8th Orphan Drugs & Rare Diseases 2018 Americas East Coast

Strategies and Innovations Driving Access and Commercialization of Orphan Drugs

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25th - 26th September 2018, Hyatt Regency Boston, Boston, MA, USA

Key Industry Speakers:

- Lori Shafner, VP, Global Development Team Leader, Alexion Pharmaceuticals, Inc.
- Alison Schecter, Global Rare Diseases, Clinical Dev't and Strategy, Sanofi Genzyme
- Tim Miller, Co-Founder, President , Chief Scientific Officer, Abeona Therapeutics Inc.
- Kei Kishimoto, Chief Scientific Officer, Selecta Biosciences
- Robert Hollowell, VP, Head, Corporate Strategy and Commercial Assessment, Shire
- Philip J. Brooks, Program Director, Division of Clinical Innovation , NCATS, NIH
- Arndt Rolfs, CEO, Centogene AG - The Rare Disease Company
- Brian Bronk, Head of External Innovation, Rare Diseases, Global BD & Licensing, Sanofi
- Alvin Shih, Chief Executive Officer, Enzyvant Therapeutics
- Matthias P. Schönermark, Managing Director, SKC
- Ken Kengatharan, CEO, Auxesia Orion and Managing Partner, Atheneos Ventures
- Radhika Tripuraneni, Vice President, Medical Affairs, Prothena Corporation Plc
- Anthony J Arleth, Managing Director, Pennside Partners Ltd
- Leslie Leahy, VP, Medical Sciences, Ovid Therapeutics
- Luke Rosen, Patient Engagement, Ovid Therapeutics
- Mike Page, Executive Director, Global Regulatory Affairs , Alexion Pharmaceuticals
- Michael A. Swit, Esq., FDA Legal Counsel, Law Offices of Michael A. Swit
- David Hogben, Head of Multichannel, Complete HealthVizion
- Jodie Gillon, Global Medical Lead, Patient Engagement Rare Diseases, Pfizer Inc.
- Khrystal K. Davis, CEO and Patient Advocate, Zebra Leaf Publishing
- Robert (Bob) Ward, Chairman, Chief Executive Officer, Eloxx Pharmaceuticals
- Tracy Zervakis, President , Patient for Clinical Research , PaCe Member, AstraZeneca
- Barry Ticho, Chief Medical Officer, Stoke Therapeutics, Inc.
- Carolina Alarco, President, Global Marketing & International Markets , Aegerion
- Richard A. Basile, Co Founder and CEO, BioPontis Alliance for Rare Diseases
- Jodi Wolff, Head of Patient Advocacy, U.S.,Santhera Pharmaceuticals
- Isabelle Lousada, CEO & President, Amyloidosis Research Consortium
- Ray Takigiku, Co-Founder, President and CEO, Bexion Pharmaceuticals, Inc.

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Orphan Drugs & Rare Diseases 2018 Americas East Coast

CONGRESS INTRODUCTION

Dear Colleagues,

Paradigm Global Events is again proud to present our **Orphan Drugs and Rare Diseases 2018 Americas East Coast**. It's the 8th in the series of our Flagship tri-annual Orphan Drugs and Rare Diseases event, this congress will provide you with a comprehensive overview of the critical issues shaping the future of Orphan Drugs in the US market.

According to a recently published report, Orphan Drugs set to be 21.4% of worldwide prescription sales by 2022. The market for orphan drugs, based on the consensus forecast for the leading 500 pharmaceutical and biotechnology companies, will grow by 11.1% per year (CAGR) between 2017 and 2022 to $209bn. The growth of the orphan drug market is more than double that of the overall prescription drug market, which is set to grow by 5.3% over the period 2017-2022. Orphan drugs are set to account for 21.4% of global prescription sales in 2022, excluding generics, up from 6% in 2000. In 2016 orphan drug sales increased 12.2% to $114bn vs. 2015, while non-orphan drug sales increased by 2.4% to $578bn. (Evaluate Pharma).

The growth of the global orphan drugs market is increasing at a rapid pace due to the growing rare diseases. 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 are 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 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.

Orphan Drugs & Rare Diseases 2018 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. We are putting together an agenda that address the driving macroeconomic factors, policies and issues that will steer the development of orphan drugs globally including commercialization, policies, reimbursement, pricing and more.

We look forward to welcoming you at the congress!

Sincerely yours,

Jocelyn Raguindin

Jocelyn Raguindin
Conference Director
Paradigm Global Events

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

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GAIN LATEST INSIGHTS

By attending you will learn:

⇒ What is Orphan Drugs' role in sustainability of the Pharmaceutical Industry?

⇒ Finding innovative and alternative ways in funding the development of Orphan Drugs

⇒ How commercially viable Is It to Engage in the Development of Orphan Drugs?

⇒ What Do Developers Look for When Looking for an Outsourcing Partner?

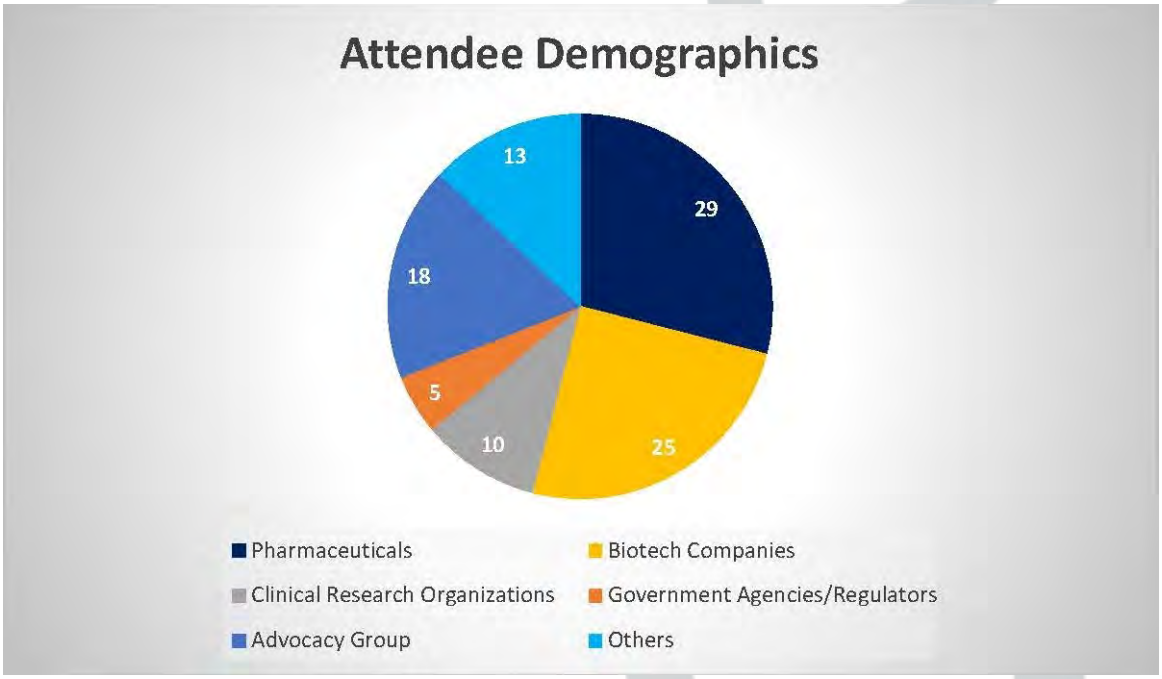
⇒ Coming Together in Developing Orphan Drugs and Crossing Borders

⇒ Trends and Deal Structures in Licensing Agreements

⇒ How Can the Developer and the Patient Assist in the Evolution and Development of Orphan Drugs?

⇒ Patient Access: How Can This Continue to Improve?

⇒ What Do Insurance Companies Think About Orphan Drugs? Will They Make Modifications to Their Policies to Support Patients with Rare Diseases?



MEDIA PARTNERS:

1st DAY OF CONGRESS | TUESDAY, 25TH SEPTEMBER 2018

08.00 - **Registration**

08.20 - **Chairperson's Opening Remarks**

MACRO OUTLOOK, TRENDS AND REGULATORY STRATEGIES

08.30 - **How can Orphan Drugs become a successful industry***

- Commercialisation strategies towards a more competitive orphan drugs industry
- Overcoming the downside trend of abuse on the financial incentives for rare-disease drug development
- What are innovative business models to guarantee a successful industry

Carolina Alarco, President, Global Marketing & International Markets , **Aegerion Pharmaceuticals**

09.00 - **KEYNOTE PANEL DISCUSSION: Addressing the biggest challenges for rare diseases and finding the solutions**

- What are the challenges and how they can be addressed?
- Overcoming the issues of misdiagnosis
- Challenges facing clinicians who care for affected individuals
- Challenges to investigators regard the difficulty and expense of assembling large cohorts of affected individuals for study, and garnering funding for research

Moderator:
Alison Schecter, Global Rare Diseases, External Opportunities, **Sanofi Genzyme**
Panelist:
* **Radhika Tripuraneni,** VP, Medical Affairs, **Prothena Corporation Plc**
* **Barry Ticho,** Chief Medical Officer, **Stoke Therapeutics, Inc.**
* **Ray Takigiku,** Co-Founder, President and CEO, **Bexion Pharmaceuticals, Inc.**

09.40 - **Essentials to Successfully Launch an Orphan Drug**

- In this talk we shall explore:

⇒ The key attributes of a successful orphan drug launch
⇒ Examples of a successful orphan drug launch

Anthony J Arleth, Managing Director, **Pennside Partners**

10.10 - **SHARED PLATFORM: Orphan Drugs Designation and Differentiation Strategies**

- Demonstration of clinical benefit and contribution to patient care
- Regional differences and requirements to achieve Orphan Drug Designation.

Lori Shafner, VP, Global Development Team Leader, **Alexion Pharmaceuticals**
Mike Page, Executive Director, Global Regulatory Affairs , **Alexion Pharmaceuticals**

10.40 - **Morning Break & Networking**

11.20 - **REGULATORY KEYNOTE: The evolving landscape of Rare Disease Policy: Drug approval and recent developments**

- Understanding the global rare disease policy landscape
- New steps to meet the challenges of rare diseases
- What are the key elements across diverse rare disease programs and how this can affect patients access to care
- Bridging gaps between policy and practice to guarantee access for patients

Reserve for FDA Speaker (TBC)

11.50 - **PANEL DISCUSSION: What Are the Trends and Deal Structures in Licensing Agreements and the M&A for Orphan Drugs?**

- Trends in Orphan Diseases partnering deals
- Orphan diseases partnering agreement structure
- At which stage of the drug development does in-licensing occur and what is the reason for this trend?
- What criteria do partners look for when partnering for in and out-licensing?

Moderator:
Brian Bronk, Head of External Innovation, Rare Diseases, **Sanofi**
Panelist:
* **Robert Hollowell,** VP, Head, Corporate Strategy and Commercial Assessment, **Shire**

12.30 - **Developing therapies for rare diseases: navigating the pathway to market**

- Case study: RVT-802 for the treatment of Complete DiGeorge Anomaly (cDGA)
- Overview of expedited regulatory pathways available for innovative rare disease therapeutics
- Managing multi-stakeholder collaboration including academia, patient advocacy, government, and industry
- Novel strategies to promote patient/market access

Alvin Shih, CEO, **Enzyvant Therapeutics**

1st DAY OF CONGRESS | TUESDAY, 25TH SEPTEMBER 2018

DISCOVERY AND CLINICAL DEVELOPMENT

13.00 - **Networking Lunch**

14.00 - **Re-dosing AAV gene therapy vectors – the elephant in the room**

- To follow....

Kei Kishimoto, Chief Scientific Officer**, Selecta Biosciences**

14.30 - **Global strategies to help diagnose patients with rare hereditary diseases**

- Screening strategies and logistics to reach patients
- Biomarker development and implementation as a diagnostic
- Understanding the epidemiology of the disease to streamline a diagnostic strategy

Arndt Rolfs, CEO, **Centogene AG -** The Rare Disease Company

15.00 - **High – throughput screening for drug repurposing**

- "Basket" trials of drugs targeting shared molecular etiologies
- Gene therapy and gene editing
- Platform approaches to clinical trials

Philip J. Brooks, Program Director, Division of Clinical Innovation , **NCATS, NIH**

15.30 - **Patient-centric clinical development**

- **TBC**

Senior Representative, Complete HealthVizion

15.30 - **Tea Break & Networking**

16.10 - **Unique approaches in clinical trial design for rare diseases***

- Various aspects for clinical trials in rare diseases
- Innovations and flexibility required for appropriate clinical research programs
- Totality of clinical data and patient insights related to therapies for rare diseases
- How clinical research and regulatory review in rare diseases can be improved in the years ahead

16.40 - **Leveraging on Technology for Development of Orphan Drugs**

- What innovative technologies in clinical trials on rare diseases are available to speed-up development?
- Limitations and challenges of these technologies
- Considerations for developing orphan drugs
- required to advance biomarker and endpoint use in clinical development

Tim Miller, Co-Founder, President and Chief Scientific Officer, **Abeona Therapeutics Inc**.

17.10 - **PANEL DISCUSSION: Accelerating clinical development of orphan drugs to facilitate patient access**

- Patient recruitment and retention for a successful clinical trial in rare diseases
- How can you prepare healthcare providers to know about the therapy and identify the right patients for your therapy?
- How can developers make research goals and use of data to be transparent and to be informed about progress or roadblocks in order to build a relationship based on trust with patients?
- What enhanced ongoing patient support beyond the clinic can be provided to encourage positive relationships between patients and healthcare providers?

Panelists:

* **Leslie Leahy,** VP, Medical Sciences, **Ovid Therapeutics**
* **Luke Rosen,** Patient Engagement**, Ovid Therapeutics**
* **Khrystal K. Davis,** CEO and Patient Advocate, **Zebra Leaf Publishing**

17.50 - **Chairperson's Closing Remarks**

18.00 **- Networking Drink Reception**

19.00 - 21.00 - **Networking Gala Dinner**

End of Day 1

2nd DAY OF CONGRESS | WEDNESDAY, 26TH SEPTEMBER 2018

08.00 - **Registration**

08.30 - **Chairperson's Opening Remarks**

ACCESS & COLLABORATIONS

08.40 - **Putting a name to the rare disease**

- Growing up with a fatal hereditary rare disease in my family and knowing I had a 50/50 chance of getting it
- The disease took the lives of 12 family members before we were given a name Hereditary Sensory Autonomic Neuropathy Type 1E (HSAN1E)
- It was through genetic testing from my brother and sister we were able to get a diagnosis.
- Caregiving for my brother and sister
- Deciding to get tested for HSAN1E, living with a negative result
- Starting a non-profit to find other families with HSAN1E

Rachelle Dixon, President / Co-Founder, **HSAN1E Society**

09.10 - **Breaking down barriers to Patient Access: Bringing the stakeholders together**

- How can stakeholders work together to remove barriers to patient access
- How can developers make research goals and use of data to be transparent and to be informed about progress or roadblocks in order to build a relationship based on trust with patients?
- How can you prepare healthcare providers to know about the therapy and identify the right patients for your therapy?
- What enhanced ongoing patient support beyond the clinic can be provided to encourage positive relationships between patients and healthcare providers?

09.50 - **Philanthropy and Impact Investing: A new patient integrated partnership needed to turn academic discovery research into treatments for rare disease patients**

- Why must we ask why there is no treatment?
- How can we achieve the goal?
- The way forward

Richard A. Basile, Co Founder and CEO, **BioPontis Alliance for Rare Diseases**

10.20 - **Patient Access to medicines for rare diseases***

- 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?

⇒ Evidence of safety and efficacy

- How can patient access larger number of medicines in shorter time?

Tracy Zervakis, President, **Patient for Clinical Research ,** PaCe Member**, Astra Zeneca**

10.50 - **Morning Break & Networking**

11.30 - **SHARED PLATFORM: How cross stakeholder collaboration drives better outcomes for rare Disease patients?**

- How to improve knowledge and recognising a rare condition for patients to gain a sense of ease and trust
- Addressing issues on practical challenges
- Improving access and supporting patients

Jodie Gillon, Global Medical Lead, Patient Engagement Rare Diseases, **Pfizer Inc.**
Isabelle Lousada, CEO & President, **Amyloidosis Research Consortium**

12.00 - **KEYNOTE PANEL DISCUSSION: The Importance of Patient Networks and Advocacy Groups in Designing of Clinical Trials and Patient Recruitment?**

- How are activists carrying the collective voice of the patients and represent the patients'
- How can leading groups advance understanding and awareness of rare diseases by unifying patients

Panelists:

* **Jodi Wolff,** Head of Patient Advocacy, U.S.,**Santhera Pharmaceuticals***
* **Khrystal K. Davis,** CEO and Patient Advocate, **Zebra Leaf Publishing**

12.30 - **Networking Lunch**

MARKET ACCESS & REIMBURSEMENT

13.30 - **Orphan Drugs in Europe – Conquering the largest pharmaceutical market**

- Current situation in Europe from a regulatory and a market access perspective
- Pricing and reimbursement: key success factors
- Case studies: stories of success and failure
- Strategic implications for biopharma companies, planning to expand into Europe

Matthias P. Schönermark, Managing Director, **SKC**

14.00 - **Strategy and Value of Early Access in the US and EU**

- Strategy of providing early access in OD
- Key logistics in providing early access
- From early to greater access

Stephan Toutain, SVP, Operations**, Anavex Life Sciences**

14.30 - **Networking Break**

15.20 - **Building a Sustainable Growth Biotech**

- We are committed to treating rare and ultra-rare diseases
- Drug development is a team sport and it is critical to attract and retain top talent
- Collaboration across the organization is necessary for advancing programs effectively

Bob Ward, Chairman, Chief Executive Officer, **Eloxx Pharmaceuticals**

15.50 - **Evolving market access landscape for orphan drugs**

- Payers are becoming more aggressive in scrutinizing price and implementing restrictions to patient access that have traditionally been reserved for competitive, high budget impact drug classes
- Recent development in orphan drugs pricing and reimbursement and the impact of those changes
- Addressing some of the major issues facing payers and orphan drug manufacturers, highlighting the way these have been handled by both parties.
- Implementing measures designed to limit exposure to costs associated with orphan drug reimbursement.

(RESERVED)

16.10 - **PANEL DISCUSSION: Strategies for Implementing value-based pricing and reimbursement for orphan drugs**

- Successful example of value-based pricing in Rare Disease
- Potential changes in treatment paradigms to improve effectiveness and reduce cost of treatment
- Implementing measures designed to limit their exposure to costs associated with orphan drug reimbursement.
- Legislative incentives to encourage development of orphan rare diseases therapies
- Impact of orphan diseases treatment on healthcare payment system

Moderator:

Ken Kengatharan, CEO, **Auxesia** Orion and Managing Partner, **Atheneos Ventures**

Panelists;

* **Matthias P. Schönermark,** Managing Director, **SKC**
* **Stephan Toutain,** SVP, Operations**, Anavex Life Sciences**

16.50 - **Chairperson's Closing Remarks**

END OF CONFERENCE

KEY EXPERT SPEAKERS

Alison Schecter, Global Rare Diseases, External Opportunities, Clinical Development and Strategy, Sanofi Genzyme

Alison is currently the Global Program Head for the Rare Disease Development at Sanofi Genzyme. She is responsible for defining the strategic focus of Rare Disease development in collaboration with Head of Commercial. She works closely with external innovation and BD to make review and decide on opportunities to be brought forward for consideration including Bioverativ. In addition, Alison now leads the acid sphingomyelinase deficiency (ASMD) (formerly known as Niemann-Pick) Global Team. She coordinates the regulatory, clinical operations, medical affairs, legal and CMC to allow submission for the first in class therapy for this ultra-rare diseases with high mortality and morbidity and no known treatment.

Alison completed her Internal Medicine residency at the Johns Hopkins Hospital and her Cardiology fellowship at Massachusetts General Hospital with a research fellowship at Mount Sinai. She also co-founded the Women's CARE (Cardiac Assessment and Risk Evaluation) Program to educate and treat under-served women in Spanish Harlem, NY and has appeared on national, local TV and radio as a medical expert on cardiovascular and women's health.

Philip J. Brooks, Program Director, Division of Clinical Innovation , NCATS, NIH

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.

Lori Shafner, VP, Global Development Team Leader, Alexion Pharmaceuticals, Inc.

Lori Shafner is a dynamic development leader with proven track record for advancing products across multiple therapeutic areas through preclinical stages to commercialization. Lori is currently Vice President, Global Development Team Leader at Alexion and previously held various leadership positions at Pfizer, Inc for 22 years. She has demonstrated expertise in executing Phase III clinical development programs and achieving pharmaceutical product approvals globally. Lori is recognized for building high performing multi-functional teams with innovative approaches to delivery with industry leading cycle times. Additional capabilities include:

•Development Program Design •Global Regulatory Submissions
•Quality & Risk Management •Alliances & Acquisitions
•Competitive Analysis •Commercial Strategy
•Team Leadership •Goal Setting & Value Metrics

Lori has a Ph.D in Pharmacology and Experimental Therapeutics from Boston University and completed her postdoctoral fellowship at Emory University.

KEY EXPERT SPEAKERS

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

Mike Page has more than 25 years' experience in the pharmaceutical industry with over 20 years in regulatory affairs. At Alexion, Mike is responsible for the developing and executing regulatory strategy across the portfolio of development and approved products. Prior to joining Alexion, Mike was US Regulatory Lead for the oncology therapeutic area at Eisai and was also responsible for regulatory aspects of the company's biologics subsidiary, Morphotek. Prior to joining Eisai, Mike was a regulatory affairs consultant at United BioSource Corporation before which was a Director of Regulatory Strategy and Registration at Pfizer, both in the United Kingdom and the United States. Focusing mainly on late stage development and product registration, Mike has global registration experience in various therapeutic areas including oncology, psychiatry, sexual health and addiction disorders.

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.

Barry Ticho, Chief Medical Officer, Stoke Therapeutics, Inc.

As Chief Medical Officer Barry is responsible for Stoke's efforts to develop first-in-class therapeutics to treat orphan diseases. Prior to joining Stoke Dr. Ticho was Head of Development of mRNA treatments for Cardiovascular and Metabolic Diseases at Moderna Therapeutics. He was previously Head of External R&D Innovation for Cardiovascular and Metabolic Diseases at Pfizer and prior to that he was Vice President of Clinical Development at Biogen. Barry obtained his M.D. and Ph.D. degrees from the University of Chicago and completed Pediatrics training at Northwestern University and a Cardiology fellowship at Children's Hospital in Boston. He was on clinical staff at Harvard Medical School and Massachusetts General Hospital and conducted laboratory research on the regulation of cardiac development.

Alvin Shih, Chief Executive Officer, Enzyvant Therapeutics

Alvin has served as CEO of Enzyvant since November 2016. Prior to joining Enzyvant, Alvin was the Chief Operating Officer for Rare Diseases at Pfizer, and the global head of R&D at Retrophin. Prior to joining biopharma, he was a healthcare consultant at LEK Consulting and McKinsey & Company. Alvin has an MD from the University of Alabama, an MBA from Northwestern University, and completed his training in internal medicine at the Massachusetts General Hospital.

KEY EXPERT SPEAKERS

Tim Miller, Co-Founder, President and Chief Scientific Officer, Abeona Therapeutics

Timothy J. Miller, Ph.D. is co-Founder, President and Chief Scientific Officer of Abeona Therapeutics Inc. He has over 20 years of business development, scientific research, product development and clinical operations expertise, with a focus on transitioning novel biotherapeutics through pre-clinical phases and Phase 3 human clinical trials. As a C-level executive in public and private companies, he has driven multiple inflection points through innovation and operational excellence in rare disease companies. Dr. Miller was President & CEO of Red5 Pharmaceuticals from 2013 until 2015 and was CEO-in-Residence at BioEnterprise Inc in 2015. He was Senior Director of Product Development at SironRX Therapeutics from 2010 to 2013. Between 1996 and 2010 Dr. Miller held various positions at several biotech companies focusing on gene therapy and regenerative medicine. Dr. Miller earned his Ph.D. in Pharmacology with a focus on Gene therapy/Cystic Fibrosis from Case Western University. He also holds a B.S. in Biology and M.S. in Molecular Biology from John Carroll University (Cleveland, OH).

Leslie Leahy, VP, Medical Sciences, Ovid Therapeutics

Leslie is a neuropsychologist with over 20 years of experience in academia, pharmaceutical and biotech neuroscience research. She is the VP of Medical Science at Ovid Therapeutics. She, along with her fellow Ovidians, are dedicated to developing medicines that significantly impact the lives of patients with rare neurological disorders.

Leslie started her pharmaceutical career in clinical development. She has led Phase 1 through 4 clinical development programs across a wide range of CNS conditions including schizophrenia, bipolar disorder, traumatic brain injury and Alzheimer's disease. While at MGH in the Department of Psychiatry, Leslie was the Program Director for the first public health study of bipolar disorder, the Systematic Treatment Enhance Program for Bipolar Disorder (STEP-BD). Leslie holds a PhD from the University of Pittsburgh, where she also completed her post doc in adult and pediatric epilepsy. Her research interests in CNS are guided by the vision and passion of bringing innovative treatments to patients.

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.

KEY EXPERT SPEAKERS

Brian Bronk, Head of External Innovation, Rare Diseases, Global Business Development & Licensing, Sanofi

Brian is the Head of External Innovation for the Rare Diseases Therapeutic Area at Sanofi. In this role, he and his teams are responsible for working with senior leadership to define an overall portfolio strategy and execute on the external activities, with accountability for identification and securing of external innovation solutions from around the globe that meet the needs of the R&D portfolio. Prior to this role, Brian was a member of the Sanofi Sunrise team, where his responsibilities spanned a range of activities related to building and maintaining a vibrant portfolio of investments, including Warp Drive Bio and Thermalin.

Prior to joining Sanofi in 2014, Brian served as an advisor to a wide array of life science organizations, along with venture capital groups, where he contributed to multiple teams reaching key R&D and financing milestones.

From 2009-2013, Brian was the Vice President of R&D at Satori Pharmaceuticals. Before Satori, Brian worked at Pfizer, rising to the level of Senior Director. Brian and his teams have been involved in the discovery of more than 20 development candidates, including Draxxin™, Convenia™, Cerina™ and Slentrol™
Brian earned his bachelor's degree from Colgate University in 1989. Following a year as a Fulbright Fellow in Dortmund, Germany, he initiated his doctoral work at the Massachusetts Institute of Technology, receiving his doctorate in chemistry in 1994.

Arndt Rolfs, CEO, Centogene AG - The Rare Disease Company

Arndt Rolfs, M.D., PhD, Professor for Neurology and Psychiatry, Dr. Arndt Rolfs is the CEO and Founder of Centogene AG. He received his approbation for human medicine in 1985 from the University of Mainz, Germany and the University of Vienna, Austria. Arndt is a principle investigator of several international multicenter studies in the area of rare diseases and actively engaged in biomarker research for several metabolic diseases, including the Sifap project (www.sifap.eu), the world's largest study in young stroke patients related to Fabry disease, several biomarker studies (e.g. BioHAE, BioGaucher, BioHunter, BioMorquio) and epidemiological studies in neurogenetic aetiologies. Dr. Rolfs has an extensive track record in medical and scientific publications with over 300 papers published in peer-reviewed scientific journals.

Ray Takigiku, Co-Founder, President and CEO, Bexion Pharmaceuticals, Inc.

Previously, he was at Procter & Gamble Pharmaceuticals, and was also interim co-Director of the Genome Research Institute (now the Reading Campus) of the College of Medicine, University of Cincinnati. Dr. Takigiku's life-long research interests have been in the origin, evolution and sustainability of life. His earliest work focused on elucidating the interplay of the oceans, atmosphere, and early life forms. Later work encompassed toxicology, pharmaceutical discovery and development, and consumer product innovation. Founding Bexion in 2006, the company now has a novel compound in clinical development for solid tumors, including glioblastoma multiforme, a deadly form of brain cancer. Dr. Takigiku received his undergraduate degrees from University of Colorado in Chemistry and Molecular Biology, his PhD in Chemistry from Indiana University, and business training from the Kellogg School of Management.

KEY EXPERT SPEAKERS

Radhika Tripurareni, VP, Medical Affairs, Prothena Biosciences

Prior to joining Prothena, Dr. Tripuraneni was Vice President, Medical Affairs at MyoKardia where she was responsible for the development of the global medical affairs strategy for mavacamten, a compound entering Phase 3 development for hypertrophic cardiomyopathy and MYK-491, in Phase 1 studies for dilated cardiomyopathy. Before MyoKardia she was Vice President, Medical Affairs at Synageva (which was acquired by Alexion), where she led a large global team of Medical Science Liaisons and supported the global launch of Kanuma, an FDA approved product for lysosomal acid lipase deficiency. Dr. Tripuraneni has experience across therapeutic areas in both orphan and non-orphan disease areas and has also worked in positions in Medical Affairs at Gilead and Genzyme, as well as in Corporate Development at Genzyme. Throughout her career she has demonstrated a commitment to science-led decision making and cross-functional collaboration that are hallmarks of our approach. Dr. Tripuraneni received her Bachelors' in Business Administration (BBA) and Liberal Arts (BLA), Doctorate in Medicine (MD) from University of Missouri and her Master in Public Health (MPH) from Harvard University.

Anthony Arleth, Managing Director, Consultancy Practice, Pennside Partners Ltd.

Anthony (Tony) joined Pennside in 2008 and has over 25 years of pharmaceutical industry experience including 20+ years at GlaxoSmithKline. He began his career at as a bench scientist and is a trained biochemist/molecular pharmacologist. Tony brings a wide range of competitive intelligence experience to Pennside, with specialization in the areas of orphan diseases, immunology/dermatology, cardiovascular, oncology and vaccines. His drug development experience includes work on COREG®, TVEVETEN® and TYKERB®. Tony holds 5 US Patents in novel gene discovery and is an author on more than 50 peer-reviewed publications and medical conference abstracts.

Matthias P. Schönermark, Managing Director, SKC

Prof. Matthias P. Schönermark, 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 2003, 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.

KEY EXPERT SPEAKERS

Luke Rosen, Patient Engagement, Ovid Therapeutics

In his role at Ovid Therapeutics, Luke works closely with patient and family organizations to foster innovative, cross-functional collaboration between the Ovid team and members of the rare disease community. He sits on the Board of Directors of Parents for Inclusive Education (PIE) and works to ensure children with disabilities have equal access to New York City Public Schools. Luke is the Founder of KIF1A.ORG, a non-profit foundation working to support research and eliminate challenges for families affected by KIF1A Associated Neurological Disorder.

Robert Hollowell, VP, Head of Corporate Strategy and Commercial Assessment, Shire

Robert is a Group Vice President of Corporate Development and Head of Corporate Strategy and Commercial Assessment at Shire. Since joining Shire, Robert has played a leading role in several major strategic programs including the Baxalta and Dyax acquisitions, Baxalta integration, divestiture of Shire's Oncology business, and the review of strategic alternatives for the Neuroscience division. In addition, Robert has participated in numerous product acquisition and licensing deals ranging from early stage research collaborations to late stage clinical programs. Prior to Shire, Robert was a Principal in the San Francisco and Boston offices of the Boston Consulting Group (BCG). While at BCG, Robert served a range of clients n the biopharma and MedTech sectors with a focus on developing corporate and commercial strategies as well as leading integration and restructuring programs. Robert earned his undergraduate degree in Molecular, Cellular, and Developmental Biology from Yale University and his M.D. degree from Duke University.

Richard A. Basile, Co Founder and CEO, BioPontis Alliance for Rare Diseases

Richard A. Basile is Co-Founder and CEO of BioPontis Alliance Rare disease Foundation, a novel hybrid philanthropic/impact investment international platform with a mission to improve the flow of promising clinical candidates for the many rare disease with no current treatment, and serves on the board of the Foundations investment subsidiary, BioPontis Alliance Rare Therapeutics LLC. Mr. Basile is a biopharmaceutical executive with 35 years of international biopharmaceutical industry experience with early-stage and Fortune 100 Pharma/Biotech companies, including licensing, mergers and acquisitions, product development, sales, marketing and launch in anti-infective, cardiovascular, immunological, dermatologic, CNS and metabolic disease segments, including biotechnology and protein therapeutic products such as recombinant Factor VIII for treatment of a rare clotting disorder, Hemophilia. Mr. Basile served as the global Vice President of corporate strategy, finance, and business development of a $1.2 billion international biologics business unit at Bayer AG (Bayer Biologicals). Mr. Basile also served in several senior marketing, sales and strategic marketing capacities' at Bayer Pharmaceuticals, and was President and General Manager of a Bayer subsidiary, Rhein Chemie Corporation. Mr. Basile was the founding CEO of Entegrion , an early stage biotech company founded on UNC-CH invention in homeostasis, and was the global commercial head of Diosynth Biotechnology, part of Organon Biosciences where he also served on the executive management team of Organon (now Merck & Co) responsible for biotechnology business development, with focus on oncology and immunology. As a founder of BioPontis Alliance for Rare Diseases, Mr. Basile has frequently advocated for a new paradigm in advancing academic research into cures for rare diseases, and has presented to the National Academy's (Government University Industry Research Roundtable) among

KEY 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.

Rachelle Dixon- President / Co-Founder of HSAN1E Society

Rachelle Dixon grew up in the shadow of rare disease. She was born into a family affected by a rare disease, HSAN1E. As an adult, Rachelle was the caregiver for her older brother, and sister. In 2015, Rachelle lost her sister to HSAN1E, and eleven months later she lost her brother to the disease as well.
Rachelle, along with other members of her family formed the HSAN1E Society. HSAN1E Society is a nonprofit organization, that's mission is to raise awareness for HSAN1E and help provide support for other families that are affected by this horrible disease.

Khrystal K. Davis, CEO and Patient Advocate, Zebra Leaf Publishing

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.

KEY EXPERT SPEAKERS

Ken Kengatharan, CEO, Auxesia Orion and Managing Partner, Atheneos Ventures

Dr. Kengatharan is currently a co-founder and Chairman of Renexxion, and Manag ng Director at Atheneos Capital, an evergreen healthcare incubator fund going fo ward focusing on opportunities in orphan drugs and rare diseases. He is a co-founder and former President & CEO of Armetheon and a co-founder and former President & CSO of Altheos, and a co-founder and former Vice President, Pre Clinical R&D of Athenagen (re-named CoMentis).

During the last 17 years, Ken has been critical to the development of multiple drug candidates (NCEs and re-purposed drugs) from concept to Phase II/III. Since setting-up his fi rst company in the late 1990s, working with a great team in each case, Ken has been instrumental in raising more than $160 MM in private equity for seven start-up companies and for closing partnership transactions in excess of $800M in deal value. He is currently a board member at Armetheon and at Renexxion, a venture advisor at SPIRE BioVentures, and an advisor to Stanford University's SPARK program. He has served on the boards of EPi3 (UK), Athenagen and Altheos, been an advisory board member at 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.

Dr. Kengatharan obtained his PhD in pharmacology from the University of London at the William Harvey Research Institute under the supervision of Nobel laureate Sir John Vane and Professor Christoph Thiemermann. Thereafter, he held a post doctoral position at the same institute as a recipient of a British Heart Foundation Fellowship. He obtained his MBA (with Distinction) from Durham University in Eng and, where he focused on Biotech Finance and Entrepreneurship.
Since 2016, Dr. Kengatharan has been an advisor and co-chair of PGE's Orphan Drugs & Rare Diseases Global Congress held in Europe and in the United States.

Michael A. Swit, Esq., FDA Legal Counsel, Law Offices of Michael A. Swit

Michael Swit has been addressing critical U.S. Food and Drug Administration (FDA) legal and regulatory issues since 1984. Before returning to private law practice in late 2017, he served for three years at Illumina, Inc., the world's leading developer of gene sequencing technology, as its chief regulatory counsel. Prior to that, Swit was a special counsel in FDA Practice at the global law firm of Duane Morris LLP in it's San Diego office.
Before joining Duane Morris in March 2012, Swit served for seven years as a vice president at The Weinberg Group Inc., a preeminent scientific and regulatory consulting firm in the Life Sciences. His expertise includes product development, compliance and enforcement, recalls and crisis management, submissions and related traditional FDA regulatory activities, labeling and advertising, and clinical research efforts for all types of life sciences companies, with an emphasis on drugs, biologics, therapeutic biotech products, medical devices, and IVDs, but also has experience with foods, cosmetics, and clinical research issues. His FDA legal experience has included tenures in the food and drug law practices McKenna & Cuneo (now Denton's) and Heller Ehrman, and as vice president, general counsel, and secretary of Par Pharmaceutical, a top public generic and specialty drug firm, where he helped spearhead the company's recovery from prior management's involvement in the Generic Drug Scandal of the late 1980's. He also was, from 1994 to 1998, CEO of FDANews.com, a premier publisher of regulatory newsletters and other specialty information products for FDA-regulated firms. He has taught and written on many topics relating to FDA regulation and associated commercial activities and is a past member of the Food & Drug Law Journal Editorial Board. He earned his AB, magna cum laude, with high honors in history, at Bowdoin College, and his law degree at Emory University.

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