

## Vienna BioCenter, IMP Lecture Hall | Vienna, Austria | November 11–14, 2018

Scientific Organizers:

**Josef M. Penninger**, IMBA – Institute of Molecular Biotechnology GmbH, Austria **Kym Boycott**, CHEO Research Institute, Canada

Developed in collaboration with Vienna BioCenter research institutes GMI, IMBA, IMP and MFPL

Work over the past 30 years has resulted in the identification of genes for approximately 50% of the estimated 7,000 rare genetic diseases; it is predicted that most of the remaining disease genes will be identified in the next 10 years. Approximately 500 medicinal products are currently on the market for rare diseases. The accelerating pace of rare disease gene identification means, in effect, an almost commensurate increase in molecularly defined, readily diagnosable, but nonetheless poorly understood and untreatable diseases. This symposium will examine the current and future bottlenecks to gene discovery, disease modeling and therapeutic approaches and suggest strategies to enable progress in this regard. Ultimately, successful deployment of precision medicine for rare diseases will inform such approaches more broadly.

## Plenary Session Topics:

- Approaches to Discover the Causes of all Rare Diseases
- Organoids to Model Rare Disease
- Therapeutic Approaches to Rare Diseases
- Translation of Discoveries to Treatments for Immunological Disorders
- Discovery to Mechanism for Rare Diseases
- The Future of Rare Disease Therapeutic Translation

Scholarship/Discounted Abstract Deadline: July 25, 2018 Abstract Deadline: August 13, 2018 Discounted Registration Deadline: September 18, 2018

Visit **www.keystonesymposia.org/18S4** for more details and visit **bit.ly/KSpenninger** to watch a video with Dr. Josef Penninger.













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