



**IXth ICORD 2014, 7-9 October**  
**ReeHorst Hotel, Ede, Netherlands**  
**Preliminary Conference Program**  
(Aug, 29)

\* *Joint sessions*

\*\* *On-line transmission to pre-registered attendees*

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

(\*\*) 9:00-11:00

**Session I: Opening and Plenary Lectures: Policies: The main step for Recognizing the Societal Value of Diagnosis, Prevention and Treatment for the Rare Diseases**

09:00-09:30 **Welcome addresses**

Coordinator: Remco de Vruh (NL)

Sonja van Weely (ZonMw)

Domenica Taruscio (ICORD Past-President), ICORD's capabilities

John Forman (ICORD President-Elect), ICORD's immediate challenges

Virginia A. Llera (ICORD-President), ICORD a platform for bridging and cooperation

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

Coordinator: Sonja van Weely (NL)

09:35-09:55 Nicola Magrini, WHO (TBC)

09:55-10:15 EURORDIS (TBC); European level

10:15-10:35 Virginia A. Llera, ICORD; The challenging process from a global vision to a global social value impact

10:40-11:00 Veronique Esman-Peeters (TBC), Dutch Ministry of Health, An overview of the Dutch National Plan on Rare Diseases

*11:00-11:30 Coffee break*

11:30-13:00

**(\*) Session II: Key-notes lectures**

Moderator: Jan-Inge Henter (Sweden)

11.30-11.35 Introduction to ICORD, Jan-Inge Henter (SE)

11:35-12:15 ICORD Lecture: Jan Smeitink; Mitochondrial Drug Development: from bench to bedside

12:15-12:20 Introduction to TI PHARMA speaker

12:20-13:00 TI PHARMA Plenary lecture: Russell Stothard (NL)

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

14:00-15:30

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

Moderators: Stephen Groft (USA) and Hans Büller (NL)

14:05-14:25 Evaluation of Orphan Drugs: Ways forward, Carla Hollak (AMC, NL)

14:25-14:45 Novel developments in HTA methodology (Health Technology Assessment),  
Leona Hakkaart (iBMG, NL)  
14:45-15:05 The politics of rare diseases and orphan drugs, Rob Hagendijk (UvA, NL)  
15:05-15:25 Novel business models for Orphan Drug development, Hans Büller  
(ErasmusMC, NL)  
15:25-15:30 Discussion, Stephen Groft and Hans Büller

*15:30-16:00 Coffee break*

16:00-17:30

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

17.30-18.45

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

Moderator: Emilio Roldan

17:35-17:55 The Biopontis Alliance for Rare Diseases (BARDF) - a new collaborative  
model for bridging from discovery to partner to cures candidates, Erik Tambuyzer  
(Belgium)

17:55-18:15 International school of public health for rare diseases, Domenica Taruscio  
(Italy)

18:15-18:35 New strategic direction at GSK, Martin Andrews (UK)

(\*) **18:45-19.30 Walking dinner** with FIGON-DMD

19:30-21:30

**General Assembly (for ICORD members)**

Coordinator: Manuel Posada (Carlos III, Spain)

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

09:00-09:45

(\*) **Session VI: Orphan drugs and personalized medicine (part I). Joint session with  
Clinical trial Foundation (DCTF) / ICORD**

Moderators: Marlene Haffner (USA) and Izaak den Daas (NL)

ICORD-DCTF Keynote Lecture

*09.45-10.30 Coffee Break*

10:30-12:00

**Session VII: Primary prevention of neural tube defects; Making a common disease rare**

Moderator: John Forman

10:30-10:50 Folate and prevention of neural tube defects: Tracking red blood cell  
concentrations will help guide policy decisions about fortification, Dr Robert Clarke  
(University of Oxford, UK)

10:50-11:10 Could folic acid supplementation bridge the gap of the results of fortification?  
State of the art in Europe and future perspectives, Prof Stefania Ruggeri (Agricultural  
Research Council (CRA), Italy)

11:10-11:30 Patient and family perspectives, Lieven Bauwens (International Federation for  
Spina Bifida and Hydrocephalus, Belgium)

11:30-11:45 Public health decision making frameworks, John Forman (New Zealand  
Organisation for Rare Disorders, NZ)

11:45-12:00 Q&A

12:00-13:00 Lunch break (*Exhibition and Posters*)

13:00-16:00

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

Moderators: Manuel Posada (Carlos III, Spain) and Izaak den Daas (NL)

Presentations (TBD)

Round table discussion- patient, academic, clinical trial and regulatory perspectives

16:00-16:30 *Coffee break*

16:30-18:30

**Session IX: ICORD Working Groups**

General Coordinator: Désirée Gavhed (Karolinska Institute, Sweden)

-Research Working Group: Research Collaborations in Rare Diseases  
Coordinator: Emilio Roldan (Argentina)

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

Coordinators: Virginia Llera (Argentina) and Sharon Terry (USA)

-Working Group on International School Public Health on Rare Diseases  
Coordinator: Domenica Taruscio (Italy)

- Working Groups for Practitioners: Guidelines for medical practice on Rare Diseases: international initiatives

Coordinator: Rumen Stefanov (Bulgaria)

20:00 **ICORD Dinner** (ticket)

- Acknowledging values in rare diseases

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

09:00-10:20

**Session X. Registries and Biobanks**

Moderators: Domenica Taruscio (Italy) and Stephen Groft (USA)

09:05-09:25 PPa-21. Registries for rare diseases: the experience of a regional network.  
Arrigo Schieppati (Italy)

09:25-09:45 PPi-07. National Registries of rare diseases in Europe: an overview by the EPIRARE project. Domenica Taruscio and Giorgio Vitozzi (Italy)

09:45-10:05 PPa-18 Spanish national rare disease biobank. Veronica Alonso (Spain)

10:05-10:20 Q&A

10:20-10:50 *Coffee break*

10:50-12:20

**Session XI: IRDIRC**

Moderators: Ségolène Aymé and Sonja van Weely

10:50-11:20 Recommendations to speed up the R&D process in the field of orphan drugs.

Gert-Jan van Ommen (NL)

11:20-11:50 Initiatives to speed up data mining in the field of rare diseases. (TBC)

11:50-12:20 Initiatives to speed up data sharing. *Antony Brookes (UK)*

12:20-13:20 *Lunch (Exhibition and Posters)*

13:20-15:20

**Session XII: Patient views about the societal value of rare diseases and orphan drugs**

Moderators: Virginia Llera and Sharon Terry

13:00-13:05 Introduction

13:25-13:45 Societal Value of Rare Diseases and Orphan Drugs, the perspective from USA to an international scenario; Sharon Terry, President and CEO Genetic Alliance (USA)

13:45-14:05 Societal Value of Rare Diseases and Orphan Drugs, the perspective from Japan: The activity of Werner syndrome patient/family group in Japan. Satoshi Tanaka, Chief secretariat of Werner syndrome patient/family group in Japan (Japan)

14:05-14:25 Societal Value of Rare Diseases and Orphan Drugs, the perspective from Mexico , David Pena, México, FEMEXER president, Pide un Deseo , President , GEISER Delegate.

14:25-14:45 Societal Value of Rare Diseases and Orphan Drugs, the perspective from Iran, Prevention, diagnosis and treatment of rare disease, Maryam Banikazemi, New York Medical College (USA)

14:45-15:05 Societal Value of Rare Diseases and Orphan Drugs, the perspective from Netherlands.

15:05-15:15 Societal Value of Rare Diseases and Orphan Drugs, the perspective from Russia, Albina Makaeva (Russia), Vice-president of National Association of organizations of patients with rare diseases "Genetics" Russia (Russia)

15:15-15:20 Conclusions

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

Moderator: Désirée Gavhed

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

Moderator: Manuel Posada

Virginia Llera

John Forman

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