

## RD-ACTION, EMA and DG SANTE Workshop: How ERNs can provide added-value in the area of clinical research

Dates: 29<sup>th</sup> -30<sup>th</sup> May 2018

Venue: European Medicines Agency, London

Day 1: 9.00 – 17.30

Day 2: 9:00 – 14:00

### Agenda DAY 1\* – ‘State of Play’

#### SESSION 1: SETTING THE SCENE – AIMS AND CONTEXT OF THIS WORKSHOP

**Session Chairs: Isabelle Moulon (EMA) and Valentina Bottarelli (RD-ACTION)**

**8:30 Registration**

**9:00: Welcome** by the EMA (Guido Rasi) and DG SANTE (Hélène le Borgne)

**9:10 Introduction to the Workshop – Aims and expected outputs:** Victoria Hedley (RD-ACTION)

**9:25: ERNs in 2018 – Where we are and where we hope to go:** Maurizio Scarpa (Chair of the ERN Coordinators’ Group and Coordinator of the MetabERN)

**9.55: ERNs and Research: state of play from the European Commission perspective:** Iiro Eerola (DG RTD) and Hélène le Borgne/ Anneli Torronen (DG SANTE)

**10:20: Questions and Discussion**

**10:30: Coffee Break**

#### SESSION 2: HOW ARE ERNS PLANNING TO ADD-VALUE TO CLINICAL RESEARCH?

**Session Chairs: Hélène le Borgne (DG SANTE) and Victoria Hedley (RD-ACTION)**

**10:45: ‘Snapshot’ of Transversal activities regarding ERNs and Clinical Research:** Eduardo López Granados (Chair of the ERNs’ Research Working Group); Eileen Treacy (Board of Member States of ERNs); Daria Julkowska (E-Rare and Coordinator of European Joint Programme Co-Fund proposal)

**11:00: Status Quo of ERNs and Clinical Research: Results of the ERN-Wide survey** (Luca Sangiorgi, EMA Liaison Chair for ERNs)

**11.20: Questions and Discussion**

**11:30: ERN Case Studies:**

- MetabERN (ERN on hereditary metabolic disorders) (Jean Michel Heard)
- ERN EURO-NMD (ERN on Neuromuscular Diseases) (Teresinha Evangelista)
- ERN BOND (ERN on Bone Disorders) (Luca Sangiorgi)
- ERN EURACAN (ERN on adult cancers (solid tumours)) (Stephane Lejeune)

**12:30: Questions and Discussion Session**

**12:50: Lunch (included)**

**13:40: Patient expectations for meaningful engagement in ERN-related clinical research:** Russell Wheeler and Alain Cornet (European Patient Advocacy Group (ePAG) representatives for ERN-EYE and ERN ReCONNECT respectively)

**14:00: Questions and Discussion Session**

### **Session 3: Overview of EMA role and support tools in medicine development**

**Session Chairs: Isabelle Moulon (EMA) and Luca Sangiorgi (ERN-BOND, alternate EMA CAT member)**

**14:15: General presentation of EMA, engagement with stakeholders, the role of academic experts:** Ivana Silva (EMA)

**14:45: Early engagement and support for medicine development:** Stiina Aarun (EMA)

**15:30: Coffee break**

**15:45: Advanced Therapy Medicinal Products (ATMPs):** Patrick Celis (EMA)

**16:15: Clinical trials in the EU and EMA registries initiative** Laura Pioppo and Xavier Kurtz (EMA)

**17:15: Questions**

**17:30: Wrap up and end of Day 1**

**17:45: Drinks Reception (Drinks and Light Refreshments served from 17:45 until 19:15)**

---

## **Agenda DAY 2 – ‘Where do we want to go, and how do we get there?’**

**09:00: Overview of Day 1 and Introduction to Day 2 Discussions** Victoria Hedley (RD-ACTION) and Luca Sangiorgi (EMA Liaison Chair for ERNs)

**Session 4: How can ERNs impact positively on future clinical research, with a particular emphasis on forging strategic EMA engagement?**

**09:30: Discussion Topic 1 - What sorts of activities under the heading of ‘clinical research’ will ERNs engage in, and what are the advantages of the ERN structure?**

**Discussion Facilitators:** Maurizio Scarpa (Chair of the ERN Coordinators’ Group and Coordinator of the MetabERN); H el ene le Borgne (DG SANTE) Victoria Hedley (RD-ACTION)

**10:30: Coffee Break**

**10:45: Discussion Topic 2 - What opportunities exist under current EMA structures and resources presented on Day 1, and how might ERNs engage with these?**

**Discussion Facilitators:** Enrica Alteri (EMA); Luca Sangiorgi (EMA Liaison Chair for ERNs and Coordinator of ERN BOND); Virginie Hivert (EURORDIS)

**11:30: Discussion Topic 3: Identifying concrete roles and recommended practices to involve patients in the various types of ERN-related Clinical Research**

**Discussion Facilitators:** Isabelle Moulon (EMA); Yann le Cam (EURORDIS); Andrea Gressani (ePAG representative for ERN-RITA)

**12:15: Lunch (Provided)**

**12:45: Discussion Topic 4 - How can ERNs generate/link/exchange data to support the planning and execution of clinical trials and studies?**

**Discussion Facilitators:** Fergus Sweeney (EMA); Ana Rath (RD-ACTION)

**13:30: Concluding Remarks and Next Steps:** Victoria Hedley (RD-ACTION), Luca Sangiorgi (EMA Liaison Chair for ERNs), Isabelle Moulon (EMA)

**14:00: Workshop Ends**

**\*N.B. – Day 1 sessions will be live-streamed. There will be no streaming on Day 2**