



3rd International Conference on
Rare Diseases and Orphan Drugs

Brussels, September 14-15, 2007

PROGRAMME

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ICORD 2007: Aims, Mission and Vision

The overall aim of ICORD 2007 is to develop constructive international collaborations that will result in true advantages for patients with rare diseases. This will be the 3rd Annual ICORD-conference aiming at facilitating international cooperation in the field of rare diseases and orphan drugs. Please note that the venue has limited number of seats, if many register the principle is first come-first served.

The 1st International Conference on Rare Diseases and Orphan Drugs was held in Stockholm in 2005 (ICORD 2005). It was co-organized by the European Commission, NIH and Karolinska Institutet, and at the conclusion of the conference it was decided to plan for future ICORD meetings.

ICORD 2006, held in Madrid, had the major aim to stimulate constructive international collaborations. The meeting was built on a number of sessions on various topics and the idea was to catalyze the formation of Working Groups (WG) on these topics, with the mission to take steps during the subsequent year in the direction of improved international collaboration.

The European Commission (DG Research) organizes a visibility conference Sept 13, 2007 in Brussels on rare disease research. The ICORD 2007 Annual Meeting will be held in back-to-back with this conference, on Sept 14-15, 2007. ICORD 2007 is an open 2-Day-Meeting. Day 1 (Sept 14) will host plenary presentations and discussions on various advances made in the field of international collaboration on Rare Diseases, in separate areas including Gaining Regulatory Approval, Linking the Academic Research Community to Industry, Rare Diseases Research Tools (with focus on biobanks), Genetic Testing for Rare Diseases, and Patient and Family Needs Across the Lifespan.

On Day 2 (Sept 15), there will be additional plenary presentations and discussions, now with focus on Recruiting Patients for Clinical Research Studies and on Expanding the Borders geographically. In the afternoon, the Working Groups are provided time to discuss the steps taken during the past year, minor or major advantages or improvements, and plan their aims and activities for the upcoming year. There will first be parallel break-out sessions, followed by brief presentations by each Working Group.

There will be an opportunity for anyone interested to volunteer for Working Group participation and if you want to volunteer for this work, tick one Working Group on the Registration Form. The basic idea is that if all Working Groups develop minor or major accomplishments in international collaboration in their respective field, this would in total be a great step ahead.

Each Working Group will structure their sessions. There are no predefined duties to accomplish; on the contrary the groups themselves are to define what would be the best to accomplish for the near and more remote future. The important thing is moving ahead together, also internationally / globally. In our vision this movement may grow slowly but steadily so that it in a few years really will facilitate improved treatments on rare diseases world-wide and better care for affected patients.

For 2008, ICORD will be held at NIH, as a 25th Anniversary of the Orphan-Drug-Act, with the preliminary dates May (19)-20-21, 2008.

Warmly welcome to ICORD 2007!

Jan-Inge Henter,
MD, Ph.D.

Stephen Groft,
Pharm.D.

Maria Wästfelt,
Ph.D.

Programme

Friday September 14

- 8:30 Introductions and Welcome**
Jan-Inge Henter, Karolinska Institutet, Sweden
Stephen Groft, ORD, NIH, USA
- 8:45 WG I Gaining Regulatory Approval: Establishing and Meeting Regulatory Requirements**
Progress Report: Developing Data Elements for the Orphan Product Designation
Marlene Haffner, Amgen, USA
Stephen Groft, NIH, USA
Yann Le Cam, Eurordis, France
Barbara Wuebbels, BioMarin, USA
- 09:45 WG II Product Discovery and Development: Linking the Academic Research Community to the Pharmaceutical and Biotechnology Industries**
Licensing of Discoveries and Inventions from National Governments, Academia, and Not-for-Profit Institutions to Industry
Maria Wästfelt, Karolinska Institutet, Sweden
Bonny Harbinger, NIH, USA
Torbjörn Ingemansson, DG Research, EC
James Cloyd, University of Minnesota, USA
Erik Tambuyzer, Genzyme, Belgium
Barbara Wuebbels, BioMarin, USA
- 11:00 Coffee**
- 11:30 WG III Development of Rare Diseases Research and Orphan Products**
Development Assessment Tools: Possibilities, Restrictions, and Solutions
Biobanks and Human Bio-Specimen Repositories
Elisa Eiseman, RAND Corp, USA
Yann Le Cam, Eurordis, France
Stephen Groft, NIH, USA
- 13:00 Lunch**
- 14:15 Establishing a Forum for Global Collaborations**
Jan-Inge Henter
Stephen Groft
- 14:30 WG V Genetic Testing for Rare Diseases in International Settings**
A. Linking the Databases of Gene Tests and EuroGentest
Ségolène Aymé, INSERM and Orphanet, France
Roberta Pagon, University of Washington, USA
B. Developing Standards for Genetic Testing Procedures and Materials
Lisa Kalman, CDC, USA
C. Developing Genetic Tests from the Research Laboratory to the Clinic – A Model for Consideration: The CETT Program
Giovanna Spinella, NIH, USA
- 15:45 Report from the ICD-11 Rare Diseases Classification Project**
Ségolène Aymé, INSERM and Orphanet, France
- 16:00 Coffee**
- 16:30 WG VI Identifying and Meeting Patient and Family Needs Across the Lifespan**
Anders Olauson, Ågrenska Academy, Sweden
Diane Dorman NORD, USA
Emilio Roldán, Gador S.A., Argentina
Victoria Llera, Geiser Foundation, Argentina
John Forman, NZORD, New Zealand
- 17:30 Adjourn for the day**

Programme

Saturday September 15

8:00 Introductions and Welcome

Jan-Inge Henter
Stephen Groft

08:15 WG IV Recruiting Patients for Clinical Research Studies and the Value of International Collaboration

Current Procedures and Clinical Trial and Statistical Considerations

Domenica Taruscio, ISS, Italy
Annalisa Trama, ISS, Italy
Wolfgang Köpcke, University of Münster, Germany
Joachim Gerss, University of Münster, Germany
Lawrence Friedman, NIH, USA
Simon Day, Roche, UK
Rumen Stefanov, ICRDOD, Bulgaria
Arrigo Schieppati,, Mario Negri Institute, Italy

10:00 *Coffee*

10:30 WG VII Rare Diseases Research and Orphan Products: Expanding the Informational and Geographical Borders

Stephen Groft, NIH, USA
John Forman, NZORD, New Zealand
Virginia Llera, Geiser Foundation, Argentina
Rumen Stefanov, ICRDOD, Bulgaria

12:15 *Lunch*

13:15 Parallel Workshops on Planning Future Activities

Work-Group (WG) I: Gaining Regulatory Approval: Establishing and Meeting Regulatory Requirements

WG II: Product Discovery and Development: Linking the Academic Research Community to the Pharmaceutical and Biotechnology Industries
Chair: Maria Wästfelt, Karolinska Institutet, Sweden

WG III: Development of Rare Diseases Research and Orphan Products Development Assessment Tools: Possibilities Restrictions, and Solutions

WG IV: Recruiting Patients for Clinical Research Studies and the Value of International Collaboration
Chair: Lawrence Friedman, NIH, USA

WG V: Genetic Testing for Rare Diseases in International Settings
Chair: Roberta Pagon, University of Washington, USA

WG VI: Identifying and Meeting Patient and Family Needs Across the Lifespan

WG VII: Rare Diseases Research and Orphan Products Development Activities: Expanding the informational and Geographical Borders
Chairs: Anders Olauson, Ågrenska Academy, Sweden; Stephen Groft, NIH USA

14:15 *Coffee*

14:30 Report and Discussion Work-Group I – Work-Group VII

15:30 Summary

Jan-Inge Henter
Stephen Groft

Future Meeting: 2008: Washington, DC
(May 20-22 Approximate Dates)

16:00 *Adjourn*

List of participants

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Notes

