KEYSTONE SYMPOSIA
on Molecular and Cellular Biology

From Rare to Care: Discovery, Modeling and Translation of Rare Diseases (S4)

November 11-14, 2018 • Vienna BioCenter, IMP Lecture Hall • Vienna, Austria

Scientific Organizers: Josef M. Penninger and Kym Boycott

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

Abstract & Scholarship Deadline: July 11, 2018 / Abstract Deadline: August 13, 2018 / Discounted Registration Deadline: September 18, 2018

SUNDAY, NOVEMBER 11

Arrival and Registration

MONDAY, NOVEMBER 12

Welcome and Keynote Address

Speaker to be Announced

Approaches to Discover the Causes of all Rare Diseases

William A. Gahl, NHGRI, National Institutes of Health, USA
The NIH Undiagnosed Diseases Program, National Network, and International Network

Kym Boycott, CHEO Research Institute, Canada
International Coordination to Solve the Unsolved Rare Diseases

Jun Wang†, iCarbonX, China
Omics Technology Combed with Artificial Intelligence and Machine Learning to Understanding the Basis of Human Health and Disease

Short Talk(s) Chosen from Abstracts

Poster Session 1

Organoids to Model Rare Disease

Josef M. Penninger, Institute of Molecular Biotechnology GmbH, Austria
Growing Human Blood Vessels into Complex Human Organoid Disease Models

Jürgen A. Knoblich†, IMBA, Institute of Molecular Biotechnology, Austria
Human Brain Organoids to Model Rare Neurological Diseases

Sergiu Pasca, Stanford University, USA
Assembling Tridimensional Models of the Human Brain to Study Disease

Short Talk Chosen from Abstracts

TUESDAY, NOVEMBER 13

Therapeutic Approaches to Rare Diseases

Speaker to be Announced

Sanjay S. Shukla†, aTyr Pharma, Inc, USA
Immunomodulation with Resolaris to Treat Rare Muscular Dystrophies

Speaker to be Announced

Brian Kaspar†, AveXis, Inc, USA
Gene Therapy for Spinal Muscular Atrophy (SMA)

Short Talk(s) Chosen from Abstracts

Poster Session 2

Translation of Discoveries to Treatments for Immunological Disorders

Speaker to be Announced

Michael J. Lenardo, NIAID, National Institutes of Health, USA
Monogenic Disease Genes in T Cells and Autoimmunity

Christoph Klein, Ludwig-Maximilians-University Munich, Germany
Treating Rare Immunological Disorders

Short Talk Chosen from Abstracts

WEDNESDAY, NOVEMBER 14

Discovery to Mechanism for Rare Diseases

Botond Roska, Friedrich Miescher Institute, Switzerland
Cell Type Targeted Gene Therapy

Bruno Reversade, Institute of Medical Biology ASTAR, Singapore
Rare Begets Common: The Power of Genetic Orphan Diseases

Speaker to be Announced

April Pyle, University of California, Los Angeles, USA
Development of Therapeutic approaches for Muscle Disease using Human Pluripotent Stem Cells

Short Talk(s) Chosen from Abstracts

The Future of Rare Disease Therapeutic Translation

Diego Ardigò, Chiesi Farmaceutici, Italy
International Efforts to Increase Therapies for Rare Diseases

Christiane Druml†, Ludwig Boltzmann Institute for Rare and Undiagnosed Diseases, Austria
The Ethical Dimensions of Rare Disease Research and Gene Repair

Closing Keynote Address

Meeting Wrap-Up: Outcomes and Future Directions (Organizers)

THURSDAY, NOVEMBER 15

Departure