ePTRI

European Paediatric Translational Research Infrastructure

This project has received funding from the European Union's Horizon 2020 research and innovation Programme under Grant Agreement n. 777554

EPTRI

European Paediatric Translational Research Infrastructure

EPTRI AIMS AND CONTEXT

Minors represent 20% of the European population and their health protection is one of the most important priorities and challenges for Europe. Nevertheless, children are often excluded from the advantages of the scientific innovation, such as new medicinal products, while around 50% of the medicines administered to young patients have been developed for adults and not even been tested specifically for paediatric use.

The needs of babies, children and adolescents need to be met on their own terms. Considering the many biological and physiological changes occurring during human development they represent a "special" population that is indeed made of several different age groups with variable pharmaco-toxicological characteristics. For this reason, it is important to **include babies, children and adolescents in the development of new medicines and therapies by performing paediatric research that is adapted to the needs of babies, children and adolescents within, or parallel to research conducted in adults.** The many innovative technologies currently adopted in the drug development process will facilitate this work.

EPTRI (European Paediatric Translational Research Infrastructure) is an EU funded project aimed to design the framework for a new infrastructure dedicated to paediatric research that will work to accelerate the paediatric drug development processes **from medicines discovery, biomarkers identification and preclinical research to developmental pharmacology, age tailored formulations and medical devices. The final goal** is to facilitate the translation of the acquired new knowledge and scientific innovation into paediatric clinical studies phases and medical use.

EPTRI will be an **open science space allowing researchers to work together** without geographical, institutional or financial barriers and a system of many interconnected research areas, that will, contribute to bring new paediatric medicines on the market according to recognised medical priorities and innovation trends. EPTRI will also promote processes allowing **knowledge translation to the industrial sector** and supporting paediatric drugs development within the existing industrial strategies.

EPTRI is also expected to positively impact on the social and ethical aspects, since it will address the theme of research for a vulnerable and neglected population and will involve paediatric patients' representatives and Young Persons Advisory Groups (YPAGs) in its advisory bodies to include their point of view in the different activities planned by the future paediatric research infrastructure.

EPTRI is a pan-European initiative which involves 29 partners from 21 EU and non-EU countries, including non-profit research organisations, top-level universities, scientific and clinical centers of excellence. According to the survey conducted to map paediatric research facilities and competences, **more than 300 research units declared their availability to provide technologies, services and paediatric expertise**, and have been grouped in the following Thematic Research Platform: Paediatric Medicines Discovery, Paediatric Biosamples and Biomarkers, Developmental Pharmacology, Paediatric Medicines Formulations and Medical Devices.

Three types of services are foreseen in EPTRI: centralised services, integrated services, common services.

For more details please consult our web site where you will find all the information about the EPTRI project.

https://eptri.eu/

EPTRI SINGLE ACCESS POINT AND CENTRALISED SERVICES

EPTRI operational activity will be based on a HUB and Spoke model with coordination and networking activities managed at Central HUB level. **The central HUB will also act to bridge EPTRI and the paediatric initiatives focused on clinical steps to make the whole paediatric drug developmental process more efficient and strategic.** IT technologies and tools will be used intensively to maintain a seamless communication and data sharing within the distributed research units.

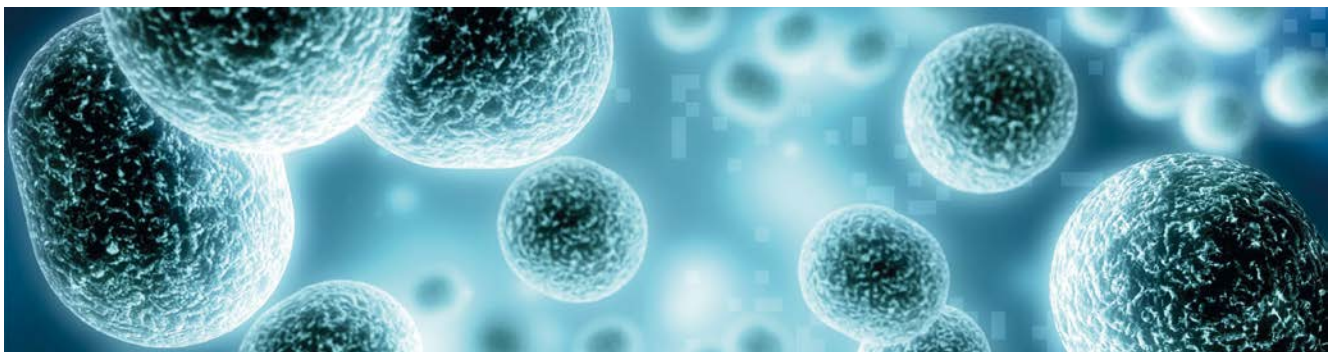
In addition to the coordination and networking activities, the Central HUB will be in charge of:

- **Single Access Point (SAP) management**, including provider identification and contract facilitation activities.
- Management of the legal aspects regarding **contracting and IP protection** between users and research units providing services.
- Management of operational contacts with the concerned paediatric initiatives to translate EPTRI capacities in **support to clinical paediatric studies.**

Requests for service will be received and managed through the Single Access Point that will distribute them according to the nature of the request.

At Central Hub level, some **'Centralised Services'** are foreseen. Those include services which for their own nature are best supplied in a centralised manner. Those are:

- Knowledge and technology transfer
- Document Repository and Libraries for scientific documents archiving and consultation
- Service to provide scientific advice on EPTRI specific issues (studies dedicated to human development, identification of new strategic lines of interventions, application of new technologies to drug development, etc.)
- Service for scouting of research funding opportunities in topics related to the activities of EPTRI
- Training and education, including staff exchanges and e-learning modules.



EPTRI THEMATIC RESEARCH PLATFORMS AND INTEGRATED SERVICES

Integrated Services will be provided through the thematic research platforms in which the key enabling technologies and expertise are grouped and integrated by each research unit under a specific framework agreement, using their own scientific competencies, facilities and equipment.

The main research areas and related integrated services that will be offered by the thematic research platforms are summarized in the following:

1. Paediatric Medicines Discovery

Within this platform, bioinformatics and computer modelling, accelerated target identification, imaging techniques, etc., will be applied in the paediatric sector. In particular, the research units adhering to this Platform are able to offer new emergent technologies currently recognised as pillars of novel precision medicine, such as use of pluripotent stem cell, 3D cell cultures, target validation, patient derived cell assays, micro-fluidics, high-throughput cell image analysis.

2. Paediatric Biomarkers and Biosamples

Within this Platform, three areas of paediatric dedicated activities will be carried on: **a)** Validation of paediatric biomarkers, **b)** Identification/characterisation of novel paediatric biomarkers, and **c)** Access to/deposit of annotated paediatric biological samples in a core group of identified biobanks and sample collections dedicated to paediatrics.

3. Developmental Pharmacology

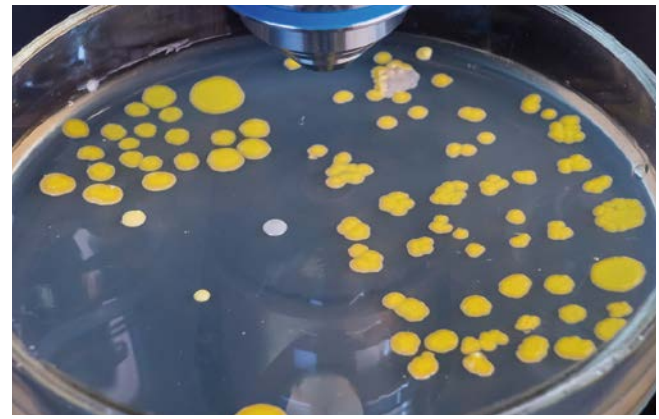
The Platform includes many expert centres focused on developmental pharmacology, specifically those bridging basic and clinical pharmacology science to determine maturation effects on drug disposition and effect with impact on drug dosing. The platform will enable the design and conduct of paediatric studies in early drug development, as well as the design of first-in-child approaches, the generation of new physiological data including ex vivo human and animal data and placental platforms, as well as the development of innovative population and physiology-based models that focus on growth and maturation and other relevant factors in paediatrics.

4. Paediatric Medicines Formulations and Medical Devices

The Platform includes research units able to work on different types of active pharmaceutical ingredients, varying from small molecules to biologicals and vaccines. The wide array of traditional and innovative technologies available can support, not only the development new neonatal and paediatric formulations and drug delivery solutions for the enteral and non-enteral routes of administration, but also reformulation to improve the quality of the medicines used off label or unlicensed in children. The platform also encompasses capabilities in taste-masking technologies, biopharmaceutics and medical devices.

COMMON SERVICES

Common Services to be provided in collaboration with already established Biomedical Research Infrastructures (BBMRI, EATRIS, ECRIN, ELIXIR, INFRAFRONTIER, EU-Openscreen, etc.) and other international and national infrastructures to harness efficiency and delivery of paediatric research activities and services and to strengthen collaboration within the scientific community. A complete analysis of the opportunities offered by the existing RIs already enabled determination of two common services, as described in the boxes below.



Common service for data interoperability in paediatric research

A common service to ensure data interoperability in paediatric research will be created in collaboration with ELIXIR (A distributed infrastructure for life-science information). It will require the creation of an EPTRI Catalogue of data and it is intended as a tool to facilitate sharing and re-use of data according to the principles of open access.

The interoperability service will be based on a common methodology for representing data collected for specific purposes and the relationships between them in order to standardise the exchange of data from multiple sources.

Common service for ELSI support in paediatric research

A common service to ensure ethical, legal and societal support and guidance for those conducting research with children, both biomedical and translational, from the preclinical phase to clinical studies, access policies and education. Specifically devoted to paediatrics, such a service will range from promoting best practices on shared decision-making to training, communication, information and empowerment of children and their families, as research participants, especially in some contexts that raise specific issues, such as rare diseases. It will cover paediatric research activities directly involving patients, patients' biological samples and data, emerging technologies, genetics and advanced therapies.

This service will be linked with existing ELSI services, such as the BBMRI-ERIC Common Service ELSI.

EPTRI

European Paediatric Translational Research Infrastructure

Coordinated by CVBF - Consorzio per Valutazioni Biologiche e Farmacologiche

Via N. Putignani, 178.
70122. Bari (Italy)
Email: coordinator@eptri.eu
Tel: +39 080 97 51 974
www.eptri.eu