PITCH & PARTNER

Conference Day One- Thursday, April 23rd 2015

Plenary session in the main conference room:

8.50 **Terrapinn Welcome Remarks**
8.55 **Chairperson's Opening Remarks**
Hans Schikan, Former Chief Executive Officer, **Prosensa**

9.00 **Keynote: Data driving diagnostics - Clinically relevant information from ordinary photos, and an algorithm to aid in diagnoses of rare diseases**
Christoffer Nellaker, Research Fellow, Medical Research Foundation's Functional Genomics Unit, **Oxford University**

9.30 **Keynote: Is the market sustainable?**
Henri Termeer, Former Chairman, President and CEO, **Genzyme** (on video)

10.00 Speed Networking & Networking coffee break

Sessions below take place in Pitch & Partner conference room:

11.00 **Using simple genetic model organisms to speed up orphan drug discovery**
Sangeetha Iyer, Scientist, **Perlstein Labs**

11.10 **PSL-001 for treatment of Pancreatic Cancer**
Jeff Geschwind, Founder, Chief Executive Officer, **Prescience Labs**

11.20 **Developing a mutant protein stabilizing platform based on a small repurposed molecule for orphan diseases**
Zohar Argov, Chief Medical Officer, **BioBlast Pharma**

11.30 **KRN5500 and two orphan designations**
Dr. David Drutz, Chief Medical Officer, **DARA Biosciences**

11.40 **NH001 for cognitive recovery from a coma**
Neal H. Farber, Chief Executive Officer, **Neurohealing Pharmaceuticals, Inc.**

11.50 **Phenylbutyrate therapy for Maple Syrup Urine Disease**
Jeff Davis, Head of Corporate Development, **Acer Therapeutics**

12.00 **Networking Lunch**

1.30 **NLX-101 for the treatment of Rett Syndrome**
Mark Varney, Co-Founder, President and Chief Executive Officer, **Neurolixis**

1.40 **Treatment of patients with hand and finger involvement due to scleroderma and symptomatic knee osteoarthritis**
Marc Hedrick, Chief Executive Officer, **Cytori Therapeutics**

1.50 **Human Neural Stem Cells and possible orphan applications**
Ann Tsukamoto, Executive Vice President, Scientific and Strategic Alliances, **StemCells Inc.**

2.00 **Stem Cell treatments for orphan designations**
Teresa Leezer, Chief Executive Officer, **Rhinocyte Inc.**

PITCH & PARTNER

2.10 **Pneumostem for the prevention of Bronchopulmonary Dysplasia (BPD)**
Antonio Lee, Chief Executive Officer and Managing Director, **Medipost America**

2.20 **OS2966: multiple-MOA platform therapeutic for orphan solid cancers**
Shawn Carbonell, Co-Founder, President & CEO, **Oncosynergy**

2.30 **Rare skin and connective tissue diseases**
David Pernock, Chief Executive Officer, **Fibrocell Science**

2.40 **AT-100 and the prevention of bronchopulmonary dysplasia**
Jan Rosenbaum, Chief Scientific Officer, **Airway Therapeutics**

2.50 **Andexanet Alfa: breakthrough-designated Factor Xa Inhibitor antidote**
Jeet Mahal, Head of Corporate Development, **Portola**

3.00 **Networking coffee break**

3.40 **OPN-305 for delayed graft function (DGF) in renal transplantation**
Martin Welschof, Chief Executive Officer, **Opsona Therapeutics**

3.50 **Tozaride targeted peptide therapy for small cell lung and neuroendocrine cancers**
Christopher P. Adams, Founder and CEO, **Andarix**

4.00 **NikZ for treatment of Valley Fever**
David Larwood, Chief Executive Officer, **Valley Fever Solutions**

4.10 **Massive parallelization of orphan drug discovery**
Christopher Gibson, Co-Founder & CEO, **Recursion Pharmaceuticals**

4.20 **Tapping on the brakes: small molecule therapy for the RASopathies**
Lisa Schill, Vice President, **RASopathies Network USA**

4.30 **Inhibitors to restore the function of lekti peptide in Netherton Syndrome lacking the skin barrier**
Jean Nordstrom, Chairman & CEO, **Sixera Pharma AB**

4.40 **Nucleic acids as promising candidates for rare disease treatments**
J. Michael French, President & CEO, **Marina Bio**

4.50 **Promising orphan therapeutics: an overview of Karyopharm's SINE™ compounds**
Margaret Lee, VP, Product Leadership & Biology, **Karyopharm Therapeutics**

5:00 **PCDH19 for the treatment of pediatric epilepsy**
Christopher M. Cashman, Chairman & CEO, **Marinus Pharmaceuticals**

5.10 **Chairperson's recap of the day**

5.25 **Roaring '20s-themed Cocktail Party sponsored by** multicare

7.00 **Gala Dinner*** sponsored by: PROMETIC
This requires separate registration.

PITCH & PARTNER

Conference Day Two- Friday, April 24th 2015

Plenary session in the main conference room:

8.55 **Chairperson's opening remarks**
Kristine Dorward, Director, Marketing & Business Development, **ProMetic**

9.00 **Keynote: Building the cures of the future – FDA's programs to boost orphan drug development**
Richard Moscicki, Deputy Center Director for Science Operations, Center for Drug Evaluation and Research, **FDA**

9.30 **Keynote: RNA as an orphan drug: turning genes on or off, and correcting them to cure genetic disorders**
Daniel Anderson, Scientific Founder, **CRISPR Therapeutics**

10.00 **Morning Refreshments**

Sessions below take place in Pitch & Partner conference room:

10.35 **Chairperson's opening remarks**
Raghuram "Ram" Selvaraju, Managing Director, Equity Research, **MLV & Co.**

10.40 **Pioneering the development of gene therapy based treatments for retinal degenerative diseases**
Jean Philippe Combal, Chief Operations Officer, **GenSight Biologics**

10.50 **Oral biological candidate for the treatment of PKU**
Gjalt Huisman, VP, Pharmaceutical Technology & Innovation, **Codexis**

11.00 **IFB-088, a breakthrough therapeutic approach to treat orphan degenerative diseases caused by misfolded protein accumulation**
Pierre Miniou, Chief Business Officer, **InFlectis BioScience**

11.10 **Some orphan diseases are only skin deep: the role of Granzyme B**
Alistair Duncan, CEO, **viDA Therapeutics**

11.20 **Delivery of enzyme replacement therapies across the blood brain barrier to treat lysosomal storage diseases**
Derek Kelaita, VP, Business Development, **ArmaGen**

11.30 **AeroVanc's successful Phase II clinical trial for Cystic Fibrosis**
Taneli Jouhikainen, Chief Operating Officer, **Savara Pharma**

11.40 **The Tolerogen platform for Myasthenia Gravis**
Tina Verolin, Program Director, **Toleranzia**

11.50 **PHARNEXT Pleotherapy approach in orphan neuropathies**
Xavier Paoli, Commercialization Strategy Director, **Pharnext**

12.00 **Hereditary Neuropathy Foundation: partnered treatment for HNF**
Allison Moore, Chief Executive Officer and Founder, **Hereditary Neuropathy Foundation**

12.10 **AX-s Pharma: a unique approach to rare disease funding**
Jess Rabourn, Chief Executive Officer, **AX-s Pharma**

12.20 **Lunch**

1.50 **Selectively target signaling pathways that lead to tumor-promoting inflammation**
John Maki, President and CEO, **Vicus Therapeutics**

2.00 **A Phase 3 study of L-glutamine therapy for sickle cell anemia and sickle β◦-Thalassemia**
Charles Stark, SVP, R&D, **Emmaus Life Sciences**

2.10 **Living Rare: Families finding help and hope for Menkes Disease**
Jamie Eckman, President, **The Menkes Foundation**

2.25 **SCENESSE as an innovative therapy for EPP**
Nicoletta Muner, Director, Global Regulatory Affairs, **Clinuvel**

2.35 **Potential applications of sigma-1 receptors in orphan indications**
Christopher Missling, President and CEO, **Anavex Life Sciences**

2.45 **Chairperson's closing remarks**