Platinum Sponsors

The global orphan drug conference and expo

Post-Event Report 2015
Executive Summary

Pharmaceutical, biotechnology, academia, payers, and patient advocacy groups from the Americas, Europe, and Asia were well-represented at the World Orphan Drug Congress USA 2015.

Countries that were represented at the show in 2015:

- Australia
- Belgium
- Brazil
- Canada
- China
- Croatia
- Denmark
- Germany
- Hungary
- India
- Ireland
- Israel
- Italy
- Lebanon
- Mexico
- Netherlands
- Norway
- Portugal
- Republic Of Korea
- Saudi Arabia
- Singapore
- Sweden
- Switzerland
- Taiwan
- United Arab Emirates
- Emirates
- United Kingdom
- United States
- Uruguay

Visit [www.terrapinn.com/orphandrug](http://www.terrapinn.com/orphandrug) for more information and to get involved.

World Orphan Drug Congress USA 2015

The World Orphan Drug Congress USA gathers together key stakeholders from industry, patient groups, government, academia, and investors from across the world. In scale and experience, this event is unrivalled.

Platinum Sponsors

- Genzyme
- Premier Research

Dates

- April 22-24, 2015

Venue

- Washington Hilton, D.C.

Show Size

- 30,000 Square feet

Sponsors & Exhibitors

- 48 companies

Attendees

- 600 attendees

New for 2015

- Rare Disease Advocacy World
- 2 Day Pitch & Partner

Website

- [www.terrapinn.com/orphandrug](http://www.terrapinn.com/orphandrug)

2016 Dates


Created by

- Terrapinn, Inc.
If you are anyone involved in the rare disease space, you must attend the World Orphan Drug Congress. Every active stakeholder playing a vital role in the Orphan Drug Industry & Rare Disease Advocacy looks forward to this conference annually. - Nadia Bodkin, President & CEO, EDSers United
THANK YOU TO OUR SPONSORS AND EXHIBITORS

Platinum Sponsors

Genzyme
Premier Research

Gold Sponsors

Clinigen
Ergomed
M3 Pharma

WiFi Sponsor

Mapi

Silver Sponsors

Clinart
Catalent
Multicare
Orphan Reach

Other

Sponsor Profiles

The Exhibition

"The World Orphan Drug Conference 2014 in Washington DC—was a great landmark for hearing so many confirmations of the need to involve patient groups."
Kay Parkinson, Founder and Director, Alstrom Syndrome U.K.

"I am really glad I was able to attend the World Orphan Drug Congress. It was a great mix of sponsor companies, CRO’s, payers and patient advocacy groups. The cross functional dialogue was highly valuable and I plan to be back next year."
Kevin J. Anderson, Associate Director, Clinical, Alexion

Over 500 meetings between exhibitors and conference attendees were facilitated through the Networking Team, event portal, and mobile app.
“Workshops were small enough to enable powerful networking discussions on key issues for multiple stakeholders. Conference organizers successfully included all key and leading thought leaders, technology developers, policymakers, investors and diverse public and private stakeholders into one room for high impact connections, brainstorming for all participants. Thank you.”

Aida Aman, Co-Founder and President and CEO, Invivo Sciences

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Attendee Breakdown

<table>
<thead>
<tr>
<th>Region</th>
<th>Percentage</th>
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</thead>
<tbody>
<tr>
<td>US</td>
<td>78%</td>
</tr>
<tr>
<td>Canada</td>
<td>2%</td>
</tr>
<tr>
<td>Europe</td>
<td>11%</td>
</tr>
<tr>
<td>Latin America</td>
<td>4%</td>
</tr>
<tr>
<td>Asia</td>
<td>2%</td>
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<tr>
<td>MENA</td>
<td>3%</td>
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HOW DID THEY HEAR

<table>
<thead>
<tr>
<th>Method</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Email from organizers</td>
<td>30%</td>
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<tr>
<td>Word of mouth</td>
<td>24%</td>
</tr>
<tr>
<td>Attended another Terrapin event</td>
<td>21%</td>
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<tr>
<td>Print material</td>
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<tr>
<td>Website</td>
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<tr>
<td>Search engine</td>
<td>4%</td>
</tr>
<tr>
<td>Trade association</td>
<td>2%</td>
</tr>
</tbody>
</table>

Top reasons visitors attended the World Orphan Drug Congress USA

1. Update on new trends in orphan drugs
2. Find partners
3. Recommend/purchase products
4. Evaluate for next year

Participating organizations

AccessPharmaCon, LLC
Acer Therapeutics
Aegerion Pharmaceuticals
Aetna Pharmacy Management
AFAG
AGTC
Airway Therapeutics
AKU Society
Alexion Pharmaceuticals
Alliance For Cyroglobulinemia
ALPE Foundation
Amarex Clinical Research
Amicus Consulting
Anavex Life Sciences Corp.
Andante Pharmaceuticals
Antibodies-online
ANVISA - Agência Nacional de Vigilância Sanitária
ApoptoIntelligence
Apogee Biotechnology Corp
ArmaGen Technologies, Inc.
Arrowhead Research
Ashfield Healthcare
Asklepios Pharmaceuticals LLC
Atlantic Healthcare
aTyr Pharma
Avevella
Ax-S Pharma
B&G Medicom
B.D.C. Venture Capital
B.M.S.
Barth Syndrome Foundation
Baxter
Bellus Health Inc
Benefits Strategies LLC
BioBlaze Pharma
BioBlaze Pharma Ltd
Biocodex USA
Biocompare
BioMarin Pharmaceutical Inc
Biommann Pharmaceutical Inc.
BioPonte Alliance for Rare Disease
BioPortfolio
Bluebird Bio
Brace Pharma Capital
Brazilian Federal Council of Medicine
Bristol Myers Squibb
C1 Consulting
CATHD
Caligor Rx
Cardinal Health
Caring Voice Coalition
Casa Hunter
Catalent Pharma Solutions
Catalyst Pharmaceuticals
CDER FDA
Certa
CheckOrphan
Children’s Tumor Foundation
China Dolls Center For Rare Disorders
Clear Pharma
Clementia Pharmaceuticals Inc.
Clinart USA
Clinerion Global Access Program
Clinivel Pharmaceuticals Ltd
Coalition for Clinical Trials Awareness
Compass
Compass Strategic Consulting, Inc.
Comradis
Concordia Healthcare Inc.
Connexion Healthcare
Coordination of Rare Diseases at Sanford
CORAM Clinical Trials
Cortendo
Cote Orphan Consulting
Coulter Partners
Covance
Cowen and Co
CPI International
CurePP
Cydan Development
Cydan Development Inc
CytoKinetics
CytoTherapeutics Inc
D.A.R.A. BioSciences Inc
DARA BioSciences, Inc
Deerfield Institute
Defiba Designs
Dexcom
Dicema Pharmaceuticals
Discovery USA
Dohmen Life Science Services
DrugDev
Edinburgh BioQuarter
EDisers United
Eli Lilly
Emerge Health Pty Ltd
Emmanuel Life Sciences, Inc
Ergomed Group
EvuroDS
Expressa Group
F. Hoffmann-La Roche Ltd
Fauchier Export Packaging Ltd
FDA
Fibrocell Science Inc
Foundation for Research on Rare Diseases and Disorders
Genetic Alliance
Genova Pharmaceuticals
Genpharm
Genpharm services
GenSight Biologics
Genzyme
Genzyme Corp
Genzyme, A Sanofi Company
Georgia Bio
GF Communications
GFX
Gidi Pharma
GlaxoSmithKline Rare Diseases Global Genomics | RARE Project
Grunenthal GmbH
Health Advances
Healthcare at Home
Hedera Neuruphy Foundation
Hovionale
Hyperion Therapeutics
I.S.M. Therapeutics
Idea Pharmaceutical
Idis Inc
IDC Pharma
In Flectis Bio Science
Inflection Pharmaceuticals
Inflrcts Bioscience
InnovateDB
Innomed Incorporated
Inspir

Institute For Medical Engineering and Science Harvard M.I.T. Division of Health Sciences and Technology
Institute for Optimizing Health Outcomes
Interfaca - Associação da Indústria Farmacêutica e Útica de Pesquisa
International Business Consultants inVentiv Health Clinical
IPEX S.A.
Jazz Pharmaceuticals, Inc
Juice Pharma Worldwide
K.B.I. Inc
Kaiser Permanente
Karyopharm Therapeutics
KBI Biopharma Inc.
Kedrin Biopharma
Kids v Cancer
Lapidus Data
LGS Foundation
Lifesience Leader
LINK Healthcare
LipoMed Inc
Lumos Pharma
M3 Pharma S/A
Macy’s
Managed Care Management
MAPI
Marathon Pharmaceuticals LLC
Marina Biotech Inc
Marinus Pharmaceuticals
Mark Krueger & Associates Inc
Marshfield Clinic
Mast Therapeutics Inc
Max Neeman international
MEDExpansion
Medicaid Health Plans of America
Medical Marketing Economics
MEDIPOST America, Inc.
Medivir AB
Metron Research Group LLC
Ministry Of Health Brazil
MLV & Co.
MMF LLC
MMRG
Mahawat
Momenta Pharmaceuticals Inc

“If was a great opportunity for rare syndrome groups meeting with orphan drug pharma and investors to discuss the value proposition of collaborating on research. It structured quite a bit of meeting/ networking time for regular meetings, roundtables, and informal meetings which led to productive and promising future collaboration opportunities.”

Carol Hoxie, Communication Specialist, Platelet Disorder Support Association

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Speakers at this year’s meeting included C-level executives and decision makers from the leading orphan drug manufacturers, policy makers and influencers, patient advocacy group leaders from different regions of the world.

Over 600 conference delegates attended the keynote sessions.

Top speakers

- **Henri Tenenne**, Chief Executive Officer, Genzyme
- **Christoffer Neilaker**, Research Fellow, Medical Research Foundation’s Functional Genomics Unit, Oxford University
- **Jeff Myers**, President, Chief Executive Officer, Medicaid Health Plans of America (MHPA)
- **Mark Rechera**, Chief Commercial Officer, PTC Therapeutics
- **Richard Moscicki**, Deputy Center Director for Science Operations, Center for Drug Evaluation and Research, FDA
- **Daniel Anderson**, Scientific Founder, CRISPR Therapeutics
- **Stephen Greff**, Former Director of the Office of Rare Disease Research, NIH
- **Eric Hoffman**, Director, Research Center for Genetic Medicine, Children’s National Hospital
- **Kathleen Coolidge**, Director, US Patient Advocacy, Rare Disease, Genzyme
- **Peter Saltenstall**, President & CEO, NORD
- **Yann Le Cam**, Chief Executive Officer, EURORDIS
- **Julie Stoss**, Vice President, Government Relations, Kaiser Permanente

**Conference Highlights**

- Three tracks across two days delivering high quality content
- Focus on commercialization and marketing of orphan drugs
- Highlights from the payers about their perspective
- Pitch and partner showcasing industry innovation
- Patient groups get their own event – showing how to build them from the ground up, and how they can help pharma to help them

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<tr>
<td>18%</td>
<td>Patient groups</td>
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<tr>
<td>10%</td>
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<tr>
<td>7%</td>
<td>Academia</td>
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It’s all about choice

We provided 2 days of jam-packed content providing attendees with the choice of multiple conferences to attend based on interest and need.

Over 200 presentations across 3 tracks

**World Orphan Drug Congress**
- Showcased the most important issue facing the Orphan Drug industry today: are the high prices sustainable?
- Focus on commercialization and what it takes to get ROI from your orphan drugs across multiple markets
- Hearing from the payers themselves what it is they are looking for in the pricing debate, and how pharma and payers can work together earlier to get these drugs to patients
- Truly global perspective: over 29 countries represented at the event.
- FDA highlighting what they are doing to push the industry forward through incentives, fast-tracks and pediatric vouchers.

**Rare Disease Advocacy World**
- How to start and organize a patient group, build a registry, and grow your natural history
- Advancing early diagnosis, awareness, and access to care for rare diseases
- Understanding the scientific mindset – working with pharma and academia
- Where to get funding and how best to utilize it in a crowded marketplace
- How can we work together to achieve greater cooperation across rare disease advocacy groups

**Pitch & Partner - for over 50 indications such as:**
- Maple syrup urine disease
- Sickle cell disease
- Menkes Disease
- Cystic fibrosis
- Rare cancers
- Pediatric epilepsy
- Netherton syndrome

“World Orphan Drug Congress is the place to be for leaders in the rare disease community.” Hans Schikan, Chief Executive Officer, Prosensa Therapeutics Bv

1-2-1 Partnering

1-2-1 Partnering in our Networking Lounge

To ensure that sponsors and exhibitors received the most out of their participation, our dedicated Networking Manager, Alina Kandinova, facilitated over 80 prearranged 1-2-1 (face-to-face) networking meetings with key prospects. Meetings took place in a designated and reserved sponsor area with facilitated introductions. Additionally, email introductions were facilitated to ensure that connections were prospered postevent.

Sponsors had one-on-one meetings with some of the following industry heavyweights. Especially in demand were meetings: Mast therapeutics, Retrophin, Marathon Pharmaceuticals, Dicerna Pharmaceuticals, Aegerion Pharmaceuticals, Recordati Rare Diseases, and Vertex Pharmaceuticals.

The Networking Portal and Mobile App

Our Networking Mobile App provides access to the individual profiles of attendees, sponsors and exhibitors. All registered attendees and sponsor representatives can personalize their agendas by favoring a session, connect with each other via personal messaging prior to the event, and arrange meetings with each other. The app remains live for a year post-event, allowing attendees to continue networking beyond the onsite networking coffee and lunch breaks. In total, the Networking Mobile App had 515 number of meetings organized.

To top off conference day one, our Networking Party allowed attendees to unwind and relax with live music from the Washington Jazz Band. Networking connections and introductions spread quickly through the room, with continuous exchange of contact information!
A Global Marketing Campaign

World Orphan Drug Congress USA once again delivered a powerful multichannel marketing campaign that achieved impressive results. Investment in the right media to reach the right audience ensured the event stayed prominent in the minds of pharma, biotech, patient groups, academia, regulators, payers and investors alike.

The marketing campaign used every channel possible and reached an audience of over 750,000 industry stakeholders:

- 456,000 industry members were sent email blasts through the use of our own database and those of our event media partners
- 20,000 people received newsletters via our media partners
- The event was listed on 60 partner websites
- 5 eBooks were produced and distributed through the Total Orphan Drugs
- The event website generated over 42,000 visits

“Beautifully produced with top speakers in the field and a healthy fostering of interaction among attendees.”
William Repicci, Executive Director, Lymphatic Research Foundation

“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
Join us at the 6th annual World Orphan Drug Congress USA 2016!

Here’s what’s more and why you can’t miss next year’s conference:

- A focus on bringing together brand managers of marketed orphan drugs
- Orphan Drugs 101 and what a rare disease looks like for doctors and clinicians which will attract pharma with more advanced orphan products
- Presenting academic medical centers who can bridge the communication gap between patients and scientists

Get your brand, your message and your expertise highlighted.

www.terrapinn.com/orphandrug
April 20-22, 2016

“I thought it was an excellent conference with the selected topics addressing a broad range of pertinent interests...the pitch and partner sessions were very good.”
Richard Lauricella, Business Development Manager, CligearSys LLC