

EPTRI Virtual meeting

December 1st, 2020

REPORT

The EPTRI virtual meeting was held virtually on December 1st 2020. It was addressed to all the 112 Research Institutions signed the EPTRI Memorandum of Understanding which aims at organising the large existing community of paediatric researchers and facilities to impact the R& in the drug discovery, design and development processes.

The meeting was opened by **Donato Bonifazi**, EPTRI Coordinator, who provided a brief overview of the recently concluded ID-EPTRI Project, aimed to design the framework for the new Research Infrastructure to accelerate paediatric research in the drug development process. D. Bonifazi went over focusing on the opportunities that Horizon Europe will offer within the Pillar 1 for the infrastructure preparatory activities, and within Pillar 2 for paediatric research projects which will support EPTRI Platforms feasibility and strengthen the wide panEuropean partnership. It will be also possible to get funding at National level for the creation of the EPTRI nodes. Further opportunities under Horizon Europe will be given through the creation of institutionalised Public-Private Partnerships. D. Bonifazi concluded informing the audience that EPTRI participated in the consultation 'Pharmaceutical Strategy for Europe' underlining the necessity to stress new adequate health policies and actions promoting the development and access to safe and efficacious medicines specific for children and will participate to the joint consultation on orphan and paediatric EU regulations (Comments to the EC - Inception Impact Assessment - Ref. Ares ((2020)7081640 - 25/11/2020).

The meeting proceeded with the speech of **Mariangela Lupo** (TEDDY Network), who presented the European Strategy Forum on Research Infrastructures (ESFRI Roadmap) as a strategic instrument to develop the scientific integration of Europe and to strengthen its international outreach. EPTRI submitted its application to the ESFRI Roadmap 2021 on 9th of September 2020 receiving Letters of Political support from 16 National Authorities relevant for the Research Infrastructures (RIs). M.

Lupo then illustrated the activities foreseen in the EPTRI Preparation phase (PP), starting in 2021 and planned to end in 2024. The EPTRI PP is expected to be funded by the Horizon Europe program, national research plans and in-kind contribution from the research organisations participating in EPTRI. An attention was given to the setting up of the EPTRI National nodes.

The strengthening of the paediatric initiatives and collaboration with the public-private sector was presented by **Carlo Giaquinto** from PENTA Foundation. He provided a brief analysis on the paediatric research, underlining some critical points. Even though there are many industry studies on paediatrics, these are often distinguished by a poor design, poor delivery, poor engagement, fragmentation and inefficiency. C. Giaquinto stressed the importance of the need to have a collective effort and shared the experience of the c4c-IMI2 Project. Alliances and collaborations are key to successful consortia, continued C. Giaquinto and explained that partnerships only work when both parties benefit from the relationship. Both public and private partners can benefit from a structural collaboration where the expected benefits are made clear in advance.

A proposal of a platform dedicated to the Child Health Technology within EPTRI was raised by **Paul Dimitri** (NIHR Children and Young People MedTech Cooperative) who stressed the importance of having the expertise with unmet needs aligned with the funding roadmap across the innovation pathway. P. Dimitri explained how the strategic framework is fundamental in defining and developing the proposed platform. He illustrated the process capability of defining unmet needs (What?) as well as the relation capability (How?). It is of high importance to develop a solid governance structure including the young patient's advisory groups (YPAG), integrate with the exciting paediatric initiatives such as EPTRI, and understand the commercial opportunities across Europe, concluded P. Dimitri.

Another interesting experience on building a digital space for Life Science was shared by **Giacinto Donvito** (National Institute for Nuclear Physics - INFN) and **Marco Tangaro** (CNR-IBIOM) who outlined the EOSC-Life Project coordinated by ELIXIR. The European Open Science Cloud (EOSC) is the envisioned federation of research (data) infrastructures that will enable the Web of FAIR Data and Services, help researchers to perform Open Science, and open up and exploit their data, publications and code. An overview of other main Projects ELIXIR is involved in, with a focus on some important services, were also provided by G. Donvito and M. Tangaro.

Adriana Ceci (EPTRI-IT Scientific Committee Chair) shared with the audience the positive experience in creating the EPTRI Italian node bringing together the different expertise at national level. She explained the EPTRI-IT composition, governance and activities. The same approach assumed in Italy is applying in all Countries involved in EPTRI for setting up the National nodes, continued A. Ceci, and explained the important contribution the Nodes may have on the paediatric Research Landscape. So far, organisational call-conferences have been done with the Institutions from the following Countries: Austria, Greece, Israel, Germany, Romania, Ireland, Ukraine, Luxembourg and Norway. At National level, there is a need that the paediatric research is marked as Priority in the National Plans. In that direction, EPTRI should be acknowledged, at least in those Countries who have provided a Letter of Political Support for inclusion in the ESFRI Roadmap 2021, in the national plans for 2021 concluded A. Ceci.

The EPTRI Drug Discovery platform was discussed by **Emmanuel Mikros** (ATHENA Research Center) who underlined that the Platform will be able to offer access to academic drug discovery facilities specialised in paediatric diseases and will provide integrated services in the areas of bioinformatics, chemical probes, target characterisation and genetics. The peculiarity of the Platform comes from the fact that it covers specific cell models (e.g. for muscular dystrophies, brain injury, apoptosis and cancer), specialised cell technologies (e.g. 3-dimensional cell cultures and organoids, microtissue products, nano-culture spheroid plates, micropattern plates, etc), and a unique collection of animal models from zebrafish to swine specific to paediatric diseases. Implement cutting edge technologies, to accelerate discovery and preclinical drug development in paediatrics is another strength of the Platform, concluded E. Mikros.

The meeting proceeded with the presentation on the EPTRI Placenta platform made by **Frantisek Staud** (Charles University) who started with an overview of the current team involved in the Platform. F. Staud explained the importance of studying the placental patho-physiology, pharmacology and toxicology as its functions are crucial to maintain optimal conditions for proper fetal development and programming. The different methods to study placenta were shown by F. Staud who terminated his presentation with the list of some current projects on placenta and recent publications.

Nunzio Denora (University of Bari 'Aldo Moro') presented the Special Issue: '*Scientific Highlights in the First European Paediatric Translational Research Infrastructure Open Meeting*'. This Special Issue

is addressed to all researchers and experts involved in the development of paediatric medicines. Manuscripts can be submitted online at www.mdpi.com by registering and logging into the website. Original research articles, reviews, opinion papers, editorials, and short communications are all welcome, explained N. Denora, on the following topics: paediatric medicines drug targets and methods of identification; advanced cellular/in vitro models for paediatric drug targeting; paediatric formulations and toxicology of drugs; drug delivery design in paediatrics; translational research. The last presentation was held by **Giovanni Migliaccio** (CVBF) who described the ERIC (European Research Infrastructure Consortium) as a long-term legal status suitable for EPTRI. The ERIC is a specific legal form that facilitates the establishment and operation of Research Infrastructures which owners are the Member States (MS). During the transition phases of development of EPTRI, in order to be able to apply for operation and service funding, EPTRI may assume a temporary legal form. One of the possibilities, continued G. Migliaccio is the European Economic Interest Grouping (EEIG) which is designed to minimise the legal, fiscal and psychological difficulties that natural persons, companies, firms and other bodies face in cooperating across borders. G. Migliaccio concluded that EPTRI will continue working with the ambition to facilitate the basic, preclinical and translational paediatric research in order to create a strong consortium able to offer access to all relevant expertise.