

## **Agenda**

# **International Conference on Clinical Research Networks for Rare Diseases**

**Le 253**  
**253 Rue du Faubourg Saint-Martin**  
**Paris, France**

**December 1-2, 2022**

## Thursday December 1<sup>st</sup>, 2022

### Opening

13h45-14h00: Welcome

- **Daria Julkowska** - *Scientific Coordinator of the European Joint Programme on Rare Diseases, France*
- **David Pearce** - *Chair of the IRDiRC Consortium Assembly, President of Research, Director of Sanford Children's Health Research Center, USA*

14h-14h30: Presentation of the IRDiRC Task Force Findings and Recommendations on Clinical Research Networks for Rare Diseases

- **Rima Nabbout** - *Department of Pediatric Neurology, Reference Center for Rare Epilepsies, Hôpital Necker-Enfants Malades, Université Paris Cité, France*

### Session 1: Presentation of Established Clinical Research Networks

14h30-15h: Presentation of the Rare Diseases Clinical Research Network

- **Tiina Urv** - *Program Director, Division of Rare Diseases Research Innovation, National Center for Advancing Translational Sciences, National Institutes of Health, USA*

15h-15h30: Presentation of the European Rare Disease Research Coordination and Support Action consortium

- **Alberto Pereira** - *Head of the Department of Endocrinology & Metabolism, Amsterdam University Medical Centers, Coordinator of the European Reference Network on Rare Endocrine Conditions, Coordinator of the European Rare Disease Research Coordination and Support Action.*

15h30-16h: Presentation of the Initiative on Rare and Undiagnosed Diseases

- **Hidehiro Mizusawa** - *National Center of Neurology and Psychiatry, Tokyo, Japan*

### Session 2: Clinical Research Networks: New Needs, New Dimension

16h30-17h: RDI-WHO Collaboration toward Global Rare Disease Networks

- **Matt Bolz-Johnson** - *Program Director, Collaborative Global Network for Rare Diseases, Rare Diseases International*

17h-18h00: Recommendations for the creation and the coordination of Clinical Research Networks for Rare Diseases: Lessons learned from major initiatives

**Moderator: Daria Julkowska** - *Scientific Coordinator of the European Joint Programme on Rare Diseases, France*

- **Marshall Summar** - *George Washington University, Children's National Hospital, USA*
- **Maurizio Scarpa** - *Udine University Hospital, European Reference Network for Hereditary Metabolic Disorders, Italy*
- **Hyun-Young Park** - *Korea National Institute of Health, South Korea*

## Friday December 2<sup>nd</sup>, 2022

### Session 3: Multi-Stakeholder Perspective on Engagement in Clinical Research Networks

9h-10h40: Pathways to stimulate interaction between academia, industry, regulators and patient groups

**Moderator: Virginie Hivert** - *Therapeutic Development Director, EURORDIS-Rare Diseases Europe, France*

- Patient perspective: **Edward Neilan** - *National Organization for Rare Disorders, USA*
- Clinical/Academia perspective: **Birute Tumiene** - *Vilnius University Hospital, Lithuania*
- Regulator perspective: **Kyriaki Tzogani** - *European Medicines Agency, The Netherlands*
- Industry perspective: **Samantha Parker** - *Innoskel, France*

### Session 4: Panel Discussion - Networking Pathways and Collaboration between Clinical Research Networks

11h-12h30: Pathways to stimulate communication between the Clinical Research Networks, cross-network training, identification of common goals

**Moderator: David Pearce** - *Chair of the IRDiRC Consortium Assembly, President of Research, Director of Sanford Children's Health Research Center, USA*

- **Katherine Beaverson** - *Pfizer, USA*
- **Marisol Montolio** - *Duchenne Parent Project, ePAG representative, Spain*
- **Franz Schaefer** - *Heidelberg University Hospital, European Rare Kidney Disease Reference Network, Germany*
- **Mark Turner** - *University of Liverpool, connect4children Consortium, United Kingdom*
- **PJ Brooks** - *Office of Rare Diseases Research, National Center for Advancing Translational Sciences, National Institutes of Health, USA*

### Concluding Remarks