



Paradigm Global Events
BUILDING PARTNERSHIPS ACROSS THE GLOBE



9th - 11th September, 2019
Hilton Boston Back Bay,
Boston MA, USA



11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

Current Trends and Innovations driving access to meet unmet medical needs of rare disease patients

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Key Expert Speakers

- > **Pankaj Agarwal**, Senior Fellow, Computational Biology, **GSK**
- > **Derek Ansel**, Director of Rare and Orphan Diseases, **Syneract**
- > **Samuel Agus**, Chief Medical Officer, **Biophytis – New Therapeutics for Aging Diseases**
- > **Daniel Anderson**, Head of Commercial Partnerships, **Invitae**
- > **Subha B. Basu**, Managing Director, **Converge Advisory Group**
- > **Megan O'Boyle**, Principal Investigator, **Phelan-McDermid Syndrome International**
- > **Philip John (P.J.) Brooks**, Program Director, **Office of Rare Diseases Research, NCATS**
- > **Anne B. Cropp**, Chief Scientific Officer, **Early Access Care LLC**
- > **Khrystal Davis**, Founder & President, **Texas Rare Alliance**, Advisory Member, **Texas Newborn Screening**
- > **Lisa Deck**, Committee Member, **Rare New England**, Co-Founders and Director, **Sisters@Heart, Inc.**
- > **Dan Donovan**, Founder & CEO, **rareLife solutions**, creators of onevoice
- > **Robert Donnell**, Head of Business Development, **Durbin PLC**
- > **Jocelyn Duff**, Co-Founder/Executive Director, **CureCMT4J/Talia Duff Foundation**
- > **Ashish Dugar**, VP, Global Medical Affairs, **Sarepta Therapeutics**
- > **Todd Galles**, Business Development Principal, **Durbin**
- > **Anthony Gucciardo**, SVP, Strategic Partnerships, **National Kidney Foundation**
- > **Femida Gwadry-Sridhar**, CEO & Founder, **Pulse Infoframe Inc.**
- > **Aileen Healy**, Exec. Director & Global Head, Rare Diseases, Worldwide R&D, **Pfizer, Inc.**
- > **Pedro Huertas**, Chief Medical Officer, **Sentien Biotechnologies**
- > **Hannah Kane**, State Representative, **Eleventh Worcester District**
- > **Ken Kengatharan**, Managing Partner, **Atheneos Ventures**, CEO, **Auxesia Orion**
- > **Takashi Kei Kishimoto**, Chief Scientific Officer, **Selecta Biosciences**
- > **David Lapidus**, President, **LapidusData Inc.**
- > **David Litwack**, Director, Regulatory Strategy and Communications, **Prevail Therapeutics**
- > **Qing Liu**, Founder & Principal **QRMedSci**, former Program Strategy Lead, **Amicus Therapeutics**
- > **Isabelle Lousada**, President & CEO, **Amyloidosis Research Consortium**
- > **Elena Lungu**, Manager, Policy Development, **Patented Medicine Prices Review Board / Canada**
- > **Neena Nizar**, Founder and Executive Director, **Jansen's Foundation**
- > **Mike Page**, Executive Director, Global Regulatory Affairs Portfolio Products, **Alexion Pharmaceuticals**
- > **Maria Picone**, Co-Founder/CEO, **TREND Community**
- > **Amit Rakhit**, Chief Medical Officer and Head of Research & Development, **Ovid Therapeutics**
- > **Julie Raskin**, Executive Director, **Congenital Hyperinsulinism International**
- > **Luke Rosen**, Founder, **KIF1A.ORG** / VP, Patient Engagement & Gov't. Affairs, **Ovid Therapeutics**
- > **Matthias Schoenermark**, Professor, President and CEO, **SKC Beratungsgesellschaft mbH**
- > **Samuel Seward, Jr.**, Professor and Chair, Dept of Medicine, **Mount Sinai St. Luke's & West Hospitals**
- > **James P. Shaffer**, Chief Business Officer, **Eiger BioPharmaceuticals**
- > **Kristin Smedley**, President, **Curing Retinal Blindness Foundation**
- > **Haya Taitel**, SVP, Chief Commercial Officer, **Kadmon Pharmaceuticals, LLC**
- > **Joseph Ternullo**, Head, Business Development, **Pulse Infoframe**
- > **Vladimir Tomov**, Chairman, **National Alliance of People with Rare Diseases – Bulgaria**
- > **Stephan Toutain**, Sr. Vice President, Operations, **Anavex Life Sciences**
- > **Sara Tylosky**, CEO and President, **Farmacon.co**
- > **Robert Ward**, Chairman & CEO, **Eloxx Pharmaceuticals**
- > **Patricia Weltin**, CEO and Founder, **Beyond the Diagnosis**

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INTRODUCTION:

Dear Colleagues,

Paradigm Global Events is again proud to present our **Orphan Drugs and Rare Diseases Global Congress 2019 Americas**. It's the 11th in the series of our Flagship tri-annual Orphan Drugs and Rare Diseases event, this congress will provide you with current trends and innovations driving access to meet unmet medical needs of rare disease patients.

According to a recent report, North America continues to be anticipated to stay dominant in the global orphan drugs market between 2018 – 2025. According to the report, the global orphan drugs market was valued at US\$ 125000 Mn in 2017 and is anticipated to reach US\$ 294000 Mn by 2025. The report also stated that the market will exhibit a remarkable CAGR of 11.4% during the forecast period. As per Fortune Business Insights, the North America orphan drugs market was worth US\$ 67,136.0 Mn in 2017 and will continue to rise at a steady pace until 2025.

The growth of the global orphan drugs market is increasing at a rapid pace due to the growing awareness of people concerning the use of novel drugs. Government in North Americas are also increasingly investing in the research and development and of orphan drugs and innovative technologies. With further growth anticipated to meet the high unmet demand for more efficacious drugs with very little side effects. Although the number of people affected by rare diseases is considerably low, the return of investment is high due to the high cost of orphan drugs. Global collaboration is also likely to fuel growth. FDA recognizes the significance of orphan drugs in the treatment of rare debilitating, life-threatening diseases, therefore, supporting stakeholders to promote research and development in this area.

However, some factors such as high initial investment that leads to higher per patient treatment cost, reimbursement uncertainties and high cost of drug development are hindering the growth of orphan drugs market. North America registered significant growth for the market during the forecast period due to rising healthcare spending, constructive government initiatives, growing occurrence of chronic diseases and small timeline required for orphan drug development.

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas will provide a unique platform for the convergence of stakeholders in the orphan drugs industry to discuss and network with top tier government, hospitals, pharmaceuticals, biopharmaceuticals, non-profit organizations, orphan drugs developers as well as regional and local manufacturers.

Introducing a new format this year, we are putting together an agenda that addresses "**Current Trends and Innovations driving access to meet unmet medical needs of rare disease patients**". We have gathered together 50+, high caliber expert speakers to participate and share their knowledge and expertise through Panel discussions, keynote presentations, round table discussions and 4 major agenda streams addressing all aspect of the industry.

We look forward to welcoming you at the congress!

Sincerely yours,



Jocelyn Raguindin
Conference Director
Paradigm Global Events

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

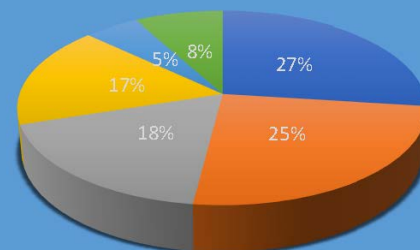
Gain Latest Insights on:

- Current Orphan Drugs landscape and major drivers in global rare disease market
- Key developmental strategies carried out and Influencing factors that may affect market share to stand out in this industry
- Current FDA thinking regarding common issues encountered in rare disease drug development
- Finding innovative and alternative ways in funding the development of Orphan Drugs
- Patients perspective: what really matters to rare disease patients and caregivers?
- Key authorities facilitating development and approval of diagnostic products/services
- Coming Together in Developing Orphan Drugs and Crossing Borders
- Trends and evolution of Advance Therapy Medicinal Products
- How Can the Developer and the Patient Assist in the Evolution and Development of Orphan Drugs to make it accessible to patient in shorter period of time?
- Strategies to improve Access and Affordability
- What Do Developers Look for When Looking for an Outsourcing Partner?
- What Do Insurance Companies Think About Orphan Drugs? Will They Make Modifications to Their Policies to Support Patients with Rare Diseases?
- Opportunity to network with Peers, potential Partners and Investors

The two-day Congress will provide an interactive and intimate discussion and networking format led by key expert speakers with extensive knowledge in the industry. Gain practical strategies and best practices on challenges, innovations, technologies and concepts surrounding orphan drugs and rare diseases.



Attendees Demographic



- Pharmaceuticals
- Biotech
- Service Providers
- Advocacy Groups
- Government/Regulators
- Others

WHO WILL YOU MEET:

This congress is specially created for valued stakeholders in the Rare Disease community:

Presidents, Heads/Chiefs, Directors, VPs and Managers in the are of:

- Research and Development
- Personalised Medicine
- Regenerative Medicine
- External R&D Innovation
- Innovative Medicine
- Rare and Ultra-Rare Diseases
- Cell and Gene Therapy
- Translational Science
- Molecular Geneticist
- Program Management
- Patient Advocacy Groups
 - Public Affairs
 - Medical Affairs
 - Regulatory Affairs
- Clinical Research Organizations
 - Market Access
- Pricing and Reimbursement
- Health Economics Outcomes Research
- Commercial Development
- Investments and Funding
 - Product Specialist
 - Global Strategic Services
- Business Planning and Operations
 - Pharmacies
 - Academia

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

SPONSORS AND EXHIBITORS

Gold Sponsor



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collegen

SKC consulting is the leading and most reputable strategy consultancy in the German health care sector. The company was founded in 2005 as a spin-off of the Boston Consulting Group and the Hannover Medical School (MHH) with a focus on companies, organizations and institutions in the German and international health care markets.

Our managing directors and colleagues are experienced and reliable advisors to pharmaceutical, medical and biotechnological companies as well as health insurances. With our profound knowledge of the German and the European health care systems, we support our clients in meeting their strategic challenges, especially in Market Access, Pricing and Reimbursement and Strategic Communication.

We at SKC think that every activity in the health care industry should focus on the improvement of the patient's situation. We are convinced that the best solution for a health care problem can only be found in a competitive landscape, shaped by innovation and empowered by trust. We believe a better, more effective, efficient and fair health care system is possible due to digital technologies, interdisciplinary networks and highest professional standards.

Visit: skc-consulting.de

Gold Sponsor



Invitae Corporation (NYSE: NVT) is a genetics company whose mission is to bring comprehensive genetic information into mainstream medical practice to improve the quality of healthcare for billions of people. Invitae's goal is to aggregate most of the world's genetic tests into a single service with higher quality, faster turnaround time and lower prices.

<https://www.invitae.com/en/>

Gold Sponsor



Eiger is a late-stage biopharmaceutical company focused on the accelerated development and commercialization of a pipeline of targeted, first-in-class therapies for rare and ultra-rare diseases. The company's lead program is in Phase 3, developing lonafarnib, a first-in-class prenylation inhibitor for the treatment of Hepatitis Delta Virus (HDV) infection. The company is also preparing an NDA with plans to file in 2019 for lonafarnib in the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and Progeroid Laminopathies. For additional information about Eiger, please visit: www.eigerbio.com

Gold Sponsor



Durbin is one of the leading providers of comparator sourcing and managed access programs to pharmaceutical and biotech companies around the world. In its 55th year of business, Durbin is pleased to look back on a history of sourcing pharmaceuticals for physicians and patients around the world and even in 3rd world countries, providing urgent and cost effective supply of pharmaceuticals to global charity and relief organizations and of course its pharmaceutical services division who source and distribute comparator drugs for clinical trials and run managed access programs that provide access to needed pharmaceuticals in non-approved markets. Durbin's distribution network includes regular shipments to over 180 countries globally. Their managed access programs include orphan, gene, and controlled products. Durbin has full service offices in the United Kingdom, European Union and United States. <http://www.durbinglobal.com/>

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

SPONSORS AND EXHIBITORS

Silver Sponsor



Pulse Inframe Inc (Pulse) believes that collaboration cures disease and Pulse enables such collaboration. A new style of healthcare informatics company, Pulse builds and supports global, healthcare ecosystems. It's solution, healthie™, empowers consortia of pharmaceutical and medical device companies, researchers, clinicians, advocacy groups and patients, by aggregating information from a variety of sources and then, delivering key healthcare insights – real time -- derived from these data. For patients with rare diseases, Pulse's global coverage is critical. Already great strides have been made in characterizing rare diseases using Pulse's global and national registries. These insights help professionals to advance medical research, focus clinical trial recruitment, assist advocacy groups in their dealings with policy makers and provide real-world evidence.

<https://www.pulseinframe.com/>

Bronze Sponsor



rareLife solutions is the rareForward scientific agency built on the insight that rare patients, advocates, and caregivers (PACs) are the primary shapers of their rare disease ecosystem. But we do more than talk about them - we action them, regularly engage them, and integrate them in everything that we do to create a truly collaborative, responsive multi-stakeholder, patient-driven rare community. We have developed thinking, services, tools, and solutions, including our flagship onevoice.world community platform, that can help you rareForward too.

<http://www.rarelifesolutions.com/>

Lead Exhibitor



Early Access Care helps international clients with effective integrated solutions customized to their early access needs. Early Access Care specializes in pre-approval early access, managing the end to end (request intake to delivery at physician) process. Our goal is to Make Access More Accessible™. We offer comprehensive high-touch services to manage requests globally.

<https://www.earlyaccesscare.com/>

Basic Exhibitor



PerkinElmer Genomics is a state-of-the-art biochemical and molecular genetics laboratory that provides newborn screening and genomic testing services around the world. With over 7.5 million newborns screened since 1994, our laboratory pairs decades of newborn screening experience with a leading-edge clinical genomics program to offer one of the world's most comprehensive programs for detecting clinically significant genomic changes.

<https://www.perkinelmergenomics.com/>



11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

SPONSORS AND EXHIBITORS

Associate Sponsor



Farmacon is a novel research consulting group of medical and logistics experts that specialize in Latin America & Latino populations to get enrollment and market access results in complicated medical projects, including rare and orphan disease and other medical projects. Our unique concierge approach includes building strong relationships with key stakeholders, such as Principal Investigators, Sub-Investigators, referring doctors, KOLs, patient advocacy groups, and families to provide market research intelligence, access and strategies. Our unique feedback mechanism allows sponsors the needed assurance to get the results they seek in a patient-centric world within clinical research and market access preparation.

<http://www.farmacon.co/>

Associate Sponsor



Synteract is an innovative, full-service contract research organization supporting biopharmaceutical companies in all phases of clinical development to help bring new medicines to market. Synteract has conducted 4,000+ studies on six continents and in 60+ countries, and contributed to more than 240 product approvals.

The CRO offers a notable depth of expertise in rare and orphan diseases, having conducted 130+ clinical trials in the last 5 years alone. In addition, Synteract focuses in the areas of oncology, dermatology, pediatric, and neuro degenerative drug development. Learn more at

<https://www.synteract.com/>

Associate Sponsor



TREND Community is a digital health company with a mission to improve the quality of life for everyone living with rare and chronic disease while accelerating the discovery, development, and delivery of new treatments. Using artificial intelligence, we capture and quantify the perspectives and lived experiences of the community from their social data streams.

<https://trend.community/>

Associate Sponsor



David Lapidus founded LapidusData Inc. in 2008 to help orphan drug companies analyze patient populations for rare diseases where the population dynamics are complex or poorly characterized. LapidusData brings a commercial perspective to epidemiology and market research in support of M&A activity, market forecasting, sales, and marketing. Our analyses are used to support the entire drug development cycle, from pre-clinical planning through commercialization and beyond.

EVENT PARTNERS:



11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

PRE-CONFERENCE WORKSHOP
9TH SEPTEMBER 2019

Opportunity for Real-World Evidence in Improving Patient Outcomes

ABOUT

Observational studies using real-world evidence (RWE) provide significant opportunities to gain insight into treatment patterns and outcomes in clinical practice outside randomized controlled trials. RWE is being widely used to gain an understanding of disease epidemiology, patient journey, real-world use and effectiveness of treatment options, unmet patient needs and the value that products offer in sub-populations. In this workshop, you will learn the opportunities and benefits of RWE.

WHO IS IT FOR ?

This workshop is ideal for anyone who wishes to become familiar with key issues in the areas of real-world evidence. It is particularly relevant to individuals working for Pharma/Biotech companies, regulatory authorities, HTA bodies, patient organizations, consultancy and academic institutions.

AGENDA

9:00 – 10.30am - Interactive presentation
10:30 – 11:00am - Networking break
11:00 – 12:30pm - Interactive presentation and Discussion

Led by:

Femida Gwadry-Sridhar, CEO & Founder
Joe Ternullo, Head, Business Development



Managed Access/Early Access Program

ABOUT

This interactive presentation will provide you with an opportunity to learn more about how EAP's provide pre-launch access for drugs in advance of their Marketing Authorization (MA) for patients with life-threatening conditions and no approved treatment options. Best practices in EAP planning, Set-Up and management, and provide information on current regulatory changes

WHO IS IT FOR ?

This workshop will be extremely beneficial to those individuals considering an EAP as part of their drug development strategy. Project Managers and Executives in Pharma/Biotech/Advocacy interested in knowing how to set-up an Early Access Program.

AGENDA

13.30 - EAP Perceptions, fears, hopes & knowledge
14.00 - Case- study – GENE THERAPY MAP
14.15 - Introduction to MAP – regulatory overview and patient and HCP perspective
15.00 - Break
15.30 - Case – study – GLOBAL CNS MAP
15.45 - Implementing a MAP

- Functions
- Timings
- Costs
- Considerations
- Charging

16.30 - Case – study – GLOBAL Oncology MAP
16.45 - How we go forward

Presenter:

Robert Donnell, Head of Business Development
Supported by: Dan Piggott / Todd Galles



11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

DAY 1, 10TH SEPTEMBER 2019

8:00 am

Conference Registration and Continental Breakfast

8:50 am

Chairpersons Welcome and Opening Remarks



MATTHIAS SCHOENERMARK
PROFESSOR, PRESIDENT AND
CEO, SKC
BERATUNGSGESELLSCHAFT MBH

9:00 am

Opening Keynote Panel Discussion: Patient perspective on Living with Rare disease: What really matters?

- > Unique challenges in living with a rare disorder
- > Social implications and aspects of living with a rare disease
- > Consequences of living with a rare disease
- > Experience with the healthcare system
- > Challenges in bringing treatment to Ultra rare disease

Moderator:



FEMIDA GWADRY-SRIDHAR
CEO & FOUNDER,
PULSE INFOFRAME INC.

Panelists:



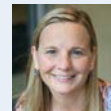
PATRICIA WELTIN
CEO & FOUNDER,
BEYOND THE
DIAGNOSIS



NEENA NIZAR
FOUNDER & EXECUTIVE
DIRECTOR, JANSEN'S
FOUNDATION



MARIA PICONE
CO-FOUNDER/CEO,
TREND COMMUNITY



LISA DECK
COMMITTEE MEMBER,
RARE NEW ENGLAND

9:40 am

Industry Keynote Panel Discussion: Current Orphan Drugs Landscape and What must be done by Stakeholders to accelerate access and affordability while continuously advancing in R&D and innovations

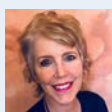
- > Legislation incentivizing the pharmaceutical industry to invest in therapeutics for rare diseases
- > Advances in sequencing that made orphan drugs development faster and cheaper
- > What can be done by healthcare providers to be proactive in monitoring side effects that can put patient safety at risk and effectively communicate results to the developer?
- > Why should patients' information about their treatment experience be collected and how can this steer the evolution of orphan drugs?
- > How social media plays a role for developers and patients to communicate and how companies can utilize social media to innovate and create new drugs for patients?

Moderator:



FEMIDA GWADRY-SRIDHAR
CEO & FOUNDER,
PULSE INFOFRAME INC.

Panelists:



SARA TYLOSKY
CEO AND PRESIDENT,
FARMACON.CO



DANIEL ANDERSON
HEAD OF COMMERCIAL
PARTNERSHIPS,
INVITAE



JIM SHAFFER
CHIEF BUSINESS OFFICER,
EIGER
BIOPHARMACEUTICALS



SUBHA B. BASU
MANAGING DIRECTOR,
CONVERGE ADVISORY
GROUP

10:20 am

Morning Networking & Refreshment Break

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

DAY 1, 10TH SEPTEMBER 2019

11:00 am

Plenary Keynote: Risky business: success and failure in orphan drugs' business model

- > The ODRD dilemma – population size vs. price premium
- > Key success factors for a powerful and sustainable market access
- > Learning from failure: avoidable roadblocks and pitfalls
- > Collaborative approaches for a complex environment



Matthias Schoenermark, Professor, President and CEO,
SKC Beratungsgesellschaft mbH

11:30 am

Plenary Keynote: Top 5 Game Changers for Rare Disease Treatment as We Know It Today

Ø **What are the top issues facing the rare disease community today and who/how will they be addressed?**

- > What innovative changes are coming in research, clinical development, manufacturing, registrational paths, and commercial models that will impact the rare disease community
- > How will patient advocacy and healthcare policy in the US healthcare environment change
- > Will the future be a place without rare diseases



Amit Rakhit, Chief Medical Officer and Head of Research & Development,
Ovid Therapeutics

12:00 am

Plenary Keynote: Maintaining global access to treatment for an ultra-rare disease through EAP

- > Identifying a pathway and treatment for an ultra-rare disease
- > Working through extraordinary circumstances
- > Ensuring access to patients around the world through a well-designed and clearly-communicated EAP



Jim Shaffer, Chief Business Officer, **Eiger BioPharmaceuticals**

12:30 pm

Plenary Keynote: Managed Access Programs – integral to Orphan launches and long term rest of World supply.

- > How MAPs fit with an orphan launch strategy
- > How to design them effectively taking all stakeholders into account
- > Best practice in implementation and management
- > When does a MAP end?



Robert Donnell, Head of Business Development, **Durbin PLC**

1:00 pm

Networking Lunch

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

2:00 pm - Attend any of the 3 Topic Focus Streams

Regulatory, Commercial and Current Trends

2:00 pm - Chairperson's Opening Address



2:10 pm – Improving the precision of precision medicine: scientific and policy challenges

- > Despite the rapid growth of precision medicine, its use in the clinic is still limited.
- > Technical issues, including a lack of standards and issues surrounding data reproducibility, slow the discovery and translation of targeted therapies.
- > Translation of promising therapies to the clinic requires novel approaches to regulation, payment, and patient/provider education.
- > Expansion of precision medicine will rely on the development and effective use of real world-data, open-access resources, and other innovations.

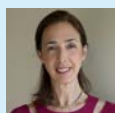


DAVID LITWACK
DIRECTOR, REGULATORY STRATEGY AND COMMUNICATIONS, PREVAIL THERAPEUTICS



2:40 pm – The role of patient organizations in approval, pricing and reimbursement of new therapies

- > Acting as the honest broker
- > Patient preferences
- > Improving endpoints



ISABELLE LOUSADA
PRESIDENT & CEO, AMYLOIDOSIS RESEARCH CONSORTIUM

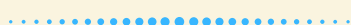


Access and Collaborations

2:00 pm - Chairperson's Opening Address



MIKE PAGE,
EXECUTIVE DIRECTOR, GLOBAL REGULATORY AFFAIRS, ALEXION



2:10 pm - Global early access programs to support rare indications

This session will provide key insights into the development and execution of global pre-approval access programs, including:

- > Considerations for successful program
- > Runway for effective early planning
- > Scope considerations - individual vs. cohort programs in global territories
- > The skinny of data in early access programs



ANNE B. CROPP
CHIEF SCIENTIFIC OFFICER, EARLY ACCESS CARE LLC



2:40 pm - Mobilizing Your Rare Disease Community: A Patient Organization's Role in R&D

Tools for patients and caregivers to play an active role in accelerating therapeutic development.

- > How to identify rare communities with similar challenges
- > Forging efficient collaborations within the scientific, biotech and patient communities
- > A foundation's role in pre-clinical research
- Importance of clinical trial readiness
- > Advocacy for regulatory approval

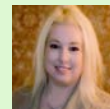


LUKE ROSEN
FOUNDER, KIFIA.ORG /VP, PATIENT ENGAGEMENT, OVID THERAPEUTICS



Innovations, Clinical Development and Advance Therapeutics

2:00 pm - Chairperson's Opening Address



KHRYSTAL DAVIS, FOUNDER & PRESIDENT, TEXAS RARE ALLIANCE, ADVISORY MEMBER, TEXAS NEWBORN SCREENING



2:10 pm – Platform Approaches to Rare Disease Therapeutics and the NIH Common Fund Somatic Cell Genome Editing Program

- > Grouping multiple rare diseases by underlying molecular etiology to accelerate clinical trials: learning from oncology
- > Overview of the NIH Common Fund Program on Somatic Cell Genome Editing
- > The Rare Disease Clinical Research Network as a platform for clinical trials



P.J. BROOKS
PROGRAM DIRECTOR, OFFICE OF RARE DISEASES RESEARCH, NCATS



2:40 pm - Rethinking clinical trial design in Duchenne Muscular Dystrophy (DMD)

- > The future of DMD treatment is multimodal
- > Selection of study population and study design should be fit-for-purpose
- > Disease progression is an individual struggle with an increasing burden
- > Participation is key to success



SAMUEL AGUS
CHIEF MEDICAL OFFICER, BIOPHYTIS



11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

2:00 pm - Attend any of the 4 Topic Focus Streams

Regulatory, Commercial and Current Trends

3:10 pm - When building a commercialization platform for an asset for the management of rare disease, **Insight Mining associated with the Patient Path, Disease Burden and Unmet Needs are critical to establishing a product value proposition**

- > Need for Education Platforms generating Disease awareness, and facilitating path to early diagnosis
- > Referral to Centers of Excellence offering a multi-disciplinary expert management and collaboration with the patient advocacy.
- > Case studies will be highlighted



HAYA TAITEL,
SVP, CHIEF COMMERCIAL
OFFICER, KADMON
PHARMACEUTICALS

3:40 pm – Networking Break

4:20 pm - Asia – the rare disease final frontier – is this where innovation will be found in the 2020's?



ROBERT (BOB) WARD
CHAIRMAN & CEO,
ELOXX
PHARMACEUTICALS

Access and Collaborations

3:10 pm - Why Do Sponsors with Orphan Disease Therapies need to be in Latin America?

- > Opportunities – Landscape and Logistics
- > Challenges – Culture and Complexities
- > Case studies: Pediatric & Adult



SARA TYLOSKY
CEO AND PRESIDENT,
FARMACON.CO

3:40 pm – Networking Break

4:20 pm - Raring to Go for CHI!: Collaborations to Improve the Lives of People with Congenital Hyperinsulinism

- > A Patient Partnership with Clinicians, Researchers, Nonprofits, and Biotech/Pharma
- > Patient-Powered Natural History for New Treatments and a Cure
- > Access to Genetic Testing and Existing Treatments
- > Improved Clinical Trial Operations, Protocol Design, and Expanded Access
- > Pilot Research Projects
- > Travel funds at Centers of Excellence
- > Physician and Patient Education Awareness through Conferences and Social Media
- > Family Support Forum



JULIE RASKIN
EXECUTIVE DIRECTOR,
CONGENITAL
HYPERINSULINISM
INTERNATIONAL

Innovations, Clinical Development and Advance Therapeutics

3:10 pm - Hermansky-Pudlak Syndrome, Trial Recruitment and Inclusivity

- > Brief description of this rare congenital syndrome and its phenotypic presentation
- > Lessons learned from past incomplete recruitments
 - Natural History protocols
 - Pulmonary Fibrosis Drug Trial
 - GI Trial
- > Brief description of two case histories
- > Inclusivity in recruitment with respect to
 - Cultural difference
 - Language
 - Socio-Economic difference and “Big Medicine”
 - Disability

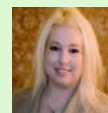


SAMUEL L. SEWARD, JR.
PROFESSOR & CHAIR,
DEPT OF MEDICINE,
MOUNT SINAI

3:40 pm – Networking Break

4:20 pm – Patients and the Value of Rare Therapies

- > Comprehensive Economic Impact Equation vs QALY
- > Recognizing Barriers to Access Rare Therapies
- > Patient Advocacy to Improve Access to Rare Therapies
- > New Therapies and New Payment Models



KHRISTAL DAVIS, FOUNDER & PRESIDENT, TEXAS RARE ALLIANCE, ADVISORY MEMBER, TEXAS NEWBORN SCREENING

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

2:00 pm - Attend any of the 3 Topic Focus Streams

Regulatory, Commercial and Current Trends

4:50 pm - Challenges around the pricing and reimbursement of EDRDs

- > EDRDs are a fast-growing market segment
- > EDRDs are more likely to generate high sales than commonly-used drugs
- > The opportunity costs for payers are significant
- > Pricing and affordability are barriers to access
- > The proposed Canadian pricing framework expected to make prescription drugs more affordable



ELENA LUNGU
MANAGER, POLICY DEVELOPMENT,
PATENTED MEDICINE PRICES REVIEW BOARD, CANADA

5:30 pm –Speakers Forum: Q & A - Orphan Drugs: Current landscape, Drivers and Future trends.

- > What are the growing trends in Rare Disease treatment?
- > How can governments and orphan drug companies help support each other's initiative to provide access to patients?
- > Stakeholders collaboration to continue fulfilling unmet needs
- > What R&D strategies can be implemented to accelerate the market access for orphan drugs?
- > Challenges in bringing orphan drugs to patients after approval

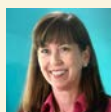
PANELIST CONSISTS OF ALL SESSION SPEAKERS

6:00 pm - Chairperson's Closing Remarks

Access and Collaborations

4:50 pm - Drug Development Resources for Rare Disease Advocates: from Pre-Clinical to Post-Market

- > How the PMS Foundation has navigated the discovery stage of drug development
- > New Resources available to rare advocacy groups trying to navigate the drug development process
- > When should groups start working toward drug development? NOW
- > How do they get started? - Ask for help and check out the resources from NCATS



MEGAN O'BOYLE
PRINCIPAL INVESTIGATOR, PMS INTERNATIONAL

5:20 –Speakers Forum: Q & A Ensuring Patient access and affordability for Rare diseases

- > Identifying barriers for access to orphan drugs
- > Addressing the challenges associated with patient access to these medicines
- > High cost associated with the medicines: Is paying for expensive treatment for a few patients sustainable?
- > Means of ensuring access and affordability of Orphan drugs
- > Establishing a reasonable price for orphan drugs

PANELIST CONSISTS OF ALL SESSION SPEAKERS

6:00 pm - Chairperson's Closing Remarks



MIKE PAGE, EXECUTIVE DIRECTOR, GLOBAL REGULATORY AFFAIRS ALEXION

Innovations, Clinical Development and Advance Therapeutics

4:45 – Gene Therapy Trial with Randomized Enrichment Design with integrated Real-World Evidence

- > Legal foundation and regulatory pathways for innovative designs
- > Design framework with integrated real-world evidence
- > Virtual matched controls methodology for comparative effectiveness analysis
- > Examples



QING LIU, FOUNDER & PRINCIPAL QRMEDSCI, FORMER STATISTICAL SCIENCE, AMICUS THERAPEUTICS

5:30 – Speakers Forum: Q & A Strategies to promote research discoveries and development of orphan products to improve the health of people with rare diseases

- > Overview of current methods for the prevention, diagnosis, and treatment of Rare Diseases.
- > Special challenges that rare diseases create for research and product regulation
- > Current public policies relevant to product development for rare diseases
- > How are advance therapy fundamentally changing the way rare diseases patients are treated
- > What are the new development in Advance Therapeutics and incentives Supporting it?

PANELIST CONSISTS OF ALL SESSION SPEAKERS

6:00 pm - Chairperson's Closing Remarks



KHRYSYAL DAVIS, FOUNDER & PRESIDENT, TEXAS RARE ALLIANCE, ADVISORY MEMBER, TEXAS NEWBORN SCREENING

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

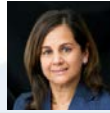
DAY 2, 11TH SEPTEMBER 2019

8:00 am

Conference Registration and Continental Breakfast

8:30 am

Chairperson's Welcome and Opening Remarks



FEMIDA GWADRY-SRIDHAR
CEO & FOUNDER,
PULSE INFOFRAME INC.

8:40 am

Keynote Panel Discussion: The role of registries in clinical research?

- > A better understanding of long-term trends in a specific patient population,
- > What makes data good data?
- > How does one harness unstructured data to provide value?
- > Can you comment on the importance of data standards and ontologies?

Moderator:

Panelist:



FEMIDA GWADRY-SRIDHAR
CEO & FOUNDER,
PULSE INFOFRAME INC.



NEENA NIZAR
FOUNDER AND
EXECUTIVE DIRECTOR,
JANSEN'S FOUNDATION



QING LIU,
FOUNDER & PRINCIPAL
QRMEDSCI,



MEGAN O'BOYLE
PRINCIPAL
INVESTIGATOR, PMS
INTERNATIONAL

9:20 am

Plenary session: Value of partnering with advocacy organizations to create innovative patient-driven solutions

- > The foundational value of building collaboration through trust, respect, transparency and empathy
- > Not all advocacy organizations are alike: assessing the landscape
- > Real-world examples of innovative digital solutions driving collaboration among multiple stakeholders
- > It isn't easy so why do it? Innovative partnering yields unique benefits boosting capabilities, medicine development and commercialization



Dan Donovan, Founder & CEO, rareLife solutions, creators of onevoice

9:50 am

Plenary Session: Innovative models to accelerate diagnosis in rare disease: Multi-partner sponsored testing programs remove barriers and speed access to a genetic diagnosis.

- > Learn how the "Invitae Detect" sponsored testing model is bringing companies together in the same disease space to generate broader awareness and faster diagnosis for more patients with rare disease.
- > Learn how expanding an existing program from one partner to three dramatically increased testing volumes and drove more diagnoses across all disorders tested.
- > Innovative new structures are increasing utility of genetic testing for patients and clinicians while decreasing risk for biopharma sponsors.



Daniel Anderson, Head of Commercial Partnerships, Invitae

10:20 am

Plenary Session: Opportunities to leverage real-world data and natural history studies for targeted drug development:

- > Unpack the meaning and utility of real-world data (RWD) & real-real world evidence (RWE)
- > Explain regulatory and legislative underpinning
- > Identify disparate sources from which RWD is obtained
- > Describe the collaborative ecosystems optimized to enable data



Femida Gwadry-Sridhar, CEO & Founder, Pulse Infoframe Inc.

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

DAY 2, 11TH SEPTEMBER 2019

10:50 am

Morning Networking & Refreshment Break

11:30 am - 12:30 pm - ROUND TABLE DISCUSSIONS - divided between 2 sessions of 30 mins. (includes 5 mins. to change table) - Attendees have 2 round table options to attend -

TABLE 1 - Orphan Drugs in Europe: Current developments in a heterogeneous market



MATTHIAS SCHOENERMARK
PROFESSOR, PRESIDENT AND CEO, SKC
BERATUNGSGESELLSCHAFT MBH

TABLE 2 - Epidemiology & market forecasting for rare diseases: value, challenges, and methods.



DAVID LAPIDUS
PRESIDENT,
LAPIDUSDATA INC.

TABLE 3 - Patient-Centric Clinical Development: The importance of patient education for rare disease and advanced therapy trials



DEREK ANSEL
DIRECTOR OF RARE AND ORPHAN
DISEASES, SYNERACT

TABLE 4 - Using AI to better understand patients and caregivers burdens and experiences from social media



MARIA PICONE,
CO-FOUNDER/CEO,
TREND COMMUNITY

TABLE 5 - Strategy and Value of Early Access in the US and EU



STEPHAN TOUTAIN
SVP, OPERATIONS,
ANAVEX LIFE SCIENCES

TABLE 6 - Incorporating natural history and registry studies of real-world evidence in clinical development



**QING LIU, FOUNDER &
PRINCIPAL QRMEDSCI,
FORMER STATISTICAL SCIENCE.
AMICUS THERAPEUTICS**

TABLE 7 - The Role of a Rare Leader: A Template for Pushing the Science and Telling Your Story



JOCELYN DUFF
CO-FOUNDER/EXECUTIVE
DIRECTOR, CURECMT4/TALIA
DUFF FOUNDATION

12:30 pm

Networking Lunch

1:30 pm

Keynote Panel Discussion: Pricing and Return of Investment (ROI) of Orphan Drugs: What is the Right Price Strategy for Orphan Drugs to Continue to Treat Patients and Remain Commercially Viable for Manufacturers?

- > Devising funding strategies to develop drugs for the treatment of rare diseases remains a challenge as patient groups are often too small
- > Bold new ways to secure investment
- > Budget impact of RD therapies create a real concern for payers
- > Responsible and evidence-based pricing, and innovative contracting
- > Forward thinking approach for better market access to Rare Disease therapies

Moderator:

Panelists:



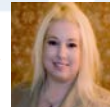
KEN KENGATHARAN,
MANAGING PARTNER,
ATHENEOS VENTURES,
CEO, AUXESIA ORION



MATTHIAS SCHOENERMARK
PROFESSOR, PRESIDENT
AND CEO, SKC



SAMUEL AGUS
CHIEF MEDICAL OFFICER,
BIOPHYTIS



KHRYSAL DAVIS
FOUNDER & PRESIDENT,
TEXAS RARE ALLIANCE

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

DAY 2, 11TH SEPTEMBER 2019

2:10 pm

Keynote Plenary Session: Challenging Rare Disease in the Commonwealth: The Effort to Create a Massachusetts Rare Disease Advisory Council

- > Overview of Representative Kane and Representative McKenna's legislation, An Act to create a Massachusetts rare disease advisory council
- > Why creating a rare disease advisory council in the Commonwealth is important and impactful
- > How stakeholders and interested parties can effectively advocate for this legislation's advancement and passage in the Massachusetts General Court



Hannah E. Kane, State Representative, Boston, Massachusetts

2:40 pm

Joint Platform: Social Listening – as a tool to enhance Big Data

- > How do we define data?
- > Gathering and mining unstructured data
- > The power of "little data"



FEMIDA GWADRY-SRIDHAR
CEO & FOUNDER,
PULSE INFOFRAME INC.



ANTHONY GUCCIARDO
SVP, STRATEGIC PARTNERSHIPS,
NATIONAL KIDNEY FOUNDATION

3:10 pm

Afternoon Networking & Refreshment Break

3:40 pm

Closing Keynote Address: Set Extraordinary Expectations

In this dynamic presentation participants will learn:

- > Explore the definition of Extraordinary
- > Understand that perception drives expectations which drive outcomes.
- > Learn the key components Ignition, Tools, & Network for achieving the desired outcomes
- > Hear stories of presenter's sons – some hilarious, some heartwarming – to prove the extraordinary formula.



Kristin Smedley, President, Curing Retinal Blindness Foundation

4:10 pm

Closing Panel Discussion: The Importance of Patient Networks and Advocacy Groups in Designing of Clinical Trials and Patient Recruitment?

- > Understanding the collective voice of the patients and represent the patients' interest
- > Patient involvement is crucial for identifying the questions to ask and the outcomes to assess
- > Patient involvement is essential for achieving true translational research
- > The identification and proper handling of patient needs in clinical research will yield advantages in terms of clinical and economic benefits.

Moderator:



FEMIDA GWADRY-SRIDHAR
CEO & FOUNDER,
PULSE INFOFRAME INC.

Panelists:



LUKE ROSEN
FOUNDER, KIFIA.ORG/
VP, PATIENT
ENGAGEMENT, OVID
THERAPEUTICS



ANNE B. CROPP
CHIEF SCIENTIFIC
OFFICER, EARLY ACCESS
CARE LLC



ANTHONY GUCCIARDO
SVP, STRATEGIC
PARTNERSHIPS,
NATIONAL KIDNEY
FOUNDATION



DAN DONOVAN,
FOUNDER & CEO,
RARELIFE SOLUTIONS,
CREATORS OF ONEVOICE

4:50 pm

Chairperson's Closing Remarks

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

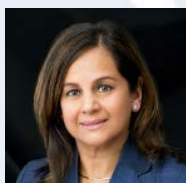
CHAIRPERSONS



Matthias Schoenermark, Professor, President and CEO, SKC Beratungsgesellschaft mbH

Prof. Matthias P. Schönemark, M.D., Ph.D., is a trained head & neck surgeon with a Ph.D. in molecular oncology. In 1998, he left his job as an Associate Professor at Hannover Medical School for a position as project leader & manager at The Boston Consulting Group, where he spent several years, supporting clients in the health care industry in Europe and overseas. In 2005, after a year as Associate Partner with A.T. Kearney, he founded the SKC Beratungsgesellschaft mbH (SKC). He was appointed as Professor of Health Care Management at Hannover Medical School in 2001. He spent several years at Columbia University in New York and at Dartmouth Medical School in Hanover, New Hampshire and lectures at universities & business schools in Europe and the U.S. on strategic issues in health care.

In his function as managing partner, he is a permanent consultant to numerous leadership personalities of international health insurance and provider organizations, as well as of medtech and pharmaceutical companies on strategic management, innovation management and change management issues. He holds an unrivalled track record of solving complex and demanding market access challenges, especially in the orphan drug and oncology sector and is one of the most experienced negotiation leaders in reimbursement and pricing procedures.



Femida Gwady-Sridhar, Founder and CEO, Pulse Inframe Inc.

Dr. Femida Gwady-Sridhar is the Founder and CEO of Pulse Inframe Inc. She has her PhD in Research Methodology, Health Economics, McMaster University. She is a pharmacist, epidemiologist and methodologist with over 25 years of experience in clinical trials, disease registries, knowledge translation, health analytics and clinical disease outcomes. She founded the first knowledge translation health informatics lab in North America in 2006, creating a collaborative ecosystem for interdisciplinary research. In 2008 this facility, I-THINK research, developed a physical and virtual platform to support multidisciplinary research. The platform has evolved to enable the integration of clinical, imaging and histopathology data.

Pulse Inframe has developed healthie™, a revolutionary evidence-based platform transforming rare disease and cancer clinical research and treatment. Pulse's SaaS data platform is becoming the de facto standard for real world evidence, meeting an urgent regulatory requirement. Pulse builds multi-stakeholder collaborative networks of pharmaceutical companies, researchers and patients and enables stakeholder access to clinical trials, input on trial design, new treatment development, and accelerated research. Pulse captures, organizes, analyzes, curates and shares existing and unique data using its industry leading and scalable data-sharing platform powered by AI and machine learning.

Under Dr. Gwady-Sridhar's guidance Pulse has developed a global presence. Pulse has collaborated with international funding agencies advancing the requirements for rare disease registries. Collecting relevant and actionable data in rare disease is essential. Pulse has addressed relevant questions about data sharing, governance, ontologies and patient reported outcomes- all key to understanding and enabling the development of real-world evidence. DrGwady-Sridhar is a life-long collaborator and has done so across different disciplines and cultures. This experience has resulted in a comprehensive understanding of the value of collaboration to fast-forward progress and improve lives everywhere.

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

EXPERT SPEAKERS



Derek Ansel, Director of Rare and Orphan Diseases, Synteract

Derek Ansel is Director of Rare and Orphan Diseases at Synteract, a CRO dedicated to rare disease patients. His career started in the laboratory, testing the efficacy of many vaccine products. After several years in clinical laboratory research, monitoring, and project management he developed a broad therapeutic experience that includes non-malignant hematology, disorders of autoimmunity, and other genetic conditions such as Cystic Fibrosis and Prader-Willi. Mr. Ansel is a member of the ACRP where he holds the CCRA credential. He is also a writer for the Vaccine Education Center at the Children's Hospital of Philadelphia, an active consultant for several start-ups in the eClinical software environment, and an IRB member at the Thomas Jefferson University in Philadelphia, PA.



Samuel Agus, Chief Medical Officer, Biophytis – New Therapeutics for Aging Diseases

Samuel Agus has served as our Chief Medical Officer since July 2018. From April 2017 to June 2018 he served as Vice President, Chief Medical Officer of Hansa Medical AB (Publ), a biotechnology company. Prior to that, he served at various leadership positions in clinical development and medical affairs, in several pharmaceutical companies, such as Teva Pharmaceuticals industries, Solvay Pharmaceuticals, Abbott, Shire and H. Lundbeck A/S. Dr. Agus holds a doctorate in Medicine from The Hebrew University of Jerusalem. He is a board-certified neurologist (from Israel) and has had academic training in biostatistics and bioinformatics.



Daniel Anderson, Head, Commercial Partnerships Invitae

Daniel leads the Commercial Partnerships group at Invitae where he is responsible for building the network of programs with biopharma companies and patient advocacy groups. Prior to joining Invitae, he worked in J.P. Morgan's healthcare investment banking group where he advised biotech and medtech companies on M&A, capital raising and other strategic transactions.

Daniel started his career at the management consulting firm ZS Associates where he specialized in addressing healthcare sales and marketing issues. He has also worked in South Africa and Tanzania to expand access to vaccination programs.

Daniel lives in Denver, Colorado with his wife and 1 year old daughter.



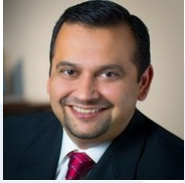
Megan O'Boyle, Principal Investigator, Phelan-McDermid Syndrome International

Megan is the parent of an 18-year-old daughter with Phelan-McDermid Syndrome (PMS). This diagnosis includes autism, intellectual disabilities, epilepsy, ADHD, and other medical conditions. She is the Principal Investigator for the Phelan-McDermid Syndrome Data Network (PMS_DN, PCORnet) and the Phelan-McDermid Syndrome International Registry (PMSIR).

Megan is passionate about the value of the patient's voice in: research, drug development, clinical trial design, development of related legislation, and quality of life decisions. She advocates for data sharing, collaborating with other advocacy groups, sharing resources and streamlining IRB practices and policies.

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

EXPERT SPEAKERS



Subha B. Basu, PhD/MBA, Managing Director, Converge Advisory Group

Subha focuses on R&D and Commercial strategies in pharma/biotech, particularly analyzing the breadth and depth of scientific, clinical and commercial information to inform drug development, business development and launch strategies. As of 2017, he started his own firm, the Converge Advisory Group (CAG), with the sole mission to bring the best assets to market faster. Subha has also held roles at Becton Dickinson, IQVIA and Frost & Sullivan, with exposure to bioprocessing, medical devices, clinical / companion diagnostics and life sciences. He has also taken two cell culture media products from concept to launch, as a marketing member on product development teams.

Over the last year, CAG has developed one of the most comprehensive data sets for rare disease and strategic analysis. Combining three large curated files, for Diseases and Epidemiology, Clinical Development / Pipeline, and Commercial / Market Access, this datamart allows just about any stakeholder, from pharma to patient advocacy groups, to better understand diseases at the indication, asset and company levels. One of the driving forces have been the lack of defensible proofs for rare disease facts and figures. So we developed our own.

Additionally, Subha is also a neuroscientist by training, having spent 10 years in academic research on rare diseases such as ALS, Batten Disease and Down Syndrome. Subha holds a bachelor's degree in biochemistry from the University of California at Berkeley, a PhD in neuroscience from the State University of New York in Brooklyn, a post-doctoral fellowship in neuroscience from Stanford University and an MBA from Carnegie Mellon University.



Philip John (P.J.) Brooks, Program Director, Office of Rare Diseases Research, NCATS

Philip John (P.J.) Brooks is a Program Director in the Office of Rare Diseases Research in the National Center for Advancing Translational Sciences (NCATS). Prior to taking on this role, he was in the NCATS Division of Clinical Innovation, where he was the lead program director for the Clinical and Translational Science Awards (CTSA) Program Collaborative Innovation Awards, designed to fund projects that will result in novel and creative approaches to overcoming roadblocks in translational science. In addition to his work in NCATS, Brooks is the Working Group Coordinator for the NIH Common Fund Somatic Cell Genome Editing Program <https://commonfund.nih.gov/editing>.

Dr. Brooks earned bachelor's and master's degrees in psychology and received his Ph.D. in neurobiology from the University of North Carolina at Chapel Hill. After completing a postdoctoral fellowship at the Rockefeller University, Brooks became an investigator in the intramural program of the National Institute on Alcohol Abuse and Alcoholism. He developed an internationally recognized research program focused on two distinct areas: the molecular basis of alcohol-related cancer, and rare neurologic diseases resulting from defective DNA repair.

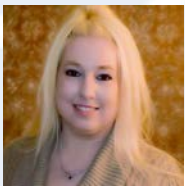
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EXPERT SPEAKERS



Anne B. Cropp, Pharm.D., Chief Scientific Officer, Early Access Care LLC

Anne has over 25 years' experience in the biopharmaceutical industry, marked by achievements in the successful development of several new drug products in adult and pediatric populations. Prior to Early Access Care, Anne was VP in Global Product Development at Pfizer Inc and led global teams from Phase 2 through NDA/MAA submission and successful registration and commercialization. She has partnered extensively with Patient Groups across a multitude of disease areas, including rare disease, to integrate patient and caregiver voice into development and pre-launch strategy. She has focused on removing barriers to the incorporation of the patient throughout the development of a compound and has created tools used by product teams to place patients at the center of development and launch strategies. She specializes in optimizing early access programs as part of the overall development strategy of new medicines in rare disease indications. Anne serves as the Chief Scientific Officer for Early Access Care, providing consultative, functional and operational support to biopharmaceutical companies for global early access programs.



Khrystal Davis, Founder & President, Texas Rare Alliance, Advisory Member, Texas Newborn Screening

Khrystal joined the rare disease community in 2011 when her newborn son, Hunter, was diagnosed with Spinal Muscular Atrophy (SMA) Type 1, the leading genetic cause of mortality in children under the age of two. SMA Type 1, often described as ALS in babies, robs the ability to move, swallow, and ultimately breathe. Khrystal is the author of *Hunt for a Cure: An Unexpected Adventure to Save a Life*. Khrystal founded Zebra Leaf Publishing to provide the rare disease community a platform to promote rare disease awareness.

In May of 2016, Khrystal advocated alongside the FAST Movement (Families for the Acceleration of Spinal Muscular Atrophy Treatments) in a meeting with top FDA representatives for access to Spinraza, an SMA treatment in clinical trials at the time. Together with other FAST members, she asked the FDA to stop placebo trials, provide a means of access for the weakest SMA patients, accelerate the approval, and approve the treatment for all SMA patients regardless of age or type of SMA. In an interim look completed on August 1, 2016, the FDA found the treatment met trial objectives. An Expanded Access Program for SMA Type 1 patients commenced August 12, 2016, and the FDA approved Spinraza for children and adults with SMA on December 23, 2016.

Khrystal is committed to improving health outcomes in those with rare diseases through improved access to rare disease treatments. She advocates for the expansion of newborn screening programs and insurance policies that conform to FDA labels for orphan drugs. She is a proponent of patient-driven access to rare disease treatments.

Khrystal holds a Juris Doctorate from Stetson University College of Law and is certified in Clinical Trial Design and Interpretation by Johns Hopkins through the Coursera program. She is a proud wife and mother of five who enjoys traveling the world with her family.

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

EXPERT SPEAKERS



Lisa Deck, Founder and Director of Sisters@Heart

Lisa Deck is a Founder & Director of Sisters@Heart, a non-profit organization that improves the lives of those affected by heart disease and stroke. Lisa is a former Go Red for Women National Spokeswomen for the American Heart Association and currently serves as an Advocacy Board Member for the American Heart Association in Boston and Southern New England.

Suffering her first three strokes twenty years ago, Lisa has been a patient activist and international speaker for the past two decades. Lisa lobbies at the local, state and Federal level for health and awareness policy development. She is a widely known advocate voice that inspires others and raises awareness of heart disease, stroke and Moyamoya disease.

In 2015, Lisa was diagnosed with Moyamoya Disease, a rare cerebrovascular disease, after an 18-year diagnostic odyssey. She underwent two brain bypass surgeries to restore blood flow to her brain. Since then, Lisa has become actively involved in rare disease advocacy, serving as a Committee member of Rare New England. Rare New England brings together New England patients, families and providers touched by rare and complex disorders. On behalf of Rare New England, Lisa produces and hosts a local cable show, The World of Rare Disease. Lisa is also active with the newly formed Moyamoya Foundation. Lisa lives in North Attleboro, MA with her husband and two children.



Dan Donovan, Founder & CEO, rareLife solutions, creators of onevoice

rareLife solutions is Dan's second software and service focused venture. Having observed the often fractured, though always passionate stakeholders in rare diseases, Dan was inspired to create onevoice, providing rare disease specific "guided communities" thoughtfully designed to address several unmet needs while improving disease understanding and accelerating research.

Dan's immersion into rare disease began in 2011 when he was appointed to the Board of Directors and later as Chief Business Officer of Cancer Prevention Pharmaceuticals (CPP). It was through his involvement with CPP that Dan discovered how incredibly fractured rare disease communities tend to be and he felt there had to be a better way to help these communities who needed help the most. Prior to rareLife and CPP, Dan founded Envision Pharma in 2001, serving as President through June 2011. Dan was the visionary behind the creation and development of Datavision, the market leader in medical publications technology. Envision Pharma was acquired by the United BioSource Corporation (UBC) in April 2008. At UBC, Dan was Senior Vice President Strategy and Market Development and a member of the Leadership Team.

Dan cut his teeth in industry at Pfizer serving in a variety of positions of increasing responsibility, ranging from sales to market research and marketing in the US domestic and international market place, culminating in his position as Director and European Team Leader for the world's largest cardiovascular portfolio, living in Belgium. During his time at Pfizer, he played a pivotal role in the commercialization of some of the pharmaceutical industry's most successful product launches.

Dan earned a Bachelor of Science degree in Finance at Lehigh University, where he was a 4-year varsity soccer player serving as captain his senior year. Dan lectures frequently at specialty industry conferences and webinars.

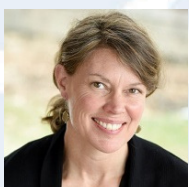
11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

EXPERT SPEAKERS



Robert Donnell, Head of Business Development at Durbin PLC

Robert Donnell has 20 years' experience in the pharmaceutical sector, working for large blue-chip companies such as Procter & Gamble and AstraZeneca. Robert joined Durbin, one of the world's leading specialist Comparator and Clinical Trials Supply companies in 2009. He is responsible for global business development across the Durbin Group, including developing relationships and devising strategic partnerships with multinational pharmaceutical and biotechnology companies. Robert has advised several UK Members of Parliament on Healthcare policy and lectures extensively on Global supply issues.



Jocelyn Duff, Co-Founder/Executive Director, CureCMT4J/Talia Duff Foundation

Jocelyn Duff is the Executive Director and co-founder of CureCMT4J, an all-volunteer non-profit dedicated to expediting a treatment or cure for Charcot Marie Tooth Disease Type 4J (CMT4J). Jocelyn and her husband, John, founded CureCMT4J in June, 2016 after receiving a diagnosis of CMT4J for their daughter, Talia, following a six-year diagnostic odyssey. CMT4J is a severe, progressive, inherited neuromuscular disease often compared to ALS.

At present there is no treatment or cure. In its initial 18 months of operation, CureCMT4J gathered world experts in CMT4J/FIG4 and gene therapy, raised over \$1 million, and funded pre-clinical work at the Jackson Laboratory, establishing efficacy using gene therapy in CMT4J mouse models. A first-in-human gene therapy clinical trial is planned for 2020.

A physician assistant (PA) by training, Jocelyn has worked in Family Practice and Internal Medicine for over 20 years. Duff was a recipient of The Science Channel's "Superheroes in Science" award, in June, 2017. Jocelyn was selected to speak at The Atlantic's "Women in Science" symposium in Washington, D.C., discussing breakthroughs in science, and at Harvard's 2018 "Precision Medicine" conference.

Jocelyn strives to connect other rare families with researchers and to assist them in expediting treatments. Jocelyn enjoys spending time outdoors with her husband and two amazing daughters.



Todd Galles, Business Development Principal, Durbin

Todd Galles, Business Development Principal at Durbin in the USA, is developing and growing Durbin's client portfolio in the US and Americas. Mr. Galles has an extensive background in the life science industry more than 30 years of experience in business development, sales, marketing, and executive management in the pharmaceutical and biotech fields. Previous companies include Syntex Laboratories, Dey LP, Santen, Mylan, and Dow Pharmaceutical Sciences. Todd has also served on numerous industry and company advisory boards. Todd focused in the clinical trial supply sector for 10 years working with leading CROs. He has been working with Durbin for 5+ years and specializes in both clinical trial supplies and managed access programs. Durbin is one of the world's leading specialist medical suppliers and distributors of pharmaceuticals. Durbin has full service offices in the UK and USA.

11th Orphan Drugs & Rare Diseases Global Congress 2019 Americas

EXPERT SPEAKERS



Anthony Gucciardo, Senior Vice President, Strategic Partnerships, National Kidney Foundation

Anthony is responsible for forging and maintaining relations with key external stakeholders across a wide range of industries, to advance NKF's mission and objectives, along with those of its partner organizations. Anthony oversees two national Corporate Development Teams, focused on securing revenue necessary to ensure NKF programmatic excellence and impact. He has been with the Foundation since 2002.

Prior to NKF, Anthony was a Hematopoietic Stem Cell Technologist at Memorial Sloan-Kettering Cancer Center in New York City, where he was responsible for processing autogeneic/allogeneic bone marrow and peripheral blood stem cells for transplantation. He holds a master's degree in Biochemistry from Columbia University.

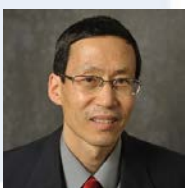


Hannah Kane (R – Shrewsbury), State Representative for the Eleventh Worcester District

Hannah Kane (R – Shrewsbury) serves as the State Representative for the Eleventh Worcester District, representing the towns of Shrewsbury and Westborough, precincts 4 and 5, and was sworn in to serve her third term in January of 2019.

Hannah graduated from Boston University's School of Management in 1993. Hannah has significant experience in both the public and private sectors. Hannah serves as a Member of the Joint Committee on Ways and Means, the Ranking Minority Member of the Joint Committee on Public Health and the Ranking Minority Member of the Joint Committee on Cannabis Policy. Hannah is a member of the Central MA Opioid Task Force, a State Director for the national Women in Government Foundation and a Board Member of the Massachusetts Caucus of Women Legislators. Hannah serves as Minority Leader Bradley H. Jones designee on the Massachusetts Food Policy Council and served on the Special Commission on Local and Regional Public Health and on the 2018-2019 Regional Transit Authority Task Force. She is a Founder and Co-chair of the first in the nation Food System Caucus. Hannah received the 2017 American Nursing Association of Massachusetts Friend of Nursing Award for her work on marijuana policy.

Hannah and her husband Jim live in Shrewsbury with their three children, one of whom has two chronic diseases.



Kei Kishimoto, Chief Scientific Officer, Selecta Biosciences

Dr. Kishimoto is the Chief Scientific Officer of Selecta Biosciences, a biotechnology company developing synthetic vaccines based on a novel self-assembling nanoparticle technology. Prior to joining Selecta, Dr. Kishimoto was Vice President of Research at Momenta Pharmaceuticals where he led multidisciplinary teams in inflammation, oncology, and cardiovascular disease. Previously he was Senior Director of Inflammation Research at Millennium Pharmaceuticals, where he provided the scientific leadership for four programs in clinical development, and an Associate Director of Immunology at Boehringer Ingelheim. Dr. Kishimoto received his doctoral degree in Immunology from Harvard University and his post-doctoral training at Stanford University.

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EXPERT SPEAKERS



M. (Ken) Kengatharan, PhD, MBA, CEO, Auxesia Orion, Executive Chairman, Helios Orion and Managing Partner, Atheneos Ventures

Dr Kengatharan is a co-founder of several San Francisco bay area based biopharmaceutical companies where he held responsibilities in R&D and corporate development. He is currently President & CEO of Auxesia Orion, Executive Chairman of Helios Orion and a member of the board of Renexxion. He is also managing partner at Atheneos Ventures, a San Francisco bay area based evergreen healthcare incubator fund focusing on opportunities in orphan drugs and rare diseases. Previously he was President & CEO of Armetheon (renamed Espero Biopharma following a merger) and prior to that President & CSO of Altheos. He is the former Vice President, Pre-Clinical R&D of Athenagen (re-named CoMentis following a merger).

During the last 20 years, Dr. Kengatharan has been critical to the development of multiple drug candidates from concept to clinical development. Since setting-up his first company in the late 1990s, with a great team and highly supportive investors in each case, he has been critical to setting-up 9 companies and raising >\$160MM in private equity and for closing partnership transactions in excess of \$1bn in deal value. He has served on the boards of Epi3 (UK), Athenagen, Altheos, Armetheon, he has been an advisor/advisory board member at Spire-BioVentures, Cardinal Free Clinics (Stanford University School of Medicine), a mentor at University of California, San Francisco (UCSF)'s entrepreneurship program and a panel judge for the Stanford Business School's Ignite Program. He trained as a cardiovascular pharmacologist in London and at Stanford after obtaining his PhD in pharmacology from the University of London under the supervision of eminent British pharmacologist and Nobel laureate, Prof. Sir John Vane FRS and Professor Christoph Thiemermann. Dr Kengatharan obtained his MBA (with Distinction) from Durham University in England, where he focused on Biotech Finance and Entrepreneurship. His current interests, based on previous experience, include capital efficient R&D, application of distributed ledger technology, AI, and novel paths to raising private equity, in healthcare. Since 2016, Dr. Kengatharan has been an advisor to PGE's Orphan Drugs & Rare Diseases Global Congress held in Europe and in the United States.



Qing Liu, Founder & Principal Consultant, QRMEDSci, former Statistical Science & Program Strategy Lead, Amicus Therapeutics

Qing Liu, Ph.D. and ASA Fellow, is the Founder of Quantitative & Regulatory Medical Science, LLC. Currently, Qing is leading Statistical Science and Program Strategy at Amicus Therapeutics, which develops precision medicines for patients suffering from rare diseases.

Qing's mission is to bring innovative methods and technologies to clinical trials by leveraging his rich clinical trial experience, extensive research and publications in innovative design and statistical methods, and IT expertise in cloud based parallel- and super-computing.

Qing is a statistical expert who gained rich experience in broad disease areas in oncology, neurology, psychiatry, pain, cardiovascular, anti-infective, anti-virus, immunology and metabolic diseases from employment in academic institution, regulatory agency (FDA) and the biopharmaceutical industry. Qing's clinical research experience includes more than 30 due-diligence projects in new drug licensing & acquisitions, numerous successful dispute resolutions with the FDA, successful implementation of various regulatory acceptable innovative trial designs and strategic clinical development planning. Qing has substantially contributed to clinical trial design, protocol review, monitoring, analysis, study report and regulatory submission.

Qing provided expert consulting for small and startup companies in efficient clinical development of innovative therapies with emphasis on medical devices, immuno-oncology, breakthrough designation drugs, rare disease therapies, and individualized medicine. To address the unique challenges in these areas, Qing specializes in blinded data monitoring (BDM) using an efficacy response signature (ERS) approach to adjust sample size and optimize statistical power for ongoing trials and in developing information enriched composite endpoints for use in efficient adaptive designs.

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EXPERT SPEAKERS



David Lapidus, President, LapidusData Inc.

David Lapidus is the founder of LapidusData Inc., a consulting firm specializing in market validation services for companies developing orphan drugs. He brings a commercial perspective to epidemiology and market research to help clients analyze patient populations for therapies where underlying population dynamics are complex or poorly characterized. LapidusData's clients include dozens of orphan drug companies and patient organizations; these projects have analyzed the epidemiology of over 50 rare diseases.

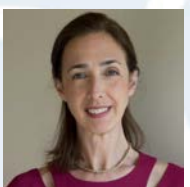
David's analyses have been used to support successful M&A activity in the orphan drug field. In addition, established orphan drug companies with marketed products use LapidusData market forecasting systems for sales, marketing, and budgetary planning.

Before founding LapidusData in 2008, David began his pharma career at Decision Resources, a pharmaceutical consulting firm. After contributing several innovations to this company's forecasting process, he moved to Genzyme, where he built a market forecasting system for the enzyme replacement therapy business unit. He holds a BA in Social Studies from Harvard University.



David Litwick, Director, Regulatory Strategy and Communications, Prevail Therapeutics

Dr. Litwick received a B.S. in Chemistry from the University of Chicago, and a Ph.D. in Biology from MIT. After postdoctoral studies at the Salk Institute for Biological Studies, he joined the faculty of the University of Maryland School of Medicine as an Assistant Professor in the Department of Anatomy and Neurobiology, a member of the Program in Neuroscience, and a founding member of the School's Center for Stem Cell Biology and Regenerative Medicine. In 2010, Dr. Litwick was awarded an AAAS Science and Technology Policy Fellowship in NCI's Office of Biorepositories and Biospecimen Research. In that role, he led several efforts to develop policy and programs to advance the use of biobanking for personalized medicine. In 2012, Dr. Litwick joined the Personalized Medicine Staff of the Office of In Vitro Diagnostics and Radiological Health at the FDA, where he developed policies to guide the review of investigational biomarker tests, companion diagnostics, and next generation technologies. He is now Director of Regulatory Strategy and Communications at Prevail Therapeutics.



Isabelle Lousada, President & CEO, Amyloidosis Research Consortium

Isabelle Lousada is the founder and CEO of the Amyloidosis Research Consortium (ARC) formed in 2015 and located in Boston. As President & CEO of ARC, she designs and leads innovative, global programs: Bringing together doctors, researchers, patients, industry, and regulators in order to build meaningful programs and break down the barriers that prevent successful drug development for the amyloidosis diseases. Ms. Lousada leads a team of dedicated employees and volunteers and oversees ARC's progressive initiatives.

In 1996, Ms. Lousada was diagnosed with AL amyloidosis, and was one of the first patients to successfully undergo a stem cell transplant. For the past twenty years, she has been committed to empowering other patients while serving on the boards of a number of non-profits. She developed programs to encourage research, increase access, and support the critical and unmet needs of patients.

Now in its fourth year, with Isabelle Lousada at the helm of the ARC, successful collaborations across the sectors have been created to advance the science and understanding of the amyloidosis diseases.

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EXPERT SPEAKERS



Elena Lungu, Manager, Policy Development, Patented Medicine Prices Review Board / Government of Canada

With both public and private sector experience, Elena Lungu is an expert in the area of pharmaceutical policy, pricing and reimbursement in Canada. Currently, she is the Manager of the Policy Development with the Patented Medicine Prices Review Board (PMPRB) and in this role, she coordinates the development of strategic policy advice related to price regulation for the Board. Previously, Elena served for a decade as the Manager of the National Prescription Drug Utilization Information System (NPDUIS) of the PMPRB, building this initiative into a leader in economic analyses related to price, utilization and cost trends in support of policy decisions in Canada. Prior to her career in the public service, Elena held various management and senior economist positions in private consulting related to market access as well as public and private reimbursement markets. Elena holds a Masters of Arts in Economics from Carleton University and has 16 years of pharmaceutical market experience.



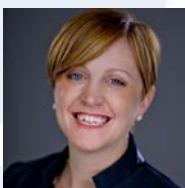
Dr. Neena Nizar, Founder and Executive Director, Jansen's Foundation

Dr. Nizar serves as Founder and Executive Director of the Jansen's Foundation. She is a rare disease advocate and Change Leader in the rare disease community, specifically in the field of ultra-rare skeletal dysplasias. Dr. Nizar has a Doctoral degree in Educational Leadership from Creighton University, Nebraska, is a TEDx speaker, a blogger and passionate voice for the special needs community. She is a Trailblazer for the Rare Advocacy Movement (RAM) and Nebraska's Mother of The Year, 2018. Dr. Nizar is also a rare disease patient and a mother to two boys with Jansen's Metaphyseal Chondrodysplasia – a disease that affects less than 30 people worldwide.



Mike Page, Executive Director, Global Regulatory Affairs Portfolio Products, Alexion Pharmaceuticals

At Alexion, Mike Page leads a global team which develops and executes of regulatory strategies across the company's rare disease product portfolio. Prior to joining Alexion, Mike led the US Regulatory team for oncology at Eisai and managed the regulatory aspects of Eisai's monoclonal antibody products. Prior to that, Mike was a regulatory affairs consultant at United BioSource Corporation, before which he was a Director of Regulatory Strategy at Pfizer, both in the UK and the US. Focusing mainly on late stage development and product registration, Mike has global experience in therapeutic areas including oncology, hematology, psychiatry, neurology, sexual health and addiction disorders.



Maria Picone, Founder and CEO, TREND Community

Maria Picone is the Founder and CEO of TREND Community. In 2012, Maria's daughter was born with Prader-Willi Syndrome. TREND Community was inspired by her journey raising a child with a rare disease. With over ten years of experience managing operational and strategic ventures in the biotech and pharmaceutical industries, Maria is bringing the patient and community voice to medical product development.

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EXPERT SPEAKERS



Amit Rakhit, Chief Medical Officer and Head of Research & Development, Ovid Therapeutics

Amit Rakhit is Chief Medical Officer and Head of R&D at Ovid Therapeutics (NASDAQ: OVID), a publicly traded biotechnology company focused on developing medicines for people living with rare neurologic conditions. In this role Amit oversees research, drug development and commercialization for the Ovid portfolio. Amit has over 20 years' experience including both clinical practice and pharmaceutical/biotechnology industry experience. Amit earned his B.A. in molecular biology from the University of California, Berkeley, his M.D. from Tufts University, his M.S. in clinical investigation from Vanderbilt University and MBA from Columbia University. He completed his fellowship in pediatric cardiology and was subsequently on staff at The Children's Hospital, Boston affiliated with Harvard University.

Prior to Ovid, he was SVP and Head of Worldwide Medical at Biogen where he led the medical function in disease areas such as multiple sclerosis, neurodegenerative diseases, and hemophilia, and orphan diseases such as spinal muscular atrophy and amyotrophic lateral sclerosis. Prior to Biogen, Amit was part of the R&D and medical organization at Bristol-Myers Squibb. Amit is an Advisor Trustee for the Liberty Science Center in NJ as well as an Advisory member of the Healthcare Board of the Partnership Fund for New York City.



Julie Raskin, Executive Director, Congenital Hyperinsulinism International

Julie Raskin is a founder and the executive director of Congenital Hyperinsulinism International (CHI). CHI, a 501(c)3, is a lifeline to those born with congenital hyperinsulinism (HI) and their families. CHI is the global organization dedicated to supporting children and adults born with HI. CHI supports research for better treatments and cures, HI families every step of the way, and is the foremost advocate for increased awareness and better medical protocols for HI to reduce preventable brain damage and death from prolonged hypoglycemia. Julie works with a collaborative team to fulfil CHI's mission to improve the lives of people born with HI.

Julie is also the Rare Action Network State Ambassador for New Jersey. The Rare Action Network is the nation's leading advocacy network working to improve the lives of the 30 million Americans living with a rare disease at the state level. The Rare Action Network is powered by NORD.



Samuel Seward, Jr., MD, FAAP, FACP, Professor and Chair, Dept of Medicine

Dr. Seward received his M.D. from the University of Texas Southwestern School of Medicine in 1990. He joined the Sinai faculty, for the first time, in 1994, as an Associate and, later, Program Director of the Combined Internal Medicine-Pediatrics Residency. In 2004, he joined the faculty of Columbia University Medical Center, ultimately rising to Associate Vice President of Columbia Health which provided a broad array of outpatient services and programs to the Columbia community. Dr. Seward returned to Mount Sinai in 2016 as the Site Chair, Department of Medicine, Mount Sinai St. Luke's and West hospitals. Dr. Seward is internationally known for his work with the Hermansky-Pudlak Syndrome patient population; HPS is a rare autosomal recessive disorder. In addition, his clinical interests include the care of adult patients who are survivors of congenital and pediatric disorders.

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EXPERT SPEAKERS



Luke Rosen, Founder, KIF1A.ORG / Vice President, Patient Engagement & Government Affairs, Ovid Therapeutics

Luke Rosen and Sally Jackson founded KIF1A.ORG in 2016. His mission is to accelerate biotech innovation and forge efficient collaborations within the scientific and patient communities, resulting in discovery of treatment for children living with KIF1A Associated Neurological Disorder, a rare neurological disease affecting his daughter.

With a focus on developing meaningful outcome measures, Luke works to educate and empower families affected by rare genetic diseases. By making sure rare disease families play an active role in discovery, from pre-clinical research through clinical trial readiness, Luke helps drive our mission to find therapeutic options for children living with KIF1A Associated Neurological Disorder.

In addition to his role with KIF1A.ORG, Luke serves as the Vice President of Patient Engagement & Government Affairs at Ovid Therapeutics, a biotech developing treatment for people living with rare neurological disorders.

Luke sits on the Board of Directors of Parents for Inclusive Education (PIE) and works to guarantee children with disabilities have equal access to New York City Public Schools. He has a Master's Degree in Bioethics, a Bachelor's Degree from Connecticut College and attended Balliol College, Oxford University.



James P. Shaffer, MBA, Chief Business Officer, Eiger BioPharmaceuticals

James Shaffer brings extensive commercial leadership and business experience with over 20 years of experience with large pharmaceuticals, emerging biotechnology and specialty pharmaceutical companies in Infectious Disease, Neurology, Oncology, GI and Pulmonary Care.

Prior to joining Eiger, Mr. Shaffer served as Vice President and Chief Commercial Officer for Halozyme Therapeutics from 2011 to 2014 where he was responsible for Sales, Marketing and Business Development overseeing the re-launch of Hylenex® and product development in Diabetes and Oncology. From 2007-2011, Mr. Shaffer was Executive Vice President and Chief Commercial Officer responsible for Sales, Marketing, Business Development and Manufacturing with Clinical Data, Inc., who developed Viibryd®, a novel antidepressant, which was acquired by Forest Laboratories for \$1.2 billion. Mr. Shaffer was Vice President of Commercial Operations for New River Pharmaceuticals which developed Vyvanse® for the treatment of ADHD and was acquired by Shire for \$2.6 billion in 2007. From 2004-2007, he was Senior Director of Commercial Operations for Prestwick Pharmaceuticals overseeing the collaboration with Cambridge Labs for Xenazine®, an Orphan CNS specialty product, and responsible for the development of the companies commercial capabilities in the US and Canada. From 2001-2004, Mr. Shaffer was the National Sales Director for InterMune. Mr. Shaffer spent the first 10 years of his career in numerous sales and marketing positions of increasing responsibility with Merck and GlaxoSmithKline.

James Shaffer is a graduate of the Ohio State University where he earned a B.S. Degree in Economics and an M.B.A..

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EXPERT SPEAKERS



Kristin Smedley, President, Curing Retinal Blindness Foundation

Kristin Smedley's two sons were diagnosed soon after birth with a rare blindness. Although Kristin was initially paralyzed with fear of raising two blind children, she launched an extraordinary mission to change their bleak future ahead. She worked with education systems in multiple states in which she lived to get her sons the tools they needed to achieve in public schools. Her children have become popular, accomplished athletes, high achieving students, talented musicians, and International Braille competition finalists. Her oldest son, Michael has achieved Dean's List in his first two college semesters.

In 2011 Kristin founded the only patient organization in the world for her sons' inherited retinal disease. CRB1.org is a global leader in changing what a rare eye disease diagnosis means, and Kristin is a sought after speaker and collaborator in the field.

Kristin recently published her first book, *Thriving Blind: Stories of Real People Succeeding Without Sight*. It hit #1 New Release in Kindle and Paperback on Amazon.com.



Haya Taitel, SVP, Chief Commercial Officer, Kadmon Pharmaceuticals, LLC

Ms. Taitel is Senior Vice President, Chief Commercial Officer at Kadmon, where she is responsible for the strategy, tactical execution and forecasting of in-line promoted products, portfolio contracting strategies and the launch readiness of pipeline assets.

Haya leads commercial operation disciplines including marketing, sales, national accounts and contracting strategies across several therapeutic areas. She also oversees the commercial operation infrastructure, including the business analytics, market segmentation and customer service units. Prior to joining Kadmon, Haya led the U.S. commercial franchises of both the Pharma and Device sectors of Johnson & Johnson. In her 25-year tenure with J&J, she launched new chemical entities in several therapeutic areas including neurology, analgesia, peripheral diabetic neuropathy, women's health, and urology, with focus on interstitial cystitis.



Joseph L. Ternullo, JD, MPH, Head, Business Development, Pulse Inframe

Joe has over 20 years' experience in healthcare. Joe is board co-chair of the Society for Participatory Medicine, a non-profit devoted to helping patients and healthcare professionals work together as equal partners. Previously, Joe served for nearly 18 years as associate director of Partners HealthCare's Center for Connected Health. He founded the Connected Health Symposium, a prominent two-day international annual event and co-founded Continua Health Alliance, a standards and interoperability organization, both now associated with HIMSS. Joe has served on several domestic and international boards and on two federal advisory committees - the US Department of Health & Human Service's American Health Information Communities Chronic Care Workgroup, and the US Department of Commerce International Trade Advisory Committee on Information and Communication Technologies, Services, and Electronic Commerce. Joe has authored several publications focused on healthcare technology. Both an attorney and certified public accountant, Joe holds degrees from Boston College, Bentley University, Boston University School of Law, and Harvard T.H. Chan School of Public Health.

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EXPERT SPEAKERS



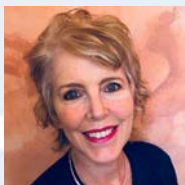
Vlademir Tomov, Chairman, National Alliance of People with Rare Diseases – Bulgaria

Chairman of the National Gaucher Association 1999-2004
President of the National Organization of the Gaucher Patients 2004-2007
Chairman of the National Alliance of People with Rare Diseases 2007- until now
Chairman of the Euro-Asian Alliance for Rare Diseases 2012- until now
President of the Confederation of Health Protection – Largest Patients Assembly in Bulgaria 2010-2013 2018 - until now
Member of the Advisory Council on Rare Diseases at the Ministry of Health - 2009-2014.
Member of the Commission for Rare Diseases at the Ministry of Health from 2015 - until now
Member of the Advisory Council on Treatment for Children at the Ministry of Health -2015 - 2018
Education: MA in Design, Pedagogy and Health Management
Speaks Russian and English.



Stephan Toutain, SVP, Operations, Anavex Life Sciences

Stephan Toutain, Senior Vice President of Operations, brings more than 25 years of drug development, general management, operations, commercial development, market access, and sales and marketing leadership with particular expertise in neurology and orphan drug markets globally. Before joining Anavex, he held the role of CCO at Interleukin Genetics. He also worked with Alnylam Pharmaceuticals to build its early access program. Previously, he led Global Commercial Development for Sarepta Therapeutics and served as General Manager for Alexion Pharmaceuticals in Europe. Mr. Toutain has also held various U.S. commercial, marketing and product management positions with Alexion Pharmaceuticals, Celgene Corporation, and Johnson & Johnson. He received a Master of Business Administration from the University of North Carolina, and a Master of Engineering in Biotechnology from the University of Nancy II in France.



Sara Tylosky, CEO and President, Farmacon.co

Sara Tylosky is an experienced Global Executive in the healthcare field with over 20 years of success leading diverse teams in a variety of competitive fast paced environments within the medical industry. As the CEO of Farmacon, a novel research-consulting firm, Sara and her medical expert network have successfully helped Pharms and Biotechs complete enrollment goals and develop strategic plans for market access, with a particular focus in Orphan Disease projects in Latin America. Farmacon has an established record as an expert problem solver with an ability to get results by mobilizing the right groups and providing the cultural feedback and intelligence so that data endpoints can be properly met. She holds an MBA from Florida Atlantic University, is fluent in Spanish and has lived in 3 continents so far, with a passion for helping others. She is also an accomplished dressage horse rider.

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EXPERT SPEAKERS



Robert (Bob) Ward, Chairman, Chief Executive Officer, Eloxx Pharmaceuticals

Mr. Robert (Bob) E. Ward is the Chairman of the Board and Chief Executive Officer of Eloxx Pharmaceuticals, Inc. ("Eloxx"). He previously served as the Chief Executive Officer and President at Radius Health, Inc. (NASDAQ: RDUS) successfully completing the initial public offering that became the top performing IPO; raised over \$780M from private and public sources; achieved FDA approval and launch of the new drug TYMLOStm injection while gaining Fast Track development status for the Elacestrant oncology program. Prior to joining Radius, Mr. Ward held a series of progressive management and executive roles with established companies such as NPS Pharmaceuticals, Schering-Plough (Merck), Pharmacia (Pfizer), Bristol-Myers Squibb and Genentech. Mr. Ward has been a Director of Akari Therapeutics, Plc since October 14, 2016 where he chairs the Governance Committee. He served as a Director of Radius from December 2013 until July 16, 2017. Mr. Ward serves as a Director of the Massachusetts High Technology Council. Mr. Ward received a B.A. in Biology and a B.S. in Physiological Psychology, both from the University of California, Santa Barbara, an M.S. in Management from the New Jersey Institute of Technology and an M.A. in Immunology from the John Hopkins University School of Medicine.



Patricia Weltin, CEO and Founder, Beyond the Diagnosis

Patricia Weltin is the CEO and Founder of the Beyond the Diagnosis. Beyond the Diagnosis unites art and science to inspire research and innovation of treatments for people living with orphan and neglected diseases.

Beyond the Diagnosis began working on a project to increase research and raise awareness of rare diseases within the medical community through art. Professional artists paint portraits of children living with a rare disease, the portraits then become part of a traveling exhibit for medical schools, research institutes and hospitals. This exhibit has also touched the hearts and minds of the general public. Since the debut, Beyond the Diagnosis has visited the NIH, Broad Institute, Hofstra Medical, Harvard Medical, the FDA and many more.

Patricia, the mother of two children with Ehlers-Danlos Syndrome, began working in this space by creating a new business model of working by state. Her award winning work in state-level advocacy grew to national advocacy efforts. Today, Patricia proudly works to successfully use art as a powerful tool to create awareness and increase innovation into orphan and neglected diseases.

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