

Breakthrough Technologies Accelerating Diagnosis and Filling the Therapeutic Gap for Rare and Genetic Diseases

According to a 2013 Rare Disease Impact Report conducted by Shire, the economic impact of diagnosing and management of rare disease is substantial. One of the key findings the report revealed is that it takes, on average, more than seven years for a rare disease patient in the US to receive an accurate diagnosis. There are 6,000 rare disease recognized by NIH and yet only approximately 200 of these illnesses have effective pharmacologic treatment coverage. There is a gap of medicines and therapies that can address certain unmet medical needs of rare disease patients that has major growth potential for pharma and device companies in the market for orphan disease.

Sino-American Biotechnology and Pharmaceutical Professional Association (SABPA) Medical Device and Diagnostic (MDD) Forum will bring together stakeholders of in research, academia, regulatory and investment to foster partnerships that will boost the development of medical device, drug, and biological products for rare disease patients; and ultimately improving the diagnosis, treatment and care of patients and their families.

Agenda

- 8:00 am **Registration & Breakfast**
- 8:45 am Welcome and Opening Remarks
- 8:55 am Documentary Trailer on "Undiagnosed" Patients
- 9:00 am Keynote Speaker: Research and Drug Development for Rare Diseases: NIH Initiatives and Resources

Rashmi Gopal-Srivastava, Ph.D., Director, Extramural Research Program, Office of Rare Diseases Research (ORDR), NCATS, NIH

- 9:30 am Solving the Unsolvable: the Genomics Revolution at Our Fingertips Ryan Taft, Ph.D., Director Scientific Research, Illumina, Inc.
- 9:55 am *Key FDA Challenges in Bringing Orphan Drugs to the Market in the United States* **Michael Swit,** J.D., Senior Director, Compliance & Regulatory, Legal, Illumina, Inc.
- 10:20 am Coffee Break & Networking
- 10:50 am *Home-based Non-invasive Brain Monitoring for Individuals with Rare Neurological Conditions*

Philip Low, Ph.D., Founder, Chairman & CEO, Neurovigil Inc.

- 11:15 am Venture Philanthropy: A Novel Approach for Funding Rare Disease Research John Wetherell, Ph.D., Partner, Pillsbury Winthrop Shaw Pittman
- 11:40 am Panel Discussion: New approaches to overcome obstacles and drive discovery and innovation for faster patient impact

Moderator: Christina Waters, Ph.D., CEO & Founder, Rare Science

- Panelists:
 Christian Braemer, CEO & CO-Founder, Benefunder

 Hudson Freeze, Ph.D., Director, Human Genetics Program, Sanford Children's

 Health Research Center

 John Walmsley, Vice President of Product Development, Starfish Medical

 Kim Goodsell, Patient Advocate
- 12:40 pm Closing Remarks
- 12:45 pm Lunch & Networking

2015 MDD Forum: Caroline Cheung (Chair), Hua Gong, Ph.D. and Lei Zhang, Ph.D. (Co-Chairs)

 When
 8:00 am - 2:00 pm, Saturday, February 7, 2015

 Where
 Knobbe Martens (12790 El Camino Real, San Diego, CA 92130)

 Registration
 https://s01.123signup.com/servlet/SignUp?P=15200891911428764000&PG=1520089182300&Info=