E-Rare SYMPOSIUM

30 October 2014

Casa dell'Aviatore
Viale dell'Università, 20
Rome, Italy

Fostering collaboration in rare diseases research

09:30 - 09:45 Welcome by Representatives of the Italian Government and Istituto Superiore di Sanità

Session I: Fostering transnational collaboration for rare diseases research funding

Chairs: Ralph Schuster, DLR, Germany and Domenica Taruscio, ISS, Italy

09:45 - 10:00 Presentation of E-Rare, Daria Julkowska, E-Rare coordinator, INSERM, France

10:00 - 10:20 Rare diseases research funding - cross-sectional analysis, Domenica Taruscio, ISS, Italy

10:20 - 10:40 EC funding for collaborative projects, Irene Norstedt, DG Research & Innovation, European Commission

10:40 - 11:00 IRDiRC: global efforts for 200 new therapies and diagnosis for most rare diseases by 2020, Paul Lasko, Chair of IRDiRC, CIHR, Canada

11:00 - 11:20 COFFEE BREAK

Session II: Success stories

Chairs: Milan Macek, Charles University, Prague, Czech Republic and Filippo Belardelii, ISS, Italy

11:20 - 11:40 Assessment of E-Rare funded projects, Anabela Isidro, FCT, Portugal

11:40 - 12:00 PODONET, "Consortium for Clinical, Genetic and Experimental Research into Hereditary Diseases of the Podocyte", Franz Schaefer, Heidelberg University Hospital, Heidelberg, Germany

12:00 - 12:20 CRANIRARE, "An integrated clinical and scientific approach for craniofacial malformations", Bernd Wollnik, University of Cologne, Cologne, Germany

12:20- -12:40 MTM-PATHIES, "MTM1 and MTMR2 myotubularins: biochemical activity and the regulation of membrane trafficking in health and disease", Alessandra Bolino, Fondazione Centro San Raffaele del Monte Tabor, Milan, Italy

12:40 - 14:00 LUNCH

14:00 - 14:20 CURE-FXS, "Targeting Rho-signalling, a new therapeutic avenue in fragile X syndrome", Mara Dierssen, Center for Genomic Regulation Genes and Disease Program, Barcelona, Spain

14:20 - 14:40 TRANSPOSMART: "An innovating platform using transposon and S/MAR for von Willebrand disease gene therapy", Daniel Scherman, CNRS, Paris, France

14:40 – 15:20 Panel discussion: framework requirements for successful research collaboration

Session III: Partners for collaboration

Chairs: Daria Julkowska, INSERM, France and Hugh Dawkins, Office of Population Health Genomics, East Perth, Australia

15:20 – 15:40 Innorare: towards the formation of a virtual institute of translational research in rare diseases, Victoria Hedley, University of Newcastle, UK

15:40 – 16:00 European Medicines Initiative, Segundo Mariz, EMA, London, UK

16:20 – 16:40 COFFEE BREAK

16:40 – 17:00 Innovative Medicines Initative, Carlo Incerti, Head of Global Medical Affairs, Genzyme, Modena, Italy

17:00 - 17:20 Involvement of patients' organizations in research funding, Yann Le Cam, EURORIDIS, France

17:20 – 17:40 RE(ACT) Community, Olivier Menzel, Blackswan Foundation, Lausanne, Switzerland

17:40 – 18:00 E-Rare-3 outlook, Daria Julkowska, E-Rare coordinator, INSERM, France

18:00 – 18:30 Conclusions