16-17 November 2016
Pre-congress workshops: 15 November
Sheraton Airport Hotel, Brussels, Belgium

Strategy, advocacy and partnering for the orphan drug industry

Created by
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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

![Pie chart showing the distribution of attendees]

- 75% end users
- 30% Pharma
- 25% Biotech
- 20% Patient Advocacy Groups / Non-Profit / Charity
- 10% Government Organization and Payers
- 5% Market Access Providers
- 5% Other supply offerings
- 5% Clinical Research Organisations

Book your place early for the best rates terapinn.com/orphan2016
“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.

<table>
<thead>
<tr>
<th>Pre-congress workshops</th>
<th>Rare cancer</th>
<th>Gene therapy</th>
<th>Clinical Trial Symposium</th>
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<tr>
<td>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 &amp; market access for cell and gene therapies for rare diseases – pick this option on your registration to join.</td>
<td>orphan designsations 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.</td>
<td>Gene therapy could revolutionalise the orphan sector but where are we? How would you price a cure?</td>
<td>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.</td>
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| 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. |

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

Book your place early for the best rates [terrapinn.com/orphan2016](http://terrapinn.com/orphan2016)
# 2016 Agenda Overview

## Pre-Congress Workshops
**15th Nov**
- Morning Pre-Congress Workshop

## Day 1
**16th Nov**
- Plenary Sessions
- Networking Break
- Roundtables

## Day 2
**17th Nov**
- Plenary Sessions
- Networking Break
- Roundtables

## Afternoon Pre-Congress Workshop
**15th Nov**
- Clinical Development
- Patient Access
- Gene Therapy
- Science & Strategy
- Rare Advocacy
- World
- Pitch & Partner
- Rare Cancers

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Book your place early for the best rates [terrapinn.com/orphan2016](http://terrapinn.com/orphan2016)
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.

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

Networking lunch followed by afternoon workshop
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 from 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

DAY ONE
Wednesdays, 16th November 2016

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

14:00 CLINICAL DEVELOPMENT

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

Innovative Clinical Trial Designs
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 Area, GSK
Scott Schliebner, Vice President, Scientific Affairs, PRA Health Sciences

14:25 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, 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 Kopecková, Czech representative of COMP & Senior Medical Oncologist, Comprehensive Cancer Centre, University Hospital of Motol, Czech Republic

14:50 Developing potential one-time, life-altering gene therapies for rare blinding conditions
Dr Romuald Corbau, Translational Research Lead, Spark Therapeutics

15:15 Gene therapy: The cure?
Lynne Fahy McGrath, Vice President Regulatory Affairs, RegenXBio

15:30 Strengthening stakeholder relationships: Industry perspective on working with KOLs
Dr Celine Pålsson, Vice Chairman, Recordati Rare Disease Foundation & Board of Directors & Medical Director, Orphan Europe

16:15 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

16:30 Our strategy in implementing innovation in our R&D approaches to help develop more treatments to rare and orphan diseases
Dr Celine Pålsson, Vice Chairman, Recordati Rare Disease Foundation & Board of Directors & Medical Director, Orphan Europe

17:00 PATIENT ACCESS

17:15 CHAIR OPENING REMARKS:
Alastair Kent, Director, Genetic Alliance UK

Global Access Strategies and Payers

17:25 European perspective followed up by individual presentations:
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, University of Plovdiv, Bulgaria
Francois Meyer, Advisor to the President, International Affairs, HAS
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Dr Celine Pålsson, Vice Chairman, Recordati Rare Disease Foundation & Board of Directors & Medical Director, Orphan Europe

19:00 SCIENCE & STRATEGY

19:15 CHAIR OPENING REMARKS:
Alastair Kent, Director, Genetic Alliance UK

The Need to Collaborate to Commercialise

19:25 What incentives are there in Europe to continue to encourage orphan drug development?

19:50 Gene therapy: The cure?
Lynne Fahy McGrath, Vice President Regulatory Affairs, RegenXBio

20:00 Strengthening stakeholder relationships: Industry perspective on working with KOLs
Dr Celine Pålsson, Vice Chairman, Recordati Rare Disease Foundation & Board of Directors & Medical Director, Orphan Europe
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<tr>
<td>15:40</td>
<td>Access to Treatment Catch 22: Removing barriers to develop competing products, the patient recruitment trap</td>
<td>Daniel Mazzolenis, Senior Medical Director, Global Oncology-Hematology, INC Research</td>
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<td>16:05</td>
<td>Networking refreshment break</td>
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<tr>
<td>16:35</td>
<td>Adaptive pathways in rare disease</td>
<td>Stella Blackburn, Vice President, Global Head of Risk Management, Real-World &amp; Late Phase Research, Quintiles</td>
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<td>16:40</td>
<td>Patient Finding: How to target your campaign to find new patients? What can be done to actively improve diagnosis of orphan diseases and increase number of patients in care?</td>
<td>Dr Jama Nateqi, Chief Executive Officer, SYMPTOMA Pharmaceuticals</td>
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<tr>
<td>17:00</td>
<td>Outcome measures and studies to support clinical development</td>
<td>Senior representative, Mapi Group</td>
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<td>17:05</td>
<td>An evolving model: Incorporating registries and effective life cycle management when choosing an adaptive pathway approach</td>
<td>Dr Vinciane Pirard, Co-Chair Task Force RDs &amp; ODs of FPFA-EuropaBio and Director of Public Affairs EMEA, Genzyme</td>
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<td>17:25</td>
<td>The evolving relationship between researchers and patient advocates – Where we’ve been, where we are now, and what can we expect?</td>
<td>Juliet Moritz, Executive Director, Strategic Development, Premier Research</td>
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<td>17:50</td>
<td>Treating SMA using an antisense oligonucleotide (ASO) platform</td>
<td>Robert Bezar, Director Medical, SMA Lead Europe and Canada, Biogen</td>
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<td>18:15</td>
<td>Chair’s closing remarks</td>
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<td>18:30</td>
<td>Networking Drinks Reception</td>
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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

To host or lead a roundtable please contact wing-yun.cheung@terrapinn.com
## OPENING KEYNOTE PLENARY

### Chair’s opening remarks

**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*

### International cooperation on research into rare diseases

**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?

*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?

**09:55**

**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*

## DAY TWO

### THURSDAY, 17TH NOVEMBER 2016

#### NETWORKING BREAK

**10:20**

**Networking refreshment break**

#### INTERACTIVE ROUNDTABLES

**11:20**

**You must register to attend the roundtables, for more information please go to the end of the programme.**

#### NETWORKING LUNCH & POSTER SESSION

**12:05**

**Networking lunch & poster session**

#### RARE DISEASE

<table>
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<tr>
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<tr>
<td>13:35</td>
<td>CHAIR OPENING REMARKS:</td>
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<td>How early is early? Why patient engagement should be made early and part of a successful product plan</td>
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<td>The patient’s voice: The future way forward for the pharma community</td>
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<td>Does rare disease care always have to be DIY care for parents and carers?</td>
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#### PITCH & PARTNER

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<tr>
<td>13:40</td>
<td>Establishing international partnerships to leverage expertise of drug development and commercialisation</td>
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<tr>
<td>14:05</td>
<td>Pitch &amp; Partner 1</td>
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<td>14:20</td>
<td>Pitch &amp; Partner 2</td>
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<td>14:35</td>
<td>Pitch &amp; Partner 3</td>
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#### RARE CANCERS

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<td>Do we need to alter the pricing and reimbursement systems for rare cancers?</td>
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#### CHAIR OPENING REMARKS:

- **13:35**
  - Dr Paolo Casali, Head of the Adult Mesenchymal Tumour Medical Oncology Unit, Istituto Nazionale Tumori

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  - 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**

**14:05**

- **The patient’s voice: The future way forward for the pharma community**
  - **Alan Thomas, Founder, Atacsia a Fi / Ataxia and Me**

**14:20**

- **Does rare disease care always have to be DIY care for parents and carers?**
  - **Kay Parkinson, CEO, Cambridge Rare Disease Network, Director, Alstrom Europe, Founder, Alstrom Syndrome UK**

**14:35**

- **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, Fazio Therapies**

**14:35**

- **Pitch & Partner 2**

**14:35**

- **Pitch & Partner 3**

**14:35**

- **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**
DAY TWO  
THURSDAY, 17TH NOVEMBER 2016

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

15:20
Pitch & Partner 5

15:35
Networking refreshment break

16:05
Importance of partnering with patient groups for driving forward rare disease research
Flóra Raffai, Executive Director, Findacure

16:35
Organising and running clinical trials as a patient group: What are the possibilities?
Avril Daly, CEO, Retina International

16:50
Investor panel: Hear from a number of VCs to better understand what they are looking for before they invest
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
Dr Sara Nunez-Garcia, Principal, Sofinnova Partners

16:50
Compassionate use enabling the successful delivery of UCART19 therapy for paediatric cancers
Mathieu Simon, Executive Vice President, Chief Operating Officer, Cellectis

17:05
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

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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

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

DIRECTOR, VALUE EVIDENCE LEAD RARE DISEASES, GSK
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, EUROMIDIS, 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 revolutionise 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 Area, 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
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

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
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- CMOs
- Market Access Consultancies
- Regulatory Affairs Consultancies
- Big Pharma and Biotech
- Platform Technology Providers
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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
--- | ---
11 | Orphan Reach
15 | PRA Health Sciences
21 | Ergomed
12 | PSR – Agility
16 | World Courier
22 | WE Pharma
13 | PPD
20 | Coté Orphan

For more information, contact Andy Mears 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

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**

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<tr>
<th>Benefits</th>
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YOUR NETWORKING OPPORTUNITIES

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
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

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

[terrapinn.com/orphan2016](http://terrapinn.com/orphan2016)

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

**BRING YOUR TEAM**
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Bring your team and get an extra discount.
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