – 28th September 2020

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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 Infrastrtucture

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

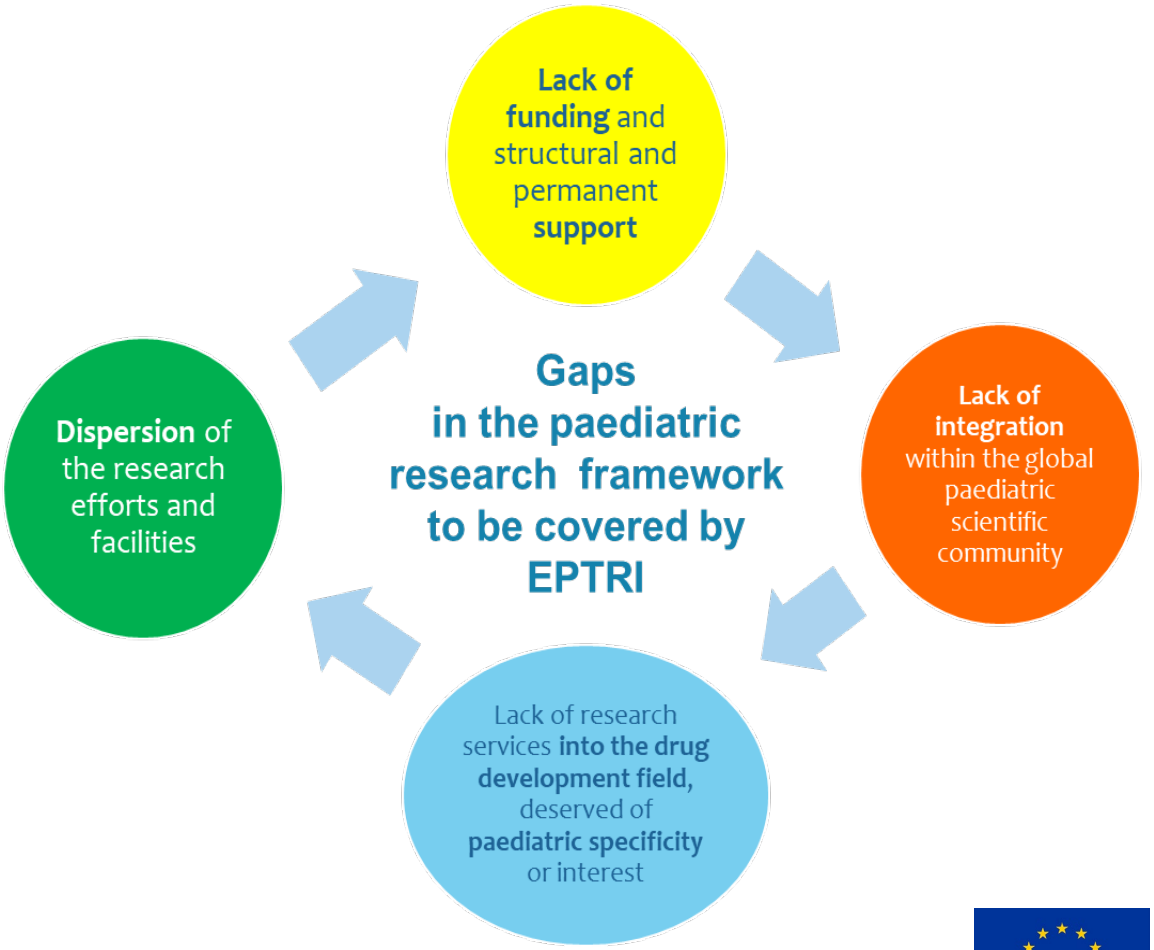
EPTRI aims to provide

-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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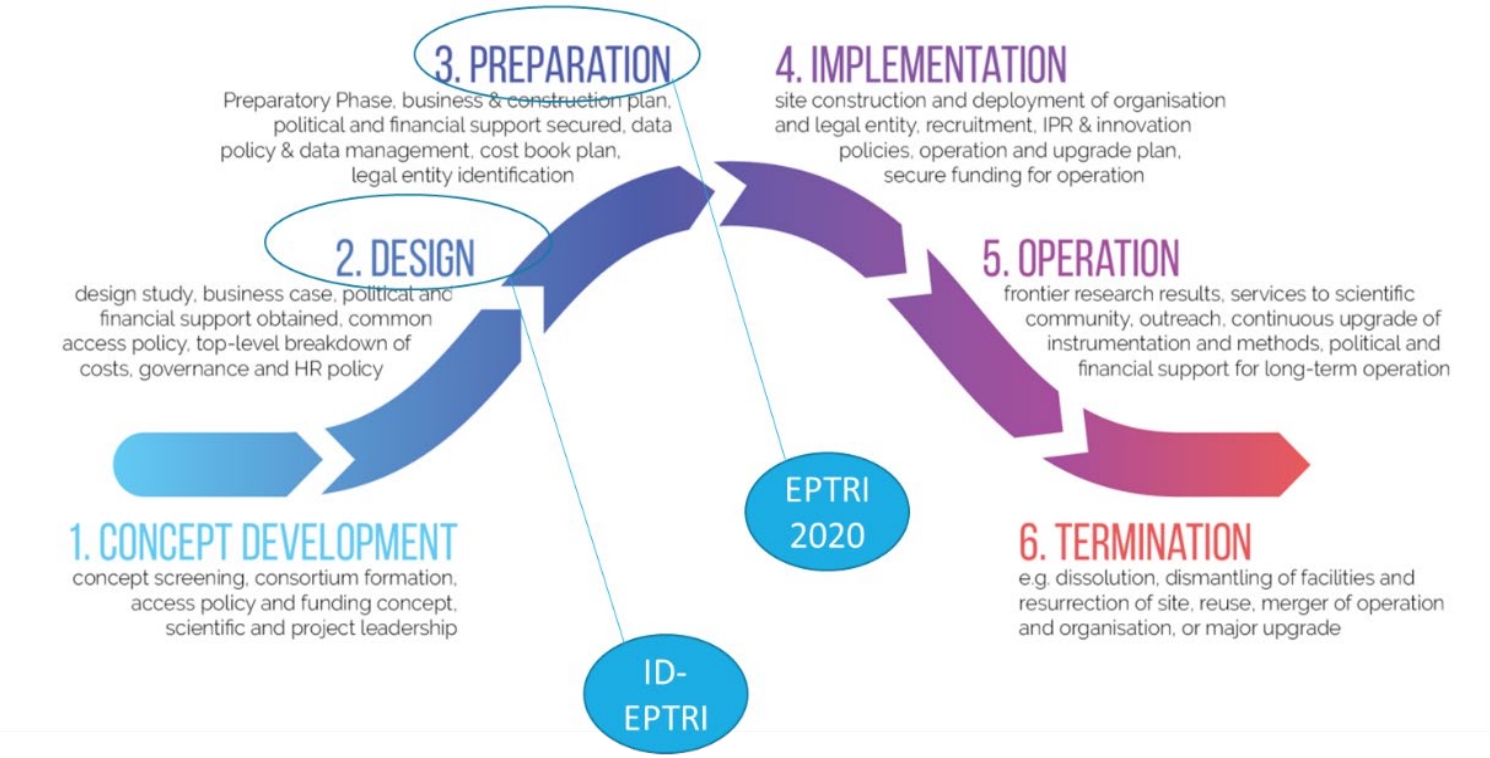
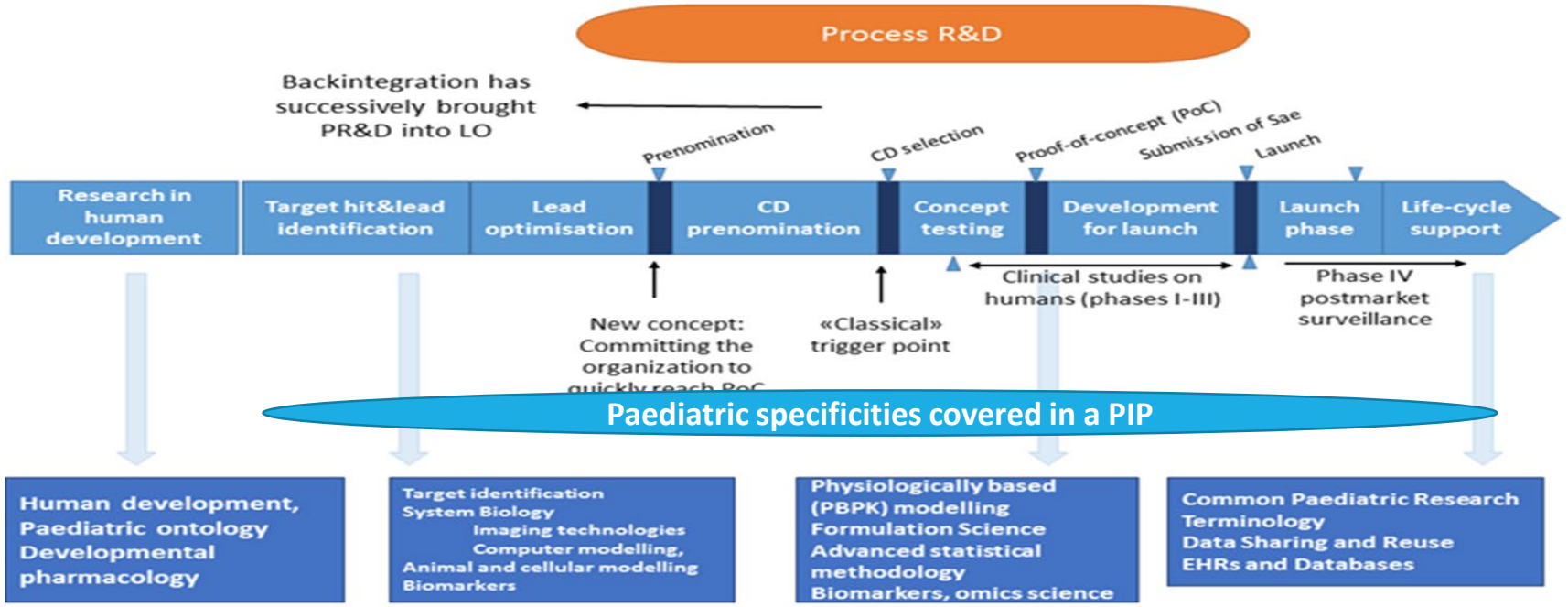
The different phases of a research infrastructure

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



EPTRI in the Paediatric medicines development process framework



Medicines development is a complex process involving different disciplines and competences in different stages.
In the paediatric setting it **SHOULD** include basic knowledge on human development and ontogeny effects evaluation.

Each stage needs information from previous stages.

In particular, the clinical stage can only start when a good consolidated basis of information exists

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.**

EPTRI contribution

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

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

To 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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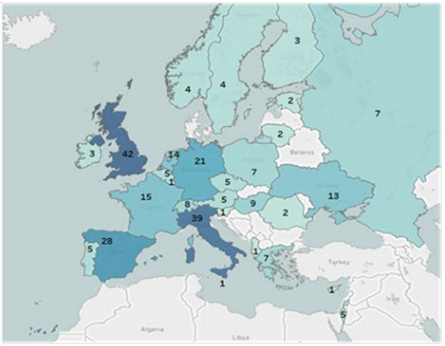
EPTRI- CONCEPTUAL DESIGN REPORT
Context analysis results

EPTRI scientific community, users and stakeholders identification

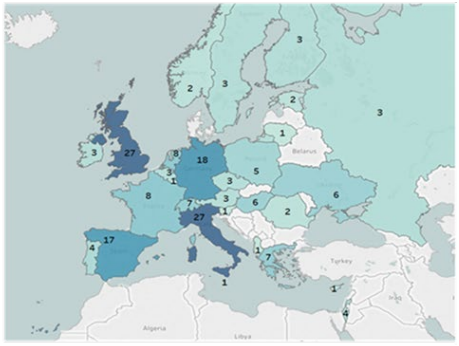
259 research Institutions, distributed across **29 countries** in the European- non European area
337 research groups providing indication on scientific services possibly offered by EPTRI
Stable **relationship with research initiatives** having paediatric interest (c4c; EJP-RD; ERNs)
Collaboration established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

Map of Research Institutions and Units in EPTRI

Research Units by country

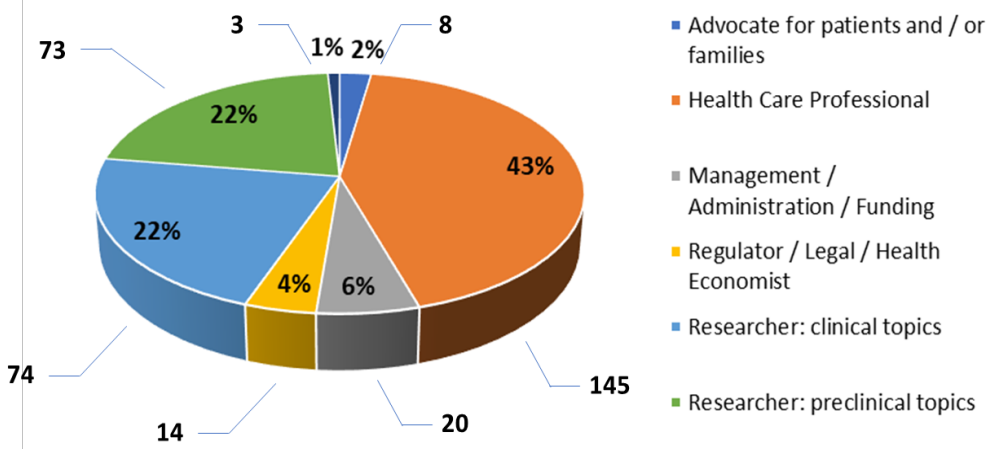


Research Institutions by country



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 counnted once.

. Distribution of the targeted users divided by sort



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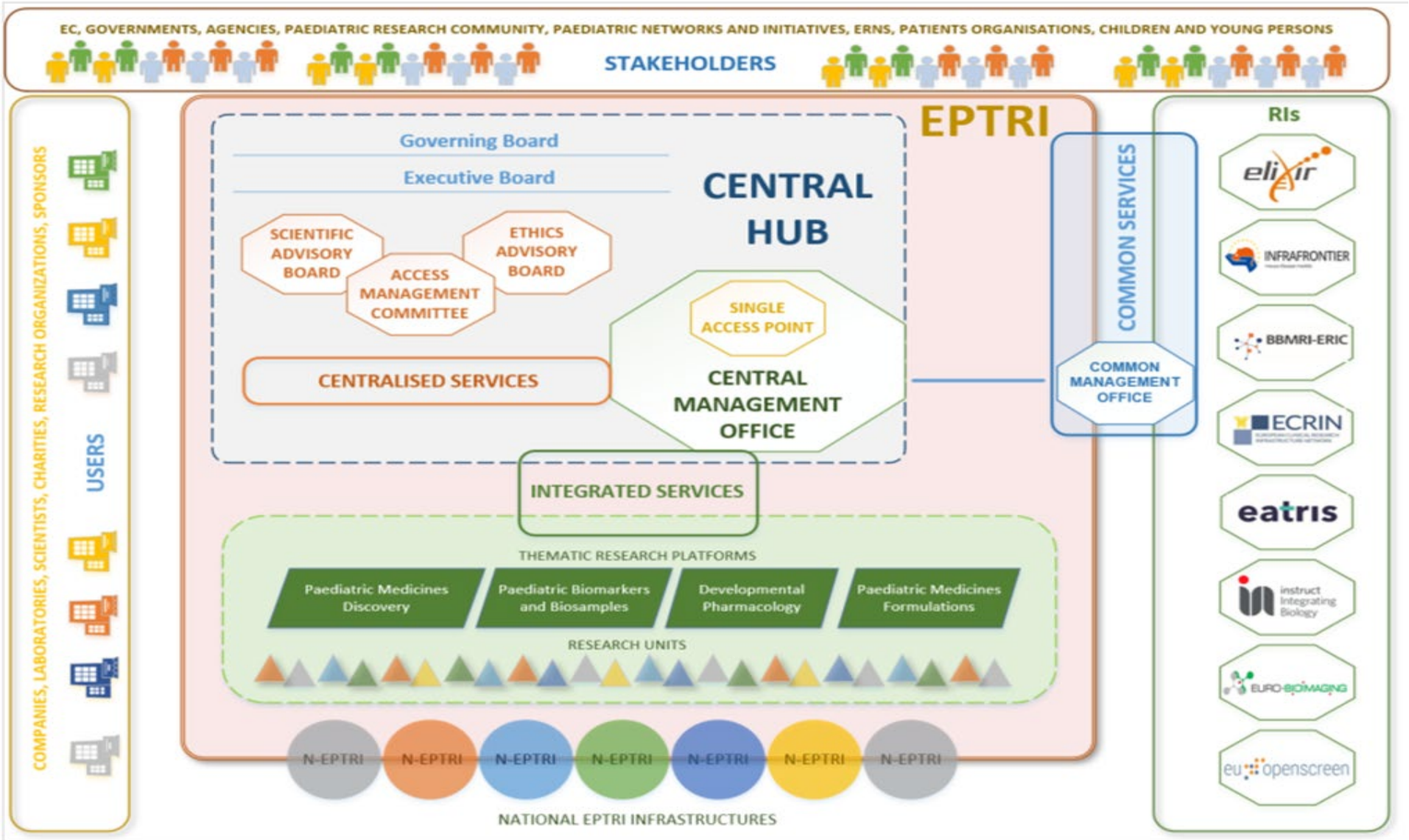
EPTRI- CONCEPTUAL DESIGN REPORT
Architecture Design



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**

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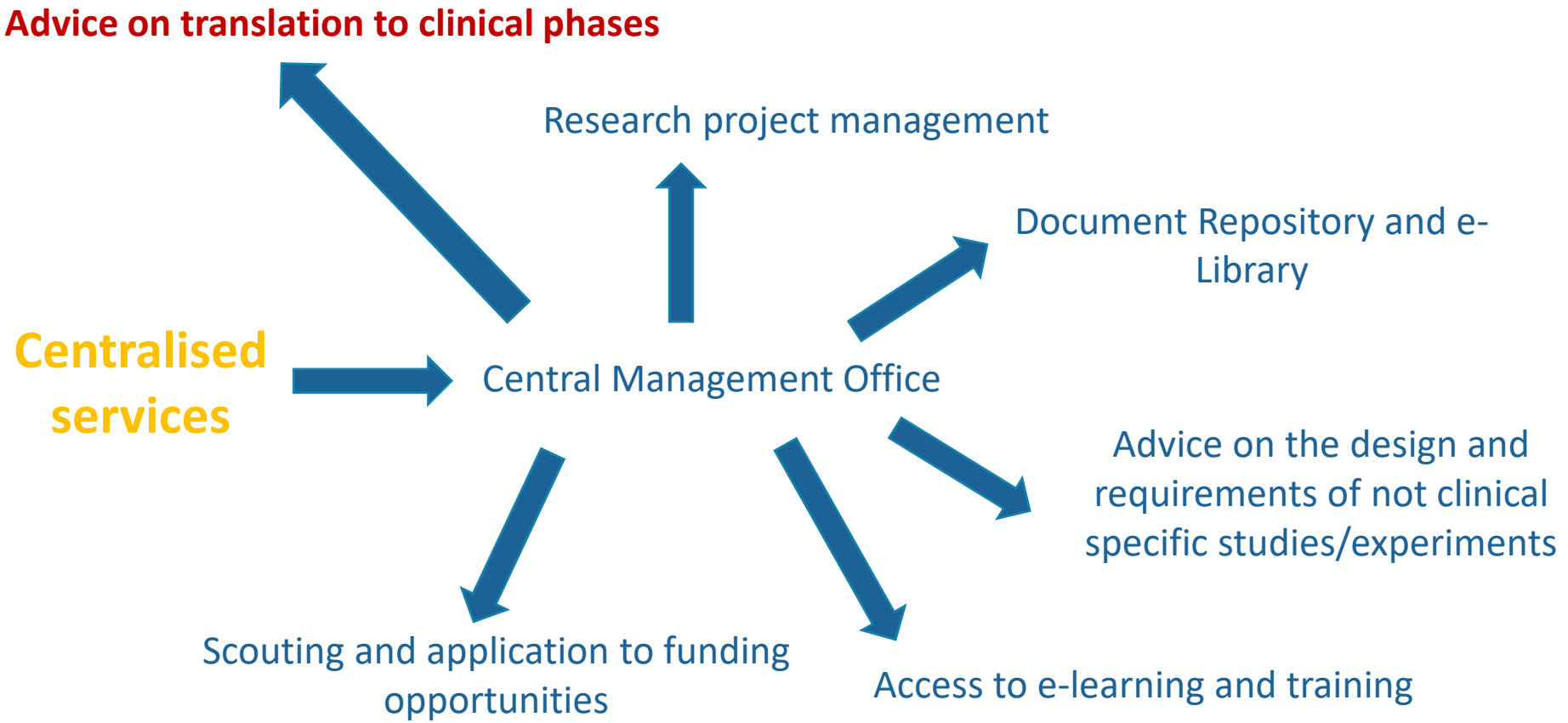
EPTRI integrated services

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

EPTRI centralised services

The services planned to be provided by EPTRI are:



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)

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Where we are now

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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**

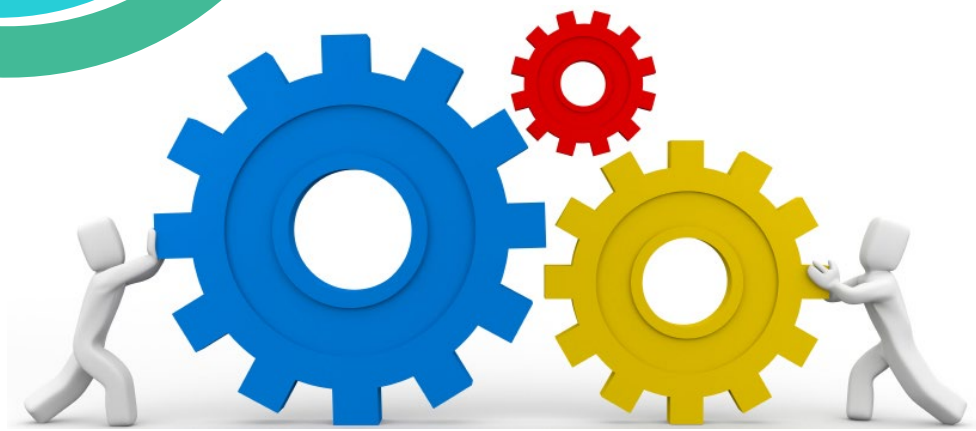
Trans-national access services

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

EPTRI INFRAIA 02-2020

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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Thank you for the attention