

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

Dates: 29th -30th 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 ReCONNET 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