WORLD OrphanDrug Congress USA

Platinum Sponsors

genzyme
A SANOFI COMPANY

premier research

The global orphan drug conference and expo

Post-Event Report 2015

Created by

terrapinn
use your brain

600 attendees from 29 countries

Representing global orphan drug and rare disease organizations

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

Visit www.terrapinn.com/orphandrug for more information and to get involved

Executive Summary

Show Name	**World Orphan Drug Congress USA 2015**
About	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 A SANOFI COMPANY; 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
2016 Dates	April 20-22, 2016, The Washington Hilton, Washington, DC, USA
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 A SANOFI COMPANY

premier research

Gold Sponsors

CLINIGEN Global Access Programs

ERGOMED

M3 PHARMA

PROMETIC

PROSENSA

PTC THERAPEUTICS

Uno Healthcare

WiFi Sponsor

Mapi Insight for solutions

Silver Sponsors

clinart MENA

Catalent

multicare

orphan reach

Omnicare Specialty Care Group

Pfizer Rare Disease

QUINTILES

SAREPTA THERAPEUTICS

CardinalHealth Essential to care

SonexusHealth

Other

Assistance Fund

GENPHARM

lapidusdata

COVANCE SOLUTIONS MADE REAL

Giddi

Karyopharm Therapeutics

OneWorld

PatientCrossroads™

Solpharm

COMRADIS

caring voice coalition We're Here to Help.

gsk

Inspire

emerge health

MMPG Healthcare Innovation Specialists

DOHMEN LIFE SCIENCE SERVICES

Ashfield Commercial & Medical Services

CLEARPHARMA SCIENCE · COLLABORATION · ACCESS

AGILITY CLINICAL PSR

COTÉ orphan consulting

JUICE PHARMA WORLDWIDE

KBI BIOPHARMA

Pii

ORPHAN DRUG SOLUTIONS

VERISTAT

Quay Pharma

CORAM CVS/specialty infusion services

MPC Masnad Pharma Consult Center

The Exhibition

600 attendees – up **19%** from last year

50% of attendees were from pharma, biotech and research institutions

50 VIPs, including patient advocacy groups and investors

48 exhibitors showcasing their solutions – up 15% from last year

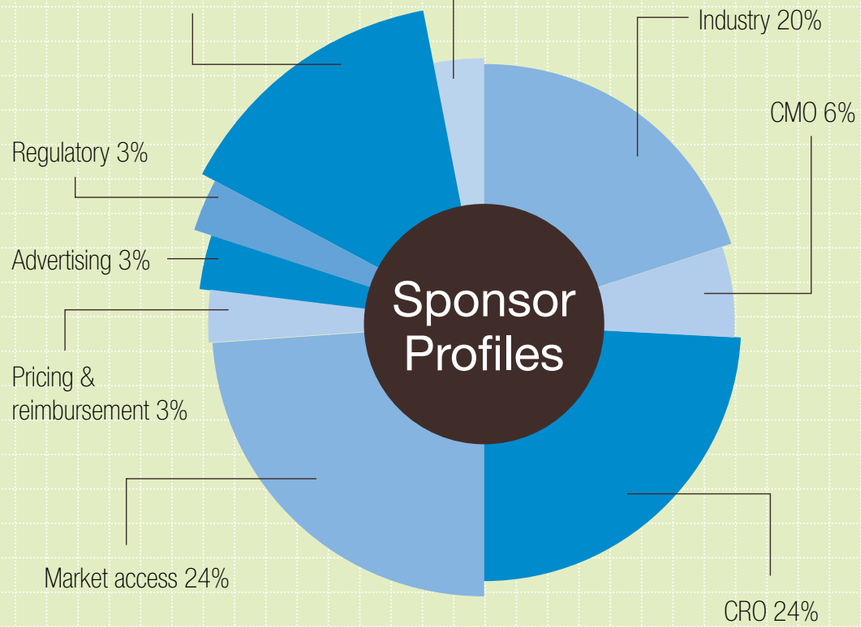
"I am really glad I was able to attend the World Orphan Drug Congress. It was a great mix of sponsor companies, CRO's, payers and patient advocacy groups. The cross functional dialogue was highly valuable and I plan to be back next year."

Kevin J. Anderson, Associate Director, Clinical, Alexion

"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.

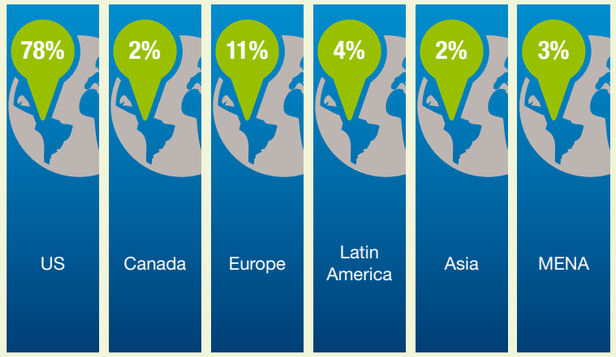
Sponsor Profiles



Over **500** meetings between exhibitors and conference attendees were facilitated through the Networking Team, event portal, and mobile app.

Attendee Breakdown

BY REGION



HOW DID THEY HEAR

- 30% Email from organizers
- 24% Word of mouth
- 21% Attended another Terrapinn event
- 10% Print material
- 9% Website
- 4% Search engine
- 2% Trade association

"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."

Ayla Annac, Co-Founder, President and CEO, Invivo Sciences

"As a representative from a patient advocacy organization, I found the meeting most interesting from the point of view of how pharmaceutical and other research organizations who are related to the medical therapies industry determine what types of therapies to develop and what obstacles, etc. they face and find ways to overcome...I feel it was worthwhile to attend. There were many outstanding speakers. I would encourage others to attend similar in the future."

Carol Hoxie, Communication Specialist, Platelet Disorder Support Association

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

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

Cherie Takemoto, Vice President, Costello Syndrome Family Network

AccessPharmaCon,LLC
Acer Therapeutics
Aegerion Pharmaceuticals
Aetna Pharmacy Management
AFAG
AGTC
Airway Therapeutics
AKU Society
Alexion Pharmaceuticals
Alliance For Cryoglobulinemia
ALPE Foundation
Amarex Clinical Research
Amiculum Consulting
Anavex Life Sciences Corp.
Andarix Pharmaceuticals
Antibodies-online
ANVISA - Ag ê ncia Nacional de Vigilância Sanit á ria
Apbdrf
Apogee Biotechnology Corp
ArmaGen Technologies, Inc.
Arrowhead Research
Ashfield Healthcare
Asklepion Pharmaceuticals LLC
Atlantis Healthcare
aTyr Pharma
Avella
Ax-S Pharma
B&O Medicom
B.D.C. Venture Capital
B.M.S.
Barth Syndrome Foundation
Baxter
Bellus Health Inc
Benefits Strategies LLC
BioBlast Pharma
BioBlast Pharma Ltd
Biocodex USA
Biocompare
BioMarin Pharmaceutical Inc
Biomarin Pharmaceutical Inc.
BioPontis Alliance for Rare Disease
BioPortfolio
Bluebird Bio
Brace Pharma Capital
Brazilian Federal Council of Medicine
Bristol-Myers Squibb
C1 Consulting
CADTH
Caligor Rx
Cardinal Health
Caring Voice Coalition
Casa Hunter
Catalent Pharma Solutions
Catalyst Pharmaceuticals
CDER FDA
Certara
CheckOrphan
Children' s Tumor Foundation
China Dolls Center For Rare Disorders
Clear Pharma
Clementia Pharmaceuticals Inc.
Clinart M.E.N.A.
Clinigen Global Access Programs
Clinuvel Pharmaceuticals Ltd
Coalition for Clinical Trials Awareness
Codexis
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
CurePSP
Cydan Development
Cydan Development Inc
Cytokinetics
Cytori Therapeutics Inc
D.A.R.A. BioSciences Inc
DARA BioSciences, Inc
Deerfield Institute
DeFabio Designs
Dicerna Pharmaceuticals
Discovery USA
Dohmen Life Science Services
Dong-A
DrugDev
Edinburgh BioQuarter
EDSers United
Eli Lilly
Emerge Health Pty Ltd
Emmaus Life Sciences, Inc
Ergomed Group
Euromedica
EURORDIS
EvaluatePharma Ltd
EveryLife Foundation for Rare Diseases
Expressa Group
F. Hoffmann-La Roche Ltd
Faulkner Export Packaging Ltd
FDA
Fibrocell Science Inc
Foundation for Research on Rare Diseases and Disorders India
Friedrich's Ataxia Research Alliance
Genetic Alliance
Genoa Pharmaceuticals
Genpharm
Genpharm services
GenSight Biologics
Genzyme
Genzyme Corp
Genzyme, A Sanofi Company
Georgia Bio
GF Communications
GFK
Giddi Pharma
GlaxoSmithKline Rare Diseases
Global Genes | RARE Project
Grunenthal Gmbh
Health Advances
Healthcare at Home
Hereditary Neuropathy Foundation
Hovione Llc
Hyperion Therapeutics
I.S.M. Therapeutics
Idera Pharmeceuticals
Idis Inc
IDIS Pharma
In Flectis Bio Science
Industrifonden
Inflectis Bioscience
Infusion
Insmed Incorporated
Inspire
Institute For Medical Engineering and Science Harvard M.I.T. Division of Health Sciences and Technology
Institute for Optimizing Health Outcomes
Interfarma - Associação da Industria Farmac ê utica de Pesquisa
International Business Consultants
inVentiv Health Clinical
Iptechex Pharmalicensing
Jazz Pharmaceuticals, Inc
Juice Pharma Worldwide
K.B.I. Inc
Kaiser Permanente
Karyopharm Therapeutics
KBI Biopharma Inc
Kedrion Biopharma
Kids v Cancer
Lapidus Data
LGS Foundation
Lifescience Leader
LINK Healthcare
LipoMed Inc
Lumos Pharma
M3 Pharma S/A
Macy's
Managed Care Magazine
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
Metrum Research Group LLC
Ministry Of Health Brazil
MLV & Co.
MME LLC
MMRG
Moksha8
Momenta Pharmaceuticals Inc

MPCC
MVR
Myotonic Dystrophy Foundation
N.I.H
N.O.R.D.
N/A
National Eye Institute N.I.H.
National Institute for Health and Care Excellence
National Institute of Health and Medical Research (INSERM)
National Organization For Rare Disorders
NCATS, NIH
Neem Biotech Ltd
Nektar Therapeutics
Neuraltus Pharmaceuticals
Neurohealing Pharmaceuticals
Neurolixis
NHLBI - National Heart Lung and Blood Institute
NIH / NCATS / Office of Rare Diseases Research
Nordic Nanovector As
Octapharma
Omnicare Inc
Omnicare Specialty Care Group
OncoSynergy
One-World, inc
Oppenheimer and Co Inc
Opsona Therapeutics Ltd
Optio Biopharma Solutions LLC
Optum Labs Inc
Organization for Rare Diseases India
Orig3n
Orphan Drug Solutions
Orphan Druganaut Blog
OrphanReach
Orpharma
Parent Project Muscular Dystrophy
Parents and Researchers Interested in Smith-Magenis Syndrome (PRISMS)
Parexel
PatientCrossroads
Perlstein Labs
Pfizer
Pharmacenter Ltd
Pharmaceutics International Inc
Pharmaessentia Corp
PharmaVision
PharmaVoice
Pharnext
PharPoint Research Inc
Phase Medical
Phelan-McDermid Syndrome Foundation
Phenomenome Discoveries
Phoenix Clinical Research
PII
Portola Pharmaceuticals , Inc.
PRA Health Sciences
Premier Research
PreScience Labs
Primevigilance
ProMetic Life Sciences Inc.
Prosensa
Prothena
Provectus Pharmaceuticals Inc
PSR-Agility Orphan Drug Development
PTC Therapeutics
Public Health Alliance For Clinical Trials
Pulmonary Hypertension Association
Qol Medical Llc
Quay Pharma
Quintiles
Raptor Pharmaceuticals Corp
Rare Disease Report
Rare Disease United Foundation
RARE Science
Rare Voices Australia
RASopathies Network USA
Reata Pharmaceuticals
Recordati Rare Diseases Inc
Recursion Pharmaceuticals
Reflex Sympathetic Dystrophy Syndrome Association of America
Registrat Mapi
Retrophin
Rhinocyte Inc
Rho Inc
RKC
Roche
R-Pharm
RTI Health Solutions
Sanford C.O.R.D.S. Sanford Research
Sanofi
Sao Paulo Association of Mucopysaccharidosis and Rare Diseases
Sarcoma Foundation of America
Sarepta Therapeutics
Savara Pharmaceuticals
Science Magazine
Seattle Children's Research Institute
SelectScience
Shire
Sickle Cell Disease Association of America, Inc.
Sickle Cell Disease Association of California
Simbec-Orion Group
Siren Interactive
Sixera Pharma Ab
SmithSolve
SNW Consulting
Sobi
Solpharm d.o.o.
Sonexus Health
Stemcells Inc
Stemline Therapeutics Inc
Stevenson Group Limited
Synageva
Synlogic Inc
Taiba Pharma LLC
Technology Networks
Teva
The Assistance Fund Inc.
The Cholangiocarcinoma Foundation
The Digestive Disease National Coalition
The Erythromelalgia Association
The George Washington University Children's Hospital National Medical Center
The Menkes Foundation
The Oxalosis and Hyperoxaluria Foundation
The Sturge-Weber Foundation
Therabron Therapeutics
Threshold Pharmaceuticals Inc
Toleranzia Ab
Tolero Pharmaceuticals
UCB Biopharma SPRL
Ultragenyx Pharmaceutical Inc.
UNIFESP
United BioSource Corp
United Mitochondrial Disease Foundation
University of Minnesota
University of Oxford
University of Utah
Uno Healthcare Inc
VaccineNation
Valley Fever Solutions
Vanda Pharmaceuticals Inc
Vencore
Veristat LLC
Vertex Pharmaceuticals
Vicus Therapeutics
Vida Therapeutics Inc
Vifor Sa
Vtesse, Inc.
William Blair
X.O.M.A. Corp
Xcelience
Xeroderma Pigmentosum Family Support Group
Zogenix Inc
ZS Associates

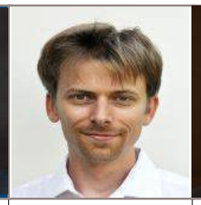
Speakers at this year's meeting included C-level executives and decision makers from the leading orphan drug manufacturers, policy makers and influencers, patient advocacy group leaders from different regions of the world.

THE VIP CONFERENCE

Over 600 conference delegates attended the keynote sessions.

Top speakers

Henri Termeer, Chief Executive Officer, Former, **Genzyme**



Christoffer Nellaker, Research Fellow, Medical Research Foundation's Functional Genomics Unit, **Oxford University**

Jeff Myers, President and Chief Executive Officer, **Medicaid Health Plans of America (MHPA)**

Mark Rothera, 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 Groft, Former Director of the Office of Rare Disease Research, **NIH**

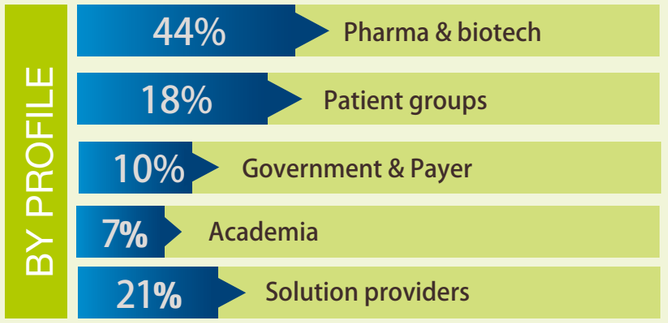
Eric Hoffman, Director, Research Center for Genetic Medicine, **Children's National Hospital**

Kathleen Coolidge, Director, US Patient Advocacy, Rare Disease, **Genzyme**

Peter Saltonstall, 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

THE PARALLEL TRACKS

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

HIGHLIGHTS

- 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
- How to market your orphan drug with and without a patient group
- 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

HIGHLIGHTS

- 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:

HIGHLIGHTS

- 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.

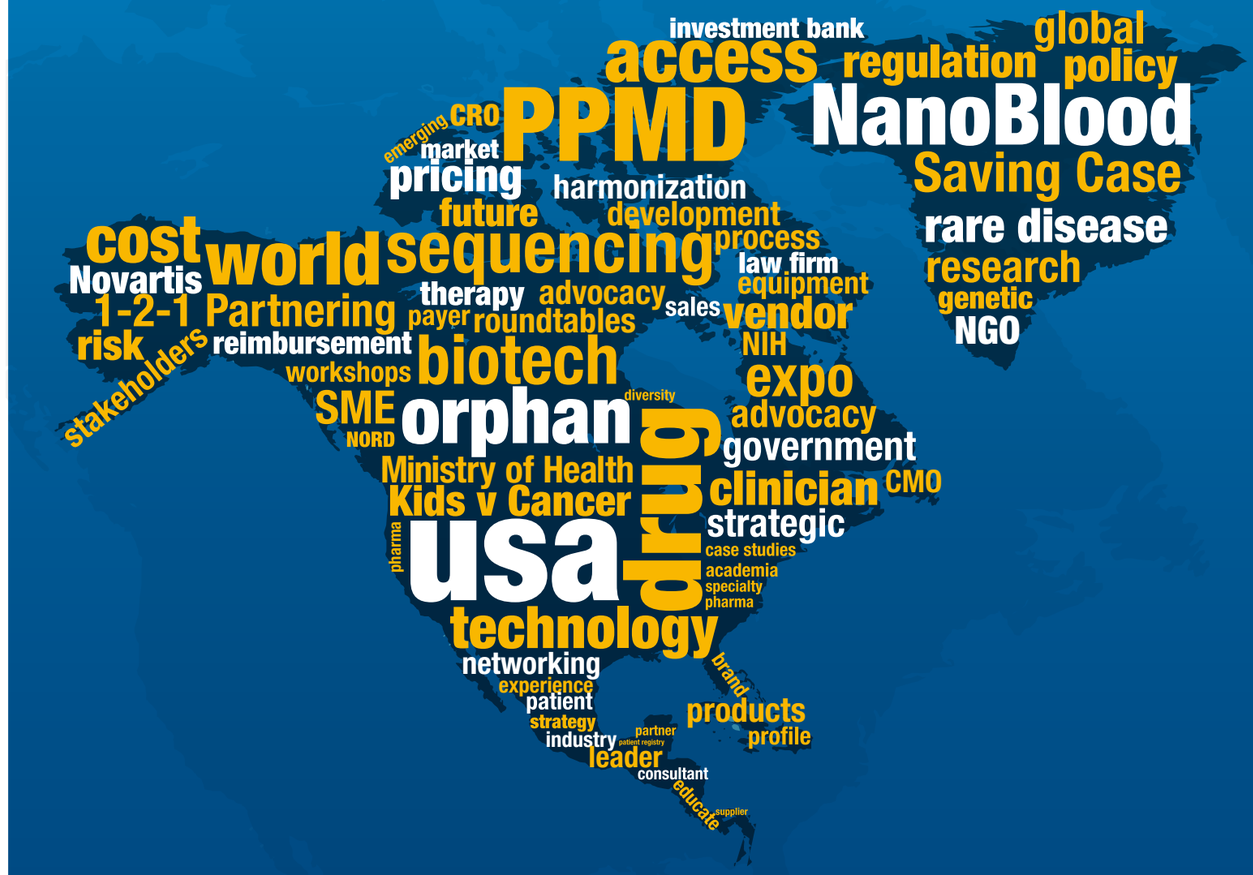
The marketing campaign used every channel possible and reached an audience of over 750,000 industry stakeholders:

- 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

Official Media Partners

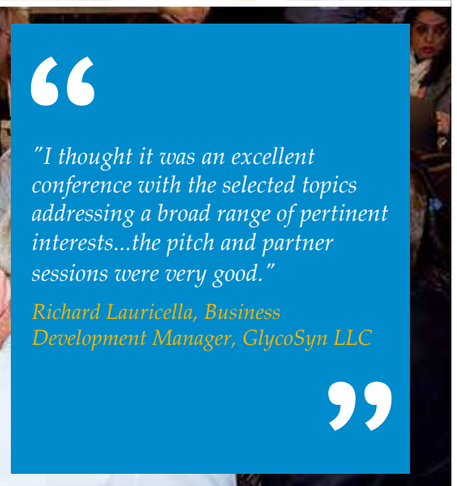
Biocompare – The Buyer's Guide for Life Scientists
Evaluate
Georgia Bio – The Life Sciences Partnership
Global Genes Project – for RARE and genetic diseases
Rare Disease United Foundation
Orphan Druganaut Blog
SelectScience – The Fastest Way to Expert Opinion
PharmaVOICE
Clinical Leader
iptechex pharmalicensing
RDR Rare Disease Report
antibodies-online.com

> *"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*



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

WORLD OrphanDrug Congress USA



"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, GlycoSyn LLC

WORLD Orphan Drug Congress USA 2016

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 advnaced 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
The Washington Hilton, Washington DC, US