



## **RARE-Bestpractices Conference**

**24 November 2016**

Istituto Superiore di Sanità  
Aula Pocchiari  
viale Regina Elena 299, Rome, Italy

organised by  
Istituto Superiore di Sanità

with the collaboration of EURORDIS



The RARE-Bestpractices project is funded by the European Union Seventh Framework Programme (FP7/2007-2013) under Grant Agreement n° 305690



The aim of the conference is to disseminate the findings from the RARE-Bestpractices project ([www.rarebestpractices.eu](http://www.rarebestpractices.eu)) and offer a forum for discussing with relevant stakeholders how this work could be taken into account in delivering better health decision making and health policies for rare diseases.

The event will bring together leading experts in the area of evidence synthesis and guideline development to discuss the methodological advancements and knowledge resources developed by the RARE-Bestpractices consortium and focus on mechanisms for ensuring the production of reliable, relevant, usable evidence in a bid to increase the value of research on rare disease.

The RARE-Bestpractices consortium has created a platform not organized around a specific disease area but rather with the flexibility to collaborate with other existing initiatives devoted to ensure the effective development and use of knowledge on rare diseases. The conference will be also dedicated to discussing ways to strengthen the partnerships that RARE-Bestpractices has already realized and the options for fostering further international collaborations to combine effort in sharing resources and making an efficient use of the knowledge available.

**Working method:** presentations of project results, lectures, Q&A moderated by chair.

## **24 November 2016 - Programme**

08.30-09.15 **Arrival and registration**

09.15-09.30 Welcome address

*Gualtiero Ricciardi (President, Istituto Superiore di Sanità)*

09.30-09.40 Opening keynote

*Jaroslav Waligora*

9.40-09.50 The Rare-Bestpractices project

*Domenica Taruscio*

9.50-10.00 Introduction to the conference

*Cristina Morciano*

10.00-11.15 **Session 1**

**HTA, clinical guidelines and orphan drugs**

Chair: *Panos Kanavos*

10.00-10.15 Valuing patients and public preferences

*Francis Arickx*

10.15-10.35 Value assessment of orphan drugs: methodological framework and empirical evidence from 8 EU Member States

*Victoria Tzouma*

10.35-10.50 Stakeholder Perspectives:

*Guillaume Dedet*

*Elena Nicod*

10.50-11.15 Q&A session moderated by chair



11.15 -11.30 **Coffee Break**

11.30-12.45 **Session 2**

**Improving trustworthiness of health care guideline development on rare diseases**

Chairs: *Joerg Meerpohl, Karen Ritchie*

11.30-11.45 Challenge in developing highly credible guidelines for rare diseases

*Joerg Meerpohl*

11.45-12.15 Lessons learnt by applying GRADE to guideline processes in rare diseases:

11.45-12.00 Sickle Cell Disease

*Andrea Darzi*

12.00 -12.15 Catastrophic Antiphospholipid Syndrome (CAPS) and models of care for Hemophilia Clinical Practice Guidelines: lessons learnt

*Alfonso Iorio*

12:15-12:30 Stakeholder Perspectives:

*Lisa Thom*

12:30-12:45 Q&A session moderated by chair

12.45 -13.30 **Lunch**

13.30-14.45 **Session 3**

**Knowledge resources for health care guideline developers and users**

Chairs: *Michele Hilton Boon, Cristina Morciano*

13.30 -13.45 Stronger together - guidelines on rare diseases as a basis for a joint European quality initiative

*Ina Kopp*

13.45 -14.00 Guidelines for rare diseases – where to find them and how to assess their quality

*Karen Ritchie*

14.00 -14.15 Resources for developers and users of health care guidelines on rare diseases: the RARE-Bestpractices courses and training tools

*Graziella Filippini*

14.15-14.30 Stakeholder Perspectives:

*Avril Kennan*

*Simone Baldovino*

14.30 -14.45 Q&A session moderated by chair



- 14.45-16.00 **Session 4**  
**Shaping the future research agenda on rare diseases: ensuring the production of reliable, relevant, usable evidence**  
Chair: *Roberto D'Amico*
- 14.45 -15.00 Avoiding waste in production, design and conduct of studies and reporting evidence on rare diseases  
*Philippe Ravaud*
- 15.00 -15.15 Identifying patients' and clinicians' priorities for rare disease research  
*Katherine Cowan*
- 15.15 -15.30 Funding relevant rare disease research: experience from E-Rare  
*Sonja van Weely*
- 15.30 -15.45 Better patient health outcomes require the best of research and healthcare: a need for a common infrastructure  
*Mathieu Boudes*
- 15.45 -16.00 Q&A session moderated by chair
- 16.00-16.50 **Session 5**  
**Foster international collaborations**  
Chair: *Domenica Taruscio*
- 16.00 -16.10 RARE-Bestpractices and Orphanet collaboration  
*Ana Rath*
- 16.10 -16.20 RARE-Bestpractices and E-Rare collaboration  
*Sonja van Weely*
- 16.20 -16.40 RARE-Bestpractices and European Reference Networks  
*Marta Mosca, Rosaria Talarico*
- 16.40 -16.50 Q&A session moderated by chair
- 16.50-17.00 **Closing remarks**  
*Egle Simelyte*, European Commission project officer  
*Domenica Taruscio* project leader
- 17.00 End of Conference



## Speakers and Chairs:

**Francis Arickx**, National Institute for Health and Disability Insurance (BE)  
**Simone Baldovino**, Università di Torino (IT)  
**Mathieu Boudes**, EURORDIS (FR)  
**Roberto D'Amico**, Cochrane Italy - University of Modena and Reggio Emilia, (IT)  
**Katherine Cowan**, James Lind Alliance (UK)  
**Andrea Darzi**, American University of Beirut (LB)  
**Guillaume Dedet**, World Health Organisation  
**Graziella Filippini**, Associazione per la Ricerca sull'Efficacia dell'Assistenza Sanitaria Centro Cochrane Italiano (IT)  
**Michele Hilton Boon**, University of Glasgow (UK)  
**Alfonso Iorio**, McMaster University (CA)  
**Panos Kanavos**, London School of economics and Political Science (UK)  
**Avril Kennan**, DEBRA International (AT)  
**Ina Kopp**, German Association of the Scientific Medical Societies (DE)  
**Joerg Meerpohl**, University of Freiburg (DE)  
**Cristina Morciano**, Istituto Superiore di Sanità (IT)  
**Marta Mosca**, Università di Pisa (IT)  
**Elena Nicod**, Bocconi University (IT)  
**Ana Rath**, Institut National de la Santé et de la Recherche Médicale (FR)  
**Philippe Ravaud**, Centre de Recherche Épidémiologie et Statistique Sorbonne Paris Cité (FR)  
**Karen Ritchie**, Healthcare Improvement Scotland (UK)  
**Egle Simelyte**, European Commission  
**Rosaria Talarico**, Università di Pisa (IT)  
**Domenica Taruscio**, Istituto Superiore di Sanità (IT)  
**Lisa Thom**, Catastrophic Antiphospholipid Syndrome patient representative  
**Victoria Tzouma**, London School of Economics and Political Science (UK)  
**Sonja van Weely**, The Netherlands Organisation for Health Research and Development (NL)  
**Jaroslav Waligora**, Policy officer for rare diseases

## Conference Chairs:

**Domenica Taruscio, Cristina Morciano** (06 49904422; cristina.morciano@iss.it)  
National Centre for Rare Diseases, Istituto Superiore di Sanità

## Scientific Committee:

**Mathieu Boudes** – EURORDIS, France  
**Roberto D'Amico** – Cochrane Italy - University of Modena and Reggio Emilia, Italy  
**Graziella Filippini** – Associazione per la Ricerca sull'Efficacia dell'Assistenza Sanitaria Centro Cochrane Italiano, Italy  
**Panos Kanavos** – London School of Economics and Political Science, United Kingdom  
**Cristina Morciano** – Istituto Superiore di Sanità, Italy  
**Karen Ritchie** – Healthcare Improvement Scotland, United Kingdom  
**Juliette Senecat** – EURORDIS, France  
**Domenica Taruscio** – Istituto Superiore di Sanità, Italy  
**Holger Schünemann** – McMaster University, Canada



### Technical Secretariat:

<b>Andrea Vittozzi</b>	Tel:4418	e-mail:contact.cnmr@iss.it
<b>Stefano Diemoz</b>	Tel: 4019	e-mail:stefano.diemoz@iss.it
<b>Patrizia Crialesi</b>	Tel: 4364	e-mail patrizia.crialesi@iss.it
<b>Giuseppe Bernardo</b>	Tel:4419	e-mail:giuseppe.bernardo@iss.it

### GENERAL INFORMATION

**Meeting Venue:** Istituto Superiore di Sanità, Aula Pocchiari, Viale Regina Elena, 299 - Rome

**Target audience:** patients, patient representatives, health professionals, researchers, policy makers, research funders and those involved in the development of guidelines and HTA for rare diseases.

**Official language of the Conference:** English

**Predicted number of participants:** 180

**CME accreditations:** NO

Participation is free of charge. The organizers will provide refreshments and lunch but will not pay for travel and accommodation expenses of attendees.

**Conference registration:** The application form, available at <https://it.surveymonkey.com/r/RBPconference> must be duly completed and sent via web **by 23/11/2016**.

The application is properly sent only when the system displays the final *thank you* page.

Submissions will be automatically accepted in chronological order of registration, until reaching the maximum meeting room capacity.

**Certificate of attendance:** Participants will receive a certificate of attendance at the end of conference

For every event relevant information please contact the Scientific Secretariat at the numbers listed above.