

ePTRI

EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE



EPTRI

Stakeholders Roundtable

Virtual meeting Report
July 9th, 2020



EPTRI STAKEHOLDERS ROUNDTABLE REPORT

The EPTRI Stakeholders Roundtable has been held virtually on the 9th of July, 2020 with 29 Stakeholders from all over Europe presenting several interesting aspects of the paediatric research and 109 active participants from EU Institutions, Governments, European Research Infrastructures (RIs) and projects, Agencies, Networks, European Reference Networks (ERNs), patients' associations, charities, research organisations and also EPTRI partners from EU and non-EU countries.

The event has represented the occasion to show the outcomes of the EPTRI design phase and identify possible ways of collaboration with other biomedical Research Infrastructures, European initiatives and networks relevant for the paediatric research.

The meeting has been organised in four sessions: two in the morning and two in the afternoon. The **morning sessions** provided an overview of the paediatric research in the European Research Area from the point of view of scientists, regulators, EU Institutions, Networks and patients organisations, followed by the **presentation of the idea of the future architecture of the EPTRI Infrastructure** in terms of IT structure, services and work performed during the feasibility phase to get to this structure. In particular, the centralised services provided at central hub level and the integrated services based on the facilities and competences of the research units that will constitute the Thematic Research Platforms have been presented.

In the afternoon, a dedicated session on the importance of the **networking and outreach** activities took place with a focus on the emerging fields identified by EPTRI to be crucial for further development (i.e. medical devices, Artificial Intelligence and placental research, etc.) and the point of view of the EU associated countries supporting EPTRI.

The last session gave the opportunity to the ESFRI Research Infrastructures in the life science domains to express their expertise in paediatrics and provide possible scenarios of collaboration with EPTRI.

Below a summary of the most interesting highlights of the presentations.

SESSION 1: The paediatric research in the European Research Area

The EPTRI project and the future of Paediatric Research: Donato Bonifazi, Consorzio per Valutazioni Biologiche e Farmacologiche

The presentation provided an overview of the work performed by the EPTRI project during its three phases and the future goals of the work to be done represented by the inclusion in the ESFRI Roadmap 2021 and the Preparatory Phase in which EPTRI will enter starting delivering services to the paediatric research community and providing a blueprint to close the gap between innovative technologies and paediatric drug development processes, using an original scientific approach that is paediatric patients-centred.

He underlined that in the pharmaceutical scenario children continues to be orphan of appropriate and innovative treatments due to a huge lack of appropriate and innovative therapies medicines tailored on children, a regulatory framework which may not keep pace with the important scientific and technological advances, and dispersed and disaggregated research' efforts.

In this framework, he identifies the EPTRI contribution in ensuring Europe's supply of safe and affordable medicines to meet patients' needs, support the European pharmaceutical industry to remain an innovator and world leader, timely access to patients, including children, and economic sustainability of paediatric research.

«The new approach should enable to promote a European strategy from development to access, mainly based on 2 assets:

*1- **a focus on traslational researchers-driven** studies to allow good ideas born in academic and non-profit laboratories to be implemented and therefore become attractive also for commercial sponsors for further development*

*2- **the increase of the public / private partnership opportunities** to avoid blocks or failures on the market .»*



The European Networks for Paediatric Research: Pirkko Lepola, Helsinki University Hospital, the European Network of Paediatric Research at the European Medicines Agency

The presentation provided an overview of the European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA) key operations, the stakeholders and the activities of the existing research networks with an overview of the European paediatric research landscape.

«The Enpr-EMA's main objective is to facilitate paediatric studies in order to increase the availability of new medicines authorised for children. Enpr-EMA focuses on enhancing and supporting network collaboration, acting as a platform for sharing good practices. The EMA provides more extensive regulatory and scientific advice for companies through PDCO, including pre-clinical medicine development, trial design and clinical trials. The EPTRI is linked with Enpr-EMA via other initiatives and works on pre-clinical activities.»

The Network of parliamentary advocates for paediatrics to promote new scenarios for a better access to medicines: Miriam Dalli, Member of the European Parliament (MEP)

MEP Dalli presented the current situation of difficulty in which children occur caused by the lack of tailored medicines due to the supply system and patient access, pricing and transparency, and rigid patenting rules. In this scenario, the idea to develop a network of parliamentary advocates for paediatrics to promote new scenarios for a better access to medicines has been prospected.

«Possible solutions:

- Create transparency and increase transparency when prices are negotiated to ensure fair deals for all patients wherever they are and the need to continue working to make joint procurement a reality. Creating transparency should hold pharmaceutical company accountable and help member states negotiate for this also the help of joint procurement.*
- Promote measures to stimulate the uptake of generic medicines, when possible which can effectively lower costs and reduce overall expenditure on medicines.*
- Monitoring system to ensure that national stockpiling initiatives are proportionate to their needs and ensure that all countries have fair access to the stockpile and contingency plan. Fair access to stockpiles will ensure that all countries can be prepared for any emergency.*
- Monitor whether patents are granted for genuine innovation and examine the practice of 'evergreening', where pharmaceutical companies get patents for inventions that are not actually new, but are 'old science' with minor modifications to existing products in order to keep their patents indefinitely.*
- Stimulate competition between generic medicines and established medicines where the patent has expired to incentivize innovation and reduce costs.»*

The highest challenge of rare diseases in children: Luca Sangiorgi, ERN-BOND, Rare Skeletal Diseases Department, Rizzoli Orthopedic Institute

He presented the rare diseases, their challenges and the issues related to paediatric research which determine that the 94% of rare diseases continue to lack of an appropriate and available therapy.

«We are supporting the fact that there is a need for evidence for basic to clinical research and the European Reference Networks (ERNs) could be one of the point in which this kind of new clinical trials can be tested. This situation can be achieved with a strong partnership between all the stakeholders. »

The patient centered research in the rare and paediatric diseases perspective: Dimitrios Athanasiou, Paediatric Committee, European Patients Forum

The presentation was focused on state of the art of paediatric research and medicines and the importance of the role of the patient point of view in developing tailored actions to foster paediatric research and make it child-friendly.

«EPTRI is part of the solution and we need EPTRI to be focused also on technical issues. Basically we want to create a sustainable global development model. We have to innovate processes and thinking processes and understand how to work together for a common future and this can be done only in trust.»

Horizon Europe overview. Paediatric developments and prospects: Alexandru Costescu, Scientific Officer of the European Commission, DG Research and Innovation, Directorate Health

The presentation provided an overview of the news in the field of paediatric developments and prospectives in the Horizon Europe programme which is aimed to strengthen the EU's scientific and technological bases and the European Research Area (ERA), boost Europe's innovation capacity, competitiveness and jobs, and deliver on citizens' priorities and sustain our socio-economic model and values in Excellent Science, Global Challenges & European Industrial Competitiveness and Innovative Europe. The missions areas identified are: Cancer; Adaptation to climate change, including societal transformation; Healthy oceans, seas, coastal and inland waters; Climate neutral and smart cities; and Soil health and food.

«What we need to say when we think of a possible change in the Regulation is that any future solutions should aim to find the balance between fostering innovation and ensure availability and access to medicines for children.»

SESSION 2: The EPTRI conceptual design

EPTRI organisation of services, data and access: Fedele Bonifazi, President of Fondazione per la ricerca farmacologica Gianni Benzi onlus

The presentation provided an overview of the EPTRI organisational model, which is based on the Hub-and-Spoke concept with a Central Hub and several Spokes represented by the Thematic Research Platforms (aggregating the research units based on their scientific field of activity) and by the National Nodes (aggregating research units at country level), and the plan of services and how they will be accessed by users.

*«Users will have access - through the Single Access Point (SAP) - to three types of services:
a) integrated services, provided by the research units organised in Thematic Research Platforms;
b) centralised services, managed and provided at Central Management Office (CMO) level;
c) common services, provided in collaboration with other Research Infrastructures.»*

Thematic Research Platform supporting Paediatric Medicines Discovery: Emmanuel Mikros, ATHENA Research and Innovation Center in Information Communication & Knowledge Technologies

The presentation provided details on the future Paediatric Medicines Discovery Thematic Research Platform which is aimed to offer access to academic drug discovery facilities specialized on paediatric diseases, provide integrated services coordinating efforts among European Centres of Excellence, and implement cutting edge technologies, in order to accelerate discovery and preclinical drug development in paediatrics.

Its uniquenesses cover 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 more importantly a unique collection of animal models from zebrafish to swine specific to paediatric disease's (e.g. mouse model of premature ageing, rabbit bronchopulmonary dysplasia model, juvenile minipig models for safety assessment, rodents model of hereditary paediatrics disease, neurodevelopmental disorders or cancer).

The presentation also detailed the services that the future platform will provide within the preclinical phase for the development of novel targeted paediatric

therapies and the distribution of the resources throughout Europe that will work in 4 research activities namely, on new target identification and validation, on cell and tissue studies, on animal studies, and in silico studies related to drug design, discovery and development.

«In the last three years, within EPTRI we identified gaps related to the understanding of diseases in paediatrics which are different between children and adults. This comparison can give the opportunity to establish targets specific to paediatric patients. We know that paediatric diseases are quite often multifactorial, so it is of paramount relevance to have polipharmacological and multitargeting approaches with new targets related to paediatric specific diseases, phenotypes and developmental stages which represent challenging domains displaying both high potential of innovation and contribution to children therapeutics. Moreover, the existing models (e.g. cell models and animal models) are inadequate to capture the unique developmental status of paediatric patients. Specific disease models and personalised medication approaches in paediatrics are needed»

Thematic Research Platform on Paediatric Biomarkers and Biosamples: Marios Phylactides, Cyprus Institute of Neurology and Genetics

The presentation introduced the need for biomarkers tailored to the paediatric population due to the frequent, but often inaccurate, extrapolation of data from biomarkers developed for adults, and the actual problems in paediatric biomarkers research, such as lower prevalence and incidence rates of disease, heterogeneous populations and more complicated bioethical considerations. Moreover, he underlined that through the EPTRI survey some unavailable expertise, resources and facilities emerged as mainly the technical resources, animal facilities, omics platforms.

The survey also helped to identify the Institutions that will contribute to the platform, which are distributed throughout Europe and the available capacities and services within EPTRI, such as the organisation and management of paediatric biosamples and related data for paediatric studies and research activities aimed to identify biomarkers of high paediatric interest (RNA/DNA/proteins/metabolite candidate biomarker identification and characterisation in paediatric samples, bioinformatics, and verification in paediatric samples of the presence and levels of biomarkers).

«The role of this platform is basically the identification and development of biomarkers to make available for paediatric drugs. Biomarkers have an enormous potential. In the last 20 years, the developed biomarkers were mainly for adults,

so the development of specific biomarkers for paediatric population is essential for many reasons. First of all, the pathogenesis of many diseases is different in children and adults and some diseases are unique to children. Moreover, children constantly grow and develop, and children's gene expression can vary during their growth.»



Thematic Research Platform on Developmental Pharmacology: Tessa van der Geest, Radboud University Medical Center

The presentation introduced the needs in developmental pharmacology as the capacity to support the underlying science necessary for clinical drug evaluation for children and the involvement of multiple experts and competences from (paediatric) pharmacologists, chemists, toxicologists, pharmacometricians and regulatory experts. She emphasizes the added value of providing access to combined paediatric expertise and technology through the future platform.

Moreover, she provided details on the distribution of the partners in Europe and their expertise, and the areas of the services that will be provided by the future Developmental Pharmacology Thematic Research Platform, as microdosing, sensitive analytical methods, placenta physiology and pharmacology, modelling and simulations, developmental animal studies, toxicity juvenile animal studies and ontogeny of drug disposition. The presentation gave also a short description on the process on 'how to get access to the provided services'.

«The developmental pharmacology platform is focusing on stimulating paediatrics within basic research at the point that patients are getting involved. The aim is to create a bridge between basic research and clinical drug development in paediatrics. When a new drug candidate is selected for a paediatric drug, data extrapolation from adults is performed, but with limits of biological processes. It is challenging not only for new drugs, but also for the ones on the market as the medicines for paediatrics

are very limited. Paediatric pharmacology is important in closing information gaps particularly regarding pharmacodynamics and pharmacokinetics. Within the platform a transnational access will be provided including access to research expertise and facilities in academia and industry and support studies underpinning optimal paediatric dose selection.»

Thematic Research Platform on Paediatric Medicines Formulations: Elisa Alessandrini, School of Pharmacy, University College of London

The presentation highlighted that the future Paediatric Medicines Formulations Thematic Research Platform has been designed to create suitable paediatric medicines in a timely and efficient way and to facilitate and accelerate development of age-appropriate formulations for children. The main research activities performed by the members of the platform are pre-formulation capabilities, formulation development, assessment of drug delivery systems, and palatability. She showed that from the results of the EPTRI survey, the following services were identified: pre-formulation advice and pre-formulation studies, formulation of drug for paediatric enteral and non-enteral routes of administration, paediatric in vivo and in vitro palatability assessment, and assessment and design of drug delivery systems for enteral and non-enteral routes of administration. Finally, the presentation gave a short description of how to get access to the services.

«The platform is aimed at creating suitable and tailored medicines for children in a timely and efficient manner. The platform will offer expertise and activities in paediatric formulation through participating research units which have a good geographical distribution accross Europe. Services will be provided under 4 main research activities and delivered through the EPTRI platform to facilitate and enable efficient and timely for age appropriate formulations for children.»

EPTRI to Underpin Paediatric Clinical Studies: Mark Turner, University of Liverpool

The presentation provided an overview of the possible EPTRI contributions to clinical research by “injecting” expertise from EPTRI into clinical studies (e.g. relevant aspects of study design and formulations) and “projecting” expertise from clinical studies into the work covered by EPTRI and the plans for medicines development (e.g. clinical need and what is possible).

«EPTRI provides horizontal integration across Europe and through central services contributes to vertical integration of medicines development.»

EPTRI Feasibility Studies to design the new scenario of paediatric research: Adriana Ceci, EPTRI Advisory Board Chair

The presentation highlighted the path performed by EPTRI during the feasibility phase focusing on feasibility studies with scientists which were aimed at demonstrating that using the large partnership set up, EPTRI would be able to provide research services necessary to the users, and providing useful information helping to prepare full-scale EPTRI proposal applications and related future grants.

«The majority of the services required by the feasibility studies are potentially covered by EPTRI research units and Centralised Services. Moreover, the process for submitting requests from users, the selection process based on transparent rules and the assignment to services' providers, also demonstrated to be suitable and manageable.»

EPTRI Feasibility Study “Melatonin administered during pregnancy as a potential neuroprotective agent against neonatal brain damage”: Eveline Jacqz-Aigrain, Assistance Publique - Hôpitaux de Paris, Robert Debré Hospital

The presentation provided an example of feasibility study received by EPTRI in the Feasibility phase aimed at evaluating the neuroprotective effect of melatonin in preterm neonates when administered to the mother during (e.g. premature labour, preeclampsia, etc) or prior to foetal distress using pre-clinical models and translational data on the physiology of melatonin during normal and complicated pregnancies.

«The proposed study identified a very innovative research need dealing with placental and neonatal studies that the EPTRI placental platform that is part of the Thematic Research Platform on Developmental Pharmacology has experts and research units capable to answer this specific need.»

The Belgian EPTRI node as an example for an integrated approach to paediatric research: Johan Vande Walle, Ghent University

The presentation provided an interesting example of organisation of an EPTRI National Node in Belgium with its transversal participation in the four Thematic Research Platforms.

«The overarching goal of EPTRI Belgian Joint Research Unit (JRU) is to create a framework for basic and translational paediatric research to facilitate the development of new innovative medicines for children. EPTRI Belgian JRU has the goal to move to a new approach characterised by medicines specifically designed to address the needs of children and to reduce the existing gap on medicines availability for babies, children and adolescents.»

SESSION 3: EPTRI Networking, Outreach and Development

EPTRI Outreach and Networking plan: Mariangela Lupo, TEDDY European Network of Excellence for Paediatric Research

The presentation gave an overview of the outreach and networking strategy for the future EPTRI Infrastructure that will allow to:

- increase the impact of EPTRI services and initiatives and its global partnership;
- reduce the barriers between basic science and citizens' wellbeing providing evidence of the direct benefits to society;
- liaise with the many paediatric initiatives conducted at national and international level as well as with the international research programmes and projects. Key to the success of these activities will be the critical involvement of the patient community and
- build liaisons with all the relevant stakeholders, including the Scientific Community, Policy and Regulatory decision makers, Pharma and Biotech Industries and SMEs.

«A lot of activities have been planned and we encourage all the partners to support us in implementing these dissemination and outreach strategies in order to make EPTRI grow and become a fully fledged research infrastructure that can come through all the phases of this research infrastructure lifecycle.»



EPTRI Toolkit for young patients: Begonya Nafria, Institut de Recerca Sant Joan de Déu, Fundacio Sant Joan De Déu

The EPTRI Patients' Toolkit aimed to educate children, young patients and families on basic and translational paediatric research has been presented. The toolkit is an interactive tool consisting of an animated video and independent clips with resources for patients and families. The video describes a case that illustrates how a paediatric medicine is developed, starting from basic research studies and going through all the steps that lead to successfully accomplish this process.

The toolkit is available at this link: <https://eptri.eu/basicresearch/>.

«The design of the toolkit took into consideration the information gathered by members of the Young Members Advisory Groups (YPAGs), who also reviewed the script of the video. The toolkit was designed incorporating the outcomes and conclusions obtained from the workshop with stakeholders and the roundtable with young people celebrated within the framework of the 3rd EPTRI General Assembly held in Madrid at the Ministry of Health, Consumption and Social Welfare on the 27th and 28th of March 2019.»

The creation of a Technology Research Platform on Medical Devices: Paul Dimitri, Sheffield Children's NHS Foundation Trust

The presentation provided the framework for the development of a European Paediatric Medical Devices Platform delivered through EPTRI. The need for this platform is characterised by lack of targeted health technology for children with previous issues relating to repurposing of adult technologies, and the emergent, localised and therefore fragmented development of health technology for children. Following the strong interest in paediatric medical devices identified by the EPTRI Stakeholder survey, alongside the breadth and depth of the expertise in medical devices community, EPTRI has proposed the development of a dedicated paediatric medical device's platform.

«The steps for the development of a Technology Research Platform on Medical Devices include:

- *Development of a Pan-European network CV that demonstrates the breadth of technology and clinical expertise in the paediatric medical devices field, aligning this expertise to the innovation pathway (developing a 3-Dimensional CV)*
- *Establish a network of partners - clinical, industry, academic, patient and families through a scoping exercise*

- *Identify unmet needs through surveys and workshops of patients and researchers to define work streams*
- *Establish within EPTRI a platform dedicated to foster medical device research in paediatrics*
- *Collaboration with funders to develop a funding roadmap to support medical devices research*
- *Develop an educational framework in paediatric medical devices for industry, clinicians and academics*
- *Create a pathway to paediatric medical device commercialisation across Europe aligning with regulatory requirements.»*

Open Lab for Robotics and Imaging in Industry and Medicine: Maria Henke, University of Lübeck

The presentation gave an overview of the expertise, equipment, and services available at the Open Lab for Robotics and Imaging in Industry and Medicine of the University of Lübeck describing some of the current projects.

«We develop individual solutions centered on patients with several possibilities in terms of equipments: more than 10 robots, tracking systems, measurement devices, 3D printers, imaging systems (US, X-ray, etc.), and different high performance cameras.»

The development of placental research services: Frantisek Staud, Charles University

The presentation detailed the idea of developing an EPTRI Placental Platform focusing on the importance of the placenta for foetal development and programming and methods to study it (in vitro, in situ, ex vivo, in vivo) on several points of view as patho/physiology, pharmacology and toxicology.

«Why to study placental physiology/pharmacology/toxicology? Prenatal period is the foundation for later life outcomes! Placenta is the first fetal organ to be created and its functions are crucial to maintain optimal in utero conditions for proper fetal development and programming.»



PlaTox – Placenta Toxicology – human placental explants for drug testing: Udo Markert, Jena University Hospital

A project on the placenta has been presented to show the possible interaction within EPTRI and the application of the placental platform. The project aims to routinely use placental explants for reproductive toxicology or general toxicology studies in terms of transfer and accumulation of test substances. The possible placenta models and the method used to work on a placental explants have been explained.

«Placenta offers enormous advantages for toxic analysis not only for the placenta itself, but as a human organ freshly available. Potential models are cultures of cell lines, isolated cells or explant fragments from placenta cotyledons (their use is the aim of the project PlaTox). The differences between them are in terms of time exposure, availability, reproducibility and work load.»

EPTRI development in EU associated countries: Daniela Nika, University Hospital Mother Teresa

The work done in Albania to support EPTRI and the achievement reached so far have been presented. In particular, the Albanian Ministry of Health and Social Protection provided EPTRI with a letter of Political support on July 25th, 2019, University Hospital Mother Teresa has been included as partner in the recently EPTRI-IA proposal submitted under the INFRAIA-02-2020 call for proposals, the National Agency of Scientific Research and Innovation has been included in the EPTRI Memorandum of Understanding, which was one of the mandatory documents for the application of EPTRI for inclusion in the ESFRI Roadmap 2021.

«One of the expected impact of EPTRI in Albania is to reduce fragmentation of the paediatric research landscape through harmonisation of procedures, implementation of common standards and the promotion of scientific excellence.»



SESSION 4: Research Infrastructures in the life science domains

How EJP-RD can support paediatric research: Daria Julkowska, EJP RD coordinator, INSERM

The presentation gave an overview of the actual situation of the rare diseases research landscape in Europe and the role covered by the European Joint Programme on Rare Diseases (EJP-RD) recognising the importance of collaboration to improve the outcomes with benefit for patients. Dr. Julkowska also underlined that at present (as based on the EJP-RD survey to more than 700 rare diseases researchers) the rare diseases (RD) community is not aware (less than 30%) about existing research infrastructures or resources that can support their research. The goal of the EJP-RD is to create rare diseases research ecosystem by connecting all stakeholders and enhancing the power of existing and new resources, services and solutions. The support to and from research infrastructures is part of this strategy.

«The Research Infrastructures are very important part of rare diseases research ecosystem, some of them being RD-specific (e.g. Orphanet, RD Connect and the new platform of the European Commission dedicated to rare diseases registries), and others covering different medical expertise but of high-added value, like EPTRI which is providing support to research in paediatric diseases.»

Ethical, Legal and social issues in paediatric research: the proposed ELSI service: Viviana Giannuzzi, EPTRI Ethical Advisory Board Member

The presentation provided an overview of the ethical, legal and social issues that apply in paediatric Research & Development and a proposal for an ELSI service to be developed in the context of a EU research infrastructure dedicated to paediatrics. Possible synergies that EPTRI can develop on this topic with other research infrastructures, such as BBMRI and ECRIN, were described, as well.

«A RI devoted to paediatrics research will help to addressing ethical and legal uncovered issues related to research with a translation approach in paediatrics:

- *to bridge between preclinical and clinical R&D*
- *to strengthen the efforts avoiding duplications in research, speeding up the availability of proper health products for children / robust data for paediatric medicines and medical devices development.»*

Interest of INFRAFRONTIER in paediatric research: Michael Raess, INFRAFRONTIER Research Infrastructure

The presentation gave an overview of the expertise and services available within INFRAFRONTIER, which provides access to more than 7300 mouse lines and to large-scale phenotyping facilities for rodent models of human diseases.

INFRAFRONTIER collaborates with several community and networks such as IMPC, EOSC-Life, EJP-RD. M. Raess provided examples of INFRAFRONTIER projects in paediatrics: INFRAFRONTIER provided mouse models for studies on novel early-onset multiorgan syndrome FINCA and phenotyped mouse models for the paediatric diseases GRACILE.

«INFRAFRONTIER is already involved in and provides resources for pediatrics research and we recognise the importance of this research. INFRAFRONTIER is open to the input from the paediatrics community on how to improve its resources and services for paediatric research and explore avenues for cooperation!»

EU-OPENSSCREEN - The European Research Infrastructure Consortium for Chemical Biology and early Drug Discovery: Bahne Stechmann, EU-OPENSSCREEN-ERIC

The presentation highlighted the importance of the Academic drug discovery efforts that provide critical contributions to the development of novel drugs as to develop chemical probes for drug target validation. Moreover, he presented the structure and the services of EU-OPENSSCREEN and the available ground for cooperation with EPTRI.

«EU-OPENSSCREEN can contribute with services that are of interest for the paediatric research community and that are not available within EPTRI as access to compound collections, high-throughput screening capabilities and medicinal chemistry optimisation, to develop 'probes' that can be relevant for the EPTRI community for target validation (i.e. in cancer cell line, immortalised cell line, etc.). EPTRI would help EU-OPENSSCREEN to engage directly with paediatric research community and EU-OPENSSCREEN can advise paediatric community on developing bioassays that are amenable to high-throughput screening.»

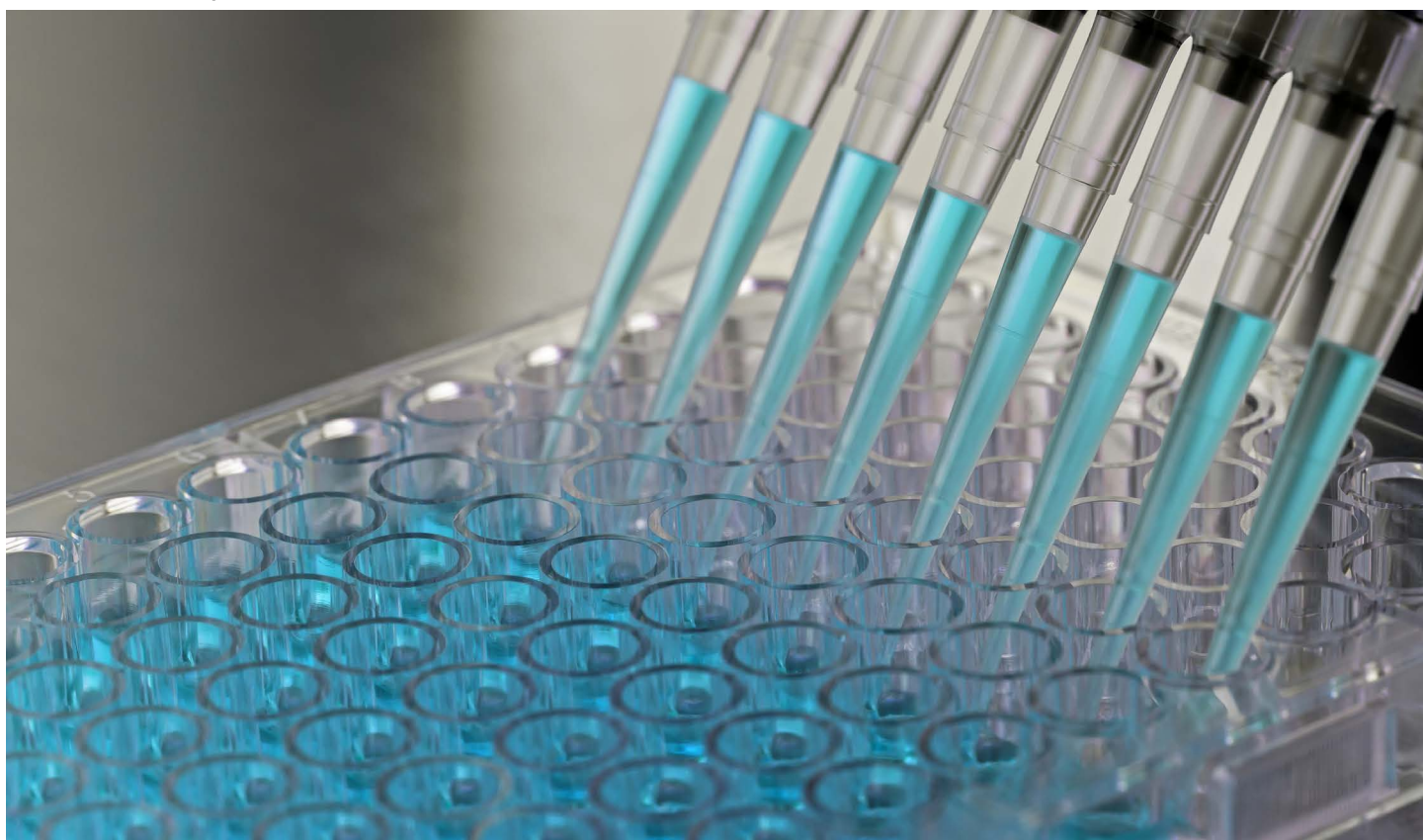


Paediatric Data Interoperability common service in collaboration with ELIXIR: Pier Luigi Martelli, University of Bologna, ELIXIR-IT

The presentation gave some information on the ELIXIR Research Infrastructure, its goal and services available. Moreover, the model for common service for data interoperability to be set up with EPTRI has been presented. The activities planned in the future to be developed with EPTRI are: electronic documents and data e-library, "Data interoperability" common service, and text mining and natural language processing tools.

«The first activities foreseen to connect EPTRI with ELIXIR are:

- *Exploratory phase to collect needs and requirements of the EPTRI community*
- *Prioritization of the possible interventions*
- *Definition of standard formats, metadata, ontologies (FAIRsharing) and implementation of new standards when needed*
- *Adoption of best practices to make existing resources findable and interoperable (Bioschemas, Identifiers.org)*
- *Inclusion in federated resources for sharing data on human variations (Beacon)*
- *Training on interoperability best practices to people involved in data management*
- *Training on best practices to release new software and analysis workflows ensuring reusability (Biocontainers, CWL).»*



The ESFRI framework to promote excellence science in Europe: Nadezda Witzanyova, Technology Center of the Czech Academy of Sciences

The presentation provided an overview of the path of ESFRI from the beginning of its history to the actual status with its objectives, methods of work and achievements reached. The importance to use a broad synergetical view for Research Infrastructure (RI) planning, construction and operation was illustrated, and the outcome of this approach was shown by comparing the situation of RIs in Czech Republic in 2008 and in 2020.

«ESFRI generally promotes excellence, where excellence begins even by the methods of work which are not bureaucratic, but based on open communication and fresh reasoning. The main outcome of such a work is that the ESFRI roadmap is a rather a process than a final set of projects, and responds to the changes of European society. What is important for a new RI proposal is to bring additional European added value, and to fit in the ecosystem of already existing projects. I congratulate the EPTRI organisers that they invited so many of the ESFRI landmarks in the biomedical area, and plan to fill in the relevant gaps of the current health RIs landscape.»

EPTRI in the ESFRI landmark: Giovanni Migliaccio, Consorzio per Valutazioni Biologiche e Farmacologiche Scientific Director

The presentation discussed the integration of EPTRI within the ESFRI landmarks identified through some targeted actions and the results of a Landmark RI survey to identify scopes and services, establish Common Services and shared expertise, and focus on unique areas of expertise and services.

«There are relevant research activities and services within the EPTRI community that are not provided by any of the existing RIs. In particular the research areas specific for the youngest element of the paediatric population from newborn to toddlers are rarely addressed in the current offer.»



SPEAKERS LIST

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EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE

EPTRI

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