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# **ID-EPTRI project**

## **European Paediatric Translational Research Infrastructure**

MEETING AGENDA

**EPTRI Open Meeting**

2<sup>nd</sup> – 3<sup>rd</sup> April, 2020

## EPTRI - European Paediatric Translational Research Infrastructure

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.

It is a pan-European initiative which involves organisations from more than 20 EU and non-EU countries, including non-profit research organisations, top-level universities, scientific and clinical centres 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 four **Thematic Research Platforms**:

- Paediatric Medicines Discovery;
- Paediatric Biomarkers and Biosamples;
- Developmental Pharmacology;
- Paediatric Medicines Formulations and Medical Devices

EPTRI will be established as a **Distributed RI based on a Hub and Spoke operational model**, which allows the participating Research Units (RUs) to be aggregated in Thematic Research Platforms (TRPs) and the Member States to organise the local resources within National Nodes able to participate to international activities in a coordinated manner.

EPTRI will **be an open science space to foster cooperation in the paediatric research** and allowing exchange of competences in a system of interconnected research areas to guarantee scientific innovation into paediatric clinical studies and new medicinal products. EPTRI will also make a very significant contribution to raising public awareness and enhancing the need of science for children's health through developing impactful activities for the target of patients and families, and the public.

**It 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.

The first day of the meeting will provide an overview of the activities carried out in this two years' project, together with a description of the future EPTRI architecture in terms of business model, governance model, tools and services to be offered, based on the current capacity and resources of the Institutions and research units that will constitute the specialized technological platforms.

During the second day 2, the oral presentations of the original abstracts and reviews, which were selected according to a call for proposals launched on several hot topics refererable to the EPTRI thematic research platforms, will be presented.

All the abstracts selected as oral or poster presentations will be published as videos in the EPTRI website.

A dedicated session on the advocacy and the importance of the networking and outreach is foreseen in the afternoon of the second day.

## EPTRI Open Meeting AGENDA

**2<sup>nd</sup> April 2020**

10.00-12.00 <sup>1</sup>	<b>SESSION 1: EPTRI Conceptual and Technical Design</b>	
	Opening remarks	
	The EPTRI project challenges	D. Bonifazi
	EPTRI Governance Model and Business Plan	G. Migliaccio
	EPTRI process towards ESFRI	M. Lupo
	EPTRI Central Hub and Single Access Point	F. Bonifazi
	EPTRI Advisory Board and centralised services	A. Ceci
12.00-14.00	<b>Break</b>	
14.00-16.00	<b>SESSION 2: EPTRI Thematic Research Platforms</b>	
	Thematic Research Platform on "Paediatric Medicines Discovery"	E. Mikros
	Thematic Research Platform on "Paediatric Biomarkers"	M. Kleanthous M. Phylactides
	Thematic Research Platform on "Developmental Pharmacology"	E. Jacqz-Aigrain S. de Wildt
	Thematic Research Platform on "Paediatric Formulations"	C. Tuleu
	EPTRI underpins medicines development to Paediatric Clinical Studies	M. Turner

**3<sup>rd</sup> April 2020**

10.00-11.00	<b>SESSION 3: EPTRI Open Scientific Session</b>	
	Human fibro-adipogenic precursor cells as a new tool for investigating antifibrotic drugs in muscular dystrophies.	J. Diaz-Manera
	Functional characterization of two genes deleted in Phelan McDermid Syndrome: SHANK3 and SULT4A1	C. Verpelli
	The neonatal Göttingen Minipig: a useful model for paediatric drug development	S. Van Cruchten
	In silico methodologies and tools for predicting developmental toxicity	C. Altomare

<sup>1</sup> All times refer to Central European Summer Time (CEST)

<b>11.00-12.00</b>	<b>SESSION 4: EPTRI Open Scientific Session</b>	
	Institute of pediatrics, obstetrics and gynecology: review, experience and prospective research	R. Marushko
	Autism Spectrum Disorder: Linked-Read Sequencing Reveals New and Undetected Variants	A. Mezzelani
	Child-appropriate multiplex mass spectrometric assay solutions to reliably monitor the maturing renin-angiotensin-aldosterone-system und the adjoining kinin-kallikrein-system in low sample volumes	B. Burckhardt
	Drug safety in translational paediatric research - How to anticipate and manage treatment related risks	B. Aurich
	Intestinal TCR $\gamma\delta$ + T cells and IL-4+ T cells: biomarkers to evaluate the transition from potential to acute celiac disease in paediatric patients	C. Gianfrani
<b>12.00-13.00</b>	<b>SESSION 5: EPTRI Open Scientific Session</b>	
	In silico modelling and simulation for dosage selection in pediatric patients including neonates	E. Jacqz-Aigrain
	Correction of the drug dosage according to changes in the weight of young children during long-term treatment can reduce the toxicity and side effects	N. Gorovenko
	Investigation and development of propranolol hydrochloride oral solid formulations designed for paediatric patients	N. Denora
	Breath-triggered aerosol release and real-time determination of the delivered aerosol for (pre)term neonates	F. C. Wiegandt
<b>13.00-14.00</b>	<b>Break</b>	
<b>14.00-16.00</b>	<b>Session 6: EPTRI Outreach and Development</b>	
	EPTRI Communication and Advocacy	B. Nafria
	EPTRI Outreach and Networking	D. Athanasiou
	Paediatric ELSI common service in collaboration with BBMRI	M. Mayrhofer
	Paediatric Data Interoperability common service in collaboration with ELIXIR	P. Martelli G. Pesole
	EPTRI GA decisions and next steps	D. Bonifazi

## EPTRI Open Meeting **SPEAKERS LIST**

**Cosimo Damiano Altomare**, University of Bari Aldo Moro, Italy

**Dimitrios Athanasiou**, Paediatric Committee, European Patients Forum, Greece

**Beate Aurich**, Robert Debré Hospital, France

**Donato Bonifazi**, Consorzio per Valutazioni Biologiche e Farmacologiche, Italy

**Fedele Bonifazi**, Gianni Benzi Pharmacological Research Foundation, Italy

**Bjoern Burckhardt**, Heinrich-Heine-University Dusseldorf, Institute of Clinical Pharmacy and Pharmacotherapy, Germany

**Adriana Ceci**, Gianni Benzi Pharmacological Research Foundation, Italy

**Daniela Corda**, National Research Council, Italy

**Nunzio Denora**, University of Bari Aldo Moro, Italy

**Jordi Diaz-Manera**, Newcastle University, UK

**Carmen Gianfrani**, National Research Council, Institute of Biochemistry and Cell Biology, Italy

**Natalia Gorovenko**, Shupyk National Medical Academy of Postgraduate Education, Ukraine

**Evelyne Jacqz-Aigrain**, Assistance Publique - Hôpitaux de Paris, Robert Debré Hospital, France

**Mariangela Lupo**, TEDDY European Network of Excellence for Paediatric Clinical Research, Italy

**Pierluigi Martelli**, University of Bologna, Italy

**Rostyslav Marushko**, State Institute of Pediatrics, Obstetrics and Gynecology, Ukraine



**Michaela Theresia Mayrhofer**, BBMRI-ERIC, Biobanking and Biomolecular Resources Research Infrastructure, Austria

**Alessandra Mezzelani**, National Research Council, Institute for Biomedical Technologies, Italy

**Giovanni Migliaccio**, Consorzio per Valutazioni Biologiche e Farmacologiche, Italy

**Emmanuel Mikros**, Athena Research and Innovation Center in Information Communication & Knowledge Technologies, Greece

**Begonya Nafria**, Institut de Recerca Sant Joan de Déu, Fundacio Sant Joan De Déu, Spain

**Graziano Pesole**, Italian National Research Council, Italian Node of ELIXIR, European Bio-Informatics Infrastructure, Italy

**Marios Phylactides**, Cyprus Institute of Neurology and Genetics, Cyprus

**Marina Kleanthous**, Cyprus Institute of Neurology and Genetics, Cyprus

**Catherine Tuleu**, School of Pharmacy, University College of London, UK

**Mark Turner**, University of Liverpool, UK

**Steven Van Cruchten**, University of Antwerp, Belgium

**Chiara Verpelli**, National Research Council, Institute for Neuroscience, Italy

**Felix Carl Wiegandt**, Fraunhofer Institute for Toxicology and Experimental Medicine, Germany

**Saskia de Wildt**, Radboud University, the Netherlands

