10th annual

WORLD Orphan Drug Congress Europe 2019

12th–14th November 2019

Pre-Congress Workshops: 12th November

Hotel SOFIA, Barcelona, Spain

Strategy, advocacy and partnering for the orphan drug industry

Created by SPARK SOMETHING TERRAPINN

Part of total orphan drugs

Including

Pitch&Partner Orphan Drugs

ClinicalDevelopment Orphan Drugs

Access&Pricing Orphan Drugs

Cell&GeneTherapy Orphan Drugs

PrecisionMedicine Orphan Drugs

ScienceStrategy Orphan Drugs

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terrapinn.com/orphandrugeu19

Cell & Gene Therapy

Orphan Drugs

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Advisory Board*Members*

The 10th World Orphan Drug Congress is taking place **12th–14th November 2019 in Barcelona, Spain;** and is not an event to be missed. Now recognised as the **largest and most established** multi-stakeholder European orphan drug event, we are once again proud to deliver another outstanding speaker line-up and a content-filled agenda that represents the whole orphan drug landscape. The 10th annual event will address the strategic and commercial aspects of bringing new treatments to **patients** who suffer from rare diseases. You'll notice that the Patient Advocacy Track is no longer here, because we've integrated the patient voice directly into every part of the agenda.

For 2019 we will be expanding the meeting further offering more dedicated content and tracks than ever before on a co-located **Cell & Gene Therapy conference,** as well as dedicated tracks in **Clinical/Product Development, Market Access & Pricing, Science & Strategy, Pitch & Partner** and **Precision Medicine**. We will also introduce new Pre-Congress Workshops.

We thank our Advisory Board members for their contribution to the agenda in 2018 - and look forward to working together to make 2019 even more successful.

Wills Hughes-Wilson
Head of Patient Access & Commercial Planning
Mereo BioPharma

Prof Michael Linden
Former VP Gene Therapy & Head, GMI
Pfizer

Yann Le Cam
CEO
EURORDIS - Rare Diseases Europe

Dr Ségolène Aymé
Founder of Orphanet, Emeritus Research Director
INSERM

Dr Alexander Natz
Secretory General
EUCOPE

Steve Groft
Senior Advisor to Director
NCATS, NIH

Sheela Upadhyaya
Associate Director Highly Specialised Technologies
NICE

Alastair Kent
Former Director
Genetic Alliance UK

Dr Carlo Incerti
Former SVP, Head of Global Medical Affairs, CMO Genzyme, A Sanofi Company

Rick Thompson
CEO
Findacure

Dr Mathew Pletcher
Head of Rare Disease Discovery
Roche

Dr M Ken Kengatharan
Managing Director
Atheneos Ventures

Dr Birgitte Volck
President, R&D
AVROBIO

Dr Pedro Franco
Former Scientific Administrator
EMA
Merck KGaA

Dr Anna Bucsics
Project Advisor
MoCA

Dr Diego Ardigo
Chair Scientific Committee, IRDiRC
R&D Rare Diseases Unit Head
Chiesi Group

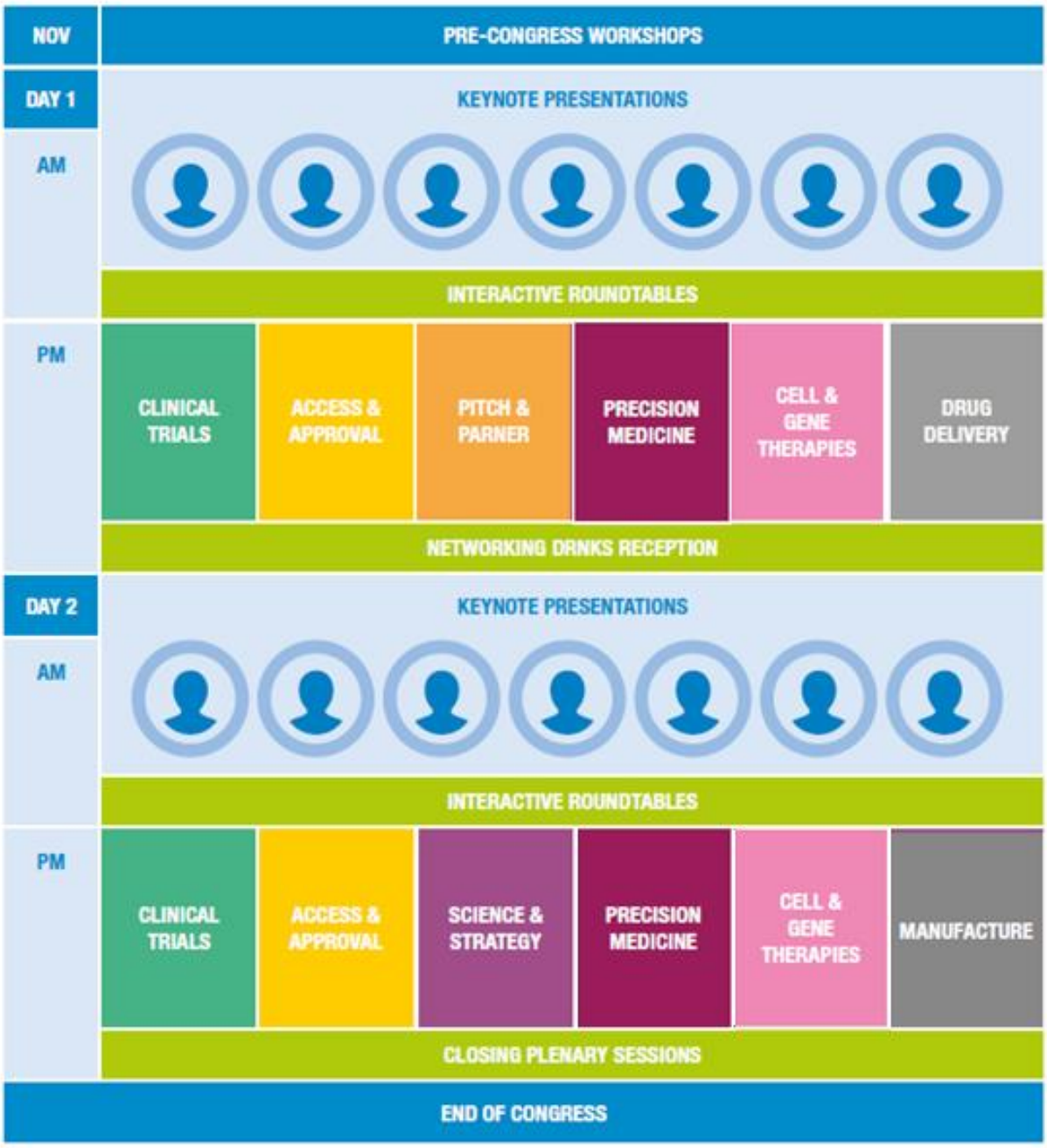
Durhane Wong-Rieger
Chair of RDI
CEO of CORD

Josie Godfrey
Former Associate Director NICE
JG Zebra Consulting

Dr Josep Torrent-Farnell
Head of the Medicines Divison
CatSalut

WORLD OrphanDrug Congress Europe 2019

Agenda*Overview*



Please contact Wing-yun.Cheung@terrapinn.com or nadia.konneh@terrapinn.com for speaking opportunities and agenda enquiries

WORLD OrphanDrug Congress Europe 2019

Morning Pre-congress Workshops *Tuesday 12 November*

10:00 – 13:00

Workshop A: Market Access Implications of Fast Track Procedures in Europe for Innovative Medicines and Orphan Drugs

MME® Learn From Our Experience, Profit From Our Thinking.®

- **Orphan Drugs in the EU: Pricing, reimbursement and market access challenges**
- **How does conditional approval vs exceptional circumstances affect market access?**
- **Alternative routes in fast track EMA accelerated approval and early consultation with Eunethta**
- **Company experiences with early dialogue and common assessment**

Workshop leader: **Dr Renato Dellamano, President, MME Europe**
Doug Paul, Partner, MME
Rudy Dupree, Senior Project Manager, EUnetHTA WP4

Industry case studies to be confirmed

Workshop B: Drug Repurposing Use for Orphan Drugs Industry vs patient rep & off-label vs label use: Transparency, incentives and approaches to help industry repurpose

Workshop leader: Rick Thompson, CEO, Findacure

Off-label Prescribing: Justifying Unapproved Medicine
Speaker TBC

Results from an off-label study and its impact in AKU patients in parallel with the international clinical trial
Prof Lakshminarayan Ranganath (Ranga) - Head of the Specialist Centre for AKU & Lead investigator the clinical trial of nitisinone for AKU

Update on STAMP
César Hernandez Garcia, Head of Department, Medicines for Human Use, AEMPS

Industry perspective
Dr Diego Ardigo, Chair, IRDiRC Therapies Scientific Committee & R&D Rare Diseases Unit Head at Chiesi Group

Incentivising generics manufacturers to repurpose drugs
Paul Flemming, Technical Director, BGMA - TBC

Generics of ex-orphan drugs: Uses and controversies
Beata Stepniewska, Deputy Director General & Head of Regulatory Affairs, MedicinesforEurope

13:00 Networking *Lunch* Break

14:00 – 17:00 Afternoon *Workshops*

Workshop C: Overcoming Clinical & Regulatory challenges in ATMPs

This workshop provides expert guidance and practical case studies to help address the major challenges of:

- **Drug candidate early development**
- **Regulatory strategy & Trial design**
- **Real patient engagement & Market access**
- **Unique challenges in the manufacturing and commercialization of gene therapies for rare diseases**

Annie Hubert, Director, European Public Policy, Alliance for Regenerative Medicine
Michela Gabaldo, Head, Alliance Management & Regulatory Affairs, Fondazione Telethon
Rune Kjeken, Scientific Director, Advanced Therapies, CAT, EMA
Simone Boselli, Director of Public Affairs, EURORDIS
Ioane Kloos, Medical Director, Cellectis

Workshop D: Gene and cell therapies in rare diseases: creating a sustainable business model and pathway to patient access

DOLON

The full potential of innovative new gene and cell therapies will only be realised through the creation of a sustainable business model and a clear pathway to access for rare disease patients. Dolon's **interactive workshop** provides you with the opportunity to explore the complexities of pricing an orphan drug, using a series of exercises that explore the orphan drug **business model,** how **pricing** and **investment decisions** are made, and how **policy impacts innovation**.

The **RARE-IMPACT initiative**, chaired by Eurordis and supported by Dolon, is a consortium of rare disease manufacturers that is engaging with HTA agencies,

	regulatory bodies, payers, patient groups, clinicians and other experts across Europe, to identify and validate the challenges to patients' access to gene and cell therapies. **Workshop leader**: **Adam Hutchings, Managing Director, Dolon** *EURORDIS & Participating partners to be announced shortly*
End of Workshops and Welcome Drinks	

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Day One – Wednesday *13* November

	OPENING KEYNOTE PLENARY
08:00	**Registration + Welcome Refreshments**
08:20	**Chair's opening remarks**
08:25	**What incentives are available for developing orphan drugs? Is the current orphan drug legislation & incentives enough?** - Findings from the commission's value assessment evaluation report – should guidelines be amended? - Feedback and alignment from regulators and industry - Considerations in the unaccounted costs for industry, society values of orphan drugs and the role of competition - Are our policy makers direction in line with what society wants? **Andrzej Rys, Director, Health Systems, Medical Products and Innovation, DG SANTE, European Commission** **Kristina Larsson, Head of Orphan Drugs, EMA** **Bernard J. Grimm, Director, Healthcare Biotechnology, Europabio** **Delphine, Senior Manager Public Affairs, EUCOPE** **Ulrika Vestin, Swedish Association of Local Authorities and Regions (SKL)**
09:10	**What guidance can we give industry when working with European Reference Networks (ERNs) and R&D?** - Roadmap for industry: How should industry be engaging with ERNs and what does each have to offer to improve clinical and research aspects? - Company and ERN perspectives, experiences so far - What are the bottlenecks preventing ERNs achieving their full potential? - What can we learn from US CRNs? - Stakeholder engagement with ERNs – industry and patients beyond academic and clinical networks **Andrzej Rys, Director, Health Systems, Medical Products and Innovation, DG SANTE, European Commission** **Prof Maurizio Scarpa, Coordinator, European Reference Network for Hereditary Metabolic Diseases (MetabERN)** **Anne Pariser, MD, Director, Office of Rare Diseases Research, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health** **Dr Vinciane Pirard, Co-Chair of Rare Disease & OMP Joint Task Force EFPIA-Europabio & Senior Director Public Affairs, Sanofi**
09:55	**Executive Insights: What technologies need to be unlocked? Discussing all the scientific aspects that are impacting the reality of gene therapy** - What is the science driving your business model, how do we overcome the technological constraints? - Challenges in creating robust pipelines sustainable to patients (selecting indications, tissue targeting/distribution, immunogenicity, limitations of vectors) - How have gene therapies in the past influenced our current pipelines?

<table>
<tr><td></td><td colspan="4">- Realities of manufacturing & commercialisation
Mark Rothera, Chief Executive Officer, Orchard Therapeutics
Stuart Peltz, Chief Executive Officer, PTC Therapeutics
Bob Smith, Senior Vice President, Global Gene Therapy Business, Pfizer
Sander van Deventer, Chief Scientific Officer, UniQure
Michael Linden, Chief Scientific Officer, Touchlight Genetics Ltd
Frederic Chereau, Chief Executive Officer, LogicBio Therapeutics</td></tr>
<tr><td>10:40</td><td colspan="4">Morning Networking Refreshment Break</td></tr>
<tr><td></td><td colspan="4">Choose 2 x Interactive Roundtables: 11:30 to 12:15 (rotation one) and 12:20 to 13:05 (rotation two).</td></tr>
<tr><td rowspan="4">Plenary follow up

All these roundtables are delivered on both times</td><td colspan="2">11:30 – 12:15: Roundtable and Q&As on plenary orphan drug legislation & incentives
European Commission, EMA, Europabio, EUCOPE, SKL and Dr Pedro Franco, Former Scientific Administrator, EMA & Merck KGaA</td><td colspan="2">12:20 – 13:05 Roundtable and Q&As on plenary ERNs
European Commission, MetabERN, NIH</td></tr>
<tr><td>Leveraging patient-focused technological solutions to support better diagnosis and access to new treatments for rare disease patients
Scott Schliebner, Vice President, Scientific Affairs, PRA Health Sciences</td><td>Expediting drug development in rare disease: Are big data and biomarkers an option for the future?
Reserved for IQVIA – title TBC</td><td>From Endpoints to Endgame: Incorporating the diagnostic odyssey and patient journey in your clinical development plan</td><td>Regulatory & operational implementation of the direct to patient trial management mode
Cinzia Dorigo, Syneos Health</td></tr>
<tr><td>Building a new infrastructure for long term evidence generation with use of registries</td><td>Balance between medical and promotional (sales force) in achieving brand objectives in rare diseases
Charles de Wet, Senior Medical Director UK, Ireland and Nordics, Alexion Pharma
Katerina Anokhina, Director, Medical Affairs, UK And Ireland, Alexion Pharma</td><td>To go or not to go for parallel consultation
Dr Gopalan Narayanan, M.D.
Vice-President, Disruptive Biologics, Voisin Consulting Life Sciences – title TBC</td><td>Challenges of developing an orphan drug worldwide
Dr Pedro Franco, Former Scientific Administrator, EMA & Merck KGaA</td></tr>
<tr><td>The impact of Brexit and Europe-wide effects from market access to the UK, patient collaborations across Europe</td><td>Market entry strategies for orphan drugs in Central Eastern Europe
Reserved for Reto Andreas Schaberl, Ewopharma AG</td><td>Developments in Eastern Europe: Unique challenges and opportunities
Reserved for Pawel Wozniak, Komtur Polska</td><td>Unique challenges in Brazil for orphan drug access and pricing
Marcela Junqueira, Head of Strategic Affairs & Health Economics, Brazil, Janssen</td></tr>
</table>

	Best practices and tools to get patients groups to fully engaged **Prof Kassim Javaid, Associate Professor, Consultant in Metabolic Medicine, Oxford University Hospitals**	**Patient organizations as partners: Including "resource" issues from support for education to support for supporting patient communities** **Durhane Wong-Rieger, President & CEO, CORD**	**The US healthcare model: Increasing patient affordability and access for oncology treatment** **Melissa Paige, UVA Health System, Patient Access Principal Coordinator, Patient Advocate**	**Use of registries on OMPs post launch registration: Experience from Germany** **Friedhelm Leverkus, Director HTS&OR Health & Value Germany, Pfizer** **Jorg Mahlich, Head of Health Economics & Outcomes Research, Janssen** **Marco Penske, Head of Market Access & Healthcare Affairs, Boehringer Ingelheim**
	The patient voice: An important consideration not only for late stage reimbursement, but also for early stage regulatory affairs **Tamsyn Frost, Regulatory Affairs, IDEA Regulatory** **Patient representative** TBC	**The Italian experience: How has our experience changed how we manage ODs?** **Andrea Mantovani, Former Value, Access and Policy Director, Amgen**	**How to build an ecosystem for rare diseases, from private to public** **Dr Diego Ardigo, Chair, IRDiRC Therapies Scientific Committee & R&D Rare Diseases Unit Head at Chiesi Group** To Invite: **IMI, European Joint Procurement-RD**	**Inclusion of "nontraditional" as well as "low and middle" income patient populations in all aspects of drug development**
	How can we overcome the clinical development challenges facing advanced therapies for rare diseases? **Dr Virginia Haurigot, Ocular Research Lead and Head of Pharmacology & Toxicology, Spark Therapeutics**	**Gene editing revolution: Hope for rare diseases, but should there be limits?**	**Pricing & reimbursement for cell & gene therapies – Are the models really that different from normal rare disease models?**	**Cell & gene therapy: Can we manufacture and commercialise in a sustainable way?** **Dr Qasim Rafiq, Associate Professor, Bioprocessing of Cell and Gene Therapies, University College London**
13:05	**Networking Lunch & Patient Poster Session**			

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14:15	ClinicalDevelopment	Access&Pricing	ScienceStrategy	PrecisionMedicine	Cell&Gene Therapy	Manufacture
	Chair opening remarks **Dr Carlo Incerti Former SVP, Head of Global Medical Affairs, CMO Genzyme, A Sanofi Company**	**Chair opening remarks**	**Chair opening remarks**	**Chair opening remarks**	**Chair opening remarks**	**Chair opening remarks**
	Patient Centricity & Trial Design	**The Value of Orphan Drugs**	**Case Studies**	**Discovery Diagnostics & Digital Health**	**Clinical Development: Cell & Gene Therapy**	**Improving Cell & Gene Therapy Manufacturing**
14:20	**The use of biomarkers to support clinical success** **Dr Steve Maricich, Medical Direction, Clinical Science, Biomarin**	**Early access strategies** **Mark Corbett, EVP, Inceptua Medicines Access**	**Case study: How a biotech can successfully raise funds for rare disease drug development** **Dr Marc Martinell**, CEO, Minoryx	**Artificial Intelligence: Promise & Reality** **Philip Nelson, Director, Software Engineering, Google Inc**	**Novartis: CAR T Innovation Journey** **Dr Emanuele Ostuni, Head of Europe, Cell & Gene Therapy, Novartis**	**Industrialization of gene therapy manufacturing** **Dr Frederic Revah, Chief Executive Officer, Genethon**
14:45	**Enabling drug discovery - the need for improved epidemiology in rare diseases** **Senior representative, Syneos Health**	**Panel: Perspectives from the industry in early access strategies** **Anant Murthy,** VP of Access and Policy for Canada, Europe and MEA, Alnylam Pharmaceuticals **Paolo Morgese,** Director, EU Market Access & Member Relations, Alliance for Regenerative Medicine	**Case study: Systematic drug re-innovation and clinical evidence in orphan diseases** **Dr Raúl Insa, CEO, SOM Biotech**	**The use of artificial intelligence and machine learning to drive innovation for rare disease treatments** **Dr Krishnan Nandabalan, CEO & President InveniAI**	**Regulatory issues in ATMPs** **Rune Kjeken, Scientific Director, Advanced Therapies, CAT, EMA**	**Developing robust manufacturing processes for novel gene therapies** - Overcoming manufacturing challenges faced by therapy developers - Effectively preparing for later stage trials and commercialization **Palani Palaniappan, SVP, Head of Global Technical Operations, Sarepta Therapeutics**

		Rute Fernandes, Head Rare Diseases Franchise, Europe & Canada, Takeda 15:30 **Panel: Commercial negotiations in HTA** Why, when and what do they achieve? The framework for conducting and tracking them **Sheela Upadhyaya,** Associate Director Highly Specialised Technologies, Centre for Health Technology Evaluation, NICE **Inneke Van De Vijver,** Acting President - Taskforce Managed Entry Agreements, NIHDI & RIZIV-INAMI, Brussels *More speakers to be announced*				
15:10	**Patient reported outcomes measures: An interpretable measure of patient benefit to demonstrate clinical effectiveness** **Thomas Morel, Director of Global Patient-Centred Outcomes Research & Policy at UCB & KU Leuven**		**How can expanded access combined with real-world data to enhance drug development** - Title TBC **Reserved for myTomorrows**	**AI and patient crowdsourcing for rare diseases diagnosis** **Julian Isla, Data and Artificial Intelligence Resource Manager, Microsoft**	**Treating Fabry disease: Phase 2 clinical updates and expansion of clinical activities into other indications** **Dr Birgitte Volck, President, R&D, AVROBIO**	**Capacity crunch? The future of manufacturing within cell & gene therapy**
15:35	**How can technology and patient-focused approaches accelerate clinical development?** **Scott Schliebner, Vice President, Scientific Affairs, PRA Health Sciences**		**Success factors for rare disease launches and partnering**	**Europe: Taking the lead on ethical AI**	**Pfizer's gene therapy pipeline** **Michael Binks, Vice President, Rare Disease Clinical Research, Rare Disease Research Unit, Pfizer** (TBC)	**Advancement of analytical tools for testing and characterization of AAV gene therapy products** **Mark Galbraith, Head of Quality Control & Analytical Sciences, Spark Therapeutics**
16:15	**Afternoon Networking refreshment break**					
16:45	**The RUDY study to inform novel biomarkers and therapeutic targets with patients**	**Tool for reducing uncertainties in evidence generation for specialised treatments for rare diseases: TRUST4RD**	**Showcases** Pitch&Partner **Showcase:** *Synthetic plasmalogen-based therapy for the treatment of Rhizomelic*	**New-born screening programs in Europe: Creating a European common model**	**LentiGlobin Gene Therapy in Transfusion Dependent β-Thalassemia**	**Defining effective manufacturing & regulatory standards** **Jack Price, Head, Division of Advanced Therapies,**

	Prof Kassim Javaid, Associate Professor, Consultant in Metabolic Medicine, Oxford University Hospitals	Tackling uncertainties for rare diseases - The use of RWE to improve access for patients **Dr Karen Facey,** Member, Scottish Health Technologies Group & Impact HTA - Co-Lead Investigator for WP10 - Appraisal of Orphan Medicinal Products & representative, FIPRA **Mercedes Echauri**, Head Patient Value and Access, Europe & Canada	*Chondrodysplasia Punctata (RCDP) and other rare pediatric peroxisomal disorders* **Dr Shawn Ritchie, CEO and CSO, MED-LIFE DISCOVERIES LP** **17:00 Showcases** **Reserved**	**Dr Giancarlo la Marca, Director of U.O. Newborn Screening, Biochemistry and Pharmacology laboratory, Meyer Children's University Hospital, Florence**	**Martin Butzal, Head of Medical, Bluebird Bio** (TBC)	**National Institute for Biological Standards and Control**
17:10	**A partnership that lasts- key considerations when working with CROs on rare disease studies** **Jonathan Kornstein Executive Director, Program Strategy, Rare Diseases & Pediatrics Premier Research**	**Value assessment: How to build a consensus around access decisions** The economic and societal impact of the orphan medicine regulation **Senior representative, Charles River Associates** – Title to be updated	**17:15 Showcases** **Reserved** **17:30 Showcases** **Reserved**	**Improving patient outcomes across the globe with the 100,000 genomes project rare disease programme** Invited: **Richard Scott, Clinical Lead for Rare Disease, 100,000 Genomes Project, Genomics England**	**Homologous recombination: A precise and nuclease-free approach to gene editing** **Dr Arthur Tzianabos, President & CEO, Homology Medicines**	**Deciding on an effective manufacturing strategy: Choosing to outsource your manufacturing**

17:35	**New models to run natural history studies and registries** Perspectives from patients, CROs and regulators on sustainable RWE Moderated by: **Martine Zimmermann, Global Head of Regulatory Affairs, Alexion pharma GmbH** **Jean-Phillipe Combal, Co-Founder & CEO, Vivet Therapeutics** *Patient rep and CRO to be announced*	**Strategies for obtaining orphan drug and other EMA designations for orphan products** **Reserved for, IQVIA -** Title to be updated	**17:45 Panel: Critique of showcases & how to attract investment and progress your pipeline?** Private investment sources for orphan drugs Investigating the funding opportunities available in Europe and globally How to attract larger players to collaborate **Clara Campas,** Partner & Co-founder, Asabys Partners **Olaf Ritzeler,** Director of scouting external opportunities / EUROPE, S **Alan J Muir**, Director Life Science & Healthcare Investment, Seven Hills Venture Partners	**Decentralised digital trials in populations with high unmet need - Next generation approaches** **Dr Michelle Krishnan, Translational Medicine Leader in Rare Diseases, Roche**	**Translating a CRISPR platform into gene-edited cell therapies - the future is allogeneic** **Rachel Haurwitz, President and Chief Executive Officer, Caribou Biosciences**	**Strategies and advances in lentiviral vector manufacturing and scale-up** **Leonard Pattenden, Head of Biotherapeutics Development & Drug, Supply, Biotherapeutics Development Unit, Cancer Research UK**
18:00		**Coming to America? How to position your orphan and rare product for launch in the United States** **Suzette DiMascio, CHE, CMCE, CPC, President /CEO, CSI Specialty Group**		**What type of smartphone Apps are useful for patients? Empowerment through digital disease education created for patients with patients** **Lucilla Franchetta Ortelli, Senior Medical Affairs Manager, Patient Support Programs, Alexion pharma GmbH**	**Harnessing the body's natural DNA repair process to treat paediatric rare diseases** **Frederic Chereau, Chief Executive Officer, LogicBio Therapeutics**	**Advances in cryopreservation technology to support the scalable supply requirements for cell and gene therapy** **Dr Qasim Rafiq, Associate Professor, Bioprocessing of Cell and Gene Therapies, University College London**
18:25	**Chair's closing remarks per track**					
18:30	**Evening Networking Drinks**					

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Day Two – Thursday *14* November

	OPENING KEYNOTE PLENARY
08:30	Chair's opening remarks
08:35	**EUnetHTA update - Showcase of early dialogue and common assessment** - Hear from EMA's scientific advice or accelerated approval and early consultation with EUnetHTA - How does this route differ? - Should companies continue with the usual EMA and access paths in parallel? - Perspectives from industry who have engaged in early dialogue and their experience - Perspectives from patients **Marcus Guardian,** Chief Operating Officer**, EUnetHTA** **Kristina Larsson,** Head of Orphan Drugs**, EMA** **Yann Le Cam,** Chief Executive Office, **EURORDIS Rare Disease Europe** **Lonneke Timmers,** Secretary of the Scientific Committee**, ZIN -** TBC *Inviting HTA agencies & Industry representatives to share their experience*
09:35	**Transatlantic synergies between innovative payment models** - A global perspective on how split-cost and performance-based models work - What are the quality criteria used? - Investigating real-world learnings from European pricing models to incorporate into program designs - What do payers think of transformative therapies like gene therapy? Moderated by **Alexander Natz, Secretary General, EUCOPE** **Sheela Upadhyaya, Associate Director Highly Specialised Technologies, Centre for Health Technology Evaluation, NICE** **Francis Arickx, Head of Directorate Pharmaceutical Policy, RIZIV-INAMI, Brussels -** TBC **Mark Trusheim, Strategic Director, NEWDIGS, MIT Center for Biomedical Innovation** **Francis Pang, VP of Global Market Access, Orchard Therapeutics** **Doug Danison, Vice President Market Access, Value and Evidence Strategy, bluebird bio**
10:35	**Morning Networking Break**

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11:35	Clinical Development	Access & Pricing	Science Strategy	Precision Medicine	Cell & Gene Therapy	Manufacture
	Chair opening remarks	**Chair opening remarks**	**Chair opening remarks**	**Chair opening remarks**	**Chair opening remarks**	**Chair opening remarks**
	Patient Centricity & Product Development	**Cross-Country Collaboration on Evidence Generation**	**Alternative business models for ODs: Collaboration**	**Advanced Therapies for Orphan Diseases**	**Reimbursement, Patient Access & Investment in Cell & Gene Therapy**	**Scale Up of Cell & Gene Therapies**
11:40	**Improving healthcare communications in rare disease** **Gavin Jones, Director of Rare Disease, Open Health** **Co-presented with a patient representative Benjamin James, patient advocate and masters graduate**	**Are Europe's collaborative efforts working? Beneluxa & Horizon scanning** **Dr Diane Kleinermans, Advisor to the Minister of Public Health and Social Affairs, Belgian Federal Government**	**The emerging business models for commercializing ODs and gene therapy treatments** **Karen Aiach, Chief Executive Officer, Lysogene** **Patient-led organisation to successfully repurpose drugs**	**Novel Approaches to Clinical Trials in Rare Neurological Disorders** -Outcomes from OV101 STARS in Angelman syndrome and OV935 adults with developmental epileptic encephalopathies **Amit Rakhit**, Chief Medical and Portfolio Management Officer, **Ovid Therapeutics**	**Germany and the CAR T cell therapy reimbursement model: Germany's first outcomes-based deal** **Detlev Parow, Head Pharmaceutical Department, DAK-Gesundheit** **Does cross border healthcare work for cell & gene therapy? The logistics and long-term treatment plan** Patient access for cell & gene therapies **Moderated by Alexander Natz, Secretary General, EUCOPE** **Michela Gabaldo, Head, Alliance Management & Regulatory Affairs, Fondazione Telethon**	**Scaling manufacturing to deal with the demands of commercial cell & gene therapy products** **Professor Brendon Noble, CSO & Director of Strategy, UK Stem Cell Foundation, The Royal Institution of Great Britain**
12:10	**A landmark case of a multi-stakeholder collaborative clinical trial delivering a repositioned drug for an ultra-rare condition**	**Update on V4+ initiative and ODs in Poland** MCDA as a tool in P&R decision making **Dr Marcin Czech, MD, Former Vice Minister, Ministry of Health, Poland, Dpt of Pharmacoeconomics,**	**Patient-led companies with alternative business models** **Jonathan Garen, CBO, uniQure** - *TBC* **Patient-led companies with alternative business models** **M4K Pharma** - *invited*	**The promise of RNA interference (RNAi) therapeutics for orphan diseases** **Bernhard Kaumanns**, VP Medical Affairs, **Alnylam Pharmaceuticals**		**Contrasting In-House and External Manufacturing Options** **Peter Olagunju, Vice President, Global Patient Operations, Europe, Bluebird Bio**

		Institute of Mother and Child & President-elect ISPOR Poland			**Leo Strican, Head of Access Value & Evidence Strategy, Europe, Bluebird Bio** (TBC) **Kevin Mayo, Head, Global Market Access - New Products, PTC Therapeutics** **Senior Representative, Novartis**	
12:40	**Networking Lunch & Patient Poster Session**					
14:10	**The role of patient advocacy in ensuring access to effective and transformative therapies** **Amanda Bok, CEO, European Hemophilia Consortium**	**Nordic HTA collaborations** **Sune Lindgaard,** Head of Section Business Intelligence and Health Economics, Amgros **Einar Andreassen,** Senior Adviser, HTA and Reimbursement, Norwegian medicines Agency **Ulrika Vestin,** Swedish Association of Local Authorities and Regions (SKL)	**Patient-led organisation with an alternative business model** **Majid Jafar, LouLou Foundation**	**Genetic surgery: Allele-specific gene editing using engineered CRISPR nuclease** **Rachel Diamant MSC Eng,** VP Intellectual Property & Portfolio Development, **Emendo Biotherapeutics**	**Discover what payers really think of gene therapy** **Sophie Schmitz, Managing Partner, Partners4Access**	**Overcoming bottlenecks in AAV manufacturing for gene therapy**
14:40	**Industry panel: How are we incorporating the patients in a meaningful way?**	**Valetta Declaration** **Hon. Christopher Fearne, Deputy Prime Minister, Ministry for Health, Malta** - *invited*	**Panel from our companies/organisations: what are the long-term implications of**	**Developing precision genetic medicines through base editing**	**Panel: European Investment landscape for cell & gene therapy**	**Strategic partnerships between CDMO's and industry to achieve commercialisation**

15:10	**Sonali Chopra,** Director, Alliance and Advocacy Relations, Genentech, Inc. **Gail Moore,** Director, Global Patient Advocacy, Horizon Pharma **Emanuele Degortes,** Head of Patient, Innovation and Access Policy, Vifor Pharma	**Baltic Procurement Initiative** **Agnese Vaļuliene, Deputy State Secretary for Resources and Changes Management, Ministry for Health, Latvia -** *invited*	**these alternative business models?** What are the differences to the more traditional model of pharma? What are the long-term implications? How do you collaborate, especially with very rare diseases to be sustainable?	**Therapeutic genome editing of Charcot-Marie-Tooth 1A disease** **Seokjoong Kim**, R&D Managing Director, **ToolGen**	**Bob Smith,** Senior Vice President, Global Gene Therapy Business, Pfizer **Dr Sander van Deventer,** CSO, UniQure & Operating Partner, Forbion Capital Partners **Stijn Vanacker** Portfolio Manager, Robeco Global Stars **Owen Smith,** Principal, 4BIO Capital	**How can advanced therapy companies make their manufacturing process more cost effective and sustainable?** **Lior Raviv, Vice President of Development, Pluristem Therapeutics**
15:30	**Networking refreshment break**					
16:00	**CLOSING PLENARY SESSIONS**					
	The impact of digital & computational tools on diagnostics and patient care - How will new diagnostic technologies in genomics, sequencing and AI change the way patients access diagnostics? - What impact will it have to physicians and at a hospital level? - Looking into the future, will these advances mean that treatments will become more accessible for all? **Yann Le Cam,** Chief Executive Office, **EURORDIS Rare Disease Europe** **Hartmann Wellhoefer,** VP, Head Rare Metabolic Diseases, Global Medical Affairs**, Takeda** **Dr Ryan Taft,** Senior Director of Scientific Research**, Illumina** Senior representative, **Microsoft**					
16:45	**What are the real impacts & benefits of cross-border collaborations?** - Measurable effectiveness to patient access to orphan medicines - How to navigate through Europe's complex HTA landscape? - Bringing together perspectives from multiple collaborations **Sabine Vogler, Director of a WHO Collaborating Centre affiliated to the Austrian Public Health Institute** **Dr Diane Kleinermans, Advisor to the Minister of Public Health and Social Affairs, Belgian Federal Government** **Dr Marcin Czech, MD, Former Vice President, Ministry of Health, Poland, Dpt of Pharmacoeconomics, Institute of Mother and Child & President**					

	Representing the Nordic Pharmaceutical Forum: **Sune Lindgaard,** Head of Section Business Intelligence and Health Economics, Amgros **Einar Andreassen,** Senior Adviser, HTA and Reimbursement, Norwegian medicines Agency **Ulrika Vestin,** Swedish Association of Local Authorities and Regions (SKL),
17:30	**Chair's Closing Remarks & Close of congress**

ExhibitionFloor

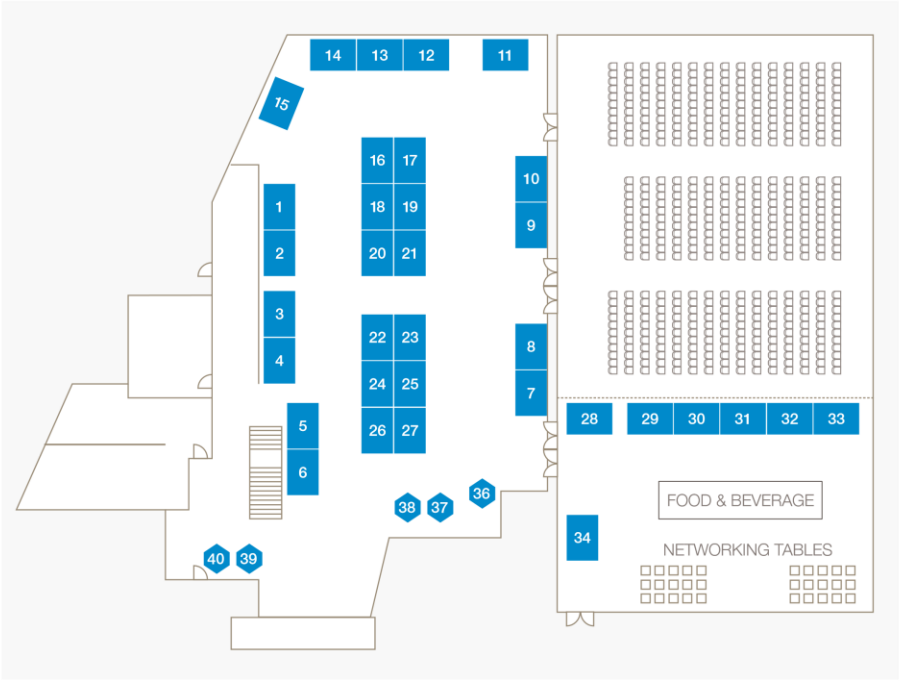
Here's an overview of the floor plan. See the website for the most up-to-date version.

YOUR CUSTOMERS WILL BE THERE TO:

- Hear insights from the world's leading thinkers, practitioners and process disrupters
- Evaluate and buy the latest technologies
- Create new partnerships and gain investment
- Have fun and do business

WHAT THIS MEANS FOR YOUR BUSINESS:

Emerging science, technologies and collaborations are needed to make the necessary moves forward. As a provider of solutions to these issues, the event will provide a space for you to get in front of potential new customers from all walks of the orphan drug industry.



WHAT A GREAT OPPORTUNITY TO:

- Debut new solutions
- Improve your brand awareness
- Meet new prospective clients
- Maintain relationships

PURSUE AND PARTNER

- Thousands of formal and informal meetings over just three days
- 30+ scientific posters
- Three engaging networking lunches
- Two networking drinks receptions

WHO SHOULD SPONSOR?	Pharma	Biotech	CROs	Market Access	Consultancies	CMOs	Platform Technology

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SupportingPartners

For more information please contact Michael.Hodge@terrapinn.com