PITCH & PARTNER
Conference Day One
Thursday, April 21, 2016

Primarily for biotech and pharma developing orphan drugs looking to raise capital or find partners, these 10-minute presentation opportunities can also be used to create awareness.

8:00 **Registration & Networking Breakfast**

8:50 **Terrapinn Welcome Remarks**

8:55 **Chairperson's Opening Remarks**

STATE OF THE INDUSTRY

9:00 **Keynote: Creating medicines with real value for orphan diseases**

- The 21st century cures and Innovations acts: Progressing the science of treatment development for the rarest patient
- Approaches to development for the rarest of diseases
- Directing treatment to the disease burden of patients to optimize the development of meaningful drugs

Emil Kakkis, President, **EveryLife Foundation for Rare Diseases**

9:20 **21St Century Cures and Innovations Acts: Opening the door to faster treatment development for devastating diseases or increasing the risk of approving drugs that are not effective or safe?**

- What are the specific provisions for orphan drug development in the 21st Century Cures Act?
- What are the general provisions that can be applied to orphan diseases and aid in drug development?
- Is the act setting overly optimistic expectations to think that drugs can be approved easily?
- What will be the benefits for patients and drug manufacturers?

Laurie Letvak, Head, Clinical Policy and Medical Ethics, Chief Medical Office, **Novartis Pharmaceutical Corporation**
Emil Kakkis, President, **EveryLife Foundation for Rare Diseases**

Moderator: **Peter Saltonstall,** President and CEO, **NORD**

10:00 Speed networking

10:30 Networking coffee break

10:55 **Chairperson's Opening Remarks**

Ross Silver, Principal Analyst, **Vista Partners**

11.00 PBT2, a Phase II/III Huntington's drug candidate with benefits in cognition and function

Birgit Anderegg, VP, Business Development, **Prana Biotechnology**

11.10 Trehalose IV Solution as a Potential Therapy for PolyA/PolyQ Neuromuscular Diseases

Colin Foster, CEO, **BioBlast**

11.20 RP103, a novel and potentially disease-modifying therapy for Huntington's Disease

Antoun Nabhan, VP Corporate Development, **Raptor Phamaceuticals**

11.30 Nanotechnology- based targeted medicines for rare diseases: the good, the bad and the ugly

Stanley Satz, Chief Scientific Officer & Founder, **Advanced Imaging Projects**

11.40 Recombinant human secretoglobins: therapeutic applications in orphan respiratory disease

Thomas Miller, Chief Executive Officer, **Therabron Therapeutics**

11.50 Aerovance for MRSA in CF

Rob Neville, Chief Executive Officer, **Savara Pharmaceuticals**

12.00 Enabling optimal oncology treatment for patients suffering from severe renal disease

Magnus Corfitzen, Chief Executive Officer, **CMC Contrast AB**

12:10 GPX-150 (5-imino-13-deoxydoxorubicin) for the treatment of soft tissue sarcoma

Arthur Klausner, Chief Executive Officer, **Gem Pharmaceuticals**

12.20 **Networking lunch break**

1:30 **Lunch Keynote: FDA's perspective on pediatric and tropical priority review vouchers**

- Differences and similarities of the rare pediatric and the tropical disease voucher system
- What are the benefits and drawbacks associated to using a priority review voucher?
- What is the future of priority review vouchers and what implications will this have in drug development, specifically in the orphan drug space?

Gayatri Rao, Director for the Office of Orphan Products Development (OOPD), **FDA**

1.50 Umbilical cord blood-derived allogeneic stem cell product for the prevention of broncho-pulmonary dysplasia (BPD)

Antonio Lee, CEO, **Medipost America**

2.00 An N of 1 matters when it affects you

Gerald Commissioning, President & CEO, **Amarantus Bioscience**

2.10 Substrate replacement therapy for congenital disorder of glycosylation type Ia

Agnes Rafalko, Ph.D.,Founder & CEO, **Glycomine**

2.20 Inhalation of GM-CSF for the treatment of Pulmonary Alveolar Proteinosis (PAP)

Kim Arvid Nielsen, CEO, **Serendex Pharmaceuticals**

2.30 Perhexiline for the treatment of Hypertrophic Cardiomyopathy

William Daly, President & Chief Executive Officer, **Heart Metabolics**

2.40 OPN-305 a novel anti-inflammatory therapeutic compound

Martin Welschof, CEO, **Opsona Therapeutics**

2.50 Advanced Lipid Technologies™ (ALT™) Platform: improving bioavailability and reducing food effect of Lipophilic API's

Marc Wolff, EVP & CFO, **Sancilio Pharmaceuticals**

3.00 TP-252 for the Treatment of Familial Adenomatous Polyposis

Frank C. Sciavolino, President and Chief Scientific Officer**, Thetis Pharmaceuticals**

3.10 **Networking coffee break**

3.50 Dodecafluoropentane emulsion (NVX-108) as a radiosensitizer in treatment of glioblastoma multiforme

Evan Unger, President & CEO, **Nuvox Pharma**

4.00 A novel AAV-based gene therapy platform for the treatment of rare diseases

Eric Olson, Senior Director, Corporate Development, **AGTC**

4.10 **Mucolytic and xanthine for the treatment of bronchiectasis**

William Howard, President and CEO, **Alitair**

4.20 Lesson's learned from the successful development of drug products for rare diseases and unmet medical needs

David Young, CEO, **Promet Therapeutics**

4.30 A platform technology for treatment of autoimmune diseases: Focus Myasthenia Gravis

Tina Verolin, Program Director, **Toleranzia AB**

4.40 JBPOS0101: treatment candidate for unmet needs in infantile spasms and super-refractory status epilepticus

Harvey Kupferberg, Executive Consultant, **Biopharm Solutions**

4:50 Cell therapy for scleroderma: uniquely positioned for regulatory and commercial success

Marc Hedrick, President and CEO, **Cytori Therapeutics**

5:00 **Chairperson's closing remarks**

PITCH & PARTNER
Conference Day Two
Friday, April 22, 2016

INNOVATION: PROCESSES & DRUG DISCOVERY DEVELOPMENT APPROACH

9:00 **Keynote: Adaptive biomedical innovation: re-engineering how we innovate**

- The era of blockbusters, RCTs-only, and average patients is over!
- What is required to drive meaningful value for patients as innovation is "orphanized"?
- View this evolution through the design lens of MIT NEWDIGS – a global "think and do" tank for collaborative systems engineering across the traditional siloes of the innovation chain.

Gigi Hirsch, Executive Director, **NEWDIGS and CBI, MIT**

9:20 **Keynote: A systematical approach to orphan drug discovery: starting with the unmet needs of payers and patients**

- Has anything changed in orphan drug reimbursement?
- Moving away from target discovery: how to meet payer's and patient's needs
- How can pharma justify the high prices of orphan drugs to payers?

Edmund Pezalla, Vice President, National Medical Director, Pharmacy Policy and Strategy, **Aetna**

9:40 **Tackling the rare diseases therapy deficit - the nonsense mutation read-through agent**

Mark Rothera, Chief Commercial Officer, **PTC Therapeutics**

10:00 **Networking coffee break**

10:35 **Chairperson Opening Remarks**

Jim DeMesa, CEO, **Integene International**

10.40 Sarasar® (lonafarnib) in Hepatitis Delta Virus (HDV) Infection: The next frontier in viral hepatitis.

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

10.50 Single Domain Antibody (SBT-100) Inhibits Triple Negative Breast Cancer Growth by Targeting Intracellular STAT3

Sunanda Singh, CEO & Founder, **Singh Biotechnology**

11.00 Ecopipam for the Treatment of Tourette's Syndrome

Richard Chipkin, President and CEO, **Psyadon Pharmaceuticals**

11.10 AEB1102 as a potential enzyme replacement therapy for Arginase 1 Deficiency

David Lowe, CEO**, Aeglea Biotherapeutics**

11.20 Treating rare diseases and enhancing their therapies: clearance of senescent cells with SIWA Corporation's NeaVira™ monoclonal antibody.

Lewis Gruber, CEO, **SIWA Corporation**

11.30 Targeted treatment for Netherton Disease

Jean Nordstrom, Chairman & CEO, **Sixera Pharma AB**

11.40 Intelligent gene targeting: a novel therapy to prevent amputations in patients with critical limb ischemia

Jim DeMesa, CEO, **Integene International**

11:50 Transporting enzyme replacement therapies across the blood brain barrier for treatment of lysosomal storage diseases

Derek Kelaita, Vice President, Business Development, **ArmaGen Technologies**

12:00 **Chairperson's closing remarks**

12.05 **Networking lunch**

3:00 End of conference