

## EJP RD Policy Meeting Draft Agenda

**12 of January 2021 (13:00 – 18:30)**

### DAY 1

The focus of day one is to provide a comprehensive state of the art of existing or forthcoming initiatives and inquire about the progress expected by 2024 when the new Partnership on Rare Diseases should be implemented. Each session will bring not only information but also perspectives from different stakeholders.

12:45 – 13:00 **Welcome and introduction from EJP RD coordination** (objectives of the meeting & where do we stand with EJP RD)

*Gilles Bloch, CEO & president of INSERM & Daria Julkowska, EJP RD coordinator*

13:00 – 13:45 **Holistic approach to rare diseases care & research – the European Reference Networks** (10 min presentations + 15 min overall discussion)

ERNs – the challenge of equal care for all, *DG Santé, speaker TBC*

ERNs in 2024 - *Chair of the ERN coordinators group, Nicoline Hoogerbrugge, Radboud UMC*

The European Patient Advocacy Groups (ePAGs) – the roles and responsibilities of patients in ERNs – from today to tomorrow - *ePAG representative, Allison Watson, Ring20 Research & Support UK*

13:45 – 14:45 **The power of data** (10 min presentations + 20 min overall discussion)

European Data Strategy, EU Health Data Space, *DG CONNECT, speaker TBC*

European Open Science Cloud, *Sarah Jones, EOSC Engagement Manager*

The potential of Artificial Intelligence, *speaker TBC*

The future of regulatory science in Europe, *Jordi Llinares, EMA*

EJP RD Virtual Platform, *Ana Rath, INSERM-Orphanet & Franz Schaefer, Heidelberg University Hospital*

**14:45 – 15:05 Coffee break**

15:05 – 16:30 **Research & innovation for the benefit of rare diseases** (10 min presentations + 20 min overall discussion)

From Horizon 2020 to Horizon Europe, DG RTD, speaker TBC

13 years of multinational funding support for rare diseases, Ralph Schuster, DLR & Sonja van Weely, ZonMw

Why public-private partnerships in health are key to success, Pierre Meulien, IMI

EU Research Infrastructures, partners for RD research now and in the future, speaker TBC

Innovation potential in RD clinical trials, Rima Nabbout, APHP & Ralf-Dieter Hilgers, Aachen University

1000 new therapies by 2027, Simone Boselli, EURORDIS

### 16:30 – 16:45 Comfort break

16:45 – 17:15 **Back to basics – education & empowerment** (10 min presentations + 10 min overall discussion)

How EJP RD empowers the RD community? – Virginie Bros-Facer, EURORDIS & Biruté Tumiene, Vilnius University Hospital

EIT-Health, bridging the gap between academia and enterprise – Céline Carrera, Director of Education EIT Health, TBC

17:15 – 18:00 **Rare diseases – the global village** (10 min presentations + 15 min overall discussion)

Supporting national rare diseases strategy – opportunities & obstacles, representative of Portugal TBC

Alignment of national and EU RD strategies – where do we stand now, Domenica Taruscio, ISS, Eva Bermejo-Sanchez & Manuel Posada, ISCIII

Rare diseases without borders – IRDiRC perspective, Lucia Monaco, IRDiRC chair, FTELE & David Pearce, IRDiRC vice-chair, Sanford Health Research Center

18:00 – 18:15

### **The story of Linda Noa**

Sylvia & Edmundo Hafez, TBC

### **End of DAY1**

**13 of January 2021 (09:00 – 12:30)**

**DAY2**

The day will start with the introduction of future vision through the foresight scenarios of Rare 2030 and will set up the premises of the discussions of day 2.

The focus of day two is to identify the key components (key founding elements) of the future rare diseases partnerships and complement them by identifying key missing elements (gaps). This will be done through interactive sessions with active participation of the audience and support of moderators and driving questions. The joint restitution session will set the basis for next steps in shaping of the Rare Diseases Partnership under Horizon Europe.

**09:00 – 09:30 Investment for social justice – the future of rare diseases**

Rare 2030 perspective - *Yann Le Cam, EURORDIS*

**09:30 – 09:50 Horizon Europe Partnerships, DG RTD, speaker TBC** (10 min presentation + 10 min Q&A)

**09:50 – 10:35 Rare Diseases Partnership – key components & gap identifier interactive session**

45 min of moderated separate sessions around the 5 themes identified in day 1: ERNs, data, research & innovation, education & empowerment, national/EU/international alignment

**10:35 – 10:50 Comfort break**

**10:50 – 11:35 Rare Diseases Partnership – key components & gap identifier** - restitution of sessions outcomes

11:35 – 12:00

**Next steps & closing remarks**

*Daria Julkowska, EJP RD*

**End of DAY2**

**Moving to IRDiRC Conference & RE(ACT) Congress**

**Register at: <https://www.react-congress.org>**