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

ADVANCED PROGRAMME

DAY 1: Wednesday, 22 February 2017

09.00 to 18.30

09.00 – 13.00	***INTRODUCTION & OPENING PLENARY*** ***EXPRESSION OF STAKEHOLDER INTERESTS*** *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/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, Pictet Asset Management Investment, UK Michael Goettler, Global President, Rare Disease Business, Pfizer Emil Kakkis, President & Chief Executive Officer, UltraGenyx Tuomo Pätsi, President EMEA, Celgene **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
11.10 – 11.40	*Coffee break*

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 (Salon Adolphe Max)*
14.00 – 18.30	***PLENARY***
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 **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** **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 Q&A from on-site and online audiences
16.15 – 16.30	*Coffee break*
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 – 18.00

08.00 – 09.00	***PLENARY*** *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	***BREAKOUT SESSIONS* – Emerging options**		
	Breakout 1: Quality Data Generation	**Breakout 2: Value for money across Europe**	**Breakout 3: Outcomes**

	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; Marco Roos, Group leader and senior scientist biosemantics, Leiden University Medical Center, Netherlands; Marieke Schoonen, Observational Research Scientist, Amgen, UK	**Common principles for value determination and assessment** **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	**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; Charlotte Roberts, MPS Society, UK
11.00 – 11.15	*Coffee break*		
11.15 – 12.30	***PLENARY*** ***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)*		
11.15 – 12.30	**Feedback and discussion from morning breakout sessions** Rrapporteurs from breakouts **Introduction of afternoon breakout sessions** Moderators of breakouts		
12.30 – 13.30	*LUNCH (Salon Adolphe Max)*		
13.30 – 15.30	***BREAKOUT SESSIONS*** **- New options**		
	Breakout 4: Quality Data Generation	**Breakout 5: Value for money across Europe**	**Breakout 6: Outcomes**
	How European Reference Networks (ERNs) could become part of the solution / enablers of quality data generation?	**Proposals for coordination of HTA across Europe: implications for rare diseases**	**Potential for European collaboration among payers and companies**

	Moderator: Adam Heathfield, Senior Director, Global Health and Value Innovation Centre, Pfizer, UK **Rapporteur:** Virginie Bros-Facer, Research Infrastructure Project Manager, EURORDIS, France **Panelists:** 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	**Moderator:** Wim Goettsch, Director, EUNetHTA, Netherlands **Rapporteur:** Julia Chamova, Director, Global Networks (EMEA), ISPOR, Sweden **Speakers:** Karolina Hanslik, Health Policy Officer, DG SANTE (Directorate Health Systems and Products), European Commission; Andrea Rappagaliosi, VP, Head of Public Affairs Europe, Sanofi, France; Valentina Strammiello, Programme Officer, European Patients' Forum, Belgium; Francis Pang, Head, Global Market Access, Amicus Therapeutics UK Limited, UK	**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
15.30 – 15.45	*Coffee break*		
15.45 – 18.00	***PLENARY*** ***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 breakout sessions** Co-Chairs & 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, 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