EPTRI
European Paediatric Translational Research Infrastructure

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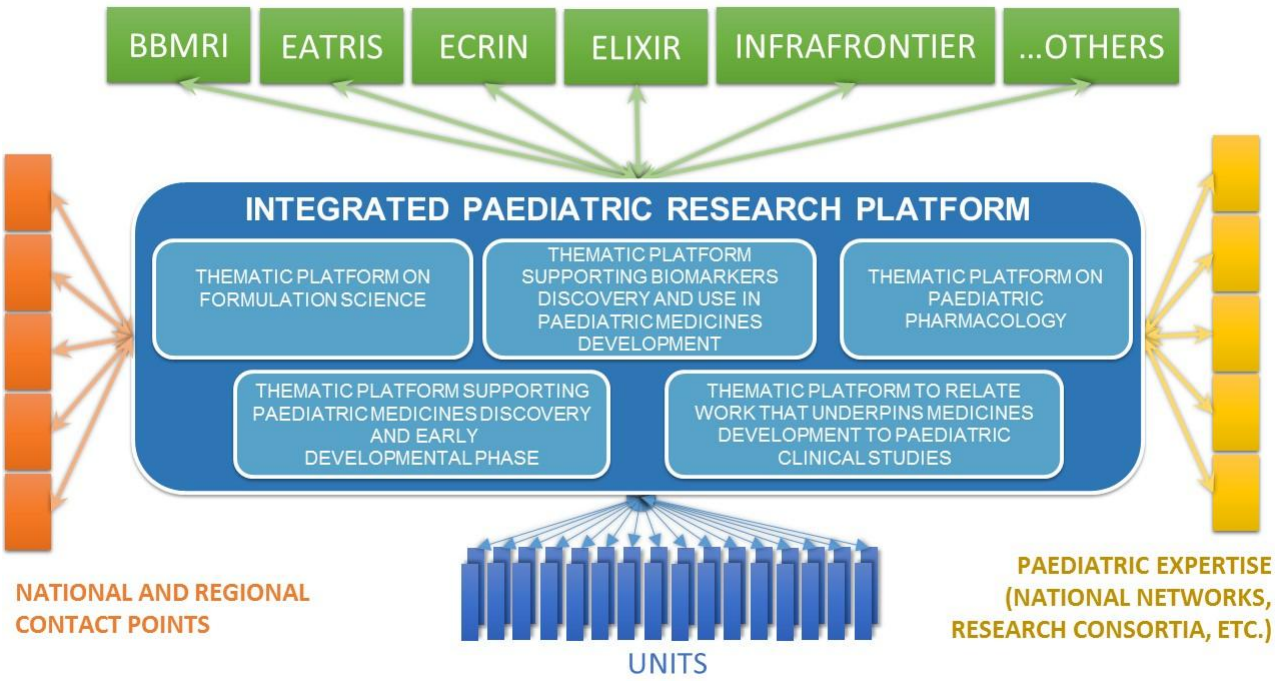
The European Paediatric Translational Research Infrastructure, EPTRI, is a **new complementary Research Infrastructure** (RI) in the context of the existing RIs intended to putting together and networking all the available competences and technologies useful to enhance paediatric research in paediatric medicines from drug discovery and early development phases to be translated into clinical phases and medicines uses.

EPTRI arises from the need to find answers to the serious lack of medicines for children in EU and worldwide and to propose developmental models for paediatric medicines that integrate technology- driven aspects with clinical trials.

The project ID-EPTRI, coordinated by Consorzio per Valutazioni Biologiche e Farmacologiche (CVBF), aims to design the framework for the new 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-Paediatric Medicines Discovery, 2- Biomarkers, 3-Paediatric Pharmacology, 4-Formulation Science, 5-Underpinning Paediatric Studies.



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To prepare the **Conceptual Design Report** (CDR), the project will encompass three phases.

During the *Context Analysis phase*, that will be performed in all the five technical and scientific domains identified, the perceived value and the possible gaps to be covered will be estimated, by enquiring the scientific Communities, the concerned national Authorities and many other Stakeholders.

During the *Operational phase*, the different components of the new RI will be planned, including governance model, strategies for interaction with national Authorities and the existing RIs, the IT- architecture model, services to be provided and a business plan.

In the second year of the project, a *Feasibility phase* will be proposed to develop virtual exercises simulating the operations of the RI to work as a "one-stop-shop" for advice in paediatric drug development. The ability of EPTRI should be tested in the following domains:

- support researchers in many methodological areas,
- foster the technology innovation in all the academic and clinical settings,
- conduct research that effectively underpins the development of medicines for children,
- increase the global effectiveness of the European research area also in favour of children and young people.

To harness efficiency in delivery of paediatric research activities and services, EPTRI plans to act as a "**paediatric common service**" with three already established RIs (BBMRI, EATRIS, ECRIN). In addition, collaboration will be strengthened within the scientific paediatric community and other existing RIs. Moreover, EPTRI will be an **open science space allowing researchers to work together without geographical, institutional or financial barriers** and a system of many interconnected areas, each of them led by a top-edge group focused on a specific topic/research area.

The project involves 26 partners from 19 EU / non EU countries including existing RIs, the major paediatric expertise to cover the scientific topics in the proposal, previous expertise and collaborative resources and partners outside Europe.

In particular, EPTRI will have the following expected impacts:

- help academic and non-academic organisations to strengthen their base of knowledge and technological know-how;
- facilitate access to progresses and results in health care and diseases' prevention for children;
- bringing together the expertise provided by the paediatric scientific community including users' communities and patients' associations,
- include into the activities and services already developed in the framework of the biomedical ESFRI Research Infrastructures a specific paediatric competence that is still underrepresented there;
- preparing the ground to increase paediatric research and reduce the serious delay to provide children with new, innovative and advanced therapies as resulting from the advancement of biological and medical sciences;
- link advancements in paediatric research to the rapid development of regulatory

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provisions, to favour the development of more demonstrative projects based on innovative study design;
- map the paediatric needs as well as the needs of technical research facilities.

It is important to highlight the positive impact that EPTRI will have on children's health. Despite EPTRI will not deliver new medicinal products on the market, such initiative will establish the right framework to speed-up the drug development process in paediatrics and to include the top-level research innovations in research for children's health. This will end in an increased offer of appropriate efficacious and safe medicines for children in all the paediatric ages (from neonates to adolescents).
A positive impact is foreseen on the scientific community, as it is expected that, following the rising integration of the different research units within the new establishing RI, the scientific relevance of the paediatric research at national and international level will increase. 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.

Activities carried out so far

- *ID-EPTRI Kick-off Meeting*

The ID-EPTRI project has been launched in Rome on 15th-16th January, 2018, at the Ministry of Education, University and Research (MIUR), with more than 100 attendees.
The meeting was the occasion to present the project, to put together all the partners and stakeholders involved and to discuss on how to implement the project in the 2-years of work ahead of us.

During the meeting, it was given an overview on the biomed Research Infrastructures in the European framework aimed to improve patients' health underlining the EPTRI role within this fragmented scenario. EPTRI will be a complementary RIs in the European scenario avoiding any overlapping with the already existing initiatives.

The first day was focused on several aspects related to the project management and coordination with a brief presentation of all the partners involved. It was discussed about the ID-EPTRI General Assembly and Bodies, requirements and timelines, governance and sustainability, administrative, legal, financial aspects and ethical issues. During the second day, the discussion moved towards the heart of project with specific presentations about the five thematic platforms and the IT model, whose results will converge, together with those of the context analysis phase, in the Conceptual Design Report to realize EPTRI. Finally, it was addressed the importance of setting-up an effective communication strategy, networking activities and patients involvement to assure a long-lasting sustainability of the project.

Thanks to the active participation of all the attendees, the meeting provided productive discussions and was the occasion for all the partners involved to meet each other and to know a bit more about all the institutions involved in the project.

The press release of the meeting has been published on the Cordis website and available here.

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A presentation video of the day is available here.

- *Presentation of the project at the University of Bari (Italy)*

The next meeting of the ID-EPTRI project, entitled "EPTRI: a new European Research Infrastructure to foster the translation reserach in paediatrics", will be held on April 10th in Bari (Italy) at the Salone degli Affreschi of the University of Bari, in Piazza Aldo Moro, 1.

The meeting will be aimed to give all participants a better understanding of the tasks and goals of the project and to illustrate the key elements of the new research infrastructure. The first part of the meeting will be focused on the global scenario around EPTRI and the role of RIs to strengthening research outcomes to improve patients' health. The second part of the meeting will be dedicated to the presentation of the technical component of the infrastructure, with a particular focus on the operational plans of the five thematic platforms composing EPTRI as well as the importance of the patient engagement. The meeting agenda is available here.

The meeting in Bari will also be the occasion to test the maturity of the regional reseach system to host the coordinating center of EPTRI thanks to the strong involvement of the main Institutions in the Apulia region: the regional Directorates for Health, Research and Development, the University of Bari, the regional Agency for Innovation and Technology, the University Hospital and the Paediatric Hospital.

- *EPTRI will be presented at the BIAT event*

The **BIAT event – Innovation and High Technology Lab** – that will take place in Naples, Italy, on 19th-20th April 2018, is an international travelling event within the initiatives "Piano Export Sud II", a program of actions for the promotion of internationalization and business innovation. The aim of the event is to enhance the innovative potential of companies and research organizations of the Italian Southern Regions (Campania, Calabria, Apulia, Sicily, Abruzzo, Molise, Basilicata, Sardinia), in order to obtain products and services to be exported on foreign markets.

Many Foreign counterparts (large companies, research centers interested in technology transfer, venture capitalists and investors) coming from the following countries: Belgium, Canada, China, Korea, Denmark, United Arab Emirates, Russia, France, Germany, Japan, India, Israel, Holland, Poland, United Kingdom, Singapore, United States, Sweden and Turkey will take part in the event and will meet the Italian participants in dedicated B2B meetings.

CVBF will attend the event promoting the EPTRI project and a dedicated page to EPTRI has been published on the official BIAT 2018 catalogue. More information on the event is available at the following link.

- *Preparation of the survey to identify potential service providers to be included in the future RI*

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In the framework of Work Package 3, a survey is under development to collect different information clarifying the scientific and technological context for paediatric research in the European landscape. This survey aims to identify potential service providers to be included in the future European Paediatric Translational Research Infrastructure.

Recipients will be asked for the following information concerning their reference centre:

- Paediatric research areas
- Existing competency framework for researchers in paediatric areas
- Gaps in the available paediatric research services and facilities
- Barriers and facilitators to allow paediatric research units to form research platforms to support EU Paediatric research
- Availability to deliver services for Paediatric Research.

To this aim, an electronically distributed questionnaire has been developed and has involved several key experts of the ID-EPTRI project, including the WP3 Scientific Reference Group, appointed during the General Assembly. Reference contacts of research units in Europe (and some also extra- Europe) have been identified thanks to a collaborative work involving the main Partners in the work packages related to the ID-EPTRI thematic platforms. This questionnaire is not intended to map clinical research units. However, it will be administered also to specialised paediatric clinical research units which could contribute by providing expertise in the fields of interest of EPTRI or by delivering it to the research units which provide themselves with the same expertise EPTRI is looking for. The survey will be launched on Monday, April 16th, and will remain open for 45 days, until end of May. Preliminary results will be available for discussion during the consortium meeting at Lobachevsky State University of Nizhni Novgorod (Russia) on June 5-6.

- *Preparation of the Second General Assembly Meeting in Russia*

The second General Assembly will be organized in Russia on June 5-6 at at the premises of the Lobachevsky University (UNN), partner in the project that has kindly offered to host the Meeting in Nizhny Novgorod. From an organisational point of view, the meeting will be a two days meeting. The first day will be dedicated to discuss about the work to be carried out in the platforms, considering that the survey and the report on survey outcomes will have been already issued and we would have received enough preliminary materials to start working on the design of the platforms themselves. The second day will be more networking focused and we could address how to interact and involve the non EU countries (as many of them are participating in the project) and the activities to be carried out by all the partners to discuss with their national government and reach the necessary political and financial support needed to apply to the ESFRI Roadmap.

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