



1st

**INTERNATIONAL CONFERENCE
ON RARE DISEASES AND
PAEDIATRIC RESEARCH**

- The future of the Regulatory framework
- Innovative technologies and Health Data
- RD and Paediatric research: the role of Networks and Infrastructures
- Supporting tools and funding in RD and Paediatric Research
- Rare and paediatric diseases during the Covid-19 pandemic

Virtual Event
18/11 - 19/11 2021

SUPPORTERS



SCIENTIFIC PROGRAM

**Day 1 – Thursday 18 November 2021**

(The time zone is Central Europe Time)

08.30-09.00 Welcome - Opening Remarks and Conference presentation (EURORDIS)

- **Marco Greco**, European Patient Forum Chair
- TBA, Eurordis
- **Katerina Koutsogianni**, Greek Patient Association President

09.00-10.00 Setting the Scene: Shaping the future of Policy and the Regulatory framework in RD and Paediatrics

Dimitrios Athanasiou, *World Duchenne Organisation (WDO), European Patient Forum (EPF), "95" Rare Alliance Greece*

- **Pharmaceutical Strategy, the European Parliament perspective**
Cyrus Engerer, European Parliament
- **The revision of both the paediatric and orphan Regulations**
Fabio D'Atri, Health and Food Safety Directorate-General, European Commission
- **Regulatory perspective on Paediatric Medicines from PDCO**
Sabine Scherer, Paediatric Committee (PDCO) at the European Medicines Agency
- **Regulatory perspective on Orphan Medicines from COMP**
Violeta Stoyanova-Beninska, Committee for Orphan Medicinal Products (COMP) at the European Medicines Agency

Panel Discussion**10.00-11.00 Promoting Research and Development of Paediatric and RD medicine**

- **Orphan Drug Development Guidebook-ODDG a tool to speed up orphan medicines development**
Virginie Hivert, EURORDIS
- **Orphan drugs and paediatric medicines: the current scenario in EU and USA**
Viviana Giannuzzi, PharmD, PhD, Fondazione per la Ricerca Farmacologica Gianni Benzi Onlus
- **Promoting Research in Europe**
Christina Kyriakopoulou, Scientific Officer at the European Commission
- **Current Challenges and Opportunities in RD and Paediatric Research for Industry**
Claudio Fracasso, Pediatric Clinical Director, Pediatric Center of Excellence, Pfizer

Panel Discussion**11.00-11.30 Break****11.30-12.30 Innovative technologies and methodologies for RD and paediatric research**

- **The child health technology frontier in easing paediatric diseases**
Paul Dimitri, Sheffield Children's NHS Foundation Trust
- **Advanced therapies for genetic diseases**
TBA
- **"Paediatric Repurposing Innovative Medicines in Europe – PRIME" project 'to meet children's needs first'**
Oscar della Pasqua, Professor of Clinical Pharmacology and Therapeutics Pharmacology, UCL School of Pharmacy
- **In silico drug repurposing in rare and paediatric diseases**
Georgia Tsiliki, Purposeful, "Athena" Research & Innovation Center
- **Modelling, simulation and extrapolation in RD and Paediatrics**
Cecile Ollivier, Regulatory Science Advisor - Europe, Critical Path Institute

Panel Discussion

**Day 1 – Thursday 18 November 2021****12.30-13.30 Health Data in RD and Paediatrics**

- *The importance of health data: paediatric databases interoperability, real-world data, data sharing and data reuse*
TBA
 - *Guidance on the Impact of EU data protection legislation on the use of health data*
Yannis Ioannidis, "Athena" Research & Innovation Center, Professor at the Department of Informatics and Telecommunications of the University of Athens
 - *Real World Evidence Generation and the benefits of Patient-Reported Outcome Measures (PROMs)*
TBA
 - *Data initiatives driven for and by patients, the Duchenne example*
Elvina Sakellariou, Research Project Manager Duchenne Data Foundation
 - *The Value of the Health Data, the Industry perspective*
Claudio Santos, SVP Global Medical Affairs, PTC Therapeutics
- Panel Discussion**

13.30 -14.30 Break**14.30 – 15.30 Hot topics**

- *Roundtable with Kids #KidsinResearch*
Mariangela Lupo, Networking and Patients Advocacy Manager, TEDDY European Network of Excellence for Paediatric Research
- *Children participation in decision making in health: the CoE initiative to promote children rights*
Annagrazia Altavilla, Chair of TEDDY European Network of Excellence for Paediatric Research
- *Young patients involvement in the European Reference Networks*
Maurizio Scarpa, Coordinator of MetabERN, European Reference Network for Rare Hereditary Metabolic Disorders
- *Optimising patients' involvement in paediatrics and Rare Diseases*
Nathalie Bere, Public Engagement Department, European Medicines Agency

15.30-16.30 #SHARINGISCARING – An open multi-stakeholder discussion**Topic 1 - What about patient data?**

- *For Patients* – TBA
- *For HCPs* - Antonio Marques Atalaia, EURO NMD Institut-Myologie
- *For Academia* - Carine de Beaufort, Clinique Pédiatrique/CH de Luxembourg, International Society for Pediatric, Adolescent and Young Adult Diabetes, DECCP
- *For Industry* – George Faidon Kalomiris, General Manager Greece, Malta & Cyprus, TAKEDA HELLAS SA
- *For Regulators* - TBA

16.30 Closing Session

**Day 2 – Friday, 19 November 2021****08.30-09.00 Welcome****Opening Remarks and Conference Day 1 Recap****09.00-10.00 Clinical trial on Rare Diseases and Paediatric Research: a changing Regulatory Framework**

- **Keynote speech “The new aspects of patient involvement in the clinical trials regulation No 536/2014”**

Ann Marie Janson Lang, Swedish Medical Products Agency, Co-Chair CTFG - Clinical Trials Facilitation and Coordination Group

- **Recommendations by the European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA) Working Group on the preparedness of clinical trials about paediatric medicines process**

Angeliki Siapkara, Acting Group manager Benefit Risk Management Group, VRMM, MHRA

- **Clinical Trial Readiness: The patients’ contribution**

Martine Dehlinger-Kremer, ICON Plc & European CRO Federation (EUCROF)

- **EUCROF Code of conduct on GDPR and what it means for the patients**

Yoani Matsakis, Telemedicine Technologies, EUCROF

Panel Discussion

10.00 -11.00 Supporting tools and funding opportunities in RD and Paediatric Research

- **How EJP-RD can support Rare and Paediatric Research and the European Partnerships for RDs**

Daria Julkowska, Coordinator of EJPRD - Europe Joint Programme on Rare Diseases

- **Current and Future funding opportunities for Rare Diseases and Paediatric Research**

TBA, European Commission

- **Innovative Health Initiative – new horizons for patient-centric public-private collaboration**

TBA, IMI

- **The new EU Research framework: How the new Horizon Europe encouraging Rare and Paediatric disease research**

TBA, European Parliament

- **Plan for paediatric research in Europe in the light of the new funding opportunities**

Adriana Ceci, European Paediatric Translational Research Infrastructure, Fondazione per la Ricerca Farmacologica Gianni Benzi Onlus

Panel Discussion

11.00-11.30 Break**11.30 – 12.00 Hot topics**

- **Modelling on COVID-19 spreading worldwide**

Panos Macheras, "Athena" Research & Innovation Center

- **How lockdown is impacting on the quality of life of RD and paediatric patients**

Sandra Courbier, EURORDIS

- **The patient way is the only way to foster Research progress**

Begonya Nafria, Patient Engagement in Research Area, Institut de Recerca Sant Joan de Déu



Day 2 – Friday, 19 November 2021

12.00-13.00 **The role of RD and Paediatric Networks and Infrastructures in Research**

- 🕒 *The role of ERICA in the European Research Area*
Mari Murel, ERICA
 - 🕒 *The role of conect4children (c4c) in the development of paediatric drugs and therapies*
Carlo Giaquinto, Penta Foundation, Coordinator of Conect4children
 - 🕒 *Innovation and quality in paediatric rare diseases research*
Maurizio Scarpa, Coordinator of MetabERN, European Reference Network for Rare Hereditary Metabolic Disorders
 - 🕒 *The European Paediatric Translational Research Infrastructure (EPTRI) to integrate experiences and create a system to accelerate the availability of new paediatric therapies, medicines and devices*
Donato Bonifazi, Ceo of Consorzio per Valutazioni Biologiche e Farmacologiche, Coordinator of EPTRI, European Paediatric Translational Research Infrastructure
 - 🕒 *The value to Industry of working with European Pediatric Research Infrastructures and Pediatric Trial Networks*
Heidrun Hildebrand, Bayer AG, Pharmaceuticals, Pediatric Development
- Panel Discussion**

13.00-14.00 Break

14.00-15.00 **RD and Paediatric Research during the COVID-19 pandemic (Industry panel discussion)**

Chair: Solange Rohou, Astrazeneca

Speaker: Dr Aparna Parikh, MD, DCH, CCRA , ICON INDIA

Topics to be discussed:

- 🕒 *How COVID-19 pandemic has affected RD and Paediatric Research*
- 🕒 *COVID-19 pandemic and R&D,*
- 🕒 *Lessons learned*
- 🕒 *Planning for the day after*
- 🕒 *Opportunities for the future*

15.00-16.00 **#SURVIVINGTHEPANDEMIC – An open multi-stakeholder discussion**

Topic 2 - A real-life experience

- 🕒 *For Patients - Mary Adamopoulou, 95 Rare Alliance Greece, EUPATI*
- 🕒 *For HCPs/Academia - Collin Hovinga, Institute for Advanced Clinical Trials for Children, University of Texas at Austin*
- 🕒 *For Industry - Prabha Viswanathan, Food and Drug Administration*
- 🕒 *For Industry - Laura Fregonese, MD, PhD, MSc Orphan Medicines Office, European Medicines Agency*

16.30 **Closing session**

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