

15th Annual

WORLD

OrphanDrug

Congress Europe 2024

22 - 25 October 2024

22 October | Pre-Congress Workshops | Hotel Catalonia Barcelona Plaza

23-25 October | Main Congress | Fira Barcelona Montjuïc

Strategy, Advocacy & Partnering For The Global Orphan Drug Industry



Featuring

Access & Pricing

Cell & Gene Therapy

Clinical Development

Precision Medicine

Patient Centricity

Real World Evidence

Science Strategy

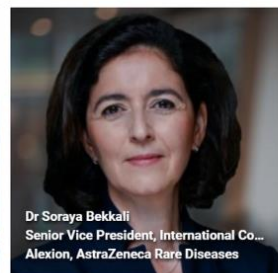
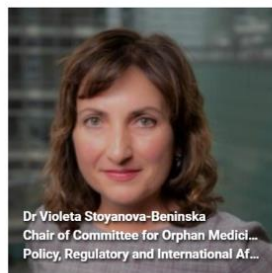
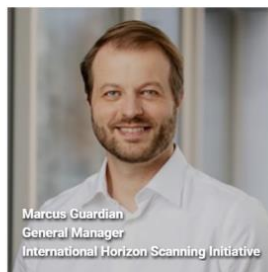
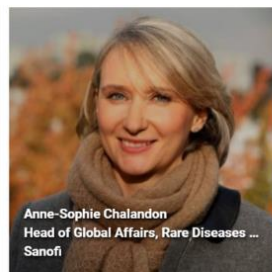
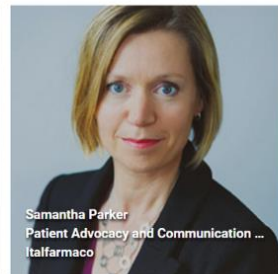
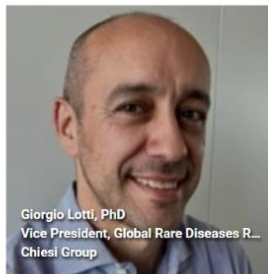
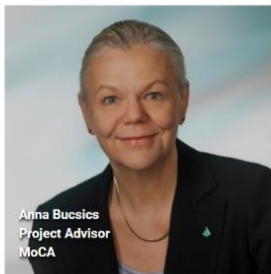
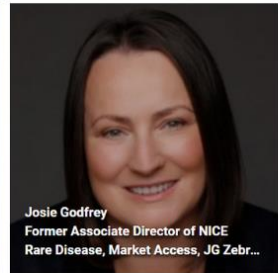
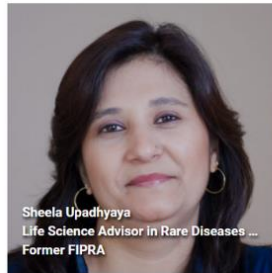
Technology Showcases

Created by

www.terrapiinn.com/WODC

SPARK SOMETHING
TERRAPINN

Advisory Board



Supporting Partners



Rare Disease Review
by World Orphan Drug Congress

A SUMMARY OF THE LATEST BREAKTHROUGHS, INSIGHTS, AND STORIES IN THE WORLD OF RARE DISEASES AND ORPHAN DRUGS

Do you want to contribute to our newsletter? Reach out to Juliana Schulte at juliana.schulte@terrapinn.com

Agenda Overview

Pre-Congress Workshops – Tuesday 22th October					
Morning Workshops: 10:30 – 13:00					
AI & Rare Disease			Holistic Approaches to RD Drug Development		
Lunch & Networking Break: 13:00-14:00					
Afternoon Workshops: 14:00 – 16:30					
The Burden of Rare Diseases & The Patient Journey		The Rare Disease Action Plan		The EUJHTA Basics for Orphan Drug Assessment	
Networking Drinks Sponsored by Ascella Health 17:00					
Day 1 – Wednesday 23rd October					
Morning Keynotes: 09:00-10:45					
Expo & Networking Break: 10:45-11:30					
Interactive Working Groups: 11:30-12:30					
Expo & Networking Break: 12:30-14:00			Poster Presentations: 13:30 – 14:00		
Conference Tracks Begin: 14:10					
Clinical Development	Access & Pricing	Cell & Gene Therapy	Real World Evidence	Precision Medicine	Technology Showcases
Expo & Networking Break: 15:40-16:10					
Track Presentations: 16:10-17:40					
Clinical Development	Access & Pricing	Cell & Gene Therapy	Real World Evidence	Precision Medicine	Technology Showcases
NEW: Company Presentations – Exhibition Stage: 15:40 – 17:00					
Off-Site Networking at the Museum Terrace Sponsored by Alexion 18:00					
Day 2 – Thursday 24th October					
Morning Keynotes: 09:00-10:25					
Expo & Networking Break: 10:25-11:10					
Track Presentations: 11:10-12:40					
Clinical Development	Access & Pricing	Real World Evidence	Science & Strategy	Precision Medicine	PatientCentricity
Expo & Networking Break: 12:40-14:10			Poster Presentations: 13:40 – 14:10		
Interactive Working Groups: 12:50-13:50					
Track Presentations: 14:10-17:40					
Clinical Development	Access & Pricing	Real World Evidence	Science & Strategy	Precision Medicine	PatientCentricity
Expo & Networking Break: 16:10-16:30					
Closing Keynote: 16:30-17:30					
EXPO Networking Drinks: 18:00					
Day 3 – Friday 25th October					
Morning Keynotes: 09:00-10:30					
Comfort Break: 10:30-10:40; Tracks restart 10:40					
Clinical Development	Access & Pricing	Cell & Gene Therapy	Science & Strategy	PatientCentricity	
Expo & Networking Break: 12:10-13:30 , tracks restart 13:30					
Clinical Development	Access & Pricing	Cell & Gene Therapy	Science & Strategy	PatientCentricity	
End of Congress 15:00					

Floor Plan



FIRA - BARCELONA
23 - 25 OCTOBER 2024

HALL 2



<https://www.terrapiinn.com/conference/world-orphan-drug-congress/floor-plan.stm>

Pre-congress Workshops Tuesday Oct 22 – Offsite at the Catalonia Plaza hotel

10:30 –
13:00
CET

ElevateHealth: Creating real impact for rare disease Patients

How can stakeholders in the healthcare ecosystem solve the complexity together?



This workshop kicks off a joint initiative between Volv Global and Novartis on how we can accelerate and optimise patient outcomes across and experience throughout the patient journey, taking particular aim at those issues which arise from insufficient coordination across stakeholders and across differences of scale.

This “ElevateHealth” project will address:

- The Micro level of the individual, i.e., the patient (also including individual physicians and patient representatives)
- The Meso level of healthcare resources, such as healthcare institutions and healthcare systems
- The Macro level of healthcare provision, including payers, insurers, regulators, taking into account impact outside the healthcare systems and its stakeholders, e.g. on society, for employers, non-paid work, etc.

It will focus on the barriers to adoption of new technology solutions that can add significant value to the healthcare ecosystem. E.g., to improve the diagnosis pathway, access to care, clinician workload and impact on healthcare system for rare diseases. The Novartis part of ElevateHealth will take the form of a parallel workshop outside the World Orphan Drug Congress programme on the evening of Thursday, 24th October, 2024.

If you are part of the healthcare ecosystem, we welcome you to join us and offer your perspectives to the discussion!

Workshop leaders: Christopher M de M Rudolf, Founder & CEO & Léon van Wouwe, Clinical Innovation Director, volv global

Hollistic Approaches to Rare Disease Drug Development: Integrating ESG, Patient Engagement, and Commercial Strategy for Improved EU Access



- Unique challenges in rare disease drug development and the need for a comprehensive approach.
- ESG in Rare Disease: Discuss how environmental, social, and governance factors are becoming increasingly important in the rare disease space.
- Emphasize the social impact of developing treatments for underserved populations
- Discuss ethical considerations in rare disease access to treatment
- Patient Engagement: Stress the critical role of patient involvement throughout the drug development process

And more...

Workshop leader: Kieron Lewis, Director of Strategic Consulting, Clinigen

Speakers:

Nicholas Brooke, Founder, The Synergist & Executive Director, PFMD
Sheela Upadhyaya, Life Science Advisor in Rare Diseases & Special Advisor, Former FIPRA & Consultant

13:00 –
14:00

Petra Nathan, CRMA, Novartis
Dr James A Levine, President, Foundation Ipsen

Networking Lunch Break
The workshops restart at 14:00

14:00 -
16:30
CET

14:00 Reducing the Burden of Rare Diseases Across The Patient Journey



People living with rare diseases face distinct and significant challenges that arise from the infrequency of their medical conditions, such as a long diagnostic journey, inadequate clinical management, and limited access to effective treatments. The burden of rare diseases on patients, their carers and families, healthcare systems, and society overall, merits greater visibility and recognition. Addressing these societal challenges requires a multifaceted approach involving stakeholders across the healthcare system, government agencies, research institutions, industry, and patient advocacy organizations.

This discussion will examine the patient perspective and the burdens faced explore what is needed to speed up and improve the patient journey of Europeans living with rare diseases, with clear recommendations for health systems and policymakers. The session will be broken into two parts:

Part 1 – The Patient Perspective: Patient experiences along the rare disease ‘journey’

Part II – ‘We all have a role’ – multi-stakeholder solutions across the rare disease ‘journey’ from

The Rare Disease Action Plan – How will we implement this?



Rare diseases pose unique challenges to healthcare systems worldwide due to their low prevalence and complex treatment requirements. Traditional financing mechanisms often fail to adequately address the needs of patients with rare diseases, leading to disparities in access to treatment and care. Last June, RDI (Rare Diseases International) launched a campaign for a World Health Assembly (WHA) Resolution on Rare Diseases in 2025. RDI is launching a campaign for a World Health Assembly (WHA) Resolution on Rare Diseases in 2025.

Objectives of the session

overall objective of the session is to discuss the different articulations needed between the several stratifications of plans, national, regional and global:

- To identify the interconnections needed
- To ensure efficiency of the system
- To avoid fragmentation of strategy and actions
- To identify the appropriate use of the resources

The session should be an active interactive session between the panellists experts and the participants.

Workshop leader introduction:

Never mind the B*****S, here’s the EUJHTA basics



A pragmatic and interactive demonstration of the essential steps required to successfully prepare an orphan oncology drug for a future EU joint clinical assessment.

This workshop offers a hands-on, practical approach to navigating the new EU joint Health Technology Assessment (EUJHTA) framework, with a focus on orphan oncology drugs. Attendees will gain a clear understanding of the fundamental principles behind the EUJHTA, including its objectives, structure, and importance in the drug assessment landscape. The session will also delve into the timelines and requirements crucial for successful EUJHTA preparation. Through interactive demonstrations and expert insights, participants will learn the necessary steps to effectively plan and execute a comprehensive strategy to meet EUJHTA standards, ensuring a smoother pathway for future joint clinical assessment.

Workshop leader: Sophie Schmitz, Managing Partner, Partners4Access

Speakers:

industry, R&D, AI, HTA and health systems. What is needed at the EU level to improve equity of access to diagnostics and treatments?

Workshop Leader: **Neil Bertelsen**, Patient Engagement Specialist and Board Member, **PFMD** & Steering Committee Member, **HTAi**

Speakers:

Peter Fish, CEO, **Mendelian**

Federica Miotta, Head of Medical Affairs, **Fondazione Telethon**

Bob Stevens, CEO, **MPS Society**

Natascha Sippel, Managing Director, **Morbus Fabry SHG**

Anne-Sophie Chalandon, Head of Rare Diseases Global Public Affairs Rare Disease and ATMP Policy at **Sanofi**, and leading the **IFPMA** Rare Diseases Group

14:10 – 16:00

Testimony and open discussion:

Alexandra Heumber Perry, CEO, **RDI**

Dr Virginie Bros-Facer, CEO, **EURORDIS – Rare Diseases Europe**

Ulrike Schwerdtfeger, Legal Technical Officer, **WHO**

Fabienne Bartoli, Inspector General, **French Ministry of Health**

Senior Representative, **ERN**

Senior MEP

invited:

Hélène Dollfus, Professor and Consultant, **University Hospital of Strasbourg** & Coordinator, **ERN-EYE**

Holm Graessner, Managing Director, **Centre for Rare Diseases Tubingen**, & Coordinator, **ERN-NMD**
Senior Representative, **European Commission**

16:00 – 16:30

Q&A & Closing remarks

Darren Callanan, Global Digital Lead, **Partners4Access**
Oriol Solà-Morales, Chair & Founder, **HiTT**

16:30

Networking Drinks Sponsored by









End of Workshops. Congress begins tomorrow 09:00 at the Fira Barcelona, Montjuic in hall 2

Day One **Wednesday 23 October**

OPENING KEYNOTE PLENARY Location: Keynote Theatre, Hall 2	
09:00	Opening remarks Soraya Bekkali , Senior Vice President, EUCAN and International Business, Alexion AstraZeneca Rare Disease
09:10	Keynote panel: How will Europe remain competitive in the C&GT and Rare Disease sector? The role of the research and innovation policies for a stronger pharmaceutical strategy in Europe <ul style="list-style-type: none"> - How can we ensure Europe's R&D and biotechnology policies remains competitive, innovative and sustainable? - How should industry respond in order to keep innovating more therapies for rare diseases? - How will the pharmaceutical legislation and EU level changes affect rare diseases regulation, affordability and accessibility to patients? - How can each multi-stakeholder contribute to a more sustainable ecosystem? <p><i>Moderator:</i> Elisabetta Zanon, Director, European Policy and Advocacy, Alliance for Regenerative Medicine Dr Violeta Stoyanova-Beninska, Senior Scientific Specialist, EMA, & Former chair of the COMP Toon Digneffe, Head Public Affairs & Public Policy, Takeda & Chair, EUCOPE Incentives Steering Group and EUCOPE Board member Dr Virginie Bros-Facer, CEO, EURORDIS – Rare Diseases Europe Stelios Kypouropoulos, Former Member of the European Parliament</p>
10:00	Keynote panel: 12 weeks to 2025's EUHTA implementation – are we ready? <ul style="list-style-type: none"> - Are we stuck in the past? Perspectives from the HTA coordination group: the historical structure of healthcare systems and how this needs to change going forward - The role of the EUHTA and the need to consider ability and willingness to pay from individual countries - How will the parallel joint collaboration work between member states? - How prepared is industry? <p><i>Moderator:</i> Sophie Schmitz, Managing Partner, Partners4Access Alexander Natz, Secretary General, EUCOPE Jon Neal, Head Pipeline & Portfolio Europe & Canada, Takeda & EFPIA Patient Access Board-Sponsored Committee Sofie Alverlind, Coordinator and Project Leader, TLV Neil Bertelsen, Patient Engagement Specialist and Board Member, PFMD & Steering Committee Member, HTAi</p>
10:45	Morning Networking Break Roundtables Begin 11:30


10:45	Morning Networking Break				
11:30 – 12:30	Interactive Working Groups:				
Interactive Working Groups					
<p><i>Working Group 1:</i> Theatre 1 Case Study: Delivering on patient centricity by systematically embedding patient insights into the lifecycle Wendy Erler, VP, Head of Patient Experience & Insights, Alexion AstraZeneca Rare Disease Gonzalo de Miquel, VP, Bone Metabolism, Rare Endocrinology and Cell Therapy Area Head, Alexion AstraZeneca Rare Disease</p>	<p><i>Working Group 2:</i> Theatre 2 Shifting Strands: Operational Challenges and Ethical Dilemmas of Genetic Testing Derek Ansel, VP Therapeutic Strategy Lead, Rare Disease, Worldwide Clinical Trials</p>	<p><i>Working Group 3:</i> Theatre 4 Leveraging Expanded Access in Rare Diseases Elizabeth Taylor, Senior Manager, Intelligence and Access Strategy, Clinigen Elaine Murphy, Senior Regulatory Affairs Strategist, Clinigen</p>	<p><i>Working Group 4:</i> Theatre 5 How can we manage the tension between immediate access to innovation and affordability? The future of rare disease policy poses a crucial question Michele Pistollato, Principal, Charles River Associates Charlotte Poon, Life Science Policy and Strategy Consultant, CRA</p>	<p><i>Working Group 5:</i> Theatre 6 Patient Engagement in Clinical Rare Diseases Luca Trentin, Senior Consultant, Alira Health Cristina Montané, Patient Advocate, ACAF Julien Delaye, Patient Engagement Manager – HTA, EURORDIS Iolanda Arbiol Rodríguez, Director, Funadció Dr. Torrent-Farnell</p>	
12:30 – 14:00	Join us in the exhibition hall for: 1-2-1 Partnering, refreshments, poster sessions				
13:30 – 14:00	Poster Presentations				
14:10	Conference Tracks Begin				

	 Theatre 2 Chair	 Theatre 6 Chair Pina Haberl, Senior Director, P4A	 Theatre 4 Chair Victoria Hedley, Rare Disease Policy Manager, Newcastle University	 Theatre 3 Chair Claire Skentelbery, Director General, EuropaBio	 Theatre 5 Chair Samantha Parker, Patient Advocacy and Communication Lead Rare Diseases Europe, Italfarmaco – TBC	 Theatre 1 Chair Daria Julkowska, Scientific Coordinator, European Joint Programme on Rare Diseases, IRDiRC/EJPRD – TBC
	From Drug Development to Trial Design	Policies, Reimbursement & Commercialisation	Evidence Generation	Gene Therapy Innovation & HTA	Newborn Screening and Diagnostics	Showcases

14:10	<p>Bridging the Gap: Clinical Trials as a Standard of Care in Rare Diseases Drew Matheson, Executive Director, Project Management, Rare Disease, Worldwide Clinical Trials</p>	<p>Panel: Acting Now for People Living with Rare Disease: The Aspire4Rare Example Moderator: Sheela Upadhyaya, Life Science Advisor in Rare Diseases & Special Advisor, Former FIPRA & Consultant Johannes Heuckeroth, Patient Advocacy and Public Affairs Lead, Germany, UCB María Peña Díaz Jara, Head of the Social and Health Care and Resources Service, Castilla-la Mancha Regional Government, Spain Vinciane Quidbach, Research Project Manager, Public Health and Policy for the Value of Treatment, European Brain Council Jorge Mestre-Ferrandiz, Associate Professor, Universidad Carlos II de Madrid</p>	<p>RWD for sustainable access and affordability Heather Masters, VP Global Pricing & Market Access, WEP</p>	<p>Launching ATMPs in the Middle East: Lessons learned and the Road Ahead Lavni Varyani, Founding Partner, Pharma BP</p>	<p>Panel: Convergence on rare disease and precision medicine – a real drive on drug development and patient advocacy Moderator: Durhane Wong-Rieger, President & Chief Executive Officer, Canadian Organization For Rare Disorders Sonia Valero, Head of Clinical Operations, Patient Advocacy Leader, Vivet Therapeutics Federica Miotta, Head of Medical Affairs, Fondazione Telethon Rachel Yang, Senior Advisor, Fudan University Zhongshan Hospital Denis Costello, Director, CML Advocates Network Marciek Klein, Global Chief Business Officer, Saventia Global Health</p>	<p>14:10 Showcase 1: Uncovering genetically defined patients for improved diagnosis, drug development and commercial strategy Karen E. Malone, CEO, GeneScape</p>
14:40	<p>Potential for optimal patient access and reimbursement Kyle Clifton, Senior Director, Red Nucleus</p>	<p>14:25: Economic Burden of Rare Disease Michele Pistollato, Principal, Charles River Associates Anne-Sophie Chalandon, Head of Rare Diseases Global Public Affairs Rare Disease and ATMP Policy at Sanofi, and leading the IFPMA Rare Diseases Group</p>	<p>14:25: Economic Burden of Rare Disease Michele Pistollato, Principal, Charles River Associates Anne-Sophie Chalandon, Head of Rare Diseases Global Public Affairs Rare Disease and ATMP Policy at Sanofi, and leading the IFPMA Rare Diseases Group</p>	<p>Innovative approaches to support access to advanced therapies: Multicriteria Decision Analysis Xavier Badia, CEO & Partner, Omakase</p>	<p>14:55 Panel: The next chapter in Newborn screening -Bridging the NBS Gap between LMIC and advanced countries -Bringing together metabolomics and genomics – how and why? Moderator: Senior Representative, Novartis Gene Therapies</p>	<p>14:25 Showcase 2: Azafaros – the latest on our clinical program and our phase 2 data results Stefano Portalano, CEO, Azafaros</p>
						<p>14:40 Showcase 3: Metabolic plasmalogen replacement therapy for a rare peroxisomal disorder Shawn Ritchie, CEO and CSO, Med-Life Discoveries</p>

15:10	<p>Diversity and Equity in Clinical Studies: Impact on Rare Disease Studies Corinne Blanchet, Executive Director, Program Delivery, Premier Research</p>	<p>Challenges of Late onset Rare diseases – recommendations from the COLLABORATE Consensus Paper Aldona Zygmunt, Public Affairs Director, Pfizer Nadiyah Hanim Abdul Latif, President, Malaysia Society for Rare Diseases Sheela Upadhyaya, Life Science Advisor in Rare Diseases & Special Advisor, Former FIPRA & Consultant</p>	<p>14:40 Leveraging Compassionate Use Programs to Accelerate Investigational Drug Development in the United States Ana Tediosi, Head of Expanded Access Program Strategy, WEP Clinical Davelyn Eaves Hood, Senior Director of Medical & Patient Affairs, Rezolute Bio</p> <p>15:10 Rare disease case-finding with the NHS: Learnings and Opportunities Dr Peter Fish, CEO, Mendelian</p>	<p>Roctavian: how NOT to launch a gene therapy Sophie Schmitz, Managing Partner, Partners4Access Olivia Johns, Associate Consultant, Partners4Access</p>	<p>James Bonham, President, International Society for Neonatal Screening Leire Solis, Health Policy and Advocacy Manager, IPOPI Ritu Jain, President, DEBRA Singapore</p>	<p>14:55 Showcase 4: Overcoming Data Silos: The Solution to Actionable Genomic Insights Laura I. Furlong, Co-Founder and Scientific Director, MedBio Informatics</p> <p>15:15 Showcase 5: ExoPTEN-First orphan designated medicinal product containing extracellular vesicles for the treatment of CNS injuries Ina Sarel, Head of CMC, Quality and Regulatory, nurExone</p>
15:40	Afternoon Networking Break					
16:10	<p>Sustainability of lifetime dosing for antisense oligonucleotides in rare diseases: The need for scalable national models Rachel Smith, Executive Director, Global Head of Rare Disease CoE, Parexel</p>	<p>From Complexity to Clarity: Overcoming Reimbursement Challenges for Orphan Drugs Elise Evers, Senior Consultant, HEOR Lead, Initiate Consultants Georgia Roberts, Senior Consultant, HTA Lead, Initiate Consultants</p>	<p>Together for Rare Diseases: Learnings from pilot projects Sheela Upadhyaya, Life Science Advisor in Rare Diseases & Special Advisor, Former FIPRA & Consultant Victoria Hedley, Rare Disease Policy Manager, Newcastle University Danielle Dong, Global Rare Medical Affairs,</p>	<p>Advancing Platform Technologies for RNA therapy development and Regulation for Rare Diseases – The ERDERA approach David Morrow, Senior Scientific Manager for Translational Medicine & Drug Development, EATRIS</p>	<p>Drive for change: paradigm shifts to accelerate rare disease drug development Christopher M de M Rudolf, Founder & CEO, volv global</p>	<p>16:10 Panel: Innovative funding mechanisms and business models in orphan and rare diseases Moderator: Daria Julkowska, Scientific Coordinator, European Joint Programme on Rare Diseases, IRDiRC/EJPRD Laura Rodriguez Gallego, Partner, Invivo Partners Catriona Crombie, Head of Rare Disease, LifeArc Dr James A Levine, President, Foundation Ipsen</p>

			Scientific Advocacy Lead, Sanofi			17:10 End of Track
16:40	<p>Leveraging Natural History Studies for Rare Disease Clinical Trial Design: Supporting the Value Proposition Juliet Mortiz, SVP, Strategic Solutions & Patient Centricity, Ergomed</p>	<p>Leading the Way: Streamlining Commercialisation of Rare Disease Therapies, Supporting Access, and Improving Patient Outcomes Gillian Molloy, Global VP of Market Access, Ascella Health</p>	<p>Panel: Evidence Assessment in rare. Why do we need a change in Paradigm. The work of HTAi RDIG Moderator: Alicia Granados, Head Scientific Advocacy & Insights, Sanofi Monica Ferrie, Director, Advocacy Beyond Borders Sheela Upadhyaya, Life Science Advisor in Rare Diseases & Special Advisor, Former FIPRA & Consultant Farzana Malik, Co-lead, Economic methods, Managing Partner, Cogience Elena Nicod, Director, Dolon</p>	<p>Panel: EU General Pharmaceutical Legislation – Europe’s Biotech Innovation for Rare Diseases in the Balance? -What would be the expected impact of the GPL on biotech innovation and innovators for rare diseases? -What improvements can be made to ensure a sustained flow and growth of biotech innovation for rare diseases? Moderator: Claire Skentelbery, Director General, EuropaBio Adrien Samson, Healthcare Policy Senior Manager, EuropaBio Enrico Piccinini, Senior Vice President, EU & International, Rare Diseases Chiesi Group Jennifer Schranz, SVP, Global Head Rare Diseases, Ipsen Manel Cascallo, General Director, Theriva Biologics</p>	<p>Exploring UAE’s Clinical Research Landscape on Rare Disease Islam Eltantawy, General Manager, IROS</p>	
		<p>16:55 EU’s Rare Opportunity: Navigating the new Joint Clinical Assessment route in Europe Adaeze Eze, Senior Research Consultant Strategic Market Access, OPEN Health Lara Groves, Associate Director, Real-World Evidence, OPEN Health Emanuele Arca, Scientific Office Lead, Strategic Market Access, OPEN Health</p>				
17:10	<p>Lessons learned from the planning and execution of a 5-year prospective natural history study in an ultra-orphan paediatric disorder</p>	<p>SmPC at the Heart of EU JCA: Shaping Decisions, Driving Access Louise Perrault, President & CEO, International Market Access Consulting</p>			<p>RARE-XTRACT: A case study of data enrichment, labelling and value extraction from unstructured Rare Disease Electronic Medical Records leveraging NLP</p>	

	<p>Tara Smith, PhD, Executive VP, Innovative Therapies, Med-Life Discoveries</p>				<p>Ian Rentsch, GM Pharma, Centogene</p>		
		<p>MoCA as an alternative as an EU HTA Scientific Consultation Šárka Kubinová, Medical Assessor, Section of Pricing and Reimbursement Regulation, State Institute for Drug Control (SUKL)</p>					
17:40	End of sessions						
19:00 – 21:00	<p>Networking Party <i>Sponsored by Alexion AstraZeneca Rare Disease</i> Join us at the Museu Nacional d'Art de Catalunya for stunning terrace views</p>						
							

***NEW: Company Presentations - Exhibition Stage

Highlighting the different rare disease programs that companies around the world have developed. Understand what are the current ongoing efforts are, available capabilities and why you should consider partnering with them.

15:40 Children born with Osteogenesis Imperfecta: building healthy bones through cell therapy Evert Koppers, CEO, Boost Pharma
15:50 What kind of data/assets can patient groups bring into pre-clinical or clinical development? Ron Jortner, Founder/CEO, Aspire Biosciences
16:00 How a patient organisation gave birth to a biotech company for CCHS Xenia Proton de la Chapelle, CEO, AtmosR
16:10 InGenuTy® - Transforming Rare Respiratory Disease Outcomes Using Inhaled Gene Therapy David Hipkiss, Executive Chair and Director, AlveoGene
16:20 Innovating for the uncommon: Almirall Commitment to Rare Skin Diseases Anna Planagumà, Associate Director, Licensing, Almirall
16:30 Data-centric drug discovery for Rare Diseases – Title TBC Jordi Carrera-Puigvert, CSO, Phenaros Pharmaceuticals
16:40 <i>Start-up pitch:</i> Cellbyte - AI in rare disease P&MA: How to enhance your launch strategy with Cellbyte Daniel Vidal Moreira, Co-Founder, Cellbyte GmbH
16:45 <i>Start-up pitch:</i> Ablevia - Injectable Decoys for the Selective and Rapid Removal of Anti-Drug and Anti-Vector Antibodies Oskar Smrzka, CEO, Ablevia Biotech GmbH
16:50 <i>Start-up pitch:</i> Red Thread - Title TBC Nick Leach, Founder, Red Thread Market Access Ltd
16:55 <i>Start-up pitch:</i>
17:00 End of company presentations
17:40 Close of Congress Day
Sponsored Off-site Networking Drinks

Day Two - Thursday 24 October

OPENING KEYNOTE PLENARY Room: Auditorium					
09:00	<p>Keynote Panel: The Rare Disease Action Plan – where are we and how to integrate this from a European to a national level for real impact?</p> <ul style="list-style-type: none"> - What have we learnt from the last presidencies – how does the future of rare diseases look with Belgium and now Hungary’s leadership? - How is each country executing their national action plan and are they involving their local policy makers? <p><i>Moderator: Alexandra Heumber Perry, CEO, RDI</i> Dr Virginie Bros-Facer, CEO, EURORDIS – Rare Diseases Europe Fabienne Bartoli, Inspector General, French Ministry of Health Daniel de Vicente, Board Member, FEDER, President, Association of Patients with ASMD Anne-Sophie Chalandon, Head of Rare Diseases Global Public Affairs Rare Disease and ATMP Policy at Sanofi, and leading the IFPMA Rare Diseases Group Gabriella Almberg, Global Head of Rare Disease Policy & Public Affairs, UCB Flaminia Macchia, Head Global Rare Disease Policy, Alexion AstraZeneca Rare Diseases</p>				
09:45	<p>Keynote panel: How much weight does the patient voice carry when it comes to influencing clinical trials, policy and orphan drug approvals?</p> <ul style="list-style-type: none"> - Are the current policy and regulatory frameworks helping or hindering patient access to medicines? - How can clinicians and healthcare professionals help in developing clinical trials and regulatory pathways? - How has the way we develop orphan drugs evolved over time (from policy, technology to engagement with patients?) <p><i>Moderator: João Duarte, VP, Regulatory Excellence, Ipsen</i> Stelios Kypouropoulos, Former Member of the European Parliament Tim Leest, Chair of Committee for Orphan Medicinal Products EMA & Federal Agency for Medicines and Health Products (FAMHP) Dr Manuel Toledo Argany, Epileptologist, Vall d’Hebron Barcelona Hospital Ella Fitzpatrick, Senior Public Involvement Adviser, NICE</p>				
10:30	Morning Networking Break; Tracks begin 11:10				
 Theatre 2 Chair Ron Jortner, Founder/CEO, Aspire Biosciences	 Theatre 6 Chair Andrew Mumford, CEO, Initiate Consulting	 Theatre 4 Chair Stefano Romanelli, Senior Government Affairs Manager, EUCOPE	 Theatre 3 Chair Dan O’Connor, Director, Regulatory Policy & Early Access, ABPI	 Theatre 5 Chair José Ángel Aibar, President, Dravet Syndrome Foundation Spain	 Theatre 1 Chair: Matt Bolz-Johnson, Mental Health Lead & Healthcare Advisor, EURORDIS - TBC

	ClinicalDevelopment <small>Children Drugs</small>	Access&Pricing <small>Children Drugs</small>	RealWorldEvidence	ScienceStrategy <small>Children Drugs</small>	PrecisionMedicine <small>Children Drugs</small>	PatientCentricity
	Chair Theatre 2 Ron Jortner , Founder/CEO, Aspire Biosciences	Chair Theatre 6 Andrew Mumford , CEO, Initiate Consulting	Chair Theatre 4 Stefano Romanelli , Senior Government Affairs Manager, EUCOPE	Chair Theatre 3 Dan O'Connor , Director, Regulatory Policy & Early Access, ABPI	Chair Theatre 5 José Ángel Aibar , President, Dravet Syndrome Foundation Spain	Chair Theatre 1 Matt Bolz-Johnson , Mental Health Lead & Healthcare Advisor, EURORDIS - TBC
	Clinical Trials and Regulation	Value & HTA Pathways	Real World Data & Advocacy	Sustainable Global Orphan Markets	Advanced Therapies for Rare Diseases	Global Initiatives and Patient Inclusion
11:10	Practical Approaches to Including Patient Advocacy Organizations in Your Drug Development Program: Not Only Why, But How Bojana Miroslavljevic , Director, Patient Advocacy Strategy, ICON Meghan Morgan-Smith , Director, Centre for Rare Diseases, ICON	Panel: Strategic Evidence Generation – Exploring Multiple Stakeholder Needs Moderator: Mariam Bibi , Practice Lead, Remap consulting Daniel de Vicente , Board Member, FEDER , President, Association of Patients with ASMD Andrew Olaye , Head of EMEA Market Access, Orchard Therapeutics Josie Godfrey , Rare Diseases, Market Access, JG Zebra Consulting Ruth Pulikottil Jacob , EMEA HEOR Lead, Cepheid	Unlocking Market Potential: Strategic Use of Real-World Data Collected During Managed Access Programs in HTA Submissions Dr Hasan Kobat , Global Business Development Lead, Real World Data, Clinigen Susanne Michel , VP Market Access, Clinigen Michael Epstein , VP & Head, Pricing and Commercial Strategy, Clinigen	Panel: What are the impacts of rare designation? Moderator: Dan O'Connor , Director, Regulatory Policy & Early Access, ABPI Durhane Wong-Rieger , President & Chief Executive Officer, Canadian Organization For Rare Disorders Frauke Naumann-Winter , Committee for Orphan Medicinal Products, Institute for Drugs and Medical Devices Jonathan Kornstein , VP, Rare Disease and Pediatrics, Caidya	Panel: Accelerating rare disease diagnosis beyond birth -Genetic newborn screening and digital technologies Moderator: Colin O'Donnell , Head of EUCAN & International Govt. Affairs, Alexion AstraZeneca Rare Disease Karen Heim , General Manager, Alexion AstraZeneca Rare Disease Stefano Portalano , CEO, Azafaros Maurizio Scarpa , Director, Coordinating Center For Rare Diseases, MetabERN Rachel Yang , Senior Advisor, Fudan University Zhongshan Hospital Julian Isla , Director, Foundation 29	Panel: The value of patient involvement: combating stigma, discrimination and celebrating strengths Debra Bellon , Strategic Engagement Manager, RDI Kirsten Johnson , President, Fragile X , Board Director, EURORDIS Association Nadiah Hanim Abdul Latif , President, Malaysia Society for Rare Diseases Felix Galarza , President, FERPOF Ecuador Ritu Jain , President, DEBRA Singapore The growing influence of Patient Voices in Rare Epilepsies Andrea Wilkinson , Global Head of Epilepsy
11:40	Diversifying Rare Disease Research: Strategies for Expanding Participation					

	<p>Almenia Garvey, Senior Director, Global Feasibility and Site Engagement, Allucent</p> <p>Begoña Nafria Escalera, Patient Engagement in Research Coordinator, SJD Children’s Hospital</p>	<p>11:55 Panel: Optimising Value Demonstration & Evidence in Rare Disease</p> <p>Moderator: Sue O’Leary, Executive VP, Evidence and Pricing, Prime</p> <p>Stuart Mealing, Associate Director, York Health Economics Consortium</p> <p>Simon Shohet, VP International Market Access & Global HEOR, Amicus Therapeutics</p> <p>Jennifer Quinn, Head of Global Value and Access, Debiopharm International</p> <p>Mukesh Sharma, Head of Early Pipeline & Business Development, Alexion Pharmaceuticals</p>	<p>11:55 Navigating the Uncharted: Patient Experience Mapping in Rare Disease</p> <p>Alexander Artyomenko, Direct Global Rare Disease Patient Affairs, Ipsen</p>		<p>11:55 Applying AI system for Diagnosing Mendelian Disorders</p> <p>Maurizio Scarpa, Director, Coordinating Center For Rare Diseases, MetabERN</p>	<p>& Rare Syndromes Patient Engagement, UCB</p> <p>Lesley Perkin, UK Patient Engagement and Policy Lead, UCB</p> <p>Lindsay Randall, Founder & CEO, Arthur’s Quest/SLCA6A1 Connect UK</p> <p>Allison Watson, CEO, Ring20 Research & Support UK CIO</p>
12:10	<p>Unique Challenges in Recruiting Patients for Rare Indication Pediatric Studies: Overcoming Difficulties through a Site and CRO Perspective</p> <p>Rachel Abu Taleb, Medical Affairs Specialist, Veristat</p> <p>Joana Claverol Torres, Clinical Research Director, Sant Joan de Déu Research Foundation</p>		<p>12:25 Strengthening Access to Medicines for Rare Diseases – Title TBC</p> <p>Sofie Alverlind, Coordinator and Project Leader, TLV</p>	<p>Leaving no one behind: UNICAS as a case study to support Health Equity in Spain</p> <p>Moderator: Flaminia Macchia, Head Global Rare Disease Policy, Alexion AstraZeneca Rare Diseases</p> <p>Leticia Beleta, VP and General Manager, Alexion AstraZeneca Rare Disease Barcelona</p> <p>Encarna Guillén, UNICAS</p> <p>Jordi Cruz, FEDER</p>	<p>Messenger RNA is a promising treatment for inborn errors of metabolic disorders</p> <p>Rena Baek, Senior Director – Rare Diseases, Moderna</p>	<p>The Rare Barometer Programme: the survey initiative of EURORDIS- Rare Diseases Europe</p> <p>Rita Francisco, Rare Barometer Survey Manager, EURORDIS</p>
12:40 – 14:10	<p>Join us in the exhibition hall for:</p> <p>Poster Presentations</p> <p>1-2-1 Partnering</p> <p>Tracks restart at 14:10</p>					
12:50 – 13:50	<p>Interactive Working Groups</p>					





	<p><i>Working Group 6:</i> Theatre 5 EUJHTA: Discussion on the practical steps to take with 70 days to implementation Sophie Schmitz, Managing Partner, Partners4Access Darren Callanan, Global Digital Lead, Partners4Access</p>	<p><i>Working Group 7:</i> Theatre 1 Use of AI – Expectations vs Reality in small data sets/populations Estelle Michael, Policy and Public Affairs Lead, UCB Ana Rath, Director, Orphanet/INSERM Bruno Sarfati, CEO, Tekkare</p>	<p><i>Working Group 8:</i> Theatre 6 RD Funding models Annamaria Merico, Head of Business Development, Fondazione Telethon</p>
14:10	Tracks restart at 14:10		

14:10	<p>Breaking Down Barriers: Leveraging Direct to Patient, Direct from Patient, and Home Healthcare in Rare Disease Studies Robin Marcus, Head of Global Decentralized Trials Market Development, Marken</p>	<p>Maximizing Value & Positioning in the Emerging Competitive Rare Disease Arena Owen Male, Senior Associate, CRA Bhavesh Patel, Principal – Life Science Strategy, CRA</p>	<p>Unlocking Imaging Biomarkers in Rare Lung Disease Through Curated Real-World Data Elizabeth Estes, Executive Director, OSIC</p> <p>14:25 Out of the box toolkit – Legal tools for patient advocacy Adrian Goretzki, President, Healthcare Education Institute</p>	<p>Panel: Interconnected Solutions: Global Networks for Rare Disease Research and Care -How do we speed up diagnosis? -Rare disease centre of excellence Mary Wang, Global Programme Director, RDI Ulrike Schwerdfeger, Legal Technical Officer, WHO David Pearce, Chair, IRDIRC Helene Cederroth, Founder, Whilem Foundation</p>	<p>Panel: Focusing on patient uptake of rare disease treatments -Patient, HCPs, clinician perspectives -how do we involve all voices in the clinical development process? Moderator: Josie Godfrey, Rare Diseases, Market Access, JG Zebra Consulting Emma Eatwell, Global Practice Director, Global Council Fleur Chandler, Head of Market Access UK and Ireland, Sanofi & Parent Board Advisor, Duchenne UK Francis Pang, SVP, Global Market Access & Geographic expansion, Orchard Therapeutics Anna Gilmore, Director of Patient Engagement, FSDH Society</p>	<p>Panel: From global to local: partnering for patient centered policies beyond Europe Moderator: Durhane Wong-Rieger, President and Chief Executive Officer, Canadian Organization For Rare Disorders Antoine Daher, President, Casa Hunter Rachel Yang, Senior Advisor, Fudan University Zhongshan Hospital Flaminia Macchia, Head Global Rare Disease Policy, Alexion AstraZeneca Rare Diseases Roser Francisco, Coordinator of Hospital Rare Diseases Units, SJD Hospital Barcelona</p>
14:40	<p>Panel: The Role of Basket & Umbrella Clinical Trials in Tackling Rare Diseases Milan Marinkov, TA Medical Lead, Allucent Genevieve Wills, Associate Director, Biostatistical Consulting, Allucent</p>	<p>Panel: Access and HTA pathways for OMPs – do they improve access and drive incentive for innovation? <i>Multiple perspectives across European countries</i> Moderator: Lisa Marsden, SVP, Market Access, Ipsen Nick Meade, Director of Policy, Genetic Alliance UK</p>	<p>Panel: Public-Private Partnerships to accelerate development and boost innovation in Rare Diseases Moderator: Victor Maertens, Government Affairs Director, EUCOPE Sheela Upadhyaya, Life Science Advisor in</p>	<p>Panel: How does access differ across Europe -How do we reinvent the healthcare system to meet the demand of rare diseases? Moderator: Samantha Parker, Patient Advocacy and Communication Lead Rare Diseases Europe, Italfarmaco</p>		





	Mila Zaballos , Senior Manager Clinical Operations, Allucent	David Kolar , Executive Director, AIFP Elena Nicod , Director, Dolon	Rare Diseases & Special Advisor, FIPRA Avril Daly , President, EURORDIS-Rare Diseases Europe Rima Nabbout , Professor Paediatric Neurology, University Paris Descartes Gabriella Almberg , Global Head of Rare Disease Policy & Public Affairs, UCB	Maria Judith Molnar , Professor, EURO-NMD Viviana Giannuzzi , Head of Department, Fondazione Gianni Benzi Inês Alves , Patient Representative, ERN BOND		
15:25	Patients voice in clinical trials design and outcomes Samantha Parker , Patient Advocacy and Communication Lead Rare Diseases Europe, Italfarmaco				Panel: Preparing the patient community for advanced therapy trials: How stakeholders can work together to ensure advanced therapy clinical trials are optimal for everyone Moderator: José Ángel Aibar , President, Dravet Syndrome Foundation Spain Emma James , VP Medical & Patient Affairs, Encoded Therapeutics Marisol Montolio , Director of Research & Technology, Duchenne Parent Project Spain Dr Manuel Toledo Argany , Epileptologist, Vall d’Hebron Barcelomona Hospital	14:55 Celebrating 5 years of EDS ECHO – Education, Empowering and Expanding Horizons Lara Bloom , CEO, Ehlers-Danlos Foundation
15:55		Joint Clinical Assessment: Aspirations and Implications for rare disease and oncology market access Emilie Neez , Associate Director, Dolon	15:25 Delivering Strategy, Advocacy & Partnering for Orphan Drugs in Spain Moderator: Alicia Gil , Partner & CEO, Omakase Dr Cesar Hernandez , General Pharmacy Director, Spanish Ministry of Health Gloria M. Palomo Carrasco , Head of Service, Advanced Therapies and Vaccines Area, Spanish Medicines Agency	15:25 Panel: Creating Pathways to meet opportunities and challenges of ultra-rare diseases Moderator: Durhane Wong-Rieger , President And Chief Executive Officer, Canadian Organization For Rare Disorders Parvathy Raman , Founder, Krishnan Family Foundation Rachel Yang , Senior Advisor, Fudan University Zhongshan Hospital		15:10 Mapping Rare – a world of rare disease advocacy Debra Bellon , Strategic Engagement Manager, RDI
						15:40 Pathways Telehealth Nurse Service for rare and genetic conditions Kate Holliday , Chief Executive, CCDR

			<p>Manel Fontanet Sacristan, CATSALUT Dr Mónica Climente Marti, Head of Pharmacy Service, Hospital Universitario Doctor Pesset (Valencia) Arantxa Sancho, Director of the Medical-Scientific Department, Farmaindustria Daniel Anibal Garcia, President, FEDHEMO</p>	<p>Nadiah Hanim Abdul Latif, President, Malaysia Society for Rare Diseases Paolo Morgese, Head of Public Affairs, Europe, Alliance for Regenerative Medicine</p>		
16:10 - 16:30	Comfort break					
	Closing Plenary Keynote					
16:30 – 17:30	<p>Keynote: How do we reinvent the healthcare system to meet the demand of not only rare diseases, but potentially all patients in future?</p> <ul style="list-style-type: none"> - By 2025 we will have many more gene therapies/ATMPs in development/approved. With this future healthcare systems cannot have bespoke solutions for each new ATMP, how can industry work with the healthcare system to build models of care that are effective and sustainable - How can industry create solutions facilitate patient access to gene therapies? - How can companies and the healthcare system work together to make it more equitable? - What role can clinicians and patients play in developing a system that is efficient and delivers the outcomes expected? <p><i>Moderator: Sheela Upadhyaya, Life Science Advisor in Rare Diseases & Special Advisor, FIPRA & Consultant</i> Monica Ines, HEOR Team Leader, Hemophilia, Pfizer Rachel Yang, Senior Advisor, Fudan University Zhongshan Hospital Antoine Daher, President, Casa Hunter Matteo Scarabelli, Associate Director Market Access HTA, EFPIA</p>					
17:30	Chair’s closing remarks and End of Day 2					
18:00	Evening Networking in the Exhibition Hall					

Day Three – Friday 25 October

	Morning Networking in the Exhibition Give yourself time to check-out and attend the morning session on-time!				
	OPENING PLENARY SESSION Keynote Theatre				
09:00 – 9:45	Keynote Panel: How to create a sustainable and robust pipeline for rare diseases and gene therapies <ul style="list-style-type: none"> - What does it take to be successful in Europe? - How will gene therapies impact future pipelines? - What are the remaining commercial and post market evidence challenges? - How will the regulatory science adapt to our innovative medicines? <p><i>Moderator: Paolo Morgese, Head of Public Affairs, Europe, Alliance for Regenerative Medicine</i> <i>Victor Maertens, Government Affairs Director, EUCOPE</i> <i>Francis Pang, SVP, Global Market Access & Geographic expansion, Orchard Therapeutics</i> <i>Daria Julkowska, Scientific Coordinator, European Joint Programme on Rare Diseases, IRDiRC/EJPRD</i> <i>Tom Butt, Senior Director, Health Economics & Outcomes Research, BioMarin</i></p>				
9:45 – 10:30	Keynote Panel: The use of RWD in orphan drug development and access pathways <ul style="list-style-type: none"> - Where does real world data fit in the evidence hierarchy for developing orphan medicines? - What are the opportunities and challenges for using real world data generated from rare disease patients? - How can innovative approaches help to integrate real world evidence in supporting effective decision making by different stakeholders? - Is there good alignment between decision makers on the value of real-world data? <p><i>Moderator: Dan O'Connor, Director, Regulatory Policy & Early Access, ABPI</i> <i>Alison Cave, Chief Safety Officer, MHRA</i> <i>Frauke Naumann-Winter, Committee for Orphan Medicinal Products, Institute for Drugs and Medical Devices</i> <i>Dr Annika Jodicke, Senior Pharmacoepidemiologist, NDORMS</i> <i>Sicily Mburu, Scientific Officer, IFPA</i></p>				
10:30 – 10:50	Comfort Break - Tracks start at 10:50				
	 Clinical Development <small>Orphan Drugs</small>	 Access & Pricing <small>Orphan Drugs</small>	 Cell & Gene Therapy <small>Orphan Drugs</small>	 Science Strategy <small>Orphan Drugs</small>	PatientCentricity
	Chair Theatre 2	Chair Theatre 6	Chair Theatre 3	Chair Theatre 4 Mary Wang, Global Programme Director, RDI	Chair Theatre 1

	Orphan Drug Innovation & Commercialisation	Creating Value & RWD	ATMPs & Manufacturing	International strategies & HTA	Mental Health Initiatives & Patient inclusion
10:50	<p>Title TBC</p> <p>Senior representative, Conect4Children</p>	<p>Challenges for defining value in Rare Diseases: the case for Duchenne Muscular Dystrophy (DMD)</p> <p>Ross Selby, Value, Access & External Affairs Director, Italfarmaco</p>	<p>Track Begins at 11:35 AM</p>	<p>Panel: Equitable genomic data principles: leveraging NGS technologies for global health equity</p> <p>Mary Wang, Global Programme Director, RDI</p> <p>Monica Ferrie, Director, Advocacy Beyond Borders</p> <p>Trudy Nyakambangwa, Founder, Child and Youth Care Zimbabwe</p>	<p>Panel: Impact of rare disease on mental health and wellbeing - building understanding of the link and impact of rare and complex diseases on mental health and improving outcomes</p> <p>Moderator: Matt Bolz-Johnson, Mental Health Lead & Healthcare Advisor, EURORDIS</p> <p>Kirsten Johson, President, Fragile X, Board Director, EURORDIS</p> <p>Lauren Roberts, Head of Development, Rareminds</p> <p>David Rintell, SVP Head of Patient Advocacy, BridgeBio</p> <p>Cristian Perez, Director of International Patient Affairs, Blueprint Medicines</p>
11:20	<p>Fostering the innovative methodological approaches in clinical trials</p> <p>Rima Nabbout, Professor Paediatric Neurology, University Paris Descartes</p>	<p>Panel: A New Beginning? The EU Biotechnology and Biomanufacturing Initiative Opportunities for Investment into Rare Disease Innovation and delivery</p>		<p>11:35 Panel: How to implement GT/ATMPs into the healthcare system, they can't make a bespoke model each time</p> <p>Moderator: Victor Maertens, Government Affairs Director, EUCOPE</p>	<p>11:35 Panel: How to create value of patient input to HTA</p> <p>Moderator: Durhane Wong-Rieger, President & Chief Executive Officer, Canadian Organization For Rare Disorders</p> <p>Alexander Natz, Secretary General, EUCOPE</p> <p>Diego Fernando Gil Cardozo, President, FECOER, Interim President, ERCAL</p> <p>Tomasz Kluszczynski, Strategy Consultant & Founder, ACESO Healthcare</p>
11:50	<p>Ethics and regulatory guidance for not-for-profit research for rare diseases: the experience of a European funded programme</p> <p>Viviana Giannuzzi, Head of Department, Fondazione Gianni Benzi</p>	<p>-What are the challenges faced by the EU investment ecosystem in rare disease innovation to market?</p> <p>-How does legislation impact investment decisions into innovation?</p> <p>-How can the EU Biotech and Biomanufacturing Initiative help</p>			

		<p>support investment opportunities into rare disease innovation?</p> <p>Moderator: Adrien Samson, Healthcare Policy Senior Manager, EuropaBio</p> <p>Claire Skentelbery, Director General, EuropaBio</p> <p>Thomas Bols, Head of Government Affairs and Public Policy, EMEA & APAC, PTC Therapeutics</p> <p>Natividad Calvente, Corporate Affairs Head Spain, Novartis</p>	<p>Matt Bolz-Johnson, Mental Health Lead & Healthcare Advisor, EURORDIS</p> <p>Andrew Olaye, Head of EMEA Market Access, Orchard Therapeutics</p> <p>Stefan Neudoefer, Head Market Access & Pricing Europe, CSL</p>		<p>Lindsay Birrell, co-CEO, Realise Advocacy</p>
12:20	<p>Networking Break</p> <p>Join us in the exhibition hall for:</p> <p>Poster Presentations</p> <p>1-2-1 Partnering</p> <p>Tracks restart at 13:40</p>				
					PatientCentricity
	Theatre 2	Theatre 6	Theatre 3	Theatre 4	Theatre 1
13:40	<p>Use of medical devices in trials – how do they change the way we receive data and the patient experience?</p> <p>Anneliene Jonker, Rare Disease Therapy Researcher, University of Twente</p>	<p>Panel: Collecting Real World Data that is useable as RWE</p> <p>Challenges of standardization and generalizability, validity, reliability and analysis</p> <p>Moderator: Durhane Wong-Rieger, President & Chief Executive Officer, Canadian Organization For Rare Disorders</p> <p>Monica Ferrie, Director, Advocacy Beyond Borders, CEO, Genetic Support Network of Victoria</p> <p>Parvathy Raman, Founder, Krishnan Family</p>	<p>Panel: Join together to optimize ATMP development pathways in the EU</p> <p>Matt Bolz-Johnson, Mental Health Lead & Healthcare Advisor, EURORDIS</p> <p>Silvia Martin Llusma, Associate Professor, Universidad CEU San Pablo</p> <p>Johan van Eldere, Academic Development Lead, University Hospitals Leuven & European University Hospital Alliance</p> <p>Adrien Samson, Healthcare Policy Senior Manager, EuropaBio</p>	<p>Panel: From evidence to practice in Rare Diseases: ERN Guidelines Programme and Patient Involvement</p> <p>-Methodical CPG development approach in Rare Diseases</p> <p>-AETSA guidelines program</p> <p>-Worldwide guidelines and evidence generation</p> <p>Moderator: Dr Beatriz Carmona Hidalgo, Senior Researcher, Junta de Andalucía</p> <p>Anabel Granja Dominguez, Researcher, Junta de Andalucía</p>	<p>The Five Principles of Self-Advocacy & Your Mental Health Toolbox</p> <p>Lauren Kopsick, Founder and Executive Director, The Healthcare Navigation Project</p>
14:10	<p>Truly Patient Centric trials – Optimizing submission to regulatory</p> <p>Bob Stevens, CEO, MPS Society</p>				<p>Supporting rare disease patient advocacy and awareness: the global challenge</p> <p>Manzi Mdamukunze, Founder, Centre Alliance Rwanda</p>

		<p>Christian Henriksz, Medical & Strategic Advisor, A Rare Cause David Duro, VP, Lyfegen Healthcare AG Peter Fish, CEO, Mendelian</p>		<p>Juan Darío Ortigoza-Escobar, Pediatric Neurologist, Sant Joan de Déu Hospital Barcelona Alicia Granados, Head Scientific Advocacy & Insights, Sanofi</p>	<p>14:25 Patients as Partners: Driving Change Together Toni Roberts, Chairman, Debra South Africa</p>
14:40	<p align="center">Close of Congress Thank you for attending!</p>				