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

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For speaking opportunities in 202, please contact Abdu.Kauroo@terrapinn.com & Wing-yun.Cheung@terrapinn.com

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

10:30 – 13:00 CET	***How to create a compelling business case for orphan drug development?***	***The Rare Disease Action Plan – How will we implement this?***
	 • How can we de-risk our efforts in orphan drug development, and change this situation? • How do we create opportunities out of the unmet need for most rare diseases? • By changing perspectives on rare disease and orphan drug development, we believe we can positively impact many more patients' lives. The panel debate will highlight the patient and societal needs we can uncover that are driving the need for industry change. We will cover factors that impede the business case or introduce risk to orphan drug development. **Workshop leaders: Christopher M de M Rudolf,** Founder & CEO, **volv global** **Léon van Wouwe,** Clinical Innovation Director**, volv global**	*Reserved for Sanofi* **10:30** *Workshop leader introduction:* **Anne-Sophie Chalandon**, Head of Rare Diseases Global Public Affairs Rare Disease and ATMP Policy at **Sanofi**, and leading the **IFPMA** Rare Diseases Group To invite: **Acko Ankarberg Johansson,** Minister for Healthcare, **Swedish Ministry of Health** **Dr Anne-Sophie Lapointe,** Rare Disease Project Manager, **Ministry of Solidarities and Health France**
13:00 – 14:00	*Networking Lunch Break* *The workshops restart at 14:00*	
14:00 - 16:30 CET	*14:00 Reducing the Burden of Rare Diseases: How increasing access to and availability of therapies creates a positive value for society* -How can we used data to successfully demonstrate the positive value that rare disease therapies bring to society and systems? -Why it's important to increase investments in R&D and screening for patients -How should the update of the current pharma legislation and a European Rare Disease plan be shaped in order to find the right balance between pharmaceutical innovation, access, affordability, and patient safety?	*14:00 – 16:00 UK Early Access to Medicines Scheme: Regulatory Process, Practical Implementation & Ethical Considerations - Title TBC*
	Hosted by 	*Hosted by* 
16:30	**Workshops end, Congress begins tomorrow 08:30 at the Fira Barcelona, Montjuic in hall 2**	

Day One *W*ednesday 23 October

	OPENING KEYNOTE PLENARY Location: Keynote Theatre, Hall 2
08:30	**Opening remarks** **Senior Representative, Alexion AstraZeneca Rare Disease**
08:40	**Opening keynote address**: **How will the current OMP amendments effect orphan drug development and patient access?** - How will revisions impact the development and availability of products for people living with rare diseases? - Do we now have the right incentives in place to stimulate the market, looking at areas of unmet need beyond cancer, alimentary conditions and metabolic disorders, which make up a large percentage of orphan medicines - Does the Pharmaceutical regulations address unmet medical need, provide a better economic balance and affordability/accessibility? **Reserved for the European Commission DG SANTE–** *pending elections*
09:00	**Keynote panel: What are the key potential benefits and challenges to be addressed in the European Commission's proposal – a multi-stakeholders' perspective** - How should industry respond in order to keep innovating more therapies for rare diseases? - How will the pharmaceutical legislation affect R&D in rare diseases, regulation, affordability and accessibility to patients? - How should each multi-stakeholder interpret and act upon the proposal and changes in order to gain a more sustainable ecosystem? - How can we boost research and development to tackle rare diseases and develop orphan medicines? **Dr Violeta Stoyanova-Beninska,** Chair of Committee for Orphan Medical Products, **EMA,** Policy, Regulatory and Internation Affairs, **CBG-MEB** **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 -** TBC *To invite:* **MEP / European Parliament perspective –** *pending elections* *Invited:* **Christina Kyriakopoulou**, Senior Policy Officer, **European Commission** – TBC
09:50	**Keynote panel: 12 weeks to 2025's EUHTA implementation – are we ready?** - 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? What are the effects of the EU Pharma package review? **Yann Le Cam,** Co-founder**, EURORDIS – Rare Diseases Europe** **Marcus Guardian**, General Manager, **IHSI,** Partner/Head, **Dierks + Company,** & former Chief Operating Officer, **EUnetHTA** *invited:* **Judith Fernandez,** Deputy Director, HTA department, **Haute Autorité de Santé**
10:45	**Morning Networking Break** **Roundtables Begin 11:30**

10:45	Morning Networking Break				
11:30 – 12:30	Interactive Roundtables:				
Interactive Working Groups					
Working Group 1: Theatre 1 **Alexion**	**Working Group 2:** Theatre 2 **Worldwide Clinical Trials**	**Working Group 3:** Theatre 3 **Clinigen**	**Working Group 4:** Theatre 4 **Volv**	**Working Group 5:** Theatre 5 **CRA**	**Working Group 6:** Theatre 6 **Alira Health**
12:30 – 14:00	**Join us in the exhibition hall for: 1-2-1 Partnering, refreshments, poster sessions**				
13:00 – 13:35	**13:00 – 13:35 Start-up pitches in Theatre 1**				
13:30 – 14:00	**Poster Presentations**				
14:10	**Conference Tracks Begin**				

	ClinicalDevelopment	Access&Pricing	RealWorldEvidence	Cell&GeneTherapy	PrecisionMedicine	TechnologyShowcase
	Chair opening remarks Theatre 2	**Chair opening remarks Theatre 6**	**Chair opening remarks Theatre 4**	**Chair opening remarks Theatre 3**	**Chair opening remarks Theatre 5**	**Chair opening remarks Theatre 1**
	From Drug Development to Trial Design		**Real World Evidence**		**Newborn Screening and Diagnostics**	
14:10	**Leveraging Genetics to Support Rare Disease Clinical Trials** **Senior Representative, Worldwide Clinical Trials**	**Rare diseases: a priority for the Spanish EU presidency – advances at national, regional and hospital level** **Alicia Gil,** Partner & CEO, **Omakase**	**15 mins: Title TBC** **Senior Representative Ergomed**	**What can we learn from past breakthrough therapies?** -how did we scale up and learn from these therapies? *Moderator:* **Anne-Sophie Chalandon**, Head of Rare Diseases Global Public Affairs Rare Disease and ATMP Policy at **Sanofi**, and leading the **IFPMA** Rare Diseases Group	**Convergence on rare disease and precision medicine – a real drive on drug development** **Durhane Wong-Rieger,** President & Chief Executive Officer**, Canadian Organization For Rare Disorders**	14:10 Showcase 1: **Title TBC** **Karen Malone,** CEO, **GeneScape** 14:25 Showcase 2:

14:40	**Minimizing patient burden through decentralised trial elements: Feasibility and Planning in a Global Rare Disease landscape** **Senior Representative, Premier Research**		**15 mins** **Senior Representative, Cascador Health**	**How to implement GT/ATMPs into the healthcare system, they can't make a bespoke model each time** *Moderator*: **Sheela Upadhyaya**, Life Science Advisor in Rare Diseases & Special Advisor, **FIPRA**	**Panel: The next chapter in Newborn screening** *Moderator:* **Charlotte Chanson,** Director, Global Diagnostics & Newborn Screening, **Orchard Therapeutics** *To invite:* **James Bonham**, President, **International Society for Neonatal Screening** **David Bick,** Principal Clinician, Newborn Genomes Programme, **Genomics England** **Maurizio Scarpa,** Director, Coordinating Center For Rare Diseases, **metabERN** **Virginie Bros-Facer,** CEO, **EURODIS**	14:55 Showcase 3:
15:10	**Using AI to find new orphan drug targets and patients** **Reserved, Red Nucleus**	**National Early Access Schemes for Gene Therapies** **Senior Representative, Pfizer**	**Art of the possible – create a winning Market Access Strategy** **Senior Representative, WEP Clinical**			15:10 Showcase 4: 15:25 Showcase 5:
15:40	**Afternoon Networking Break**					
16:10	**What can we learn across European models for early access?** **Senior Representative, Clinigen**	**Do early access programs always create value?** **Senior Representative, Partners4Access**	**Panel: Compassionate Use in the EU: Challenges and the Path to Harmonization** **Senior Representative, myTomorrows**	**Rethinking gene therapy clinical trials to meet the needs of HTA authorities and regulators**	**Drive for change in orphan drug development** **Christopher M de M Rudolf,** Founder & CEO, **volv global**	16:10 Showcase 6: 16:30 Showcase 7:
16:40	**First-of-a-kind study designs to accelerate rare disease drug development** **Senior Representative, Parexel**	**Panel: Title TBC** **Senior Representative UCB**	**Panel: Collecting Real World Data that is useable as RWE** Challenges of standardization and generalizability, validty, reliability and analysis **Moderated by Durhane Wong-Rieger,** President & Chief Executive	**The secret to convincing payers of cell and gene therapy's benefits**	**Accelerating rare disease diagnosis** Genetic newborn screening and digital technologies	16:45 Showcase 8:

			Officer, **Canadian Organization For Rare Disorders**			17:00 Showcase 9:
17:10	**Adjusting clinical development challenges for children in rare diseases** **Senior Representative, Allucent**	**Rare HTA – Payer advice in rare disease, the formal versus informal advice conundrum** **Senior Representative, Initiate Consultancy**		**Rare diseases: What factors in HTA and US payer decision making for curative vs. not curative treatments?** **Senior Representative, Ascenian**		17:15 Showcase 10:
17:40	**End of sessions**					
17:40 – 19:00	**Evening Networking in the Exhibition Hall** End of day 1					

Day Two - *Thursday 24 October*

	OPENING KEYNOTE PLENARY Room: Auditorium
08:35	**Opening remarks**
08:45	**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?** - 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? **Fabienne Bartoli,** Inspector General, **French Minstry of Health** **Dr Virginie Bros-Facer,** CEO**, EURORDIS – Rare Diseases Europe -** TBC **Senior Representative, UCB** *Invited:* **Acko Ankarberg Johanssen,** Minister for Healthcare, **Sweden** **Maggie de Block,** Chair of Representatives, **Belgium Health Ministry** **Dr Anne-Sophie Lapointe,** Rare Disease Project Manager**, Ministry of Solidarities and Health France**
09:35	**Keynote panel: How much weight does the patient voice carry when it comes to influencing clinical trials, policy and orphan drug approvals?** - Are the current policy and regulatory frameworks in the EU 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? **Martine Zimmerman,** SVP Head of Regulatory & Quality Affairs, **Ipsen** **Durhane Wong-Rieger,** President And Chief Executive Officer, **Canadian Organization For Rare Disorders** **Senior FDA Representative** *To invite:* **Michael Berntgen,** Head of Scientific Evidence Generation Department**, EMA** **Dr June Raine,** CEO**, MHRA** **MEP Stelios Kympouropoulos** **Senior Representative, WHO** **Senior Representative, HTA**
10:25	**Morning Networking Break; Tracks begin 11:10**

ClinicalDevelopment	Access&Pricing	RealWorld*Evidence*	ScienceStrategy	PrecisionMedicine	PatientCentricity
Clinical Development **Theatre 2**	**Pricing & Reimbursement** **Theatre 6**	**RWE & Value** **Theatre 4**	**Sustainable Global Orphan Markets** **Theatre 3**	**Advanced Therapies for Rare Diseases** **Theatre 5**	**Patient led initiatives** **Theatre 1**

	ClinicalDevelopmen	Access&Pricing	RealWorldEvidence	ScienceStrategy	PrecisionMedicine	PatientCentricity
	Chair Theatre 2	Chair Theatre 6	Chair Theatre 4	Chair Theatre 3	Chair Theatre 5	Chair Theatre 1
	Clinical Development	Pricing & Access Policies	RWE & Value	Sustainable Global Orphan Markets	Advanced Therapies for Rare Diseases	Patient inclusion
11:10	**Early use of historical controls to improve rare disease drug development** **Senior Representative, ICON**	**Panel: Optimizing pricing and improving patient access to Orphan Drugs** **Senior Representative, Remap consulting**	**Panel: Orphan drug policy and access - how do they prepare for RWE?** **Senior representative, Clinigen**	**WHO rare disease resolution 2025** **Senior Representative, WHO**	**How can we improve clinical outcomes by use of video and technology?** **Samantha Parker,** Patient Advocacy and Communication Lead Rare Disease Europe, **Italfarmaco**	**Patient care and the diagnostic odyssey** **Senior Representative, Mendelian**
11:40	**Reserved for Pharma BP**			**Panel: What are the impacts of rare designation?** *Moderator*: **Dan O'Connor,** Director, Regulatory Policy & Early Access, **ABPI** **Anne-Sophie Chalandon**, Head of Rare Diseases Global Public Affairs Rare Disease and ATMP Policy at **Sanofi**, and leading the **IFPMA** Rare Diseases Group **Durhane Wong-Rieger,** President & Chief Executive Officer, **Canadian Organization For Rare Disorders**	**The challenges of drug development by a patient group** **Senior Representative, Volv**	**How can we support rare disease patients on a global scale?** **Dr Violeta Stoyanova-Beninska,** Chair of Committee for Orphan Medical Products, **EMA,** Policy, Regulatory and Internation Affairs, **CBG-MEB**
			11:55 RWE in clinical evidence		**Applying lessons learned from Duchenne muscular dystrophy exon skipping therapy development to N-of-1 cases for patients with rare brain diseases**	

12:10	**Title TBC** **Senior Representative, Veristat**	**Panel: Pricing and Access Challenges for Rare Diseases and Orphan Drugs** **Senior Representative, Prime Global**	**Case study: Importance of Patient experience mapping What's the relevance of the data and how it's being used to inform decisions** **Senior representative, Ipsen**	**Patient Centricity: From Development to Dosing – Title tbc Senior Representative, Alexion AstraZeneca Rare Disease**	**Messenger RNA is a promising treatment for inborn errors of metabolic disorders** **Senior Representative, Moderna**	12:10 – 12:55 **Evolving Landscape on Patient involvement in Rare Disease HTA** *Moderator:* **Rick Thompson,** CEO, **Beacon** **Durhane Wong-Rieger,** President & Chief Executive Officer, **Canadian Organization For Rare Disorders** **Alexander Natz,** Secretary General, **EUCOPE**
12:40 – 14:10	**Join us in the exhibition hall for: Poster Presentations 1-2-1 Partnering Tracks restart at 14:10**					
12:50 – 13:50	**Interactive Working Groups**					
	Working Group 7: **Theatre 1** **Reserved for RDI**	**Working Group 8**	**Working Group 9**	**Working Group 10**	**Working Group 11**	

14:10	**Co-creating solutions with patient communities to enhance rare disease innovation** **Senior Representative, Ipsen**	**Access Opportunities for Rare Disease In and Out of Europe** **Senior Representative, CRA**	**What are the challenges to ERNs intensifying their research efforts with industry, and what are the solutions to overcome this? Findings in the Together4RD White Paper** **Sheela Upadhyaya,** Life Science Advisor in Rare Diseases & Special Advisor**, FIPRA**	**Global Network on Rare Diseases – a partnership between Rare Disease International & WHO** -How do we speed up diagnosis? -Rare disease center of excellence **Mary Wang,** Science Director, **Rare Diseases International**		**The Five Principles of Self-Advocacy &Your Mental Health Toolbox** **Senior Representative, The Healthcare Navigation Project**
14:40	**Time to See the Difference: using vCOA and video analysis to develop novel outcome measures** **Senior Representative, Aparito**	**A critique of the critique: Pharmaceuticals value, price and profitability** **Senior Representative, Dolon**	**Why Aren't Industry prepared?** -HTA & Regulators and the blackbox -the fears of what to expect and what not to expect *Moderator:* **Marcus Guardian**, Chief			**Title TBC** **Senior Representative, UCB**

<table>
<tr><td>15:10</td><td rowspan="2">Panel: Breaking Barriers: Addressing Clinical Development Challenges in Medicines for Children with Rare Diseases
Senior Representative, Allucent</td><td>EUHTA & joint clinical assessment: Did it improve access and innovation
Multiple perspectives across European countries
Moderator: Senior Representative, Ipsen</td><td>Operating Officer, EUneHTA</td><td rowspan="2">Panel: What is the value of the patient input to HTA?
Moderator: Durhane Wong-Rieger, President And Chief Executive Officer, Canadian Organization For Rare Disorders
Senior Representative, IFPMA</td><td rowspan="2">Panel: What are the barriers of drug development in ultra-rare diseases</td><td>Mapping Rare – a world of rare disease advocacy
Senior Representative, RDI</td></tr>
<tr><td>15:40</td><td>Title TBC
Senior Representative OPEN Health
15:55 TBC</td><td>RWE for gene therapies post treatment – how is this collected and assessed for reimbursement</td><td>Enabling and Enhancing Telehealth for RD Across the Globe</td></tr>
<tr><td>16:10 - 16:30</td><td colspan="6">Comfort break</td></tr>
<tr><td></td><td colspan="6">Closing Plenary Keynote</td></tr>
<tr><td>16:30 – 17:30</td><td colspan="6">Keynote: How do we reinvent the healthcare system to meet the demand of not only rare diseases, but potentially all patients in future?
- 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 to mitigate the high orphan drug prices?
- How can companies and the healthcare system work together to make it more sustainable?
- What role can clinicians and patients play in developing a system that is efficient and delivers the outcomes expected?
Moderator: Sheela Upadhyaya, Life Science Advisor in Rare Diseases & Special Advisor
Senior Representative Pfizer
To invite:
Representatives from Africa/China/Japan & Senior Clinicians</td></tr>
<tr><td>17:30</td><td colspan="6">Chair’s closing remarks</td></tr>
<tr><td>18:30</td><td colspan="6">Networking function
Sponsored by Alexion
Join us at the Museu Nacional d'Art de Catalunya for stunning terrace views
End of day 2</td></tr>
<tr><td></td><td colspan="6">ALEXION
AstraZeneca Rare Disease</td></tr>
</table>

Day Three – *Friday 25 October*

	Morning Networking in the Exhibition Give yourself time to check-out and attend the morning session on-time!				
09:00 – 9:45	**OPENING PLENARY SESSION** **Keynote Theatre**				
	Keynote Panel: How to create a sustainable and robust pipeline for rare diseases and Gene therapies - How will gene therapies impact future pipelines? - What are the remaining scientific challenges? Selecting indications, tissue targeting/distribution, immunogenicity, limitation of vectors - What are the remaining commercial and post market evidence challenges? - How will the regulatory science adapt to our innovative medicines? **Invited Gene therapy companies:** **Biomarin, Novartis,**				
9:45 – 10:30	**The use of RWD in orphan drug development and access pathways** • 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? **Dan O'Connor,** Director, Regulatory Policy & Early Access**, ABPI** **To invite:** **Daniel Prieto-Alhambra,** Professor of Pharmaco & Device Epidemiology**, NDORMS** **Harold Enzmann,** Chair, **EMA CHMP** **Sam Roberts,** CEO, **NICE** **Patient Rep – EURORDIS** **Senior Rep – C-Path**				
	Tracks start at 10:40				
	ClinicalDevelopment	Access&Pricing	Cell&GeneTherapy	ScienceStrategy	PatientCentricity
	Chair **Theatre 2**	**Chair** **Theatre 6**	**Chair** **Theatre 3**	**Chair** **Theatre 4**	**Chair** **Theatre 1**
	Endpoints	**Creating Value**	**Manufacturing and Technology**	**Global initiatives**	
10:40	**Consistent Subjectivity: How to quantify subjective endpoints in rare diseases**	**Reserved**	**Panel: Cell & Gene therapy considerations towards scalable manufacturing**	**Panel: A World Health Assembly Resolution 2025: a call to action**	**Impact of rare disease on mental health and wellbeing -**

			Manuel Carrondo, iBET	**Alexandra Heumber Perry,** CEO, **RDI** More to confirm..	**building understanding of the link and impact of rare and complex diseases on mental health and improving outcomes** **Senior Representative, EURORDIS**
11:10	**One Less Worry: Decentralizing Rare Disease Studies for Easier Patient Participation** **Senior Representative, Marken**	**Panel: Pricing models – have we made progress?**			
11:40	**How do you de-centralise trials?**		**Launching C&G in Europe – A US Biotech perspective**	**The big picture outside of Europe; EMA's innovation taskforce –** Early development on what is coming on a regular basis intended on the treatment of rare disease	
12:10	**Networking Break** **Join us in the exhibition hall for:** **Poster Presentations** **1-2-1 Partnering** **Tracks restart at 13:30**				
	ClinicalDevelopment	Access & Pricing	Cell & Gene Therapy	Science Strategy	PatientCentricity
	Theatre 2	**Theatre 6**	**Theatre 3**	**Theatre 4**	**Theatre 1**
13:30	**Clinical diversity in rare trials**	**Panel: How does access differ in across Europe (Eastern Europe)**	**CBHC for patients with rare diseases: a practical proposal**	**Strategies for study enrolment, patient engagement and recruitment**	**Panel: Access to essential medicines for rare diseases – Title TBC** **Senior Representative RDI**

14:00	**Use of medical devices in trials – how do they change the way we receive data and the patient experience?**		**Art of the possible – create a winning Market Access Strategy**	**Panel: Innovative funding mechanisms and business models** **Ken Kengatharan,** Managing General Partner, **Atheneos Ventures** To inivite: **Laura Rodriguez Gallego**, Partner, **Invivo Partners**	
14:30	**HCPs and clinician perspectives – how do we involve all voices in the clinical development process?**	**How to enable wider patient access to innovation from small biotech companies**	**Developing a RNA therapy platform for rare diseases**		12:25 – 13:10 **Panel: Rare Diseases among Older Adults** - Rare, adult onset, autosomal dominant neurological disorders - Timely diagnosis and intervention for rare diseases affecting older adults - Aging with rare disease
15:00	**Close of Congress** **Thank you for attending!**				