FIRST INTERNATIONAL WORKSHOP

CLINICAL PRACTICE GUIDELINES ON RARE DISEASES

ROME, 23RD -24TH FEBRUARY 2012

ISTITUTO SUPERIORE DI SANITÀ

VIALE REGINA ELENA, 299

AULA POCCHIARI

DAY one: 23rd February 2012

08:30 Participants registration

08:45 Welcome address
Enrico Garaci, President of Istituto Superiore di Sanità
Renato Balduzzi, Italian Minister of Health (To Be Confirmed)

09:00 Workshop objectives
Domenica Taruscio

Session 1: The state of the art
Chairs: Paola Facchin, Isabel Peña-Rey

09:10 Rare diseases: features and specificities (Domenica Taruscio)

09:30 Existing approaches to handling evidence on rare diseases for the development of Clinical Practice Guidelines (Alric Rüther)

10:00 Development of clinical practice guidelines on rare diseases: the National Centre for Rare Diseases experience (Cristina Morciano)

10:30 Development of clinical practice guidelines on rare diseases: the French National Authority for Health experience (Valérie Lindecker-Cournil)

11:00 *Coffee break*

11:30 Development of clinical practice guidelines on rare diseases: the TREAT-NMD experience (Thomas Sejersen)

12:00 General discussion

13:00 *Lunch*

Session 2: Guidelines on rare diseases and quality of care

Chairs: Kate Bushby, Alfonso Mele

14:00 Quality improvement in Scotland and rare diseases (Michele Hilton Boon)

14:30 Guidelines and cost-efficacy (Panos Kanavos)

15.00 Patients expectations: EURORDIS (Monica Ensini)

15:30 Health Institutions expectations: London Strategic Health Authority (Edmund Jessop)

16.00 Health Institutions expectations: Office of Population Health Genomics (Hugh Dawkins)

16.30 Clinical practice guidelines: link between clinical services and research in rare diseases (Luis Alejandro Barrera)

17.00 The EU point of view (Antoni Montserrat)

17.30 WHO approach to guidelines for rare diseases: the point of view of the guideline review committee (Laragh Gollogly)

18.00 General Discussion

18:30 *Conclusion of day I*

DAY two: 24th February 2012

Session 3: Developing guidelines in presence of scarce evidence

Chairs: Bruno Dallapiccola, Alric Rüther

9.00 EBM and rare diseases: how to manage the absence/scarcity of evidence (Nicola Magrini)

9.30 Narrative based medicine (Rishi Goyal)

10.00 Evidence Based Medicine - Narrative Based Medicine: a possible integration (Guido Giarelli)

10.30 *Coffee break*

11.00 GRADE system and rare diseases: how to make weak recommendations more transparent (Holger Schünemann)

11:30 Identifying and managing conflict of interest (Antonio Addis)

12.00 General Discussion

13.00 *Lunch*

Session 4: How to promote quality of guidelines on rare diseases at international level?
Chairs: Angela Brand, Holger Schünemann

14.00 The Institute of Medicine standards (Richard N. Shiffman)

14.30 What is G-I-N's role in promoting the quality of Clinical Practice Guidelines on rare diseases? (Fergus Macbeth)

15:00 Round Table "Future steps for international collaboration"
Moderators: Richard N. Shiffman, Domenica Taruscio
Discussants:
Angela Brand, Kate Bushby, Rishi Goyal, Manuel Posada, Béla Melegh, Pedro Serrano-Aguilar, Liesbeth Siderius, Rumen Stefanov, Holger Schünemann.
Patients' representatives: Monica Ensini (EURORDIS), Flavio Bertoglio (Consulta Nazionale delle Malattie Rare), Renza Barbon Galluppi (UNIAMO).

16.30 General Discussion

17:30 *Closing remarks*

SPEAKERS, DISCUSSANTS, CHAIRPERSONS AND MODERATORS
Antonio Addis, Agenzia sanitaria sociale regionale - Regione Emilia Romagna, Italy
Renato Balduzzi, Ministry of Health, Italy
Renza Barbon Galluppi, UNIAMO, Italy
Luis Alejandro Barrera, Instituto Errores Innatos del Metabolismo Universidad Javeriana, Colombia
Flavio Bertoglio, Consulta Nazionale delle Malattie Rare, Italy
Angela Brand, University of Maastricht, EUCERD Member, Netherlands
Kate Bushby, Institute of Human Genetics, Newcastle University, United Kingdom
Bruno Dallapiccola, Ospedale Pediatrico "Bambino Gesù", EUCERD Member, Italy
Hugh Dawkins, Office of Population Health Genomics, Australia
Monica Ensini, European Organization for Rare Diseases, France
Paola Facchin, Coordinamento Interregionale Malattie Rare, Registro Veneto Malattie Rare, Padova, Italy
Enrico Garaci, President of the Istituto Superiore di Sanità, Italy
Guido Giarelli, Department of Health Sciences, University "Magna Graecia" Catanzaro, Italy
Laragh Gollogly, World Health Organization
Rishi Goyal, College of Medicine, University of Arizona, United States
Michele Hilton Boon, Healthcare Improvement Scotland, United Kingdom
Edmund Jessop, London Strategic Health Authority, EUCERD Member, United Kingdom
Panos Kanavos, London School of Economics and Political Science, United Kingdom
Valérie Lindecker-Cournil, Service des Bonnes Pratiques Professionnelles, Haute Autorité de Santé, France
Fergus Macbeth, National Institute for Health and Clinical Excellence, member of the G-I-N Board, United Kingdom

Nicola Magrini, Agenzia Sanitaria e Sociale Regionale Regione Emilia Romagna, Italy
Alfonso Mele, National Guidelines System (SNLG), Istituto Superiore di Sanità, Italy
Béla Melegh, Department of Medical Genetics, University of Pécs, Hungary
Antoni Montserrat, Policy Officer for Rare Diseases, Neurodevelopmental Disorders and the European Health Examination Survey, Health and Consumers General-Directorate (SANCO), Directorate C "Public Health and Risk Assessment", European Commission
Cristina Morciano, National Centre for Rare Diseases, Istituto Superiore di Sanità, Italy
Isabel Peña-Rey, Quality Agency of the NHS Ministry of Health and Social Policy and Equality, EUCERD Member, Spain
Manuel Posada de la Paz, Istituto de Salud Carlos III - Ministry of Science and Innovation, Spain
Alric Rüther, Institute for Quality and Efficiency in Health Care – IQWiG, Germany
Holger Schünemann, Department of Clinical Epidemiology & Biostatistics McMaster University Health Sciences Centre, Canada
Thomas Sejersen, Karolinska Institute, Treat-NMD Member, Sweden
Pedro Serrano-Aguilar, Fundación Canaria de Investigación Y Salud, Canary Islands
Richard N. Shiffman, Yale Center for Medical Informatics, United States
Liesbeth Siderius, European Academy of Paediatrics, Youth Healthcare, Meppel, Netherlands
Rumen Stefanov, Bulgarian Association for Promotion of Education and Science, Bulgaria
Domenica Taruscio, National Centre for Rare Diseases, Istituto Superiore di Sanità, EUCERD Member, Italy

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Simultaneous translation service English/Italian, Italian/English will be available

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