7th Annual

World Orphan Drug Congress USA 2017

April 20-21, 2017
Washington Marriott Wardman Park, Washington DC
Pre-conference workshop & seminar day on April 19, 2017

THE GLOBAL ORPHAN DRUG CONFERENCE AND EXPO

Together with:

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THE GLOBAL GATHERING FOR ORPHAN DRUGS

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OPEN LETTER TO THE ORPHAN DRUG INDUSTRY

Dear Orphan Drug Industry,

Where to begin? Since the inception of the World Orphan Drug Congress USA, both the event and the industry itself have grown exponentially. Stimulated by legislation offering blockbuster companies attractive incentives, Orphan Drug research sparked by the 1983 US Orphan Drug Act and quickly followed with acts in Singapore, Japan, Australia and the European Union, becoming a global market set to be valued at US$181.4 billion by 2020.

Over its 7-year history, the shape of the World Orphan Drug Congress USA has changed from a small conference focused on the US market, to a 4 track event, with over 1,000 global attendees, coming together to discuss the latest trends. What, for instance, is the value of real-world evidence in Orphan Drug research? Or how commercially acceptable is an even higher priced, potentially one-time treatment gene therapy? Is the regulatory and reimbursement pathway more or less the same as other orphan drugs?

In 2017, World Orphan Drug Congress USA is bringing together the worlds of Scientific Innovation and Commercialization, with 2 tracks in the main conference. By exploring trends in Digital Health, Biosimilars, Gene Therapy, and more, we will uncover the next scientific breakthrough. Examinations of Mergers, Commercialization, Marketing and the Regional Markets will provide insight into this billion-dollar business.

Rare Disease Advocacy World has also had a makeover in 2017. Focusing on the growing trend of advocacy groups influencing regulation, we have fantastic sessions highlighting the myriad of successes of advocacy in action influencing drug development. National Patient Organizations from the US, South Africa, Australia, Colombia, New Zealand, Japan, India and Europe will also be discussing the opportunities for rare disease research and development in their respective regions.

Know a biotech developing Gene Therapies or therapies for Pulmonary, Central Nervous System or Ultra Rare Diseases? Pitch and Partner is giving the opportunity for 34 biotech companies to showcase their orphan drug development.

That’s not all! Our pre-conference workshop day offers attendees the opportunity to delve deeper into pricing & reimbursement, forecasting techniques for orphan & ultra-orphan drugs, and integration of cross-functional community voice into development & commercialization of orphan drugs. The addition of two new half-day seminars will shine a light on the forward-looking environments of Next-Gen Therapies and Personalized Medicine.

We look forward to welcoming you to DC in April.

Yours,

André Singer
General Manager – Life Sciences
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EVENT AT A GLANCE

WEDNESDAY, APRIL 19 (PRE-CONFERENCE DAY)

9.00
Workshop 1: From a whisper to a scream: amplifying the community voice - the benefits and challenges of cross-functional integration of the community voice into development & commercialization

1.00
Workshop 2: Market forecasting techniques for orphan & ultra-orphan drugs - the balance between practical and theoretical concepts for orphan pricing and reimbursement

5.00
Workshop 3: Payers

7.00
Workshop 4: Personalized Medicine

THURSDAY, APRIL 20

9.00
Preliminary Sessions - Orphan Drugs 2020

10.30
Afternoon Refreshment Break - Speed Networking Session

11.20
World Orphan Drug Congress

12.40
Rounds

1.20
Networking Lunch

2.40
Real World Evidence

4.50
Preliminary Sessions - Pricing & Reimbursement

5.45
Cocktail Reception

FRIDAY, APRIL 21

9.00
Preliminary Sessions - Gene Therapy

10.30
Afternoon Refreshment Break - Networking Session

11.20
World Orphan Drug Congress

1.00
Networking Lunch

2.30
Digital Health & Diagnostics

3.10
National Patient Organizations

4.15
End of Conference
WORLD ORPHAN DRUG CONGRESS USA SPEAKERS

INDUSTRY

Chris Garabedian
Former CEO, Sarepta Therapeutics

Kathrine Bosley
Chief Executive Officer, Editas Medicine

Stephen Marcus
Chief Executive Officer, Cantex Pharmaceuticals

Timothy Walbert
Chairman, President and Chief Executive Officer, Horizon Pharmaceuticals

Donna Armentano
Executive Director, Global Head Gene Therapy, External R&D, Innovation, Pfizer

Wills Hughes-Wilson
Senior Vice President, Chief Patient Access Officer, Sobi

François Rader, MD
Board Chairman, Acceleron Pharma (AXNR), Former President, A11Bio, NPS Pharma

Terrie Livingston
Senior Director, Worldwide Medical, Biogen

Wildon Farwell
Senior Medical Director, Clinical Development, Biogen

Jonathan Heller
Executive Director, Chief Executive Officer, Amicus Therapeutics

John Crowley
Chairman and Chief Executive Officer, Summit Therapeutics

Glyn Edwards
Chief Executive Officer, Alexion Therapeutics

Michelle Berg
Vice President, Patient Advocacy, Alexion Therapeutics

Asie Brennan
Chief Medical Officer, Syngene Therapeutics

Barbara Wuebbels
Vice President, Patient Advocacy, The American PTC Therapeutics

Eric Pauwels
Senior Vice President and General Manager, The American, PTC Therapeutics

Sven Kiil
VP and Head of Gene Therapy Development, GSK

Nick Lenchisy
Chief Executive Officer, bluebird bio

Keith Dematties
Director, Center of Sourcing Excellence, Bristol-Myers Squibb

Jeffrey D. Marrazzo
Co-Founder and Chief Executive Officer, Spark Therapeutics

Mark Rothera
Chief Commercial Officer, PTC Therapeutics

Ken Much
President & CEO, Cognition Therapeutics, Former CEO, Chimerix

Howard Mayer
Senior Vice President, Head of Global Clinical Development, Shire Pharmaceuticals

Scott Rocklage
Former Chief Executive Officer, Epirus Biosciences

Christopher Missling
President and Chief Executive Officer, Alexion Pharmaceuticals

Jayne Gershonowitz
Senior Vice President and Chief Executive Officer, Lysonne

Karen Ascal
Founder and Chief Executive Officer, Lysonne

Nancy Parsons
Vice President, Commercial Operations, Pharmaceutical Products, Sigma-Tau Pharmaceuticals

Mary Frances Harmon
Head of Global Patient Advocacy, PTC Therapeutics

Angus Hogg
Vice President, Head of Global Patient Advocacy, Shire

Francesca Cook
Director, Pricing and Market Access, Regeneron

Michael Gottlieb
Global President, Pfizer Rare Disease

Ethan Perstein
Founder and Chief Executive Officer, PeraLife

John Leonard
Chief Medical Officer, Intella

Mark De Boer
Chief Medical Officer, PF Pharma

Takashi Kinoshita
Chief Scientific Officer, Selecta Biosciences

Thomas J. Farrell
President and Chief Executive Officer, Bolusum Pharmaceuticals

Ragnar Klingberg
Chief Executive Officer, Emeralded

Amber Salzman
CEO & President, Adrenon Biotechnologies

Marc Martinelli
Chief Executive Officer, Mirysa Therapeutics

Diego Ardigo
STM Project Leader, Corporate Drug Development, R&D, Chiesi Pharmaceuticals

Molly Burich
Associate Director of Public Policy, Biosimilars, Pipeline and Reimbursement, Hematology, Global Health & Value, Pfizer

Andreas M. Platt
Senior Director, Team Lead, Outcomes & Evidence, Hematology, Global Health & Value, Pfizer

Walter Kowoniuk
Director, Strategy and Operations, Pﬁzer

Johan Heylen
Chief Commercial Officer, Ablynx

ASSOCIATION & OTHERS

Stephen Marcus
Chief Executive Officer, Cantex Pharmaceuticals

Marc Wurtz
VP, A Chair Financial Officer, Saville Pharmaceuticals

Gary Mathias
Chief Executive Officer, Thelos Pharmaceuticals

John Maslowski
Founder, KOM Biotech

Raul Insa
Founder, Sobi Biotech

S H Lau
Chief Executive Officer, New Biologic LLC

Sylvia Bella
President and Chief Executive Officer, Synlogic

Karen Aiach
President and Chief Executive Officer, PTC Therapeutics

Karen Watson
Chief Executive Officer, EveryLife

Glyn Edwards
Chief Executive Officer, Epirus Biosciences

Eric Gascho
Chief Executive Officer, EVERDRIS

Pat Furlong
Founding President and Chief Executive Officer, Parent Project Muscular Dystrophy

Kenneth Hobdy
President, Cure SMA

Vignesh Ganapathy
Associate Director of Advocacy & Government Relations, EveryLife Foundation for Rare Diseases

Join these speakers at World Orphan Drug Congress USA by booking your ticket today at www.terrapinn.com/WODC2017
The orphan drug industry has matured considerably in the past decade. With that, more orphan drugs get approved, while pharma and biotechs get closer to launch new and innovative therapies for rare disease patients. Their focus has shifted from early stage challenges to strategies on commercialization, marketing, pricing and reimbursement.

As these companies move from pre-revenue to commercial and start recouping the investments from years of R&D, more capital starts to flood into promising new biotechs, and consolidation starts to rise. VCs investment in orphan drugs biotechs has grown from $101 million in 2005 to over $346 million in 2014, according to Pitch Book.

From VCs to pharma investors, funding is a much more optimistic reality for orphan drug biotechs. However there are still major challenges and uncertainties in terms of how investors assess these investment opportunities. Evolving regulatory guidelines, increased scrutiny over orphan drugs high price, policies risk, and the common R&D failures, are some of the key areas impacting investment decisions in this market.

The lack of a platform for orphan drug biotechs to meet and present their development programs to investors contributed to the launch of Pitch & Partner at the World Orphan Drug Congress USA. In 2017, not only 20+ biotechs startups will be pitching their companies, but a groups of VCs, a new orphan drug/rare disease investment index and successfully funded biotechs will present at Pitch & Partner.

Investing in the orphan drug space – targeting big business by treating a small number of patients

- Why is this space attractive for investors?
- Leveraging faster approval times, economic incentives, lower production costs, scarcity of treatments and the increased number of diseases diagnosed yearly
- Risk assessment of potential market failure to drive successful investments
- Investing in early vs. late stage rare disease companies
- What are common mistakes companies make when pitching for investment?
- How can companies be more attractive for investment?

Presenting the Global Orphan/Rare Diseases Biotechnology Index - an investment perspective of the hottest orphan drugs biotechs

- Rare-disease biotech companies surge in value – a bubble at the horizon?
- Will the orphan drug market continue its consolidation trend?
- Have investment and activities in the orphan drug space reached its peak?
- Trends and topics that will impact the orphan drugs market in the next 5 years

From venture capital to the launch of a rare disease biotech – unlocking the druggable mechanisms that regulate disease genes

- Long-term potential to address complex genetic diseases through The Fulcrum Product Engine
- From VC to a fully operational biotechnology company. Keys to successfully establishing a new biotech in a highly competitive landscape
- Partnership and collaboration opportunities to advance rare diseases’ research and maximize the potential of the Product Engine

EXECUTIVES & BIOTECHS CONFIRMED TO PITCH INCLUDE

- Ragnar Klingberg, Chief Executive Officer, EmeraMed
- Marc Martinek, Chief Executive Officer, Minoryx Therapeutics
- Stephen Marcus, Chief Executive Officer, Cantex Pharmaceuticals
- Amber Salzman, Chief Executive Officer, Adverum Bioteotechnologies
- Marc Wolff, EVP & Chief Financial Officer, Sancilio Pharmaceuticals
- Gary Mathias, Chief Executive Officer, Thetis Pharmaceuticals
- S H Lau, Chief Executive Officer, New Biotic LLC
- John Maslowski, Chief Executive Officer, Fibrocell

Apply by contacting André Singer at andre.singer@terrapinn or call +1 646 619 1797
PRE-CONFERENCE DAY WORKSHOPS

Preconference workshops will be held on April 19th at Washington Marriott Wardman Park, prior to the official start of World Orphan Drug Congress USA 2017.

Workshops include:

**WORKSHOP 1**

From a whisper to scream: amplifying the community voice (CV) – the benefits and challenges of cross-functional integration of the CV into development & commercialization

**April 19th, 2017 at 9am**

**WORKSHOP 2**

When and how can Expanded Access Programs (EAP) support your drug development

**April 19th, 2017 at 9am**

**WORKSHOP A**

Predicting and influencing the market potential of orphan drugs: Embracing the interdependence of forecasts and early development decisions

**April 19th, 2017 at 1pm**

To attend a pre-conference day workshop, register for a 3 conference day pass at www.terrapinn.com/WODC2017
SEMINAR 1 KOL/ PHYSICIANS

08:55 Opening remarks

09:00 Improving the care of children with rare diseases identified by newborn screening
Jennifer Kwon, Associate Professor of Neurology and Pediatrics, University of Rochester

09:20 Driving research and accelerating treatments for patients with Castlemann Disease
David Fajgenbaum, Assistant Professor of Medicine, University of Pennsylvania; Executive Director, Castlemann Disease Collaborative Network

09:40 Gene therapy and other novel treatment strategies for inherited muscle and nerve disorders
Jerry R. Mendell, M.D., Director, Center for Gene Therapy, The Research Institute at Nationwide Children’s Hospital

10:00 The age of gene therapy; the impact on cystic fibrosis and other rare disorders
James Wilson, Professor of Pathology and Laboratory Medicine Director, Orphan Disease Center, University of Pennsylvania

10:20 Networking coffee break

11:00 Creating an accelerated research environment for rare neurological diseases
Florian Eichler, M.D., Associate Professor of Neurology, Massachusetts General Hospital and Harvard Medical School

11:30 Driving research and accelerating treatments for patients with Hermansky-Pudlak syndrome (HPS)
Samuel Seward, MD, Site Chair, Department of Medicine, Mount Sinai

12:00 Networking Lunch

SEMINAR 3 PAYERS

1:00 Opening remarks

1:10 Alternative funding methods that will spur innovation and not rely solely on payers for the reimbursement of orphan drugs
Peter Dehnel, Medical Director, Blue Cross- Blue Shield of Minnesota

1:35 Creating true value for patients- severity as a measurement to prioritize reimbursement of orphan drugs in Norway
Einar Andreassen, Senior Adviser, Norwegian Medicines Agency

2:00 Ultra orphan drugs: the NHS model for managing extremely rare diseases
Edmund Jessop, Medical adviser, NHS England

2:25 Networking coffee break

3:00 Proposed new approaches for the affordability of orphan drugs and the sustainability of the healthcare system in Germany
Detlev Parow, Head, Care Management Development, D.A.K.

3:25 Strengthening evidence-based decisions and the benefit assessment of orphan drugs in Germany
Meriem Bouslouk, Officer, Pharmacoeconomics Department Federal Joint Committee (G-BA), Germany

3:50 Medicaid state overview and the impact on orphan drug reimbursement in the post-election era
Jeff Myers, President & CEO, Medicaid Health Plans of America

4:15 Current landscape for tackling increased drug costs through more reasonable reimbursement
Invited: John Coster, Director, Division of Pharmacy, Center for Medicare and Medicaid Services (CMS)

4:40 Pricing and reimbursement challenges to access European markets and ways to present value and evidence to payers and regulators
Anna Bucsics, Advisor, MOCA (Mechanism of Coordinated Access to Orphan Medicinal Products)

5:00 Welcome drinks reception
SCIENTIFIC INNOVATION SEMINARS (Wednesday, April 19th, 2017)

SEMINAR 2 NEXT GENERATION THERAPIES

08:55 Opening remarks

09:00 CRISPR-engineered animals in phenotypic screens to identify orphan drug candidates
  Ethan Perlstein, Founder and Chief Executive Officer, Perlara

09:20 Using CRISPR-Cas9 to develop drugs for rare liver disorders and other rare diseases
  John Leonard, Chief Medical Officer, Intellia

09:40 Anti-CD40 monoclonal antibody therapy for the treatment of chronic inflammatory orphan diseases
  Mark De Boe, Chief Executive Officer, FF Pharma

10:00 Synthetic vaccine particles in the prevention of anti-Drug antibodies in rare disease treatments
  Takashi Kishimoto, Chief Scientific Officer, Selecta Biosciences

10:20 Networking coffee break

11:00 Synthetic biology approaches in the gut microbiome to treat inborn errors of metabolism (IEMs)
  Nikhil Nair, Assistant Professor, Department of Chemical and Biological Engineering, Tufts University

11:30 A new paradigm in Hematopoietic stem cell transplantation (HSCT) for orphan inherited blood disorders
  Thomas J. Farrell, President and Chief Executive Officer, Bellicum Pharmaceuticals

12:00 Networking Lunch

SEMINAR 4 PERSONALIZED MEDICINE

13:05 The NIH Undiagnosed Diseases Program and Network: a cross-disciplinary approach to disease diagnosis, new disease identification and the application of precision medicine to patient care
  William Gahl, Clinical Director, National Human Genome Research Institute, Head, Undiagnosed Diseases Program

13:35 Building the rare disease ecosystem and enabling innovation and drug discovery through data collaboration
  Adam Resnick, Head of Precision Medicine Group, Children’s Hospital of Philadelphia

2:05 Personalized to precision – Studying large cohorts of people under precision medicine to identify drug repurposing for rare diseases and gain insight into more common disorders
  Murray Brilliant, Director, Center for Human Genetics, Marshfield Clinic

Get your 3-day ticket early to get the best price at www.terrapinn.com/WODC2017
WHAT IS THE FUTURE OF ORPHAN DRUGS?

At this year’s event, we are diving straight into discussions around the future of this billion-dollar industry. Chief Executive Officers from Horizon Therapeutics and Aegerion Pharmaceuticals will open the conference with their predictions for a more innovative, patient centric Orphan Drug industry.

CHAIRPERSON’S OPENING REMARKS

KEYNOTE CEO PANEL: Driving the future of the orphan drug market – adapting to change, fostering innovation and transforming patients’ lives

- Building a long-term vision - bringing leadership and past experiences to guide the direction of a rare disease company
- Nurturing and growing a business while staying responsive to patients’ needs
- What does the future of orphan drugs look like and what can patients and other stakeholders expect to see in the next 5 years?

MODERATED BY: Sybrand Pretorius, Senior Vice President and Chief Scientific Officer, PAREXEL

Next, our expert panel will navigate a changing regulatory landscape with discussions into PDUFA VI and the 21st Century Cures Act; taking into account the latest in drug development and the increasing involvement of patients in legislation

KEYNOTE PANEL: Prescription Drug User Fee Act (PDUFA VI) and 21ST Century Cures – recent and pending legislative efforts to make drugs better for patients

- Making the drug development process better and more efficient for patients through biomarker development, precision medicine, patient reported outcomes and RWE for regulatory uses
- Engagement of patients in regulatory decision-making, streamlined drug development and faster applications review - what this means for drug manufacturers
- How can legislative pieces be improved to continue getting support from Congress?

Book your delegate ticket today at www.terrapinn.com/WODC2017
Pricing & Reimbursement

The FDA approved 21 Orphan Drugs in 2015, and close to half of the novel drugs approved in 2014 were to treat rare diseases. This is indicative of a new trend; biopharma companies all over the world are identifying orphan drugs as an attractive, innovative development pathway. Many factors have fueled this trend, most notably regulatory incentives such as market exclusivity, priority review and tax credits, to name a few. The emergence of Next-Gen Therapies, such as Gene Therapies, has sparked a need to foster more innovative models like pay-for-performance. Increased scrutiny on drug pricing has also called into question if current-pricing models can effectively assess the value of orphan drugs.

Keynote Address: How can different stakeholders pay for successes in gene therapy?
- How did GSK and AIFA reach a price for Strimvelis and what other pricing models were considered?
- Applicability of pay-for-performance to other parts of the world that don’t have a single payer system
- Will pay-for-performance foster gene therapy commercialization and change the evaluation of premium pricing for other drugs?

Keynote Payers’ Panel: Effectively assessing the value of orphan drugs in a time of increased scrutiny of drug pricing
- How can payers reward industry for true innovation without breaking the bank?
- Effectively assessing international referencing pricing, differential pricing, pay-for-performance and HTA to increase access for patients
- How will pay-for-performance models impact the reimbursement of all orphan drugs?
- Will the changing political landscape affect orphan drug reimbursement?
- If robust data is not yet available, why should payers reimburse at premium levels?

Make sure you don’t miss anything – bring your team by taking advantage of our group discounts on www.terrapinn.com/WODC2017
KEYNOTE ADDRESS: THE POTENTIAL OF BIG PHARMA IN ACCELERATING RARE DISEASE BREAKTHROUGHS

- Joint drug discovery program between researchers and scientists through the ‘Rare Diseases Research Consortium’
- Gene therapy as the catalyst for innovation in rare disease therapeutics
- Powering rare disease therapeutics and patient engagement through the product lifecycle
- Where will the orphan drug market be in 2020?
M&A AND COMMERCIALIZATION

The global orphan drug market is set to be worth US$181.4 billion by 2020. Industry heavyweights from Pfizer, Cello Health, Summit Therapeutics and the former President & CEO from NPS Pharma, will offer expert insight on partnering, licensing & collaboration, mergers and pre-launch strategies, as pathways to commercialization.

A pharma perspective on partnerships with external innovators in the life sciences
- Why and how pharma pursues external partners
- How to prepare for “successful” partnership discussions with pharma – it’s a matter of fit
- What you should expect from your pharma counterpart during and after diligence

Creating significant shareholder value: turning around NPS, two rare diseases products launched and a $5.2B sale to Shire
- Defining a new business model and a values-based culture
- Repurposing two products from large indications to niche orphans with no competition
- Managing the adversities along the way

Orchestrating successful pre-launch strategies for orphan drugs: overcoming challenges, optimizing approaches
- Leveraging insights – knowing your market, understanding your patients
- Demonstrating value – scientific differentiation, clinical needs, commercial relevance
- Honing strategies – assessing market dynamics and potential, optimizing your path to market
- Telling the story – multi-channel initiatives to engage and educate stakeholders

Transforming a R&D biotech into a fully vertically integrated company in the ultra-orphan space
- Setting up a commercial infrastructure (~100 people) for the launch of a treatment for acquired TTP in North America and Europe
- Implementing a supply strategy, from commercial manufacturing to last-mile distribution
- Rolling out a marketing and communication strategy and defining the appropriate evidence pricing
- Preparing out-licensing options for Japan and rest of world

PATIENT-CENTRIC RESEARCH

The Orphan Drug industry has long been influenced, and pushed forward, by patients and advocates. With the advent of technology allowing the patient to provide real-world data and the growing trend of advocacy in action, the orphan drug research ecosystem is evolving to become patient-centric.

Evolving the relationships in the orphan drug ecosystem to achieve truly patient-centric clinical research
- Where we’ve been, where we are now, and what can we expect?
- What does patient centricity really mean?
- Optimizing the relationship between researchers and patient advocates to strengthen trial design, enrollment, adherence and outcomes

Last year, we interviewed Mark Rothera, Chief Commercial Officer of PTC Therapeutics to share with us insights on the game-changer Translarna for over 2,000 rare diseases.

Similarly, patient organizations, big or small, have an important role in ensuring the patient voice and the realities of living with Duchenne on a daily basis are understood, the impact it has on families, of having a boy that loses functionality on a daily basis and that needs to be cared for on a 24-hour basis, is heard and understood.

TAKEN FROM AN INTERVIEW WITH MARK ROTHERA, CHIEF COMMERCIAL OFFICER, PTC THERAPEUTICS, BY TOTAL ORPHAN DRUGS EARLIER THIS YEAR.

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REAL WORLD EVIDENCE

Thanks to scientific and technological advances, the orphan drug industry now has access to tools that leverage Real-World Evidence (RWE). These tools are helping to overcome challenges unique to rare disease trials, such as patient recruitment. Patients can now record real-world data, providing valuable insight about the disease lifecycle; enabling more efficient orphan drug development.

From helping trial recruitment to developing natural history studies – creating patient registries that are meaningful in drug development
• Patients, industry and other stakeholders’ contribution to produce better quality registry data
• What technology developments exist to optimize data collection, analysis and curation?
• What are the characteristics of a high quality registry?

Improving the use of RWE for regulatory decisions – steps to support new indications and label expansions
• Is the regulatory environment conducive to the effective incorporation and use of RWE?
• Recent policy proposals and discussions regarding use of RWE by FDA
• Opportunities for leveraging RWE to improve evidence development for regulatory decisions in rare diseases.
• Approaches for public and private partnerships to support a national data and evidence resource for developing RWE

How can patients build better registries themselves?
• De-risking patient’s data collection, increasing clinical trial participation and supporting translational research
• Applications of registry data in industry incentives, clinical trial design and recruitment, surveys and focus groups and patient focused drug development meetings with regulators, and payers
• Overcoming challenges related to funding, type of nature of the data collected, utilization and distribution and access and location

MARKETING & COMPETITIVENESS

In any industry, staying competitive is key to maintaining or growing your market share. Once again, patient-centricity is impacting the industry by changing the way biopharma stays ahead. Strong patient engagement; digital platforms as marketing tools, to share information and amplify messages, and organic patient-industry collaborations are enabling companies such as Sigma Tau and Shire stay one step ahead.

Marketing strategies to stay competitive during post-launch, ensure brand loyalty and market share
• Leveraging patient associations, patient services, remote engagement and physician relationships to drive the ongoing use of treatments?
• How to innovate while keeping launched products competitive? How to do this in therapeutic categories with multiple treatment options?
• Effective life cycle management of orphan drugs and the integrated work with medical affairs and advocacy

Doing digital right: raising awareness of MPS through the launch of digital paper planes
• Partnering with the International MPS Society and The US MPS Society to raise the profile of MPS (mucopolysaccharide) diseases
• Enabling people to share information about MPS globally aiming to shorten the path to diagnosis
• Using a social media platform to amplify messages and post awareness messages simultaneously

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It was an impressive collection of stakeholders from both industry & patient groups and I very much appreciated the opportunity to attend with a patient pass. The roundtables featured great discussions.

Stephanie Fischer, Patient
GENE THERAPY

Identifying the cause of a disease, not just the symptoms, has been made possible through incredible scientific advancement in understanding our DNA. This in turn has led to the emergence of Gene Therapies.

Gene Therapy Giants; GSK, uniQure, Spark Therapeutics and bluebird bio will tell you how they have broken through barriers of gene therapy development for rare diseases.

KEYNOTE ADDRESS: Development of lifesaving gene therapies through collaboration between academic discovery, scientific research, medical practice and product development
- How can cell and gene therapy programs be developed through collaborative efforts with academia and lead to more approvals?
- Designing a trial when using a patient’s own gene modified stem cells to correct immunodeficiency’s
- Overcoming challenges of small population sample, skepticism of the community and high pricing reputation

KEYNOTE ADDRESS: Adeno-associated virus (AAV)-based gene therapies for rare, chronic and degenerative diseases
- Using therapeutic miRNAs for the development of a gene therapy for Huntington’s Disease
- Innovative and modular technology platform for hemophilia gene therapies
- Strategic collaboration with Bristol-Myers Squibb to advance gene therapies for cardiovascular disease

KEYNOTE ADDRESS: Driving towards success in gene therapy development: pioneering a different approach for differentiated outcomes in rare diseases
- Inherited Retinal Disease (IRD): a collaborative effort between scientists, patients and regulators during a decades-long journey
- Building a fully-integrated company to prepare for a potential first-ever U.S launch
- Considering potential new value propositions for patients, payers and the health care system
- Gene therapy platform development: lessons learned

The future of Orphan Drugs is on show at this year’s World Orphan Drug Congress USA. Bring your team by registering today at www.terrapinn.com/WODC2017

KEYNOTE ADDRESS: TRANSFORMING THE LIFE OF CHILDREN ON A GENETIC DEATH SENTENCE BY GIVING THEM A BLUEBIRD DAY
- Overview of the clinical development program for cerebral adrenoleukodystrophy and beta-thalassemia
- Overcoming challenges in the design and interpretation of the human clinical trials, immunogenicity, and the selection of the best primary efficacy end point to progress clinical development
- How to balance the science and medicine being developed with the expectations of payers and regulators?
We have already taken a look at how advancements in technology have delivered solutions to biopharma; for example, how real-world evidence is providing unique insight into a disease lifecycle. Not to be outdone, scientific innovations are also pushing the orphan drug industry forward. From the nonsense mutation read-through agent, which has been so pivotal in the search for a treatment for DMD, to the first approved stem cell treatment for a rare eye disorder in Europe; the science in orphan drugs is out of this world.

Mark Rothera  
Chief Commercial Officer,  
PTC Therapeutics

Tackling the rare disease deficit – the advent of oral mutation specific therapies
- The nonsense mutation read-through agent represents a novel platform for treating many rare diseases
- Insights from Translarna (ataluren) studies in nonsense mutation DMD, CF and beyond
- The alternative splicing platform and its application to SMA & Huntington’s Disease

Wildon Farwell  
Senior Medical Director,  
Clinical Development,  
Biogen

Antisense technology: Biogen and IONIS collaboration to develop the first treatment for Spinal Muscular Atrophy (SMA)
- Intrathecal Anti-Sense Oligonucleotides (ASOs) for CNS Diseases
- Clinical development program to demonstrate improvement in range of SMA patients
- NURTURE Study in Pre-symptomatic SMA infants: multi-disciplinary collaboration

Diego Ardigo  
ATMP Project Leader,  
Corporate Drug Development, R&D, Chiesi Pharmaceuticals

The journey towards the approval of EU’s first stem cell treatment for a rare eye disorder
- Using stem cells to reconstruct the epithelium that covers the corneal surface
- Demonstrating the safety and efficacy of stem cells in a clinical setting
- Manufacturing and logistics considerations for collection of the sample and delivery of the final treatment to the patient
- Fostering innovation in the rare disease field through stem cell therapies

The orphan drug market is truly global, and so too is the World Orphan Drug Congress USA. This is the only event where regional markets are dissected and analyzed. National Patient Organizations from the US, South Africa, Australia, Colombia, New Zealand, Japan, India and Europe provide global insight in Rare Disease Advocacy World. We will also be casting a business-eye over Europe, Canada and LatAm; dissecting market access, drug review to reimbursement, regulatory frameworks and more.

Trevor Richter  
Director, Common Drug Review and Optimal Use of Drugs, CADTH

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

Ana Maria Serrato  
Country Manager,  
Aegerion Pharmaceuticals Colombia

Evolving access for orphan drugs in Canada: orphan drug evaluation at CADTH
- Access to orphan drugs in Canada
- Assessment of orphan drugs for reimbursement at CADTH
- Current and future challenges in the evaluation of orphan drugs for reimbursement in Canada

Orphan drugs and rare diseases in Latin America
- New legislations and trends addressing the health care system sustainability of the region
- From product centric-to patient-centric
- Is LATAM still a key player for rare disease companies? Yes, but…

Join us in Washington D.C. in April by booking your ticket online today at www.terrapinn.com/WODC2017
**DIGITAL HEALTH & DIAGNOSTICS**

From global sharing of clinical trial, to analytics and the use of genomic sequencing in rare disease diagnostics, the worlds of healthcare and technology are colliding. Cloud technology, big data analytics, Telehealth and genomics are revolutionizing how we manage rare diseases. Technology is fueling the trend of patient-centricity in research and in regulation and its impacting clinical development, patient discovery, diagnostics and commercialization.

**Healthcare digitalization: Cloud technology and Telehealth revolutionizing rare disease management**

- Improving the interactions between researchers, connecting patients with clinical studies and patient groups
- Creating virtual video rooms with patients to evaluate biometric data and patient reported outcomes
- Optimized and faster diagnostics by enabling researchers at different locations to examine the same data and share real-time feedback

**BIG data in “small” diseases- increasing the likelihood of success of ALS clinical trials and its applicability to other rare disorders**

- Working with pharma and other stakeholders to make anonymized data available to the global R&D community
- Big data to understand natural history, disease heterogeneity, novel biomarkers and pathways of disease
- The ALS Prediction Prize program: using algorithms to predict the progression of the disease over time

**How technologies that monitor rare disease patients can impact clinical development, patient adherence and improve PROs?**

- Mobility platforms and wearables giving clinicians the opportunity to have a better understanding of the disease progression in order to adjust medication dosages and identify potential emergencies
- Reducing hospital visits by enabling a more real-time monitoring of the patient and the actions doctors can take before visits
- Increasing patient engagement by achieving a higher understanding and involvement in their health

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**BIOSIMILARS**

The late ’90s saw significant advancement in orphan drugs thanks to government-supported financial incentives. 15 years later, $33 billion of orphan biologics are set to go off patent opening a great opportunity for specialty pharmacy and major industry players alike – **Orphan Biosimilars**.

Despite a significant number of orphan products that have already reached loss of exclusivity, as of yet, there is no true orphan biosimilar on the market. This is largely due to a lack of a regulatory pathway, manufacturing hurdles and competition.

This is set to change. Stay ahead of this inevitable trend.

**Biosimilar orphans – preparing for an inevitable reality**

- Benefits of biosimilar trials for patients that don’t have access to orphan drugs and that know will get the drug and not a placebo
- Overcoming challenges in patient recruitment in small patient populations that are loyal to the originators and where KOLs have limited knowledge on phase III trials
- The need for clarification from regulators on interchangeability studies and the expectations for the development of orphan biosimilars

**Is the orphan drug market ready to embrace the biosimilar landscape?**

- Are orphan drug biosimilars needed in order to increase patient access to costly drugs?
- Is biosimilar development going to foster innovation in orphan drug manufacturers?
- Should the focus be on biosimilar development when there’s still so many rare diseases with no cure?
- How will the regulatory guidelines evolve to address orphan drug biosimilars?
The interactive roundtable sessions offer you the chance to focus your time at the World Orphan Drug Congress USA. With topics ranging from Gene Therapies to Orphan Biosimilars, from commercialization to collaboration, we’ve got something for everyone working in this multi-stakeholder world.
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<table>
<thead>
<tr>
<th><strong>ROUNDTABLES</strong></th>
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<td><strong>1 BIOSIMILAR ORPHANS</strong></td>
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| Preparing for an inevitable reality
Scott Schleienbecker, Vice President of Scientific Affairs, Rare Diseases, Federal Work, PRA Health Sciences
Hazel Gortham, Senior Director, Biosimilars Development, Scientific Affairs, PRA Health Sciences |
| **2 STRATEGY** |
| Successfully managing, and implementing drug development programs for domestic and international commercialization of orphan drugs
Diane White, Associate Director Development Sciences Project Management, Bimarin Pharmaceutical |
| **3 PRICING** |
| Price gouging and the dangerous new breed for pharma companies
Ed Pesalla, Former Vice President, National Medical Director for Pharmaceutical Policy and Strategy, Amia |
| **4 PARTNERSHIPS** |
| Successful collaboration between a rare disease foundation and a pharmaceutical company to advance preclinical foundation and clinical research in Rett Syndrome
Christopher Mouling, President and Chief Executive Officer, Avanos Life Sciences |
| **5 DRUG DEVELOPMENT** |
| Simplifying the complexities of rare disease drug development
Stella Blackburn, Vice President, Global Head of Risk Management, Real World Insights, QuintilesIMS |
| **6 GENE THERAPY** |
| Partnering to build capabilities in Gene Therapy
Donna Armentano, Executive Director, Global Head Gene Therapy External R&D Innovation, Pfizer |
| **7 STEERING COMMITTEES** |
| Reaching the goal of patient committees that can drive, trial design and meaningful data
Jayne Gershkowitz, Senior Vice President and Chief Patient Advocate, Amicus Therapeutics |
| **8 ACCESS** |
| How can rare disease companies, insurers and patients/families work together to ensure access to transformative and curative therapies?
Francesca Cook, Director, Pricing and Market Access, REGENXBIO |
| **9 ENHANCED COLLABORATION** |
| Collaboration between patients, researchers and government institutions to support patient-centered research
Roxani Gopal-Srivastava, Director, Extramural Research Program, Office Rare Diseases Research, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health |
| **10 GLOBAL PROCUREMENT** |
| Using your procurement function to drive innovation and not just three bids and a buy
Keith Delmotte, Director, Center of Sourcing Excellence, Bristol-Myers Squibb |
| **11 ETHICAL CONSIDERATIONS** |
| Patients’ rights when participating in rare disease trials
Claudia Hirawat, Chair, International Circle of Ambassadors, EURORDIS |
| **12 EUROPEAN MARKET ACCESS** |
| Effective strategies for setting drug prices and achieving reimbursement of orphan drugs
Matthias Schönermark, Chief Executive Officer, SKC Beratungsgesellschaft |
| **13 VIRTUAL TRIALS** |
| Can we make virtual trials a reality in orphan drug development?
Horacio Plotkin, Vice President and Global Therapeutic Head Pediatrics and Rare Diseases, PPD |
| **14 TRIAL OPTIMIZATION** |
| Unique challenges in small population rare disease studies vs. large population trials
John Bolland, Vice President of Product Development, Atlantic Research Group |
| **15 MANUFACTURING** |
| Overcoming orphan drug manufacturing challenges to scale up development and aseptic filling from pre-commercial to commercial products
Presented by |
| **16 COST-EFFICIENT TRIALS** |
| Achieving clinical trial design efficiency to reduce cash burn
Carlos Comazzoli, Group Chief Medical Officer, Simbec-Orion Group |

Places on the roundtables are limited! Book your ticket today to ensure you get your table of choice at www.terrapinn.com/WODC2017
Building a long-term vision—bringing leadership and past experiences to guide the direction of a rare disease company

Optimizing the relationship between researchers

How can legislative pieces be improved to continue getting support from Congress?

Joint drug discovery program between researchers and scientists through the ‘Rare Diseases Research Consortium’

Demonstrating value—scientific differentiation, sharing of scientific information and protocols

Providing consultation to stakeholders developing

Where will the orphan drug market be in 2020?

An overview of Patient-Focused Drug Development (PFDD) initiatives under PDUFA V to support drug development and evaluation

Using the evidence gathered from patients to conduct benefit-risk assessments and advise sponsors on their drug development programs

Providing consultation to stakeholders developing PRO tools through the Clinical Outcomes Assessment Program at OD/ERDV

Jonathan C. Goldsmith, Associate Director Rare Disease Program, FDA

Is the FDA patient-centric? Patient-focused drug development (PFDD) as a systematic approach to gather input from patients

• An overview of Patient-Focused Drug Development (PFDD) initiatives under PDUFA V to support drug development and evaluation

• Using the evidence gathered from patients to conduct benefit-risk assessments and advise sponsors on their drug development programs

• Providing consultation to stakeholders developing PRO tools through the Clinical Outcomes Assessment Program at OD/ERDV

Jonathan C. Goldsmith, Associate Director Rare Disease Program, FDA

Evolving the relationships in the orphan drug ecosystem to achieve truly patient-centric clinical research

Where we’ve been, where we are now, and what can we expect?

• What does the future of orphan drugs look like and what can patients and other stakeholders expect to see in the next 5 years?

• Building a long-term vision—bringing leadership and past experiences to guide the direction of a rare disease company

• Nurturing and growing a business while staying responsive to patients’ needs

Keynote panel: Driving the future of the orphan drug market—adapting to change, fostering innovation and transforming patients’ lives

Moderated by: Sy Pretorius, Senior Vice President and Chief Scientific Officer, PAREXEL

Katrina Bosley, Chief Executive Officer, Edna Medicine

John Creanor, Chairman and Chief Executive Officer, Amicus Therapeutics

Timothy Wullert, Chairman, President and Chief Executive Officer, Horizon Pharmaceuticals

Keynote address: The potential of big pharma in accelerating rare disease breakthroughs

Where we’ve been, where we are now, and what we can expect?

• What does the future of orphan drugs look like and what can patients and other stakeholders expect to see in the next 5 years?

• Building a long-term vision—bringing leadership and past experiences to guide the direction of a rare disease company

• Nurturing and growing a business while staying responsive to patients’ needs

Jonathan C. Goldsmith, Associate Director Rare Disease Program, FDA

A pharma perspective on partnerships with external innovators in the life sciences

• Why and how pharma pursues external partners

• How to prepare for “successful” partnership discussions with pharma – it is a matter of fit

• What you should expect from your pharma counterpart during and after diligence

Jonathan Heller, Executive Director and Global Head, External R&D Innovation, Rare Diseases, Pfizer

Creating significant shareholder value: turning around NPS, two rare diseases products launched and a $5.2B sale to Shire

• Defining a new business model and a values-based culture

• Repurposing two products from large indications to niche orphans with no competition

• Managing the adversities along the way

Francois Nader, MD, Board Chairman, Acceleron Pharma (XLRN), Former President & CEO, NPS Pharma

Orchestrating successful pre-launch strategies for orphan drugs: overcoming challenges, optimizing approaches

• Leveraging insights—knowing your market, understanding your patients

• Demonstrating value—scientific differentiation, clinical needs, commercial relevance

• Honing strategies—assessing market dynamics and potential, optimizing your path to market

• Telling the story—multi-channel initiatives to engage and educate stakeholders

Yi Han, PhD, MBA, Executive Vice President of Market Access and HEOR, Cello Health Communications

Jim McDermott, Vice President, Cello Health BioConsulting

Commitment to access: a key element of development and availability of rare disease treatments

Looking to guarantee access to available medicines through the humanitarian donation of protein factor for hemophiliacs in countries where there is no access

• Using donations to open a dialogue to allow the treatment to be included in the regular healthcare system in developing countries

• Setting the foundation to create a sustainable model for humanitarian support in the rare disease field

Wills Hughes-Wilson, Senior Vice President, Chief Patient Access Officer, SOBI

The other side—effective licensing and collaboration agreements with rare disease biotechnology companies

• Expanding operations while retaining the essence of patient engagement and tight relationships with the patient community

• Sharing of scientific information and protocols between companies to find different ways of looking at diseases

• When is the right time to engage in partnerships?

Glyn Edwards, Chief Executive Officer, Summit Therapeutics
CONFERENCE DAY ONE (Thursday, April 20th, 2017)

12.40 ROUNDTABLE 1: Building a successful launch - strategies and tactics to achieve clinical and commercial success
Paul Boulton, Vice President, Global Commercial, Patient Services, Celio Health Insight, US

1:20 ROUNDTABLE 2: New scientific platforms - scientific innovation to bring new treatments for rare disease (mAbs, gene therapy, RNAi-based therapeutics, synthetic biology, antisense)
Amin Broumand, Chief Medical Officer, Syngene Therapeutics

1:40 ROUNDTABLE 3: Pre-launch strategies - finding and engaging with rare diseases
KOL early to support prescription, reimbursement, patient identification and the whole product life-cycle

2.00 ROUNDTABLE 4: Trials innovation Innovative solutions and approaches for rare disease clinical trials
Scott Schollander, Vice President of Scientific Affairs, Rare Diseases, Pfizer, PRA Health Sciences

2.20 ROUNDTABLE 5: PRDs - defining the patient-centered experience of painful crises in Sickle Cell disease through the development of a patient reported outcomes tool
Andrea M. Pieti, Senior Director, Team Lead, Outcomes & Evidence, Hematology, Global Health & Value, Pfizer

2.40 ROUNDTABLE 6: RWD - Using real-world data and advanced analytics to find undiagnosed patients with rare disease
Keith Blackwell, Vice President, Global Head of Risk Management, RWD, Clinical, QuintilesIMS

3.00 ROUNDTABLE 7: Patient retention - retention and faster access to rare disease patients
Michael Murphy, Chief Medical and Scientific Officer, Worldwide Clinical Trials

3.20 ROUNDTABLE 8: Patient retention and faster access to rare disease patients

3.40 ROUNDTABLE 9: Orphan launches addressing unique challenges of small biotech organizations and orphan drug launches and leveraging your healthcare communications partner
Thomas Sprant, Senior Vice President, Scientific Services, ClinicianMind

4.00 ROUNDTABLE 10: Gene therapy - designing clinical development programs that support approval, reimbursement and commercialization
Karen Aisch, Founder and Chief Executive Officer, Lyoqene

4.20 ROUNDTABLE 11: Orphan drugs in Germany - market access for orphan drugs and lessons learned from the AMNOG process
Matthias Schlenkermann, Chief Executive Officer, SKG Beratungsgesellschaft

4.40 ROUNDTABLE 12: Next-Generation commercial strategy - disrupting rare/ orphan drug commercial strategy through proven, predictive modeling
Jeff Castilla, Chief Commercial Officer, MWI Patient Precision Analytics

5.00 ROUNDTABLE 13: BD & Forecasting - pricing and access considerations and understanding how to build the patient forecast based on available epidemiological information
Joseph B. Musumeci, Senior Advisor, Blueprint Orphan

5.20 ROUNDTABLE 14: Integrated medical plans - begin with the end in mind to develop an integrated medical plan to maximize your drug’s potential
Aaron Macleod, Executive Director, RML Strategy, Global, INC Research

5.40 ROUNDTABLE 15: De-risking patient’s data collection, increasing clinical trial participation and supporting translational research
Applications of registry data in industry incentives, clinical trial design and recruitment, surveys and focus groups and patient focused drug development meetings with regulators, and payers

6.00 ROUNDTABLE 16: Overcoming challenges related to funding, type of nature of the data collected, utilization and distribution and access and location
Kenneth Hobby, President, Cure SMA

3.40 AFTERNOON NETWORKING BREAK
**PRICING & REIMBURSEMENT**

**Keynote address: How can different stakeholders pay for successes in gene therapy?**
- How did GSK and AIFA reach a price for Strimvelis and what other pricing models were considered?
- Applicability of pay-for-performance to other parts of the world that don’t have a single payer system
- Will pay-for-performance foster gene therapy commercialization and change the evaluation of premium pricing for other drugs?

Luca Pani, Former General Director, Italian Medicines Agency

**Keynote payers’ panel: Effectively assessing the value of orphan drugs in a time of increased scrutiny of drug pricing**
- How can payers reward industry for true innovation without breaking the bank?
- Effectively assessing international referencing pricing, differential pricing, pay-for-performance and HTA to increase access for patients
- How will pay for performance models impact the reimbursement of all orphan drugs?
- Will the changing political landscape affect orphan drug reimbursement?
- If robust data is not yet available, why should payers reimburse at premium levels?

Einar Andreassen, Senior Adviser, Norwegian Medicines Agency
Luca Pani, Former General Director, Italian Medicines Agency
Peter Dehnel, Medical Director, Blue Cross and Blue Shield of Minnesota

**Chairperson’s closing remarks**

**5:45** NETWORKING COCKTAIL RECEPTION SPONSORED BY Leadiant

**6:45** END OF DAY 1

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How can cell and gene therapy programs be developed through collaborative efforts with academia and lead to Inherited Retinal Disease (IRD): a collaborative effort between scientists, patients and regulators during a decades-long journey? New legislations and trends addressing the health care system sustainability of the region: From product-centric to patient-centric. Tackling the rare disease deficit – the advent of oral medicines – towards European consistency. Evolution of access for orphan drugs in Canada: orphan drug evaluation at CADTH. Maximizing the pipeline for orphan drugs: manufacturing challenges to scale-up development and aseptic filling from pre-clinical to commercial products. Saving the costs of orphan drug development – effective strategies for setting drug prices and achieving reimbursement of orphan drugs. Tackling the rare disease deficit – the advent of oral medicines – towards European consistency. Evolving access for orphan drugs in Canada: orphan drug evaluation at CADTH.
1.00  NETWORKING LUNCH

1:30  Lunch Keynote: Scientific rationale – FDA’s updates and considerations to orphan drug approval
- Rigorous collection of natural history data
- Rigorous development of assets prior to start of clinical trials, particular biomarker assets
- Adoption of randomization to control groups in trials
  Richard Moscicki, Deputy Center Director for Science Operations, CDER, FDA

1.50  NETWORKING LUNCH RESUMES

2.30  DIGITAL HEALTH
Healthcare digitalization: Cloud technology and Telehealth revolutionizing rare disease management
- Improving the interactions between researchers, connecting patients with clinical studies and patient groups
- Creating virtual video rooms with patients to evaluate biometric data and patient reported outcomes
- Optimized and faster diagnostics by enabling researchers at different locations to examine the same data and share real-time feedback
  Margaret Collins, Professor of Pediatrics, Cincinnati Children’s Hospital, Pathologist, Consortium of Eosinophilic Gastrointestinal Disease Researchers (CEGIR)

2.50  BIG data in “small” diseases- increasing the likelihood of success of ALS clinical trials and its applicability to other rare disorders
- Working with pharma and other stakeholders to make anonymized data available to the global R&D community
- Big data to understand natural history, disease heterogeneity, novel biomarkers and pathways of disease
- The ALS Prediction Prize program: using algorithms to predict the progression of the disease over time
  Alex Sherman, Director, Strategic Development & Systems Neurological Clinical Research Institute, Massachusetts General Hospital
  Principal Associate in Neurology, Harvard Medical School

3.10  How technologies that monitor rare disease patients can impact clinical development, patient adherence and improve PROs?
- Mobility platforms and wearables giving clinicians the opportunity to have a better understanding of the disease progression in order to adjust medication dosages and identify potential emergencies
- Reducing hospital visits by enabling a more real-time monitoring of the patient and the actions doctors can take before visits
- Increasing patient engagement by achieving a higher understanding and involvement in their health
  If you’re interested in sponsoring this session contact André Singer at andre.singer@terrapinn.com or +1 646 619 1797

3:30  BIOSIMILARS
Is the orphan drug market ready to embrace the biosimilar landscape?
- Are orphan drug biosimilars needed in order to increase patient access to costly drugs?
- Is biosimilar development going to foster innovation in orphan drug manufacturers?
- Should the focus be on biosimilar development when there’s still so many rare diseases with no cure?
- How will the regulatory guidelines evolve to address orphan drug biosimilars?
  Scott Rocklage, Former Chief Executive Officer, Epirus Biosciences
  Leah Christl, Associate Director for Therapeutic Biologics OND Therapeutic Biologics and Biosimilars, FDA
  Hazel Gorham, Senior Director, Biosimilars Development, Scientific Affairs, PRA Health Sciences
  Molly Burich, Associate Director of Public Policy, Biosimilars, Pipeline and Reimbursement, Boehringer Ingelheim

4.10  Chairperson’s closing remarks

4.15  End of conference

Join us by booking your ticket at www.terrapinn.com/WODC2017
Join these speakers at Rare Disease Advocacy World by booking your ticket today at [www.terrapinnn.com/WODC2017](http://www.terrapinnn.com/WODC2017)
INFLUENCING DRUG DEVELOPMENT

In no other drug development arena has the patient been so influential. Patients, advocates and advocacy groups are impacting regulation and driving orphan drug development. Government, Payers and Industry alike are seeking collaborations with patients to improve clinical trials, drug development and access. The role of the patient is changing once more. Rare Disease Advocacy World will kick off with industry-led investigation into the impact of patient-industry collaboration.

Is the FDA patient-centric? Patient-focused drug development (PFDD) as a systematic approach to gather input from patients

- An overview of Patient-Focused Drug Development (PFDD) initiatives under PDUFA V to support drug development and evaluation
- Using the evidence gathered from patients to conduct benefit-risk assessments and advise sponsors on their drug development programs
- Providing consultation to stakeholders developing PRO tools through the Clinical Outcomes Assessment Program at CDER/FDA

"Focus on the Patient Program” at GSK: strengthening patient leadership to accelerate and improve clinical trials, drug development and access strategies

- Working with patients to better understand the disease burden and adjust aspects of drug delivery methods, time-frames for data collection, target population and study endpoints
- Providing evidence to payers by developing patient case studies to show the human impact of the disease and treatment
- Helping patients prepare to participate in scientific, ethical and regulatory committees with pharmaceutical and government stakeholders

Inclusion of patient reported outcomes (PROs) in rare disease trial

- Developing meaningful patient reported outcomes by gathering information about their disease journey through an independent social network
- Will PROs be used to run 3b trials to can produce further evidence to support approval?
- Strategies and tools to enhance patient engagement and patient experience

Taking on the challenges of enrolling patients in gene therapy clinical trials

- Understanding barriers and hesitations for clinical trial participation
- Creating patient awareness to join trials- weighing the benefit-risk to participate
- Educating patients to make informed decisions

ADVOCACY IN ACTION

After a general look at how patients are influencing drug development, Parent Project Muscular Dystrophy (PPMS), PTC Therapeutics and EveryLife Foundation for Rare Diseases will provide practical insight into how patient groups can impact policymaking and orphan drug development, through real-world examples.

Advocacy’s influence in regulations – how united patient groups can impact policymaking

- Increasing patient advocacy groups’ participation in congressional caucuses and how their voice can be used in PDUFA and FDASIA meetings
- Submission of Patient-centered benefit-risk assessment studies to the FDA to include patient preference information into regulatory decision-making
- Patients’ influence in regulatory guidelines, new research, and product approvals

Patient-industry partnerships to influence orphan drug development and the product lifecycle

- Raising awareness to help with clinical trial enrollment in patient communities
- Helping patients understand their disease and available treatment options, as well as serving as hubs for education and support
- Working with patients and research institutions to accelerate translational research by providing firsthand information on the disease

Newborn Screening Bill in California – saving babies through early diagnosis

- The new movement - homogenization of newborn screening laws to standardize the number of rare diseases screened at birth
- Improving early diagnosis of rare diseases and subsequent treatment through early disease screening
- Optimizing data collection, early identification of patients for clinical trials and natural history studies

For more examples of Advocacy in Action, check out Total Orphan Drugs at www.orphan-drugs.org
Are initiatives such as ‘Right to Try’ the future of approval and patient access to treatment? Rare Disease Advocacy World is facilitating a multi-stakeholder examination of the latest access strategies, and how they will impact the review of new therapies for rare diseases.

Compassionate use: patient advocacy organization helping patients get access to medication through Compassionate Use Programs

- Navigating the various compassionate use and overcoming obstacles in clarity of the programs, inclusion criteria and lack of transparency
- Educating the community on expanded access programs to ensure safe access to experimental treatments
- Liaising with pharmaceutical stakeholders to help patients gain access to potentially lifesaving treatments

Panel: Early access to experimental drugs - is ‘Right to Try’ the solution for patients with no treatment options?

- Are these laws bypassing the FDA and exposing patients to dangerous experimental treatments?
- Is building robust compassionate use programs in small, medium sized and big pharma the answer to strengthen the responsible access to experimental treatments?
- The impact of RTT on clinical trial recruitment and the drug development programs of small and medium sized biotechs
- Are changes to FDA’s compassionate use program needed?
NATIONAL PATIENT ORGANIZATIONS

Leaders from National Patient Organizations from the US, South Africa, Australia, Colombia, New Zealand, Japan, India and Europe will be discussing the opportunities for rare disease research and development in their respective regions. How has multi-stakeholder collaboration promoted research, diagnosis, treatments and service for all rare diseases in Australia? How has Japan made rare and intractable diseases an Asian health priority? How has EURORDIS unified the voice of European rare disease patients?

Peter Saltonstall
President and Chief Executive Officer, NORD

Kelly du Plessis
Chief Executive Officer, Rare Diseases South Africa

Angela Chavez
President, Colombia Rare Disorders Society

Harsha Rajasimha
Co-Founder, Organization for Rare Diseases, India

Yann Le Cam
Chief Executive Officer, EURORDIS

Amanda Samanek
Executive Director, Genetic and Rare Disease Network, Australia

Letitia O’Dwyer
Chief Executive Officer, New Zealand Rare Disorders Society

Yukiko Nishimura
Chief Secretariat of International Relations, Japan Patients Association, Abid

Making rare and intractable diseases an Asian health priority
- Japanese ‘Specified Disease Treatment Research Program’ and Orphan drug legislation to encourage orphan drug research
- J-Rare and ODDD (Open Discussion for Orphan Drug Discovery programs) to foster rare disease identification, registries and diagnosis
- Connecting Asian rare disease stakeholders to accelerate the path to treatment

Overcoming the lack of medical infrastructure, research and support to improve the quality of life of rare disease patients in South Africa
- Efforts to move rare disease policy forward in Africa
- Creating awareness and establishing a network to connect all stakeholders that can help rare disease patients
- Facilitating access and supportive care for patients and families

Unifying the voices of many rare disease organizations to advance new diagnostics, treatments and cures for rare diseases
- The NORD’s research grant program: working with patient groups for translational and clinical studies
- Campaigns to promote the awareness of rare diseases and raise funds to assist undiagnosed patients
- Educating patients in the process and eligibility for accessing unapproved therapies

Influencing public policy to achieve a true recognition and support for population with Rare Diseases in Colombia and Latin America
- Working to overcome challenges of disparity in rare disease public policies in Latam, in order to improve access and medical care
- State of the art in Colombian public policies in the recognition of rare disorders- can this be a pathway for other countries in Latam?
- Supporting the patient ecosystem to achieve accurate diagnosis and recognition of rare diseases by the professional health community

Challenges of patients with rare diseases in India and recent progress
- Awareness, Education and Advocacy in India
- Connecting patients and physicians in India with international research and clinical trials
- Facilitating coordinated care and multidisciplinary approach for proper diagnosis and treatment of patients with rare diseases in India

The voice of European rare disease patients: supporting research, networking and improving patients’ quality of life
- Seeking more collaboration between governments and pharma to streamline the pricing process and expedite access to drugs
- Medicines Adaptive Pathways to Patients (MAPpt) activities to ensure access to new treatments for patients with unmet medical needs
- EURORDIS perspective on the ethical, social, economic and scientific grounds of rare disease research

Changing the paradigm of access, recognition and collaboration in rare diseases in New Zealand
- Working with rare disease groups to ensure their concerns and interests are communicated to health officials during consultation processes
- Liaising with pharmaceutical companies and PHARMAC to facilitate access
- Providing solutions to connect patients to specialists

Leaders from National Patient Organizations from the US, South Africa, Australia, Colombia, New Zealand, Japan, India and Europe will be discussing the opportunities for rare disease research and development in their respective regions. How has multi-stakeholder collaboration promoted research, diagnosis, treatments and service for all rare diseases in Australia? How has Japan made rare and intractable diseases an Asian health priority? How has EURORDIS unified the voice of European rare disease patients?

Learn from the world's leading advocates. Register your place today by visiting www.terrapinn.com/WODC2017
Joint drug discovery program between researchers and scientists through the ‘Rare Diseases Research Consortium’
- Keynote address: The potential of big pharma in accelerating rare disease breakthroughs
  - Building a long-term vision - bringing leadership and past experiences to guide the direction of a rare disease company
  - Nurturing and growing a business while staying responsive to patients’ needs
  - What does the future of orphan drugs look like and what can patients and other stakeholders expect to see in the next 5 years?

Keynote CEO panel: Driving the future of the orphan drug market – adapting to change, fostering innovation and transforming patients’ lives
- Making the drug development process better and more efficient for patients through biomarker development, precision medicine, patient reported outcomes and RWE for regulatory uses
- Engagement of patients in regulatory decision-making, streamlined drug development and faster applications review - what this means for drug manufacturers
- How can legislative pieces be improved to continue growing support from Congress?

Keynote panel: Prescription Drug User Fee Act (PDUFA VI) and 21ST Century Cures - recent and pending legislative efforts to make drugs better for patients
- Keynote: The potential of big pharma in accelerating rare disease breakthroughs
  - Identifying patients before applying for an orphan drug designation, finding their genetic history, caregivers and other carriers of the disease
  - Early engagement to support diagnostic research, biomarker identification and early translational research
  - Understanding the economic burden of a disease from a patient perspective to build the value of the drug

Inclusion of patient reported outcomes (PROs) in rare disease trial design
- Developing meaningful reported outcomes by gathering information about their disease journey through an independent social network
- Will PROs be used to run 3b trials to can produce further evidence to support approval?
- Strategies and tools to enhance patient engagement and patient experience

Understanding the economic burden of a disease from a patient perspective to build the value of the drug
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- Inclusion of patient reported outcomes (PROs) in rare disease trial design
  - Patient engagement in regulatory decision-making, streamlined drug development and faster applications review - what this means for drug manufacturers
  - How can legislative pieces be improved to continue growing support from Congress?

Patient centricity - fundamental collaborations with patients groups to advance orphan drug development
- Patient collaboration - addressing unique challenges of small biotech organizations and orphan drug launches and leveraging your healthcare communications partner

Patient Advocacy - Patient health and care experience in product development
- Patient centricity - fundamental collaborations with patients groups to advance orphan drug development

Shefali Pathak, Chief Medical and Scientific Officer, Novartis Pharmaceuticals

Innovative solutions and approaches for rare disease clinical trials
- Scott Schiller, Vice President of Scientific Affairs, Rare Diseases, Federal Work, PIMA Health Sciences

Inclusion of patient reported outcomes (PROs) in rare disease trial design
- Developing meaningful reported outcomes by gathering information about their disease journey through an independent social network
- Will PROs be used to run 3b trials to can produce further evidence to support approval?
- Strategies and tools to enhance patient engagement and patient experience

Announcement of new orphan drug designations
- Identifying patients before applying for an orphan drug designation, finding their genetic history, caregivers and other carriers of the disease
- Early engagement to support diagnostic research, biomarker identification and early translational research
- Understanding the economic burden of a disease from a patient perspective to build the value of the drug

ROUNDTABLE 3: Pre-launch strategies - finding and engaging with rare disease KOLs, early to support prescription, commercialization, patient identification and the whole product life-cycle

ROUNDTABLE 4: Trials Innovation - Innovative solutions and approaches for rare disease clinical trials
- Innovative solutions and approaches for rare disease clinical trials

ROUNDTABLE 8: Vials are vile - how intelligent device solutions improve patient adherence while dosing reliably to prevent relapses and influence P&R?
- Moderated by Medicom

ROUNDTABLE 9: Gene therapy - designing clinical development programs that support approval, reimbursement and commercialization
- Karen-Jiach, Founder and Chief Executive Officer, Lysoptimize

ROUNDTABLE 11: Orphan launches - addressing unique challenges of small biotech organizations and orphan drug launches and leveraging your healthcare communications partner
- Thomas Sprad, Senior Vice President, Scientific Services, CionMed

ROUNDTABLE 14: Orphan drugs in Germany - market access for orphan drugs and lessons learned from the AMNOG process
- Matthias Schierwagen, Chief Executive Officer, SBC Beraubungsevassenschaft
CONFERENCE DAY ONE  (Thursday, April 20th, 2017)

1.20  NETWORKING LUNCH SPONSORED BY

2:40  ADVOCACY IN ACTION

Advocacy's influence in regulations – how united patient groups can impact policymaking
- Increasing patient advocacy groups’ participation in congressional caucuses and how their voice can be used in PDUFA and FDASIA meetings
- Submission of Patient-centered benefit-risk assessment studies to the FDA to include patient preference information into regulatory decision-making
- Patients’ influence in regulatory guidelines, new research, and product approvals

Pat Furlong, Founding President and Chief Executive Officer, Parent Project Muscular Dystrophy (PPMD)

Patient-industry partnerships to influence orphan drug development and the product lifecycle
- Raising awareness to help with clinical trial enrollment in patient communities
- Helping patients understand their disease and available treatment options, as well as serving as hubs for education and support
- Working with patients and research institutions to accelerate translational research by providing firsthand information on the disease

Mary Frances Harmon, Head of Global Patient Advocacy, PTC Therapeutics

Newborn Screening Bill in California – saving babies through early diagnosis
- The new movement - homogenization of newborn screening laws to standardize the number of rare diseases screened at birth
- Improving early diagnosis of rare diseases and subsequent treatment through early disease screening
- Optimizing data collection, early identification of patients for clinical trials and natural history studies

Vignesh Ganapathy, Associate Director of Advocacy & Government Relations, EveryLife Foundation for Rare Diseases

4:50  AFTERNOON NETWORKING BREAK

5:10  Keynote payers’ panel: Effectively assessing the value of orphan drugs in a time of increased scrutiny of drug pricing
- How can payers reward industry for true innovation without breaking the bank?
- Effectively assessing international referencing pricing, differential pricing, pay-for-performance and HTA to increase access for patients
- How will pay for performance models impact the reimbursement of all orphan drugs?
- Will the changing political landscape affect orphan drug reimbursement?
- If robust data is not yet available, why should payers reimburse at premium levels?

Einar Andreassen, Senior Adviser, Norwegian Medicines Agency
Peter Dehnel, Medical Director, Blue Cross and Blue Shield of Minnesota
Luca Pani, Former General Director, Italian Medicines Agency

5:40  Chairperson’s closing remarks

5:45  Networking cocktails sponsored by

6:45  End of day one
How can cell and gene therapy programs be developed through collaborative efforts with academia and lead to more effective therapies? How to balance the science and medicine being developed with the expectations of payers and regulators? Overcoming challenges in the design and interpretation of the human clinical trials, immunogenicity, and the selection of the best primary efficacy end point to progress clinical development.

**Overview of the clinical development program for cerebral adrenoleukodystrophy and beta-thalassemia**

Innovative and modular technology platform for hemophilia gene therapies

Considering potential new value propositions for patients, payers and the healthcare system

The impact of RTT on clinical trial recruitment and the drug development programs of small and medium sized biotechs

Educating the community on expanded access programs to ensure safe access to experimental treatments

Panel: Early access to experimental drugs - is 'Right to Try' the solution for patients with no treatment options?

- Are these laws bypassing the FDA and exposing patients to dangerous experimental treatments?
- Is building robust compassionate use programs in small, medium sized and big pharma the answer to strengthen the responsible access to experimental treatments?
- The impact of RTT on clinical trial recruitment and the drug development programs of small and medium sized biotechs
- Are changes to FDAs' compassionate use program needed?

**Roundtable sessions:**

**ROUNDTABLE 1: Biosimilar orphans - preparing for an inevitable reality**
Scott Schlabach, Vice President of Scientific Affairs, Rare Diseases, Federal Health, PPA Health Sciences
Hazel Gorham, Senior Director, Business Development, Scientific Affairs, PPA Health Sciences

**ROUNDTABLE 2: Strategy - successfully managing, and implementing drug development programs for domestic and international commercialization of orphan drugs**
Diane White, Associate Director Development Sciences Project Management, Biotechs Pharmaceutical

**ROUNDTABLE 3: Pricing - price gouging and the dangerous new breed for pharma companies**
Ed Pezzali, Former Vice President, National Medical Director for Pharmaceutical Policy and Strategy, Aetna

**ROUNDTABLE 4: Partnerships - successful collaboration between a rare disease foundation and a pharmaceutical company to advance preclinical foundation and clinical research in Rett Syndrome**
Christopher Misling, President and Chief Executive Officer, Arise Life Sciences

**ROUNDTABLE 5: R&D - Using real-world data to find rare disease patients for long-term observational studies**
Stella Blackburn, Vice President, Global Head of Risk Management, Real-World Insights, QuintilesIMS

**ROUNDTABLE 6: Gene therapy - partnering to build capabilities in Gene Therapy**
Donna Armentano, Executive Director, Global Gene Therapy, External R&D Innovation, Pﬁzer

**ROUNDTABLE 7: Steering committees - reaching the goal of patient committees that can drive trial design and meaningful data**
Jayne Gershkowitz, Senior Vice President and Chief Patient Advocate, Amicus Therapeutics

**ROUNDTABLE 8: Access - how can rare disease companies, insurers and patients/families work together to ensure access to transformative and curative therapies**
Francesco Costa, Director, Pricing and Market Access, REGENXBIO

**ROUNDTABLE 9: Enhanced collaboration - collaboration between patients, researchers and government institutions to support patient-centered research**
Rachel Gugli-Schneider, Director, External Research Program, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health

**ROUNDTABLE 10: Global procurement - using your procurement function to drive innovation and not just three bids and a buy**
Kathie DoMattina, Director, Center of Sourcing Excellence, Bristol-Myers Squibb

**ROUNDTABLE 11: Ethical considerations - patients’ rights when participating in rare disease trials**
Claudia Hiraudo, Chair, International Circle of Ambassadors, EURODIS

**ROUNDTABLE 12: European market access - effective strategies for setting drug prices and achieving reimbursement of orphan drugs**
Matthias Schönermark, Chief Executive Officer, SKC Beratungsgesellschaft
Conference Day Two (Friday, April 21st, 2017)
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OUR PACKAGES OFFER HIGH VALUE INCLUDING

- The chance to show over 200 pharma and universities what you are doing to help expedite orphan drug development
- The opportunity for you to invite your customers and prospects
- A 1-2-1 partnering service, where we set up meetings for you
- The opportunity to raise your credibility and establish thought leadership

WHY EXHIBIT?

- Debut new products
- Demonstrate existing and complicated processes
- Provide a meeting branded place for clients at the event
- Keeping your brand front of mind
- Brand and marketing exposure
- Raise credibility
- Generate leads and make sales

PURSUE AND PARTNER

- 700 of partnering meetings in 2 days
- 1,000+ attendees
- 5+ hours of assisted networking during 40-minute coffee breaks and 1 ½ hour lunch breaks
- Over 30 intimate roundtable, focus-group discussions to participate in
- Speed Networking session
- 2 Networking Cocktail Receptions

WHO YOU WILL MEET?

PHARMA - Head, SVP, VP, Director of:
- Rare Disease Unit
- R&D
- Medical Affairs
- Commercial
- Marketing

BIOTECH:
- CEO
- CSO
- VP of Business Development

UNIVERSITIES - Professors and Heads
- Academic medical center chiefs
- Payers & regulators

WHO SHOULD SPONSOR AND EXHIBIT?

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- Market Research consultants
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- Technology providers
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- Law firms
- Risk advisers
- Marketing & Communications Consultants
- Strategic Consultants
- Market Access Consultants
INTRODUCING OUR EVENT TECHNOLOGY PARTNER JUBLIA.

The World Orphan Drug Congress USA recognizes the importance of networking. Jublia offers a unique service, combining the latest in networking software with a personal touch to provide an easy-to-use approach for you to facilitate quality networking opportunities.

So, how does it work?

4 weeks before the event, you will be sent an email containing your personalized link. Click on this link to access the full attendee list. Once here, you can input search criteria. Jublia then uses this information to rank the attendee list in order of their relevance to you!

Simply click on the name of who you would like to meet and securely send them a meeting request. The system acts as your meeting diary, keeping track of your availability and avoiding double bookings. The system will also assign you a “Meeting Table”, so you are guaranteed a nice spot onsite to conduct the meeting.

Terrapinn staff will be on hand to help!

FOR MORE INFORMATION visit www.terrapinn.com/WODC2017

THIS WOULDN’T BE A TERRAPINN EVENT WITHOUT SOME LIGHT FUN AND NETWORKING. WE HAVE AT LEAST TEN HOURS OF NETWORKING INCLUDING A WELCOME DRINKS RECEPTION, NETWORKING COCKTAILS, 1-2-1 MEETINGS AND SPEED NETWORKING.

Join us once the conference sessions are finished on Day One for Networking Cocktails.

Kick off the event in style at the Welcome Drinks Reception! Join us on the evening of April 19 to pick up your badge, grab a drink and start networking early.

FOR MORE INFORMATION visit www.terrapinn.com/WODC2017
TOP 10 REASONS TO ATTEND

1. GLOBAL
Meet with 1,000+ attendees from over 42 countries, including leaders from US, Canada, Japan, Australia, South Africa, France, Sweden, UK, Italy, Netherlands, New Zealand, Colombia, and India

2. PAYERS
Hear valuable lessons from the Blue Cross Blue Shield, Norwegian Medicine Agency and Italian Medicines Agency on fostering innovative pricing and reimbursement models like pay-for-performance

3. GENE THERAPIES
Hear the CEOs of uniQure, bluebird bio, Spark Therapeutics and VP of GSK on breaking barriers for gene therapy development for rare diseases

4. DUCHENNE MUSCULAR DYSTROPHY
Join PTC Therapeutics, the former CEO of Sarepta Therapeutics and Pat Furlong from Parent’s Project Muscular Dystrophy on discussions about recent approval of medicines for DMD and how it will impact review of new therapies for rare diseases

5. COMMERCIALLY-FOCUSED
Network with Pfizer, GSK, Shire, Biogen, Alexion, Biogen, SOBI and hundreds of C-level executives from orphan drug biotechs at the annual meeting of the industry

6. ORPHAN BIOSIMILARS
Stay abreast of the inevitable trend of biosimilars for orphan drugs and how this $33bn industry will emerge as patents start to expire in the next 3-5 years

7. LEGISLATION
Navigate how PDUVA VI, 21st Century Cures Act and California’s Newborn Screening Bill will change patients involvement in orphan drugs clinical trials, access and rare disease diagnosis

8. PARTNERING AND M&A
Learn how François Nader, former CEO of NPS Pharmaceuticals, created significant shareholder value to sell the company for $5.4bn and other strategies for partnering with big pharma

9. PITCH & PARTNER
Hear from the 34 hottest biotechs in CNS and Pulmonary therapeutic areas, as well as gene therapies and Ultra Orphan Drugs

10. WORKSHOPS
Attend 8 insightful workshops on pricing & reimbursement, forecasting techniques for orphan & ultra-orphan drugs, and integration of cross-functional community voice into development & commercialization of orphan drugs

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