PROGRAMME COMMITTEE

MEMBERS

Lieven Annemans
Professor of Health Economics
Ghent University
Belgium

Nicola Bedlington
Secretary General
European Patients' Forum (EPF)
Austria

Kate Bushby
Act. Res.Chair of Neuromuscular Genetics, RD-Action
UK

Ivana Cattaneo
Europabio - EFPIA Joint Task Force on Rare Diseases and Orphan Medicinal Products
Italy

Karen Facey
Evidence Based Health Policy Consultant, HTAi
UK

Benjamin Gannon
Europabio - EFPIA Joint Task Force on Rare Diseases and Orphan Medicinal Products
UK

Yann Le Cam
Chief Executive Officer
EURORDIS
France

Francis Pang
EUCOPE representative
UK

Jean-Louis Roux
Public Affairs Director
EURORDIS
Belgium

Chris Sotirelis
Trustee Advisor
Thalassaemia UK
UK

Sheela Upadhyaya
Associate Director - Highly Specialised Technologies, NICE
UK

Stijn Vanacker
Analyst – Global Healthcare
Robeco
Netherlands

ADVISORS

Charles Barker
Partner, PrimeMover Associates
USA

Jill Bonjean
Resource Development Director
EURORDIS
France

Hans-Georg Eichler
Senior Medical Officer
European Medicines Agency

Pascal Garel
Chief Executive
The European Hospital and Healthcare Federation (HOPE)
Belgium

Virginie Hivert
Therapeutic Development Director
EURORDIS
France

Sandra Nestler-Parr
Co-Chair – ISPOR Rare Disease Special Interest Group; Trustee – Alpha-1 UK Support Group; Head of Rare Diseases – Roboleo & Co
UK

Elizabeth Vroom
President Dutch Duchenne Parent Project and Chair of UPPMD
Netherlands

Russell Wheeler
Trustee and patient advocate
Leber's Hereditary Optic Neuropathy Society (LHON Society)
UK

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#RareEU2017

EURORDIS
RARE DISEASES EUROPE

Paris Office

EURORDIS
Plateforme Maladies Rares
96, rue Didot
75014 Paris, France
Tel: +33 (0)1 56 53 52 10
eurordis@eurordis.org

Brussels Office

EURORDIS
Fondation Universitaire
Rue d'Egmont 11
1000 Brussels, Belgium

eurordis.org

Graphic design www.FabienNail.com

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2ND MULTI-STAKEHOLDER Symposium ON IMPROVING PATIENT ACCESS TO RARE DISEASE THERAPIES

22-23 FEBRUARY 2017
HOTEL LE PLAZA
BRUSSELS

EURORDIS
RARE DISEASES EUROPE

A cooperative process to reach mutually acceptable solutions that respects all stakeholders, improves our common understanding and establishes sustainable mutual trust

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A EURORDIS RARE DISEASE DAY® EVENT

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DAY 1
WEDNESDAY 22 FEBRUARY 2017
9.00 to 18.30

09.00 – 13.00 INTRODUCTION & OPENING PLENARY
EXPRESSION OF STAKEHOLDER INTERESTS Theatre room
Live video streaming
Co-Chairs: **Charles Barker**, PrimeMover Associates, USA & **Peter O'Donnell**, Politico, Belgium

09.00 – 09.15 Setting the scene **Yann Le Cam**, Chief Executive Officer, EURORDIS

09.15 – 09.30 Introduction **Charles Barker**, PrimeMover Associates, USA

09.30 – 09.40 Patient case study: The consequences of diverging and inconsistent decisions
Elizabeth Vroom, President, Dutch Duchenne Parent Project and Chair of UPPMD, Netherlands

09.40 – 11.10 PANEL discussion: moderated by Co-Chairs
What are the interests of pharmaceutical companies developing treatments/therapies for rare disease patients?
What are the challenges? What are the options moving forward? How do we work together to improve this?
Q&A from on-site and online audiences

Panelists: Martin Andrews, Senior Vice President, GSK Rare Diseases, UK
Simon Bedson, Senior Vice President and General Manager, International Commercial Operations, Vertex, UK
Marc Booty, PST Head – Healthcare, Pictet Asset Management Investment, UK
Michael Goettler, Global President, Rare Disease Business, Pfizer
Emil Kakkis, President & Chief Executive Officer, UltraGenyx, USA
Tuomo Pätsi, President EMEA, Celgene, Switzerland
Questioners: Dimitrios Athanasiou, Patient Expert, Muscular Dystrophy Association Hellas, Greece
Avril Daly, Chief Executive Officer, Retina International, Ireland
Jack Scannell, Co-head of Pharmaceuticals - Equity Research, UBS, UK
Chris Sotirelis, Trustee Advisor, Thalassaemia UK, UK

11.10 – 11.40 *Coffee break (Gallery & Adolphe Max room)*

11.40 – 13.00 PANEL discussion: moderated by Co-Chairs
What are the interests of payers and HTA bodies when considering how to improve access to rare disease therapies?
What are the challenges? What are the options moving forward? How do we work together to improve this?
Q&A from on-site and online audiences

Panelists: David Elvira, Director General, Catalonia Medicines Agency, Spain
Gottfried Endel, Department for Evidence Based Economic Health Care, Main Association of Austrian Social Insurance Institutions, Austria
Diane Kleinermans, Ministry of Public Health, Belgium
Marco Petschulies, Scientific Advisor, G-BA, Germany
Sheela Upadhyaya, Associate Director Highly Specialised Technologies, Centre for Health Technology Evaluation, National Institute for Health and Care Excellence (NICE), UK
Questioners: Martin Andrews, Senior Vice President, GSK Rare Diseases, UK
Nicola Bedlington, Secretary General, European Patients' Forum, Belgium
Yann Le Cam, Chief Executive Officer, EURORDIS, France
Stijn Vanacker, Global Healthcare Analyst, Global Equity, Robeco, Netherlands

13.00 – 14.00 *Lunch (Adolphe Max room)*

14.00 – 18.30 PLENARY : THE IMPORTANCE OF MULTI-STAKEHOLDER COLLABORATION Theatre room
Co-Chairs: **Charles Barker**, PrimeMover Associates, USA & **Peter O'Donnell**, Politico, Belgium

14.00 – 14.10 Case study: The importance of multi-stakeholder collaboration
Lucia Monaco, Chief Scientific Officer, Fondazione Telethon, Italy

14.10 – 14.25 Collaborating for success **Karen Facey**, HTAi, UK

14.25 – 14.55 Current state of the art in multi-stakeholder collaborative processes
Early dialogue initiatives & expedited regulatory pathways

Speakers: EMA, PRIME, Adaptive pathways: Hans-Georg Eichler, Senior Medical Officer, EMA
Clinical trials in small populations: Simon Day, Chair, IRDiRC Small-Patients Clinical Trial Task Force, Director, Clinical Trials Consulting & Training Limited, UK
EUNetHTA: François Meyer, Advisor to the President, International Affairs, Haute Autorité de Santé (HAS), France
Mechanism of Coordinated Access to orphan medicinal products (MoCA): Ana Palma, Global HTA & Patient Access Lead, Sobi, Belgium

14.55 – 16.15 Multi-stakeholder panel discussion: success factors for collaboration in relation to access
Q&A from on-site and online audiences

Moderator: Hans-Georg Eichler, Senior Medical Officer, EMA
Panelists: Diego Ardigo, Chair, Therapeutic Scientific Committee IRDiRC and Project Lead, Chiesi Group, Italy
Stella Blackburn, Vice President, Global Head of Risk Management, Real-World & Late Phase Research, Quintiles, UK
Ri de Ridder, Director General, RIZIV/INAMI, Belgium
Karen Facey, HTAi, UK
Ruth Ladenstein, President of Europe's Paediatric Oncology Society, Austria
François Meyer, Advisor to the President, International Affairs, Haute Autorité de Santé (HAS), France
Marco Petschulies, Scientific Advisor, G-BA, Germany
Chris Sotirelis, Trustee Advisor, Thalassaemia UK, UK

16.15 – 16.30 *Coffee break (Gallery & Adolphe Max room)*

16.30 – 18.00 A collaborative conversation for transformative operational solutions serving the interests of each stakeholder
Moderator: Charles Barker, PrimeMover Associates, USA

18.00 – 18.30 Conclusions from Day 1 **Peter O'Donnell**, Politico, Belgium

18.30 *End of Day 1*

DAY 2
THURSDAY 23 FEBRUARY 2017
08.00 to 18.00

08.00 – 09.00 PLENARY Theatre room
Co-Chair: **Sandra Nestler-Parr**, Co-Chair-ISPOR Rare Disease Special Interest Group; Trustee – Alpha-1 UK Support Group; Head of Rare Diseases – Roboleo & Co, UK and **Sheela Upadhyaya**, Associate Director Highly Specialised Technologies, Centre for Health Technology Evaluation, National Institute for Health and Care Excellence (NICE), UK

08.00 – 08.15 Trajectory from Day 1 & Aims of Day 2
Sandra Nestler-Parr, Co-Chair-ISPOR Rare Disease Special Interest Group; Trustee – Alpha-1 UK Support Group; Head of Rare Diseases – Roboleo & Co, UK

08.15 – 08.25 Patient case study: Patient Involvement as Game Changers **Angela Paton**, MPS Society, UK

08.25 – 08.45 Introduction to breakout sessions Moderators of breakouts

09.00 – 11.00 SIMULTANEOUS BREAKOUT SESSIONS – Emerging options

Versailles room
Breakout 1: Quality Data Generation

Pan-European disease & product registries to address needs of all stakeholders

Moderator: Vinciane Debroux-Pirard, Senior Director Public Affairs, Sanofi-Genzyme, Netherlands
Rapporteur: Xavier Fournie, Corporate Medical Director, Executive Vice-President, Global Medical Affairs – Real World Evidence, Mapi Group, France
Speakers: Henk Blom, Head Laboratory for Clinical Biochemistry and Metabolism, University Medical Center Freiburg, Germany; **Claudia Crocione**, Project & Communication Manager, HHT Onlus, Italy; **Marco Roos**, Group leader and senior scientist biosemantics, Leiden University Medical Center, Netherlands; **Marieke Schoonen**, Observational Research Scientist, Amgen, UK

Theatre room
Breakout 2: Value for money across Europe

Towards a common European value and funding system for orphan medicinal products: implementing the ORPH-VAL principles

Moderator: Lieven Annemans, Ghent University, Belgium
Rapporteur: Ruediger Gatermann, Director Health Policy & External Affairs Europe, CSL Behring, Germany
Speakers: Adam Hutchings, Director, Dolon Ltd., UK; **Michael Schlander**, Professor of Health Economics University of Heidelberg, Germany

Estérel room
Breakout 3: Outcomes

Innovative performance based outcome agreements

Moderator: Karen Facey, Evidence Based Health Policy Consultant, HTAi, UK
Rapporteur: Adrian Towse, Director, Office of Health Economics, UK
Speakers/panelists: Tim Wilsdon, Vice President, CRA International and Financial Services Consultant, UK; **Sheela Upadhyaya**, Associate Director Highly Specialised Technologies, NICE, UK; **Thomas Hach**, Director Healthcare Systems from Group Global Strategy, Novartis, Switzerland; **Charlotte Roberts**, MPS Society, UK

11.00 – 11.15 *Coffee break (Gallery & Adolphe Max room)*

11.15 – 12.30 PLENARY Theatre room
Co-Chairs: **Sandra Nestler-Parr,** Co-Chair-ISPOR Rare Disease Special Interest Group; Trustee – Alpha-1 UK Support Group; Head of Rare Diseases – Roboleo & Co, UK & **Russell Wheeler**, trustee and patient advocate, Leber's Hereditary Optic Neuropathy Society (LHON Society), UK

11.15 – 12.30 Feedback and discussion from morning breakout sessions Rapporteurs from breakouts
Introduction of afternoon breakout sessions Moderators of breakouts

12.30 – 13.30 *Lunch (Salon Adolphe Max)*

13.30 – 15.30 SIMULTANEOUS BREAKOUT SESSIONS - New options

Versailles room
Breakout 4: Quality Data Generation

How European Reference Networks (ERNs) could become part of the solution / enablers of quality data generation?

Moderator: Adam Heathfield, Senior Director, Global Health and Value Innovation Centre, Pfizer, UK
Rapporteur: Virginie Bros-Facer, Research Infrastructure Project Manager, EURORDIS, France
Panelists: Amanda Bok, CEO, European Haemophilia Consortium (EHC), Belgium; **Vinciane Debroux-Pirard**, Senior Director Public Affairs, Sanofi-Genzyme, Netherlands; **Ruth Ladenstein**, President of Europe's Paediatric Oncology Society, Austria; **Mauricio Scarpa**, Clinical Lead for Rare Metabolic Diseases, Director of the Centre for Rare Diseases, Helios Dr Horst Schmidt Clinic, Germany; **Luca Sangiorgi**, Head Medical Genetics and Rare Orthopaedic Diseases, Rizzoli Orthopaedic Institute, Italy; **Matt Bolz-Johnson**, Healthcare and Research Director, EURORDIS, Germany

Theatre room
Breakout 5: Value for money across Europe

Proposals for coordination of HTA across Europe: implications for rare diseases

Moderator: Wim Goettsch, Director, EUNetHTA, Netherlands
Rapporteur: Julia Chamova, Director, Global Networks (EMEA), ISPOR, Sweden
Speakers and panelists: Karolina Hanslik, Health Policy Officer, DG SANTE (Directorate Health Systems and Products), European Commission; **Trevor Leighton**, VP Pricing & Reimbursement, Shire, UK; **Andrea Rappagliosi**, VP, Head of Public Affairs Europe, Sanofi Pasteur MSD, France; **Valentina Strammiello**, Programme Officer, European Patients' Forum, Belgium; **Francis Pang**, Head, Global Market Access, Amicus Therapeutics UK Limited, UK

Estérel room
Breakout 6: Outcomes

Potential for European collaboration among payers and companies

Moderator: Ri de Ridder, Director General, RIZIV/INAMI, Belgium
Rapporteur: To be named
Panelists : Inneke Van De Vijver, Analyst Pharmaceuticals & File Manager , RIZIV/INAMI, Belgium; **Stefan Weber,** Director Payment Policy from Global Public Policy, Novartis; **Jean-Louis Roux**, Public Affairs Director, EURORDIS, Belgium; **Gottfried Endel**, Department for Evidence Based Economic Health Care, Main Association of Austrian Social Insurance Institutions, Austria

15.30 – 15.45 *Coffee break (Gallery and Adolphe Max room)*

15.45 – 18.00 PLENARY Theatre room
Co-Chairs: **Sandra Nestler-Parr**, Co-Chair-ISPOR Rare Disease Special Interest Group; Trustee – Alpha-1 UK Support Group; Head of Rare Diseases – Roboleo & Co, UK and **Dimitrios Athanasiou**, Patient Expert, Muscular Dystrophy Association Hellas, Greece

15.45 – 16.45 Feedback and discussion from afternoon breakout sessions Rapporteurs from breakouts

16.45 – 17.00 The role of the European Commission on improving access to rare disease therapies
Xavier Prats Monné, Director General, Directorate-General for Health and Food Safety, European Commission

17.00 – 17.45 PANEL discussion: Paving the way to a fair, inclusive and on-going multi-stakeholder approach with the potential to generate sustainable, affordable and actionable improvements in patient access to rare disease therapies
Co-Moderators: Charles Barker, PrimeMover Associates, USA & **Laura Batchelor**, Director, FIPRA International, Belgium
Panelists: Nicola Bedlington, Secretary General, European Patients' Forum, Belgium
Vinciane Debroux-Pirard, Co-Chair, Joint Task Force on Orphan Drugs & Rare Diseases, EFPIA-EuropaBio, Netherlands
Jo de Cock, Chief Executive Officer, National Institute of Health and Disability Insurance (NIHDI), Belgium
Karen Facey, Evidence Based Health Policy Consultant, HTAi, UK
Victoria Hedley, RD-ACTION Thematic Coordinator for Rare Diseases at Newcastle University Institute of Genetic Medicine, UK
Yann Le Cam, Chief Executive Officer, EURORDIS, France
Alexander Natz, Secretary General, EUCOPE, Belgium
Sandra Nestler-Parr, ISPOR Rare Disease Special Interest Group, UK
Mauricio Scarpa, Clinical Lead for Rare Metabolic Diseases, Director of the Centre for Rare Diseases, Helios Dr Horst Schmidt Clinic, Germany

17.45 – 18.00 Conclusions & closing remarks
Speaker to be named

18.00 *End of Day 2 – End of symposium*