

# ICORD 2014 Conference Program

**\* = Joint sessions**

## Day 1 (Tuesday, October 7, 2014)

9:00-11:00

**Session I: Opening and Plenary Lectures. Policies: The main steps for recognizing the societal value of diagnosis, prevention and treatment of rare diseases**

Room: Schouwburg

09:00-09:30 **Opening**

Room: Schouwburg

Moderators: Sonja van Weely (ZonMw, The Netherlands) and Remco de Vruh (TI Pharma, The Netherlands)

- *ICORD's capabilities.* Domenica Taruscio (ICORD Past-President, Italy)
- *ICORD's immediate challenges.* John Forman (ICORD President-Elect, New Zealand),
- *ICORD - a platform for bridging and cooperation.* Virginia Llera (ICORD-President, Argentina)

09:30-11:00 **Plenary Lectures**

Room: Schouwburg

Moderators: Sonja van Weely (ZonMw, The Netherlands) and Remco de Vruh (TI Pharma, The Netherlands)

- 09:35-09:55 *Health policy for rare diseases in The Netherlands.* Fred Krapels (Dutch Ministry of Health, The Netherlands)
- 09:55-10:15 *Major EU policies on rare diseases from patients' perspective.* Yann Le Cam (EURORDIS, France)
- 10:15-10:35 *The current state of rare diseases in Mexico.* Mario Garza Alanis (Federal Commission for Protection against Health Risks, COFEPRIS, Mexico)
- 10:35-10:55 *The challenging process from a global vision to a global social value impact.* Virginia Llera (ICORD-President, Argentina)
- 10:55-11:00 *Q&A*

11:00-11:30 *Coffee break* (Mozart Foyer)

11:30-13:00

**(\* Session II: Key-note plenary lectures**

Room: Schouwburg

Moderators: Jorg Janssen (TI Pharma, The Netherlands) and Jan-Inge Henter (Karolinska University Hospital and the Karolinska Institute, Sweden)

- 11:30-11:35 *Introduction to TI PHARMA Lecture.* Jorg Janssen
- 11:35-12:15 *TI PHARMA Lecture: Addressing the needs of the neglected: control of schistosomiasis in young African children and infants.* Russell Stothard (Liverpool School of Tropical Medicine, United Kingdom)
- 12:15-12:20 *Introduction to ICORD.* Jan-Inge Henter (ICORD Founder, Sweden)
- 12:20-13:00 *ICORD Lecture: Mitochondrial drug development: from bench to bedside* (OP-01). Jan Smeitink (Radboud University Medical Centre, The Netherlands)

13:00-14:00 *Lunch break with Exhibition and Posters* (Mozart Foyer)

14:00-15:30

**(\* Session III: Ways forward: Evaluation, development and societal impact of orphan drugs. Joint session with ZonMw**

Room: Schouwburg

Moderators: Stephen Groft (NIH, USA) and Hans Büller (ErasmusMC, The Netherlands)

- 14:05-14:25 *Evaluation of Orphan Drugs: Ways forward* (OP-09). Carla Hollak (Academic Medical Center, The Netherlands)
- 14:25-14:45 *Novel developments in HTA methodology (Health Technology Assessment)* (OP-10). Leona Hakkaart (Institute of Health Policy and Management, The Netherlands)
- 14:45-15:05 *The politics of rare diseases and orphan drugs* (OP-13). Rob Hagendijk (University of Amsterdam, The Netherlands)
- 15:05-15:25 *Novel business models for Orphan Drug development* (OP-08). Hans Büller
- 15:25-15:30 *Discussion*. Stephen Groft and Hans Büller

15:30-16:00 *Coffee break* (Mozart Foyer)

16:00-17:30

**Session IV: Poster session (Authors present by the posters)**

Room: Mozart Foyer

17.30-18.45

**Session V: Special Lecture; International collaboration**

Room: Kernhem Rooms

Moderator: Emilio Roldán (SLADIMER, Argentina)

- 17:35-17:55 *The Biopontis Alliance for Rare Diseases (BARD) - a new collaborative model for bridging from discovery to cures candidates* (OP-02). Erik Tambuyzer (BARD, Belgium)
- 17:55-18:15 *International school of public health for rare diseases* (OP-03). Domenica Taruscio (Istituto Superiore di Sanità, Italy)
- 18:15-18:35 *New strategic direction at GSK*. Martin Andrews (GSK, United Kingdom)

(\*) 18:45-19.30

**Walking dinner with FIGON-DMD**

Room: Mozart Foyer

19:30-21:30

**General Assembly (for ICORD members)**

Room: Kernhem Rooms

Coordinator: Manuel Posada (ICORD Secretary, Spain)

## Day 2 (Wednesday, October 8, 2014)

09:00-10:00

**Time for micro-meetings and interactions**

Poster Area (Mozart Foyer)/ Kernhem Rooms

10.00-10.30 *Coffee Break*

10:30-12:00

**Session VI: Primary prevention of neural tube defects. Making a common disease rare**

Room: Kernhem Rooms

Moderator: John Forman (New Zealand Organisation for Rare Disorders, New Zealand)

- 10:30-10:45 *Congenital Anomalies as Preventable Rare Diseases* (PPi-01). Amanda Julie Neville (Universitaria di Ferrara, Italy)
- 10:45-11:05 *Folate and prevention of neural tube defects: Tracking red blood cell concentrations will help guide policy decisions about fortification* (OP-06). Derrick Bennett (University of Oxford, United Kingdom)
- 11:05-11:25 *Could folic acid supplementation bridge the gap of the results of fortification? State of the art in Europe and future perspectives* (OP-07). Stefania Ruggeri (Agricultural Research Council, Italy)
- 11:25-11:45 *Patient and family perspectives* (OP-12). Lieven Bauwens (International Federation for Spina Bifida and Hydrocephalus, Belgium)
- 11:45-11:55 *Public health decision making frameworks* (OP-05). John Forman (New Zealand Organisation for Rare Disorders, New Zealand)
- 11:55-12:00 *Q&A*

12:00-13:00 Lunch break with Exhibition and Posters (Mozart Foyer)

13:00-16:00

**(\*) Session VII: Orphan drugs and personalized medicine (part II). Joint session with Dutch Clinical Trial Foundation (DCTF)**

Moderators: Manuel Posada (Instituto de Salud Carlos III, Spain) and Izaak den Daas (QPS Netherlands BV, The Netherlands)

Presentations, 3x45 min (35 + 10 min discussion)

- Marjon Pasmooij (Medicines Evaluation Board, The Netherlands): regulatory perspective
- Angela Brand (Maastricht University, The Netherlands): academic perspective
- Barbara Wuebbles (Audentes Therapeutics, USA): patient perspective

16:00-16:30 Coffee break (Mozart Foyer)

16:30-18:30

**Session VIII: ICORD Working Groups**

Room: Kernhem Rooms

General Coordinator: Désirée Gavhed (Karolinska Institute, Sweden)

16:30-16:45 *Introduction to the working groups and the ICORD workshops (ACCORDs).*

*-Research Working Group: Research Collaborations in Rare Diseases*

Coordinator: Emilio Roldán (SLADIMER, Argentina)

Presentation: *An integrative model of rare diseases management in a tertiary hospital: from basic research to clinical practice and beyond (OP-04).* Joan Comella (Vall d'Hebron University Hospital, Spain)

*-Working Group of Policies and citizen perspectives around the world: working together for ICORD 2015*

Coordinators: Virginia Llera (GEISER, Argentina) and Sharon Terry (Genetic Alliance, USA)

Presentation: *Beyond Orphan Drugs: Cooperation of a Biopharmaceutical Company with other Rare Diseases' Stakeholders (OP-11).* Fernando Royo (Fundación Genzyme, Spain)

*-Working Group on International School Public Health on Rare Diseases*

Coordinator: Domenica Taruscio (Istituto Superiore di Sanità, Italy)

*-Working Groups for Practitioners: Guidelines for medical practice on Rare Diseases. International initiatives*

Coordinator: Rumen Stefanov (Medical University of Plovdiv, Bulgaria)

20:00

**ICORD Dinner** (ticket required)

Kasteel Doorwerth

*Acknowledging values in rare diseases*

Buses leave the conference venue 19:30 and are expected to return to hotels around 22:30-23:00

## Day 3 (Thursday, October 9, 2014)

09:00-10:20

**Session IX. Registries and Biobanks**

Room: Theatre Azure

Moderators: Domenica Taruscio (Istituto Superiore di Sanità, Italy) and Stephen Groft (NIH, USA)

09:05-09:25 *Registries for rare diseases: the experience of a regional network (PPa-21).* Arrigo Schieppati (Rare Disease Unit Bergamo, Italy)

09:25-09:45 *National Registries of rare diseases in Europe: an overview by the EPIRARE project (PPi-07).* Domenica Taruscio (Istituto Superiore di Sanità, Italy)

09:45-10:05 *Spanish national rare disease biobank (PPa-18).* Veronica Alonso (Instituto de Salud Carlos III, Spain)

10:05-10:20 Q&A

10:20-10:50 *Coffee break* (Salon Claire)

10:50-12:20

**Session X: The International Rare Diseases Research Consortium (IRDiRC)**

Room: Theatre Azure

Moderators: Ségolène Aymé (IRDiRC Scientific Secretariat, France) and Sonja van Weely (ZonMw, The Netherlands)

10:50-11:20 *Recommendations to speed up the R&D process in the field of orphan drugs.* Gert-Jan van Ommen (Leiden University, The Netherlands)

11:20-11:50 *Initiatives to speed up data mining in the field of rare diseases.* Ségolène Aymé (Orphanet, INSERM, France)

11:50-12:20 *Initiatives to speed up data sharing.* Anthony J Brookes (University of Leicester, United Kingdom)

12:20-13:20 *Lunch break with Exhibition and Posters* (Salon Claire)

13:20-15:20

**Session XI: Patient perspectives on the societal value of rare diseases and orphan drugs**

Room: Theatre Azure

Moderators: Virginia Llera (GEISER, Argentina) and Sharon Terry (Genetic Alliance, USA)

13:20-13:25 *Introduction*

13:25-13:45 *Perspectives from the USA.* Sharon Terry (Genetic Alliance, USA)

13:45-14:05 *Perspectives from The Netherlands: International lessons from a Dutch reimbursement procedure on Pompe and Fabry disease (OP-14).* Cees Smit (Dutch Genetic Alliance, European Genetic Alliances Network, The Netherlands)

14:05-14:25 *Perspectives from Mexico.* Carlos David Peña (FEMEXER, México)

14:25-14:45 *Perspectives from Japan: The activity of Werner syndrome patient/family group in Japan (PPI-12).* Satoshi Tanaka, Werner syndrome patient/family group in Japan (Japan)

15:00-15:20 Q&A

**15:20-15:45 Working group conclusions**

Room: Theatre Azure

Moderator: Désirée Gavhed

**15:45-16:00 Remarks and farewell**

Room: Theatre Azure

Moderator: Manuel Posada

Virginia Llera

John Forman