7th annual

WORLD OrphanDrug Congress Europe 2016

16-17 November 2016

Pre-congress workshops: **15 November**

Sheraton Brussel Hotel, Brussels, Belgium

Strategy, advocacy and partnering for the orphan drug industry

Created by terrapinn use your brain

Part of total orphan drugs

Official Communications Partner aurora

#WODCeurope

REGISTER EARLY & SAVE MORE

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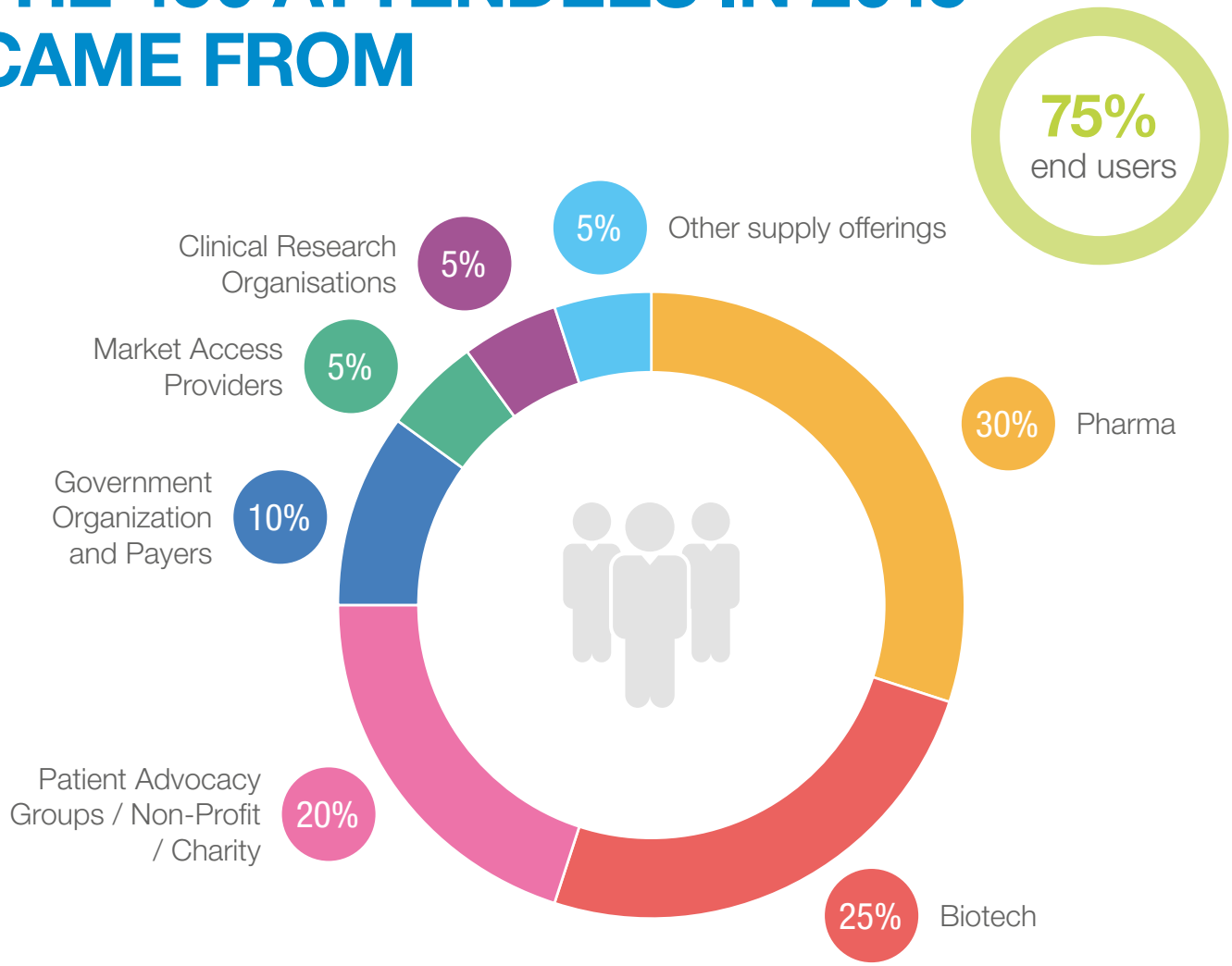
WHY ATTEND THE WORLD ORPHAN DRUG CONGRESS 2016?

Make sure you are at the forefront of the orphan drug industry. Regardless of whether your interest lies in research, clinical development, patient access, global pricing and reimbursement or just to engage with patient advocacy groups more intimately, we have content, networking and potential partners for you!

By bringing experts from patients and all stakeholders under one roof, you get to choose the sessions which are the most applicable to help your business plan for the future of orphan drug research, development and manufacture.

Take just three days out of the office to meet with 450+ potential customers who need to find solutions to challenges around the commercial, regulatory and scientific issues in orphan drugs.

THE 450 ATTENDEES IN 2015 CAME FROM



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“VERY WELL RUN WITH VALUABLE TOPICS AND CREDIBLE SPEAKERS”

EXECUTIVE VICE PRESIDENT, GLOBAL ORPHAN BUSINESS UNIT AND INTERNATIONAL OPERATIONS, **HORIZON PHARMA**

WHATS NEW IN 2016?

The 7th World Orphan Drug Congress is taking place 15 - 17 November in Brussels and is not an event to be missed this year. Now recognised as the largest and most established European orphan drug event, we are once again proud to deliver another fantastic speaker line up and content filled agenda that represents the whole orphan drug scene. The 7th annual event will address the strategic and commercial aspects of bringing new treatments to patients who suffer from rare diseases.

Pre-congress workshops on the 15th November will give you a full days' worth of content where you get the chance to learn topics from **health economics, payer** and **evidence** generation to unique challenges in **pricing, reimbursement & market** access for cell and gene therapies for rare diseases – pick this option on your registration to join.

Rare cancer orphan designations are increasing which is why we have dedicated a days' worth of content in the programme. Should the clinical end points and reimbursement structure be considered differently? Find out from **Novartis, Cellectis, Amgen, AbbVie, PPD** and many more.

Gene therapy could revolutionalise the orphan sector but where are we? **How would you price a cure?** Hear from **Spark Therapeutics, DAK-Gesundheit, GSK, Abeona Therapeutics, RegenXBio, Lysogene** and plenty more.

Clinical Trial Symposium is made up of industry experts including **Dr Paul Strijbos,** Global Head Rare Disease Clinical Development at Roche, **Martine Zimmermann,** Vice President Global Regulatory Affairs at Alexion and **Scott Schliebner,** Vice President, Scientific Affairs at PRA Health Sciences to give a 360 industry perspective.

An extended all European payers session with countries represented from **UK, France, Italy, Belgium, Bulgaria, Norway and Czech Republic.**

An investors panel has been added into the Pitch & Partner track to allow more biotechs to meet the VC and funders they need to move forward.

Rare Advocacy World Track, providing vital information regarding how patient groups are growing to co-developing orphan drugs from **EURORDIS, BMS, Findacure, Genetic Alliance UK, Retina International, Alström Syndrome UK, Cambridge Rare Disease Network, Global Genes** and many more.

Combining Science & Strategy is what our congress does best, which is why we have invited high-level speakers to give us insights into their market and company in a dedicated track, including: **Alexion, Bayer, Genzyme, California Life Sciences Association (CLSA), Orphan Europe** and many more.

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THE ADVISORY BOARD MEMBERS

Yann Le Cam

Chief Executive Officer, **EURORDIS**

Dr Ségolène Aymé

Founder of Orphanet, Emeritus Research Director, **INSERM**

Dr Bruno Sepodes

Chair of COMP, **EMA**

Dr Alastair Kent

Director, **Genetic Alliance UK**

Nicole Boice

Founder & CEO, **Global Genes**

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SPOTLIGHT ON SPEAKERS

Spotlight on INDUSTRY SPEAKERS

Dr Sven Kili
Vice President and Head of Gene Therapy Development
GSK

Dr Paul Strijbos
Global Head Rare Disease Clinical Development
Roche

Martine Zimmermann
Vice President Global Regulatory Affairs
Alexion

Dr Vinciane Pirard
Co-Chair Task Force RDs & ODs of EFPIA-EuropaBio and Director of Public Affairs EMEA, **Genzyme**

Spotlight on NON-PROFIT AND PATIENT GROUP SPEAKERS

Yann Le Cam
Chief Executive Officer
EURORDIS

Kay Parkinson
CEO & Founder, Alström Syndrome UK and Chair
Cambridge Rare Disease Network

Avril Daly
CEO
Retina International

Flóra Raffai
Executive Director
Findacure

Spotlight on GOVERNMENT AND PAYER SPEAKERS

Kristina Larsson
Head of Orphan Medicines
EMA

Andrzej Rys
Health Systems, Medical products and Innovation Director, Health and Food Safety Directorate (DG Sante)
European Commission

Maggie De Block
Minister of Social Affairs and Health, Policy unit of the Ministry of Social Affairs and Health, **Belgian Federal Government**

Sheela Upadhyaya
Associate Director Highly Specialised Technologies Centre for Health Technology Evaluation
NICE

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2016 AGENDA OVERVIEW

PRE-CONGRESS WORKSHOPS 15TH NOV	DAY 1 16TH NOV	DAY 2 17TH NOV
MORNING PRE-CONGRESS WORKSHOP	PLENARY SESSIONS	PLENARY SESSIONS
	NETWORKING BREAK	NETWORKING BREAK
	ROUNDTABLES	PANEL SESSIONS
LUNCH	LUNCH	LUNCH
AFTERNOON PRE-CONGRESS WORKSHOP	CLINICAL DEVELOPMENT PATIENT ACCESS GENE THERAPY SCIENCE & STRATEGY	PITCH & PARTNER RARE ADVOCACY WORLD RARE CANCERS

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MORNING
PRE-CONGRESS WORKSHOP

15TH NOVEMBER, 2016

HOSTED BY **MME (MEDICAL MARKETING ECONOMICS)**

09:00 - 12:00

Pricing, Reimbursement and Market Access Challenges for Advanced Therapy Medicinal Product & Orphan Drugs in the EU

Major hurdles exist even in the EU health systems delaying and preventing patient access to Orphan Drugs (ODs) approved by the EMA. If possible, such hurdles are even greater for ODs that are also Advanced Therapy Medicinal Products (ATMPs) - Cell therapies, gene therapies and hybrid cell-gene therapies.

In this workshop we will review the actual market access (MA) status and timing in the main EU countries of ODs approved by the EMA in the recent past and will then discuss the additional issues for ATMP/ODs.

Real-world experience will be presented and discussed, with the testimony of a medium-size European Company with a specific focus on ODs and, in particular, ATMP/ODs.

Finally, potential solutions will be discussed, to address key pricing, reimbursement and MA challenges for ATMP/ODs in the EU context.

Gain from a unique combination of **1)** manufacturer, customer, economic, and clinical perspectives combined with **2)** solid academic theory from attending this workshop.

Dr Renato Dellamano, President, **MME Europe**

Expertise shared from MME:

- In addition to work in oncology and orphan markets, we have helped developers and marketers of >40 ultra-orphan drugs and several ATMPs to identify, frame, communicate and capture the value of their therapies.
- Unique combination of manufacturer and customer perspectives combined with solid academic theory
- Strategy development and tactical execution to support informed decision making
- Assessment and planning of opportunities and competitive situations at every stage of the product life cycle
- Our methods are designed to develop an understanding of the value perceptions of all meaningful stakeholders

What are the benefits of attending this workshop?

- The orphan markets have examples of products that have exceeded expectations as well as some that have yet to achieve initial forecasts: pricing and reimbursement is a factor that can work in either direction.
- During this workshop we will look at the general issues and challenges for ODs and at the additional particular challenges for ODs that are also ATMPs.
- In this particularly innovative field, understanding the current orphan drug environment and the potential changes in the near future include sustainability of orphan drug pricing plus the additional key issues related to pricing and reimbursement of cell and gene therapies.

12:00

Networking lunch followed by afternoon workshop

To register visit **terrapinn.com/orphan2016** and pick the pre-congress workshop option

AFTERNOON
PRE-CONGRESS WORKSHOP

15TH NOVEMBER, 2016

HOSTED BY **MAPI GROUP**

13:00 - 17:00

Accelerating Commercialization of Rare Disease Treatments

Covering topics on compassionate use, Real World evidence, global regulatory strategies and market access.

Discussion points include:

Methodologies in developing Real World Data: Engaging stakeholders in developing evidence strategies

- How is expanded access used to provide treatment for patients with rare diseases?
- Compassionate Use Programs in the window between submission and commercialization
- What are the benefits of conducting clinical studies and registries for rare diseases?
- What are the types of studies conducted during different phases of orphan drug development?
- How are patient registries used to generate real-world data for rare diseases and orphan drugs?
- Ethical Patient Warehousing – working with Advocacy to identify populations in advance of research
- What are the unique challenges of conducting rare disease/orphan drug registries?
- How can proactive study management strategies successfully address these challenges?

Dr Will Maier, Chief Scientific Officer, **Mapi Group**

Market Access Challenges for Rare Diseases: Recent Developments in HTA and Reimbursement Decision

- Opportunities for early engagement with stakeholders, including clinical and economic evidence generation advice
- The current reimbursement environment in Europe
- Country-specific orphan drug programs and information requirements
- Existing challenges to reimbursement
- Demonstrating cost-effectiveness/budget impact
- Innovative schemes for reimbursement of orphan drugs

Céline Taveau, Associate Scientific Director, Mapi Group Real World Strategy & Analytics, **Mapi Group**

Global regulatory strategies: The emerging global Regulatory Pathway for Rare Disease treatments

- Regulatory strategies for successful orphan drug R&D
- Have regulations increased or shifted making designation or approval more difficult?
- Perspectives form Europe and US priority review vouchers

Patricia Anderson, Vice President, Regulatory Services, **Mapi Group**

17:00

End of Workshop Followed by Networking Drinks Hosted by **Mapi Group**

To register visit **terrapinn.com/orphan2016** and pick the pre-congress workshop option

OPENING KEYNOTE PLENARY

08:50 Chair's opening remarks

09:00 **ERN (European Reference Networks): A structure to promote clinical excellence, strength collaboration and facilitate research**

- Discussing the issues surrounding the DG Sante policies for a better future

Andrzej Rys, Health Systems, Medical products and Innovation Director, Health and Food Safety Directorate (DG Sante), **European Commission**

09:25 **Assessing the value for money of orphan medicines: Towards European consistency**

- Creating more effective and sustainable methods adapted to rare diseases
- Achieving both affordability of orphan medicines and sustainability of orphan innovative industry
- Getting towards transparency and consistency in frameworks on value and value for money
- Collaboration between all stakeholders, including research-based industry, payers, clinicians, and patients

Prof Lieven Annemans, Consultant in Health Economics and HTA & Professor of Health Economics, **Ghent University**

09:50 **What can the current debate in the EU tell us about the guidelines regarding 'significant benefit' (SB) of orphan medicines? Together with EMA's update on PRIME**

- Discussions on topics from the new notice
- Is innovation needed for orphan designation?
- Can SB be more closely defined?
- The need to clarify how sponsors should demonstrate SB over authorised medicines or other methods of treatment used in the EU
- How to determine when a new pharmaceutical form represents a SB?
- An update on PRIME

Kristina Larsson, Head of Orphan Medicines, **EMA**

10:30 **Networking refreshment break**

11:30 **INTERACTIVE ROUNDTABLES**
You must register to attend the roundtables, for more information please go to the end of the programme

13:00 **Networking lunch & poster session**

CLINICAL DEVELOPMENT

14:15 CHAIR OPENING REMARKS: **Dr Paul Strijbos,** Global Head Rare Disease Clinical Development, **Roche**

Innovative Clinical Trial Designs

14:25 **Small data: Navigating the challenges of rare disease clinical development to facilitate drug approval**

MODERATOR:
Dr Paul Strijbos, Global Head Rare Disease Clinical Development, **Roche**

SPEAKERS:
Martine Zimmermann, Vice President Global Regulatory Affairs, **Alexion**

Duncan Richards, VP Medicine Development Leader, MPC Therapeutic Aread, **GSK**

Scott Schliebner, Vice President, Scientific Affairs, **PRA Health Sciences**

PATIENT ACCESS

CHAIR OPENING REMARKS:

Global Access Strategies and Payers

European perspective followed up by individual presentations:

How can access for patients be improved and accelerated: What are the roadblocks?

Sheela Upadhyaya, Associate Director Highly Specialised Technologies Centre for Health Technology Evaluation, **NICE**

Carlo Tomino, Head of Clinical Research, **IRCCS San Raffaele**

Prof Rumen Stefanov, Director, **Institute of Rare Diseases & Dean,** Faculty of Public Health, **Medical University of Plovdiv, Bulgaria**

Francois Meyer, Advisor to the President, International Affairs, **HAS**

Kristin Svanqvist, Head of unit for HTA and reimbursement, **Norwegian Medicines Agency**

Kateřina Kopečková, Czech representative of COMP & Senior Medical Oncologist, Comprehensive Cancer Centre, **University Hospital of Motol, Czech Republic**

GENE THERAPY

CHAIR OPENING REMARKS: **Alastair Kent,** Director, **Genetic Alliance UK**

Unique Gene Therapy Challenges and Opportunities

Developing potential one-time, life-altering gene therapies for rare blinding conditions

Dr Romuald Corbau, Translational Research Lead, **Spark Therapeutics**

14:50 **Gene therapy: The cure?**

Lynne Fahey McGrath, Vice President Regulatory Affairs, **RegenXBio**

15:15 **GSK's journey in treating ADA-SCID through gene therapy: A regulatory perspective**

Dr Sven Kili, Vice President and Head of Gene Therapy Development, **GSK**

SCIENCE & STRATEGY

CHAIR OPENING REMARKS:

The Need to Collaborate to Commercialise

What incentives are there in Europe to continue to encourage orphan drug development?

Strengthening stakeholder relationships: Industry perspective on working with KOLs

Our strategy in implementing innovation in our R&D approaches to help develop more treatments to rare and orphan diseases

Dr Celine Plisson, Vice Chairman, **Recordati Rare Disease** Foundation',s Board of Directors & Medical Director, **Orphan Europe**

15:40	**Access to Treatment Catch 22: Removing barriers to develop competing products, the patient recruitment trap** **Daniel Mazzolenis,** Senior Medical Director, Global Oncology-Hematology, **INC Research**	**Stronger together: Belgium, Netherlands and Luxemburg acting as one to face orphan drugs** **Maggie De Block,** Minister of Social Affairs and Health, Policy unit of the Ministry of Social Affairs and Health, **Belgian Federal Government**	**Patient centric gene therapy research** **Samantha Parker,** Chief Patient Affairs/Health Policies Officer, **LYSOGENE**	**Going commercial from R&D as a small emerging company**
16:05	**Networking refreshment break**			
16:35	**Adaptive pathways in rare disease** **Stella Blackburn,** Vice President, Global Head of Risk Management, Real-World & Late Phase Research, **Quintiles**	**Orphan drug market access strategies in a generic space: The example of Homozygous familial hypercholesterolemia (HoFH)** **David Jakouloff,** Head of Global Market Access, **Aegerion Pharmaceuticals**	**What reimbursement models can we use for gene therapies?**	**How are regulatory changes affecting orphan drug approval and access** **Martine Zimmermann,** Vice President Global Regulatory Affairs, **Alexion**
17:00	**Outcome measures and studies to support clinical development** Senior representative, **Mapi Group**	**An evolving model: Incorporating registries and effective life cycle management when choosing an adaptive pathway approach** **Dr Vinciane Pirard,** Co-Chair Task Force RDs & ODs of EFPIA-EuropaBio and Director of Public Affairs EMEA, **Genzyme**	**Delivering gene therapy and plasma-based products for severe and life-threatening rare diseases** **Dr Timothy Miller,** CEO and President, **Abeona Therapeutics**	**The challenges of supporting medicines to acquired rare diseases and poor diagnosis** **Malcolm Allison,** GLT Head, Pulmonary hypertension, **Bayer**
17:25	**The evolving relationship between researchers and patient advocates — Where we've been, where we are now, and what can we expect?** **Juliet Moritz,** Executive Director, Strategic Development, **Premier research**	**Economic assessment of orphan drugs**	**An update on potentially transformative hematopoietic stem cell (HSC) gene therapy programs for severe genetic and rare diseases**	**Maintaining leadership, expertise and strategy in developing orphan drugs** **Stephanie Okey,** (Former Senior Vice President Head of North America), Senior VP, US General Manager, **Genzyme** & Board Member, **California Life Sciences Association (CLSA)**
17:50	**Treating SMA using an antisense oligonucleotide (ASO) platform** **Robert Bezar,** Director Medical, SMA Lead Europe and Canada, **Biogen**	**Do we have enough early access programmes in Europe?**	**An update on the first European Commission approved gene therapy: Lipoprotein lipase deficiency** **Dr Detlev Parow,** Head of the Department Care Management Development, **DAK-Gesundheit, Germany**	**Progress of the first and only treatment to treat the underlying cause of CF**
18:15	**Chair's closing remarks**			
18:30	**Networking Drinks Reception**			

SPOTLIGHT ON DAY 1 11:30 ROUNDTABLES

Covering a number of hot topics and delivered by patient groups, sponsors and leading solution providers, choose which **two roundtable** discussions you would like to join, for 'off the beaten track' learning and information you can't find online. Each session will last 45 minutes and will be repeated so you can join two discussions in the allotted time. **Every delegate must sign up to join,** for more information contact **Wing-yun Cheung** on **wing-yun.cheung@terrapinn.com**

ROUNDTABLES

11:30 DRUG DEVELOPMENT & MARKET CHALLENGES

Drug development in neuro-orphan diseases – Challenges & mitigations

Lynne Hughes, VP & Head, Centre of Excellence, Neurology, Therapeutic & Speciality Business Development, **Quintiles**

Marie Trad, Executive Medical Director, **Quintiles**

Building a patient centric model for repurposed drug development

Dr Tim Guilliams, Chief Executive, **Healx**

Sharing practical insights on the planning and execution of orphan drug clinical trials

Dr Stephan de la Motte, Chief Medical Advisor, **SynteractHCR**

The voice of the patient and caregiver in protocol design

Karen Kaucic, Senior Vice President & Global Head, **PPD® Consulting**

Using Real World Data (RWD) to break down barriers to diagnosing rare diseases

11:30 GENE THERAPY

The potential of CRISPR/Cas9 technology in orphan gene therapies, ethical considerations and the need to engage with rare disease patients earlier

Virginie Bros-Facer, Research Infrastructure Project Manager, **EURORDIS**

Expectation setting and educating on gene therapies for orphan diseases: Meeting people where they are

Michelle Berg, Vice President Patient Advocacy, **ABEONA Therapeutics**

Lentiviral vector gene therapy updates, challenges and opportunities

Manufacturing strategies supporting orphan gene therapies

Identifying rare disease patient populations for regulated pricing

To host or lead a roundtable please contact **wing-yun.cheung@terrapinn.com**

OPENING KEYNOTE PLENARY

09:00 — **Chair's opening remarks**

09:05 — **Shaping EU rare disease policies – What needs to change and what is the strategy behind it to achieve it?**

- Facilitating the adoption and implementation of national plans and strategies for rare diseases in European countries
- Status of patients' rights to access medicine in other European countries
- Establishing new mechanisms that would improve access while addressing the upcoming challenges in terms of sustainability of the healthcare systems

Yann Le Cam, Chief Executive Officer, **EURORDIS** – ***session to be finalised***

09:30 — **International cooperation on research into rare diseases**

- Addressing the huge unmet medical needs
- How do we overcome the challenges of small patient populations for the development of diagnostic/ therapeutic tools
- How will we achieve 200 new therapies for rare diseases by 2020? What has been achieved so far?

09:55 — **Dr Ruxandra Draghia-Akli,** Director of the Health Directorate at the Research DG, **European Commission**

Will all new medicines become orphan drugs in the era of personalised healthcare?

- How will gene therapy change the sector?
- How can personalised medicines reach patients whilst being affordable?
- When will it realistically take effect for our patients?

Dr Ségolène Aymé, Founder of Orphanet & Emeritus Research Director, **INSERM**

10:20 — **Networking refreshment break**

11:20 — **INTERACTIVE ROUNDTABLES**
You must register to attend the roundtables, for more information please go to the end of the programme.

12:05 — **Networking lunch & poster session**

	RARE DISEASE ADVOCACY WORLD	PITCH & PARTNER	RARE CANCERS
13:35	CHAIR OPENING REMARKS:	CHAIR OPENING REMARKS:	CHAIR OPENING REMARKS: **Dr Paolo Casali,** Head of the Adult Mesenchymal Tumour Medical Oncology Unit, **Istituto Nazionale Tumori**
13:40	**How early is early? Why patient engagement should be made early and part of a successful product plan** **Nicole Boice,** CEO, **Global Genes**	**Establishing international partnerships to leverage expertise of drug development and commercialisation** **Dr Klaus-Dieter Langner,** Chief Scientific Officer, **Grünenthal**	**Do we need to alter the pricing and reimbursement systems for rare cancers?**
14:05		**Pitch & Partner 1** **Overcoming FSHD together: Our goal to improve the lives of people with FSHD by developing a small molecule-based causal therapy** **David Dasberg,** Managing Director, **Facio Therapies**	**Using a platform trial model for more efficient evaluation of new agents and combinations in rare cancers** **Dirk Reitsma,** Vice President, Therapeutic Area Head, Hematology and Oncology, Global Project Development, **PPD**
14:20		**Pitch & Partner 2**	
14:35	**Does rare disease care always have to be DIY care for parents and carers?** **Kay Parkinson,** CEO & Founder, Alström Syndrome UK and Chair, **Cambridge Rare Disease Network**	**Pitch & Partner 3**	**The use of RADIANT-4 study designs for the development of drugs for rare Neuroendocrine tumours** **Alberto M. Pedroncelli,** VP and Head Rare Disease and NET, **Global Medical Affairs, Novartis**

14:50

Pitch & Partner 4

15:05

Importance of collaborative work - What pharma companies and patient advocacy groups could accomplish together in driving rare disease awareness, research and management

Taisia Isupov, Virology/Transplant Field Medical Sciences Lead, **BMS**

Pitch & Partner 5

Veneclexta/Venetoclax: A case study in development and regulatory strategy for CLL treatment

Sybil Skinner Robertson, Area Head Regulatory Affairs, AbbVie

15:20

Pitch & Partner 6

15:35

Networking refreshment break

Funding

16:05

Importance of partnering with patient groups for driving forward rare disease research

Flóra Raffai, Executive Director, **Findacure**

Investor panel: Hear from a number of VCs to better understand what they are looking for before they invest

Stephen M. Nagler, Executive Director, **MedPro Investors LLC**

Dr Raghuram Selvaraju, Managing Director, Head of Healthcare Equity Research, **Rodman & Renshaw**

Geraldine O'Keeffe, Partner, **Life Sciences Partners**

Christopher Egerton-Warburton, Partner, **Lion's Head Asset Management**

Compassionate use enabling the successful delivery of UCART19 therapy for paediatric cancers

Mathieu Simon, Executive Vice President, Chief Operating Officer, **Cellectis**

16:35

Organising and running clinical trials as a patient group: What are the possibilities?

Avril Daly, CEO, **Retina International**

An update from Amgen on BLINCYTO for paediatric ALL: Single-arm clinical trial methods

Dr Beth Barber, Therapeutic Area Head, Global Health Outcomes Oncology Therapeutics, **Amgen**

17:05

Chair's closing remarks

17:10

Close of Congress

SPOTLIGHT ON DAY 2 11:20 ROUNDTABLES

Covering a number of topics on regional provisions, patient engagement strategies and rare cancer challenges, choose one roundtable discussion you would like to join. Each session will last 45 minutes, every delegate must sign up to join, for more information contact **Wing-yun Cheung** on **wing-yun.cheung@terrapinn.com**

ROUNDTABLES

11:20 REGIONAL PROVISIONS FOR ORPHAN DRUGS

Developments in Eastern Europe: Unique challenges and opportunities

Pawel Wozniak, Managing Director, **Komtur Polska**

How to navigate the landscape of Orphan Drugs in Asia

Bruno Parenti, New Markets Director, **Orphan Europe**

HTA requirements and early access in MENA taking into account the current slowing regional economy

Tony Zbeidy, General Manager MENA, **Orphan-Europe Middle East**

11:20 PATIENT ENGAGEMENT & CLINICAL DEVELOPMENT

How to build a network of patient registries

How can a patient group more effectively approach sponsors

Vivian Fernandez, Director of Patient Advocacy, **REGENXBIO Inc.**

Challenges and guide to building a network of partners within and outside of Europe to accelerate more collaborative clinical trials

Creating collaborations among different rare disease groups that share similar mechanisms of actions, targets and/or therapies

Opportunities for greater patient engagement in the development process

11:20 RARE CANCERS

Creating reference networks and the use of data sharing in rare cancer drug development

Maximising market access of rare cancer drugs

Ulf Staginnus, Head Patient Access Oncology, **Shire**

Alternative and novel non-viral vector opportunities in orphan gene therapies

Richard Harbottle, Head of DNA Vector Research Group, **German Cancer Research Centre (DKFZ)**

Rare Cancer therapies enabling rare cancers to be become common through effective interventions: How to address this challenge for further line treatments and add-on therapies

Michael Zaiac, Head of Medical Affairs EMEA, **Celgene**

Rare cancer epidemiology - Burden of rare cancers from population based cancer registries and indicators for diagnosis and treatment

Gemma Gatta, Head of Unit at S.S. Evaluative Epidemiology, **Italian Institute of Tumors and EMA**

To host or lead a roundtable please contact **wing-yun.cheung@terrapinn.com**

“THE INFO PROVIDED WAS REALLY HELPFUL AND I WILL USE IT MY DAILY WORK AND THE NETWORKING WAD GREAT”

DIRECTOR, VALUE EVIDENCE LEAD RARE DISEASES, **GSK**

10 REASONS TO ATTEND

1. Experience the strongest agenda with our new advisory board and hear from **70+** high-level big **pharma, biotech, government, payers and regulator speakers including: EMA, European Commission, EURORDIS, Roche, GSK, Alexion, Biogen, Genzyme, Grünenthal, Celgene, Amgen, AbbVie, Spark Therapeutics, BMS, Orphan Europe, Global Genes, Findacure, Retina International** and many more

2. Unite with payers in an extended all **European payers session with countries represented from UK, France, Italy, Belgium, Bulgaria, Norway and Czech Republic** in an interactive panel to discuss patient access, assessment changes and pricing

3. Get inspired by **plenary speakers** including **Kristina Larsson,** Head of Orphan Medicines at the **EMA** addressing **significant benefit** and **PRIME, Andrzej Rys,** Health Systems, Medical products and Innovation Director at the European Commission on **European Reference Networks** to **Yann Le Cam** and **Dr Ségolène Aymé**

4. **Pre-congress workshops** will give you a full days' worth of content where you get the chance to learn topics from health economics, payer and evidence generation to unique challenges in **pricing, reimbursement & market access** for cell and gene therapies for rare diseases

5. **Rare cancer** orphan designations are increasing which is why we have dedicated a days' worth of content in the programme. Should the clinical end points and reimbursement structure be considered differently? Find out from **Novartis, Cellectis, Amgen, AbbVie, PPD** and any more

6. **Gene therapy** could revolutionalise the orphan sector but where are we? How would you price a cure? Hear from **Spark Therapeutics, DAK-Gesundheit, GSK, Abeona Therapeutics, RegenXBio, Lysogene** and plenty more

7. Our **Clinical Trial Symposium** is made up of industry experts including **Dr Paul Strijbos,** Global Head Rare Disease Clinical Development at Roche, **Martine Zimmermann,** Vice President Global Regulatory Affairs at Alexion, **Scott Schliebner,** Vice President, Scientific Affairs at PRA Health Sciences and **Duncan Richards,** VP Medicine Development Leader, MPC Therapeutic Aread, GSK to give a 360 industry perspective

8. An **investors panel** has been added into the Pitch & Partner track to allow more biotechs to meet the VC and funders they need to move forward

9. **Rare Advocacy World Track,** providing vital information regarding how patient groups are growing to co-develop orphan drugs from **EURORDIS, BMS, Findacure, Genetic Alliance UK, Retina International, Alström Syndrome UK, Cambridge Rare Disease Network, Global Genes** and many more

10. Network at the biggest and most established European Orphan Drug event with over **450+ attendees** during our extended networking breaks, interactive sessions and drink reception

Book your place early for the best rates **terrapinn.com/orphan2016**

“IT IS AN IMPORTANT MEETING THAT SHEDS LIGHT ON THE VALUE PATIENT ADVOCATES BRING TO THE DEVELOPMENT PROCESS”

MEDICINES DEVELOPMENT LEAD- RARE DISEASES, **PFIZER**

THE EXH

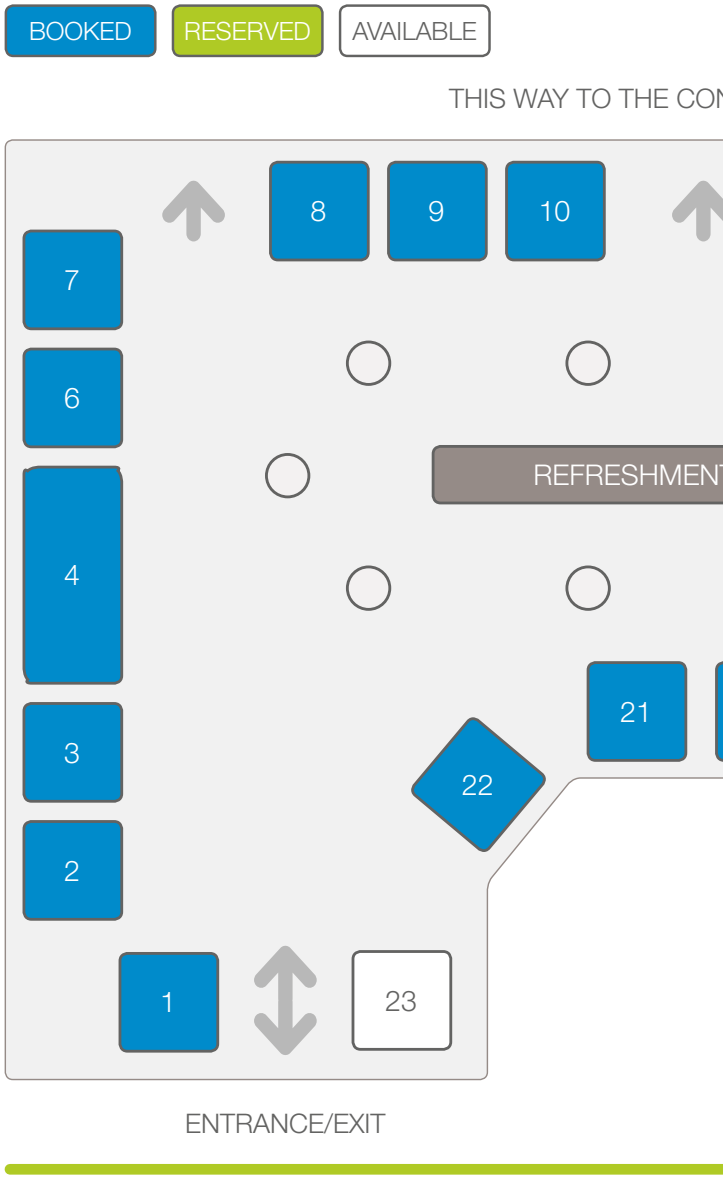
GET YOUR ORGAN

WHY SPONSOR?

- Make sales
- Debut new products
- Profile your brand
- Meet new business partners and suppliers
- Develop key relationships
- Educate pharma and biotech companies

WHO WILL YOU MEET?

- Innovators
- Disruptors
- Decision-Makers
- Influencers
- Big Pharma and Biotech
- Regulators
- Payers
- Patient Representatives
- Government
- Academia
- Non-Profit Organisations
- Industry Solution Providers

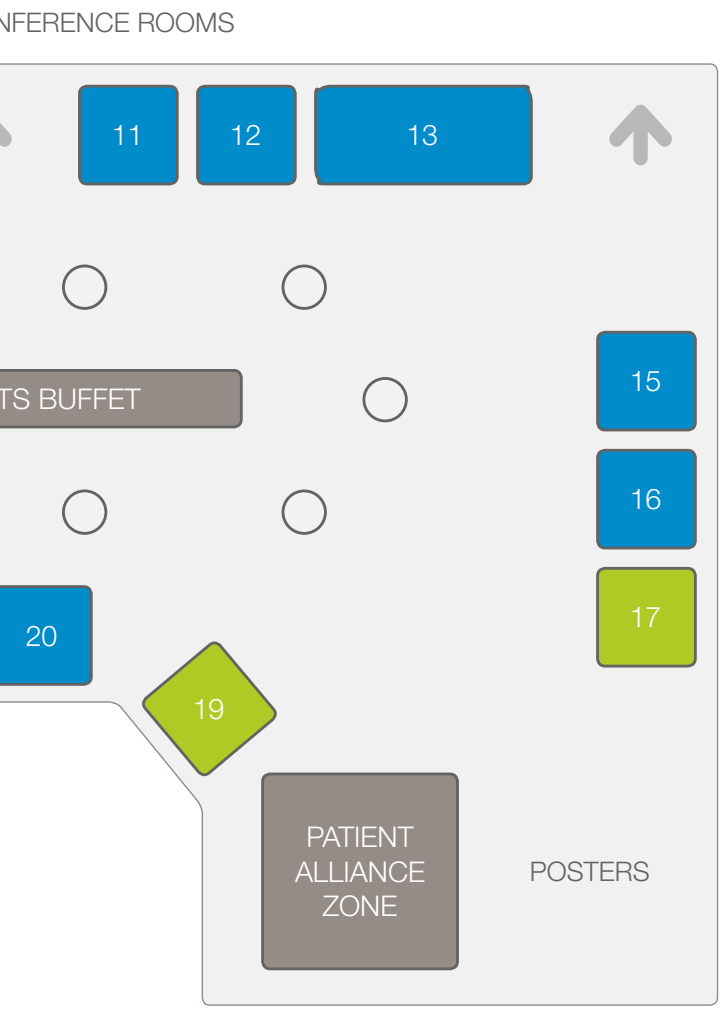


STAND	COMPANY	STAND	COMPANY	STAND	COMPANY
1	Oxford Pharmagenesis	4	Quintiles	8	Premier Research
2	SynteractHCR	6	INC Research	9	Comradis
3	Cello Health	7	Dolon	10	APL

To exhibit, sponsor or speak at next year's event call **Andy Mear**

HIBITION

IISATION NOTICED



THIS IS A PRIME OPPORTUNITY FOR:

- CROs
- CMOs
- Market Access Consultancies
- Regulatory Affairs Consultancies
- Big Pharma and Biotech
- Platform Technology Providers
- OEM / Equipment Providers

Sponsorship opportunities include:

- Programme presence – from plenary to track sessions
- Exhibition
- Drinks Reception Sponsor
- Patient Alliance Zone Sponsor
- Networking Break Sponsor
- Lanyard Sponsor
- Wi-Fi Sponsor
- Show Guide Sponsor

And many more…

STAND	COMPANY	STAND	COMPANY	STAND	COMPANY
11	Orphan Reach	15	PRA Health Sciences	21	Ergomed
12	PSR – Agility	16	World Courier	22	We Pharma
13	PPD	20	Coté Orphan		

rs on **+44 (0)20 7092 1228** or email **andrew.mears@terrapinn.com**

SPONSORSHIP & EXHIBITOR PACKAGES

Put your company at the forefront of the orphan drug industry and sponsor the World Orphan Drug Congress.

Begin reaping the benefits from…

- Showcasing new solutions
- Improving your brand awareness
- Meeting new prospective clients
- Maintaining relationships with existing customers

Benefits	Platinum	Gold	Silver	Bronze	Exhibitor
Keynote speaker slot	1				
Speaker slot		1			
Host a roundtable	1 or chair	1	1	1	
Moderate or speak on panel			1 or chair		
Chair half a day	1 or roundtable		1 or panel		
Pre-arranged onsite meetings	*	*			
Exhibition booth	12m^2	12m^2	6m^2	6m^2	6m^2
Delegate passes	5	4	3	2	2

Other sponsorship opportunities include:

- Drinks Reception (includes booth)
- Lanyards (includes booth)
- Branding throughout all refreshment breaks (includes booth)
- Exclusive Show Guide
- Bags and Badges
- Stationary
- Wi-Fi
- Water bottles

To exhibit, sponsor or speak at next year's event call
Andy Mears on **+44 (0)20 7092 1228** or email **andrew.mears@terrapinn.com**

YOUR NETWORKING OPPORTUNITIES

BIANCA GELDENHUYS
Networking manager

NETWORKING MANAGER

If you come in as a high-level sponsor, leading up to the event you will be assigned your very own Networking Manager who will be at your beck and call to help you set up meetings in advance of your arrival to the event. Your meeting schedule will be based on your desired targets on the delegate list. On-site, the Networking Manager will facilitate your meetings in a reserved area.

NETWORKING DRINK RECEPTION

It's not always about the conference sessions.

A networking drink reception in the evening of the 16th November will allow you to unwind with your peers and continue conversations in good company.

Explore the exhibition hall during dedicated breaks and meet with solution providers that offer innovative services and technologies. With over 30 exhibitors and patient groups represented, you are sure to meet those that can help you with any challenges you face.

PATIENT ADVOCACY ZONE

A great place for big pharma, biotechs, payers and more to network directly with patient representatives. A dedicated Poster Session during the lunch of day one will form the back drop to the Patient Advocacy Zone, and provide a very powerful way for Patient Advocacy Groups to display the work they undertake, the support they offer and the challenges facing their community.

NETWORKING LUNCHES

Our extended lunch periods in the exhibition hall will provide you with ample time to network between sessions. These lunch formats allow for more opportunities for casual conversations and introductions at your booth.

Book your place early for the best rates **terrapinn.com/orphan2016**

WORLD OrphanDrug Congress Europe 2016

Advancing the science and strategy of the world's future orphan drugs

ABOUT THE WORLD ORPHAN DRUG CONGRESS

The 7th annual World Orphan Drug Congress is the marketplace for orphan drug professionals looking at the complete value chain of orphan drug development, from clinical development and R&D to corporate development and market access.

Being Europe's largest Orphan Drug Congress, this event will provide a platform for you to showcase your thought leadership and expertise pharma, biotechs, payers, regulators and patient advocates

WHO SPONSORS?

- Pharma
- Biotech
- CROs
- Market Access
- Consultancies
- CMOs
- Platform Technology

WHO ATTENDS

CEOs, CSOs, Directors and Heads from:

- Big Pharma
- Biotechs
- National and International Government and Payers
- Patient Advocacy Groups and Non-Profit Organizations
- Academics and Research Institutions

JOB TITLES

- CEO/CSO/COO
- Head of (Clinical/Pre-clinical) Research
- Head of Discovery
- Head of Global Patients Insights and Engagement
- Head of Business Development
- Head of Global Market Access/Patient Access
- Head of Pricing and Reimbursement
- Head of Patient Advocacy/Patient Engagement & Public Affairs
- Head of Real-World & Late Phase Research
- Head of Risk Management
- Head of Regulatory Services

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RESERVE YOUR PLACE TODAY

The earlier you book, the more you'll save.

terrapinn.com/orphan2016

	Before 10 Jun 2016	Before 22 Jul 2016	Before 26 Aug 2016	Before 16 Sep 2016	Before 07 Oct 2016	Before 28 Oct 2016	Final Price
CONFERENCE + WORKSHOPS	€2070 **SAVE €1385**	€2430 **SAVE €1025**	€2780 **SAVE €675**	€2950 **SAVE €505**	€3100 **SAVE €355**	€3290 **SAVE €165**	€ 3,455
CONFERENCE + WORKSHOPS ACADEMIC & REGULATORY	€1035 **SAVE €695**	€1215 **SAVE €515**	€1390 **SAVE €340**	€1475 **SAVE €255**	€1550 **SAVE €180**	€1645 **SAVE €85**	€ 1,730
CONFERENCE	€1880 **SAVE €1275**	€2310 **SAVE €845**	€2670 **SAVE €485**	€2800 **SAVE €355**	€2920 **SAVE €235**	€3040 **SAVE €115**	€ 3,155
CONFERENCE ACADEMIC & REGULATORY	€950 **SAVE €630**	€1100 **SAVE €480**	€1260 **SAVE €320**	€1350 **SAVE €230**	€1420 **SAVE €160**	€1500 **SAVE €80**	€ 1,580
DISPLAY A SCIENTIFIC POSTER	€ 50						

BOOK NOW

Register at **terrapinn.com/orphan2016**

or call **+44 (0) 207 092 1210**

BRING YOUR TEAM

There's so much great content, you can't possibly cover it all alone!

Bring your team and get an extra discount.

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WORLD OrphanDrug Congress Europe 2016

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