



"European Actions to improve the life of patients living with rare diseases"

Luxembourg, 25 – 26 October 2011

JEAN MONNET building,  
Rue Alcide de Gasperi, L-2920, Luxembourg  
Meeting room JMO M1

FINAL AGENDA

1<sup>st</sup> day - 25 October 2011 (Afternoon)

Co-chairs of session: D.SPANO, Principal Advisor, Directorate General for Health and Consumers, L.BRIOL, Director, Executive Agency for Health and Consumers (EAHC)

16:00

- **Welcome and introduction to the event**  
L.BRIOL, Director, Executive Agency for Health and Consumers (EAHC)  
D.SPANO, Principal Advisor, Directorate General for Health and Consumers
- **Welcome address: "European Actions to improve the life of patients living with rare diseases"**  
Directorate General for Health and Consumers, Director General, Ms. P. TESTORI-COGGI
- **Presentation of short feature film on RD patient portraits featuring**  
Mr. J.DALLI, Commissioner for Health and Consumers

16:30 – 17:20

**Testimonials by patients or family members on concrete impact of EU funding/ policies in everyday life**

- M. Y.LE CAM, France, father of a child living with Cystic Fibrosis, Chief Executive, Rare Disease Europe (EURORDIS)
- Ms A.DALY, Ireland, patient living with Retinitis Pigmentosa, member, Irish National Alliance
- Ms L.GREENE, UK, mother of a child with Cystinosis, member of the Committee for Orphan Medicinal Products (COMP) at the European Medicines Agency (EMA)
- Ms. K. PARKINSON, mother of two children with Alstrom syndrome, President Alstrom Syndrome UK Support group
- M. Y.LE CAM, Chief Executive, EURORDIS: stories of Ms T.SCHYNS-LIHARSKA and Ms. K.AIACH

17:20 – 17:50

- Panel discussion/ questions from the floor

17:50 - 18:30

Press point: time allocated to interviews by the invited journalists

18:30- 19:15

Apéritif dinatoire

- Welcome address by M. M.di BARTOLOMEO, Minister of Health, Luxembourg

20:30

Press dinner for journalists and speakers (NOVOTEL)

Cluster meeting of Rare Diseases projects:  
Building on the achievements of the Health programme 2007-2013

09:00 – 10:30

Session 1: Projects which are structuring European action in the area of Rare Diseases

Chair of session: S. SCHRECK, Directorate General for Health and Consumers, Unit C2, Health information and knowledge

Project presentations

- Ms. S.AYME, Development of the European portal of rare diseases and orphan drugs (ORPHANET Europe)
- M. F. HOUYEZ, European Organisation for Rare Diseases (EURORDIS)
- Ms. D.TARUSCIO, European Project for Rare Diseases National Plans Development (EUROPLAN)
- M. P.KANAVOS, Social economic burden and health related quality of life in patients with rare diseases in Europe ( BURQUOL-RD)
- Ms. W. HUGHES-WILSON, Consensus and Synergies for the EU Registration of Rare Disease Patients (EPIRARE)
- Ms E.GARNE, European Network for the Surveillance of Congenital Anomalies (EUROCAT)

Panel Discussion

10:30 – 11:00

Coffee break

11:00 – 12:15

Session 2: Pilot European Reference Networks on Rare Diseases

Chair of session: J. REMACLE, EAHC

Project coordinators present

- Mr. J.-C. DEYBACH, European Porphyria Network (EPNET )
- Ms S.PARKER, European Wilson's Disease Network: Improving information, knowledge and access to expertise and care (Euro-Wilson)
- Ms. K.GRAMSCH, Dissemination and Implementation of the Standards of Care for Duchenne Muscular Dystrophy in Europe (Care-NMD)
- Ms. T. SCHYNS-LIHARSKA, European Network of Expertise for Rare Pediatric Neurological Diseases (nEUROPED)
- Ms. H. TRANG, European network for central hypoventilation syndromes: Optimizing health care to patients, (EU-CHS)
- M. L. DUBERTRET, Together Against Genodermatoses - Improving Health Care and Social Support for Patients and Families with Severe Genodermatoses (TAG)
- Ms. K. PARKINSON, An EU rare diseases registry for Wolfram syndrome, Alstrom syndrome and Bardet Biedl syndrome (EURO-WABB).
- M. J.HAEBERLE, European registry and network for Intoxication type Metabolic Diseases, (E-IMD)
- M. M. CORRONS, European reference network of expert centres in rare anaemias (ENERCA)
- M.G.GATTA, Surveillance of rare cancers in Europe (RARECARE Net)

Lessons' learnt from pilot projects

- Ms. K. BUSHBY, Centre for Neuromuscular Disease, University of Newcastle, Coordinator EUCERD JA

Panel Discussion

12:15-12:30

Closing remarks - J. REMACLE, EAHC

12:30 – 13:30 Press point: time allocated to interviews by the invited journalists