



Rare Diseases Registries Workshop

Hospital Universitario La Paz, Madrid, Spain

21-22 March 2017



PROGRAMME

Day 1

12:30-13:30 **Welcome lunch**

13:30-13:40 **Welcome and introduction**

European Commission - Consumers, Health, Agriculture and Food Executive Agency; Spanish Ministry of Health, Social Services and Equality; Instituto de Salud Carlos III and H. La Paz-CIBERER

13:40-14:00 **Overview of EU Health Programme projects funded between 2008-2015 related to Rare Diseases registries**

Speaker

Hristina Mileva – European Commission - Consumers, Health, Agriculture and Food Executive Agency

Session 1: Towards an European platform on Rare Diseases Registration

Chair: Agnieszka Kinsner-Ovaskainen – DG Joint Research Centre, Directorate F-Health, Consumer and Reference Materials, Health in Society Unit (JRC F1), Italy

14:00-14:20 **European Platform on Rare Diseases Registration**

Speaker

Agnieszka Kinsner-Ovaskainen – DG Joint Research Centre

14:20-14:40 **EUROCAT - European Surveillance of Congenital Anomalies**

Speaker

*Amanda Julie Neville, University of Ferrara
President of EUROCAT Association and leader of EUROCAT Registry, Italy*

14:40-15:00 SCPE - Surveillance of Cerebral Palsy in Europe

Speaker

*Catherine Arnaud, Toulouse III University
Chair SCPE, France*

15:00-15:20 EUCERD Core Recommendations on RD patient registration and data collection

Speaker

*Victoria Hedley, Newcastle University
RD-ACTION Thematic Coordinator, UK*

15:20-16:00 Plenary Discussion: Interoperability of RD Registries

16:00-16:30 Coffee break

Session 2: Main results of the European Union's Health Programme – Rare Diseases Registries related projects

Chair: *Eva Bermejo, Institute of Rare Diseases Research (IIER) and Research Centre on Congenital Anomalies – Institute of Health Carlos III.*

16:30-16:50 EPIRARE - European Platform for Rare Disease Registries

Speaker

*Luciano Vittozzi, National Centre for Rare Diseases, National Institute for Health.
Coordinator of EPIRARE, Italy*

16:50-17:10 PARENT Joint Action - Cross Border Patient Registries iNiTiative

Speaker

*Marija Magajne, Nacionalni Inštitut za Javno Zdr
Coordinator of the PARENT JA, Slovenia*

17:10-17:30 RD-ACTION (Rare Diseases Joint Action)

Speaker

*Victoria Hedley, Newcastle University
RD-ACTION Thematic Coordinator, UK*

17:30-17:50 Sharing experience with the European Union's Research and Innovation Programme Projects – RD-CONNECT (An integrated platform connecting databases, registries, biobanks and clinical bioinformatics for RD research)

Speaker

*Elizabeth Wood, Newcastle University
Project manager of RD-Connect, UK*

17:50-18:30 Plenary Discussion: Best practices in the field of RD Registries

19: 30 Reception cocktail / dinner

Day 2

Session 3: Example of successful EU funded registries

Chair: *Susan Webb, Scientific Assistant Director of CIBERER and coordinator of ERCUSYN, Spain*

9:30-9:45 E-HOD - European registry and network for homocystinurias and methylation defects

Speaker

M^a Luz Couce Pico, Complejo Hospitalario Universitario de Santiago-CIBERER E-HOD, Spain

9:45-10:00 E-IMD - European registry and network for Intoxication type Metabolic Diseases

Speaker

Angels García-Cazorla, Hospital Sant Joan de Déu-CIBERER E-IMD, Spain

10:00-10:10 Q & A

Session 4: Challenges encountered by RD patient registries

Chair: *Pablo Lapunzina, Scientific Director of CIBERER, Spain*

10:10-10:30 Challenge 1: Integration and interoperability of EU and national RD registries

Speaker

Manuel Posada de la Paz, Instituto de Investigación en Enfermedades Raras – Instituto de Salud Carlos III Spanish Rare Diseases Registry Coordinator, Spain

10:30-10:50 Challenge 2: Public-Private Partnership for Rare Diseases Registries

Speaker

Vinciane Pirard. Co-chair of EFPIA-Europabio Rare Diseases and OMP Task Force and Sanofi Genzyme Head of Special Care EU Public Affairs, Belgium

10:50-11:20 Coffee break

11:20-11:40 Challenge 3: Registries and patient involvement: Patient-Powered Patient Registries

Speaker

Virginie Bros-Facer, Research Infrastructure Project Manager Eurordis, France

11:40-12:00 Challenge 4: Overview of Health Policies on Rare Diseases. State of play of the European Reference Networks

Speaker

Enrique Terol - DG-SANTE

12:00-12:45 Plenary Discussion: Current and future situation of patient registries

12:45-13:00 Closing remarks

Hristina Mileva – Chafea
Susan Webb - CIBERER

13:00 Lunch
