



ePTRI

EUROPEAN **PAEDIATRIC** TRANSLATIONAL RESEARCH INFRASTRUCTURE

3rd General Assembly

Meeting Agenda

March 27th-28th, 2019

Ministry of Health, Consumption and Social Welfare

Paseo del Prado 18 - Madrid - Spain

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 drugs research** from basic science on human development and biomarkers discovery to developmental pharmacology and paediatric medicines formulations and medical devices.

EPTRI represents the answer to an unmet need, the serious lack of medicines tailored for children worldwide. The EPTRI infrastructure will be composed of five technical and scientific domains including:

- 4 thematic platforms:

- ❖ **Human Development and Paediatric Medicines Discovery**
- ❖ **Paediatric Biomarkers and Biosamples**
- ❖ **Developmental Pharmacology**
- ❖ **Paediatric Medicines Formulations and Medical Devices**

- the scientific domain "**Underpinning Medicines Development to Paediatric Studies**" acting as a bridge between EPTRI and other RIs and paediatric clinical trials networks.

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.

DAY 1

EPTRI Design and Governance

Workshop on PPI (Patient and Public Involvement in Research)

Roundtable with Stakeholders

During the Day 1 it will be provided an overview of the activities carried out in the first year of the 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.

A dedicated focus to patients will be offered with a workshop where the actors will be youth leaders of the Young Persons Advisory Groups (YPAGs) involved in the EPTRI project that, indeed, takes care of the voice of children and young patients to ensure that the design of the future Research Infrastructure (EPTRI) is patient centred.

A Roundtable with the Stakeholders will involve representatives from Paediatric Networks, RIs, ERNs, Charities, Patients' associations, YPAGs, Companies' Federations, Regulatory Agencies, Governments, Authorities, with the aim to promote the entrance of EPTRI in a global framework of partnership able to strengthen paediatric research and to support the growth of EPTRI along with the vision of the main Stakeholders.

DAY 2

Mapping of EPTRI and Sustainability

EPTRI platforms and feasibility assessment

The second day of the meeting will be mainly focused on the activities carried out in the context analysis of the project. The preliminary results of the survey aimed to collect interest and detect potential services providers to be included in the future RI will be presented and the user survey will be launched. The next session will be aimed to present the sustainability model of EPTRI in the European scenario and the activities carried out in the different levels (scientists, patients, governments, collaboration with other RIs and paediatric initiatives, etc.). It will be underlined the strategic role that each country and each top-leading research Institutions will be able to play in the building of the new RI.

Then, the design of the research activities provided by the thematic platforms will be presented as well as the feasibility studies that will allow to simulate the activities to be carried out during the next implementation phase, that will run in parallel with the proposal of inclusion of EPTRI into the 2020 ESFRI (European Strategic Forum on Research Infrastructures) Roadmap.

AGENDA

Day 1, March 27th

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| 9.00 | REGISTRATION | |
| | SESSION 1: EPTRI Design and Governance Chairs: A. Ceci, J. Demotes-Mainard | |
| | <ul style="list-style-type: none"> Draft of the Conceptual Design Report (G. Migliaccio) Draft of the Technical Design (F. Bonifazi) Communication and dissemination strategy to be included in the CDR (B. Nafria) Draft of the Governance Model (B. Lydall) Draft of the ELSI paediatric common service (S. Casati) Survey of potential users (C. Owen) | |
| 11.00 | Coffee Break | |
| | OPENING SESSION | |
| 11.30 | Welcome address | (D. Bonifazi) (M. Mellado) (R. Yotti Álvarez) |
| | Open Meeting SESSION 2: Roundtable with Patients Chairs: M. Lupo, B. Nafria, J. Claverol Torres, C. Enache | |
| 12.00 | <ul style="list-style-type: none"> Presentations of youth members of YPAGs on their contribution to the RI | |
| 13.00 | Lunch | |
| | Open Meeting SESSION 3: Workshop on Patient and Public Involvement in Research (PPI) Chairs: E. Mikros, M. Lavitrano, E. Jacqz-Agrain, C. Tuleu | |
| 14.00 | Two parallel break-out sessions will be organised to discuss about the strategies for the involvement of patients and their families in the field of paediatric research | |

Open Meeting

SESSION 4: Roundtable with the stakeholders

Chairs: M. Mellado, D. Bonifazi

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| 15.00 | Representatives from Paediatric Networks, RIs, ERNs, Charities, Patients' associations, YPAGs, Companies' Federations, Regulatory Agencies, Governments, Authorities | (A. Ambrosini, T. Andreu, D. Athanasiou, E. Bermejo-Sanchez, J. Claverol Torres, M. Dehlinger Kramer, R. Diaz, K. A. Fodor, C. Hernandez, M. Gavish, V. Kazantsev, P. Macheras, I. Palanca Sánchez, G. Pesole, N. Ruperto, F. Schaefer, M. Turner, A. M. Uranga) |
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| 17.00 | Parallel session Appointment of the Advisory Board Chair: F. La Neve | (D. Athanasiou, A. Ceci, O. Della Pasqua, C. Knibbe, G. Migliaccio, G. Pesole, C. Tuleu) |
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18.00 **END OF DAY**

Dinner and Flamenco show

Day 2, March 28th

SESSION 5: Coordination and project management

Chairs: L. Mangiarini, A. Ussi

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| 9.00 | <ul style="list-style-type: none"> EPTRI 1st year sum-up of activities and assessment (M. Lupo) EPTRI 1st year Financial and Technical Reports (G. Vecchia) |
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SESSION 6: Mapping of EPTRI

Chairs: C. Calvo, J. Kindblom

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| 10.00 | <ul style="list-style-type: none"> Gap analysis (I. Wong) Results of the reopening of the survey (A. Landi) Ethical revision in the European Paediatric Translational Research Infrastructure (A. Altavilla) |
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11.00

Coffee Break

SESSION 7: EPTRI Sustainability

Chairs: D. Bonifazi, E. Steinfelder

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| 11.30 | <ul style="list-style-type: none"> ▪ Feasibility study with the Governments (G. Migliaccio) ▪ Relationship with existing RIs (J. Demotes-Mainard) ▪ Draft of the Business Model (B. Lydall) |
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13.00

Lunch

SESSION 8: EPTRI thematic platforms

Chairs: M. Lavitrano, N. Denora

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| 14.00 | <p>Excellence and priorities of research activities provided by the thematic Platforms:</p> <ul style="list-style-type: none"> ▪ Human Development and Paediatric Medicines Discovery (E. Mikros) ▪ Paediatric Biomarkers and Biosamples (M. Phylactides) ▪ Paediatric Pharmacology (S. de Wildt) ▪ Placenta Platform Feasibility (E. Jacqz-Aigrain) ▪ Paediatric Medicines Formulations and Medical Devices (C. Tuleu) |
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16.00

Coffee Break

SESSION 9: EPTRI feasibility assessment

Chairs: C. Altomare, A. Ceci

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| 16.30 | <ul style="list-style-type: none"> ▪ Feasibility study in the haemoglobinopathies field using biomarkers and databanking (M. Kleanthous) ▪ Underpinning medicines development to paediatric clinical studies (M. Turner) ▪ Pilot study on data re-use (M. Sturkenboom) |
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17.30

Meeting closure and greetings from Ministry of Health representatives

P. Aparicio Azcárraga

LIST OF CHAIRS AND SPEAKERS

Anna Ambrosini, Head of the Neuromuscular Research Programs, Research & Development of Telethon Foundation; Scientific Director of the Italian Research Foundation for Amyotrophic Lateral Sclerosis (AriSLA), Italy

Annagrazia Altavilla, TEDDY European Network of Excellence for Paediatric Clinical Research, Italy

Cosimo Altomare, Department of Pharmacy, University of Bari "Aldo Moro", Italy

Toni Andreu, Scientific Director of the European Advanced Translational Research Infrastructure in Medicine (EATRIS-ERIC), the Netherlands

Pilar Aparicio Azcárraga, General Director of Public Health, Quality and Innovation, Ministry of Health, Consumption and Social Welfare, Spain

Dimitrios Athanasiou, PDCO EMA, EPAC/TAG Eurordis, UPPMD, MDA Hellas, Greece

Eva Bermejo-Sánchez, Tenured Scientist-Chief of Area at the Research Institute for Rare Diseases (IIER), ECEMC's Scientific Coordinator, the Research Center on Congenital Anomalies (CIAC), Instituto de Salud Carlos III, Spain

Donato Bonifazi, Chief Executive Officer of Consorzio per Valutazioni Biologiche e Farmacologiche, Italy

Fedele Bonifazi, Vice President of Gianni Benzi Pharmacological Research Foundation, Italy

Cristina Calvo, Head Clinician, Pediatric and Infectious Diseases Department, Hospital Universitario La Paz, Spain

Sara Casati, Common service ELSI expert of the Biobanking and Biomolecular Resources Research Infrastructure (BBMRI-ERIC), Italy

Adriana Ceci, President of Gianni Benzi Pharmacological Research Foundation, Italy

Joana Claverol Torres, Member of the Coordination team of the European Young Persons' Advisory Group network (eYPAGnet), Spain

Oscar Della Pasqua, University College London, UK

Martine Dehlinger Kramer, President of the European Clinical Research Organization Federation (EUCROF), Germany

Jacques Demotes-Mainard, General Director of ECRIN-ERIC, European Clinical Research Infrastructure Network, France

Nunzio Denora, Department of Pharmacy, University of Bari "Aldo Moro", Italy

Ruben Diaz Naderi, Deputy CEO, International Affairs, Secretary General of the European Children's Hospitals Organisation (ECHO), Spain

Cristina Enache, Romanian Angel Appeal Foundation, Romania

Krisztian András Fodor, Head of the Department of Strategy, Development and Methodology of the National Institute of Pharmacy and Nutrition (OGYEI), Hungary

Moshe Gavish, Technion-Israel Institute of Technology (TECHNION), Israel

César Hernández, Head of Department of Medicines for Human Use of the Agencia Española de Medicamentos y Productos Sanitarios (AEMPS), Spain

Evelyne Jacqz-Aigrain, Professor at Pediatric Pharmacology and Pharmacogenetics, Paris Diderot University, Hôpital Universitaire Robert Debré, France

Victor Kazantsev, Vice Rector National Research Lobachevsky State University of Nizhny Novgorod, Russia

Jenny Kindblom, Region Västra Götaland, Sweden

Marina Kleanthous, Head of the Molecular Genetics Thalassaemia Department, Cyprus Institute of Neurology and Genetics, Cyprus

Catherijne Knibbe, Professor of Individualized Drug Treatment, Leiden University, the Netherlands

Federica La Neve, Ospedale Pediatrico Bambino Gesù, INCIPIT – Italian Network for paediatric Clinical trials, Italy

Annalisa Landi, Researcher at Gianni Benzi Pharmacological Research Foundation, Italy

Marialuisa Lavitrano, University of Milano-Bicocca, Coordinator of the Italian node of the Biobanking and Biomolecular Resources Research Infrastructure (BBMRI-ERIC), Italy

Mariangela Lupo, Project Manager at Teddy European Network of Excellence for Paediatric Clinical Research, Italy

Ben Lydall, Finance and Sustainability Specialist at EATRIS-ERIC, European Research Infrastructure for Translational Medicine, the Netherlands

Panos Macheras, Emeritus Professor, Department of Pharmacy at the National and Kapodistrian University of Athens, Head of the PharmaInformatics Unit at ATHENA Research and Innovation Center in Information Communication & Knowledge Technologies, Greece

Laura Mangiarini, Fondazione PENTA, Italy

Maria José Mellado, Associate Professor of Universidad Autónoma de Madrid, Servicio Madrileño de Salud, Hospital University La Paz, Spain

Giovanni Migliaccio, Scientific Director of Consorzio per Valutazioni Biologiche e Farmacologiche, Italy

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

Begonya Nafria, Patient Engagement in Research Coordinator at Institut de Recerca Sant Joan de Déu, Fundacio Sant Joan De Deu, Spain

Carla Owen, University of Liverpool, c4c Research Operations Manager, UK

Inés Palanca Sánchez, Senior Advisor at Spanish National Health System Quality Agency in Ministry of Health Consumption and Social Welfare, Spain

Graziano Pesole, Italian National Research Council (CNR), Director of the Institute of Biomembranes, Bioenergetics and Molecular Biotechnologies, Head of the Italian Node of ELIXIR, European Bio-Informatics Infrastructure, Italy

Marios Phylactides, Cyprus Institute of Neurology and Genetics, Cyprus

Nicola Ruperto, Coordinator of the Paediatric Rheumatology International Trials Organisation (PRINTO), Italy

Franz Schaefer, Coordinator of the European Reference Network for Rare Kidney Diseases (ERKNet), Germany

Eric Steinfelder, General Director of the BBMRI-ERIC, Biobanking and Biomolecular Resources Research Infrastructure, Austria

Miriam Sturkenboom, University Medical Center Utrecht, the Netherlands

Catherine Tuleu, Professor in Paediatric Pharmaceutics, School of Pharmacy, University College of London, UK

Mark Turner, Conect4Children (c4c) Coordinator, University of Liverpool, UK

Amelia Martín Uranga, Responsible of the Innovative Drugs Platform of Farmaindustria, Spain

Anton Ussi, Administrative Director of EATRIS-ERIC, European Research Infrastructure for Translational Medicine, the Netherlands

Giulio Vecchia, Project Manager at Fondazione PENTA, Italy

Saskia de Wildt, Radboud University Nijmegen, the Netherlands

Ian Wong, School of Pharmacy Director, University College of London, UK

Raquel Yotti Álvarez, General Director of Institute of Health Carlos III, Spain

