KEYSTONE SYMPOSIA
on Molecular and Cellular Biology

Protein Replacement through Nucleic Acid Therapies (L3)

April 7-10, 2019 • Steamboat Grand • Steamboat Springs, CO, USA

Scientific Organizers: Christine Esau, Inder M. Verma and Melissa J. Moore

Supported by the Directors' Fund

Abstract & Scholarship Deadline: December 11, 2018 / Abstract Deadline: January 8, 2019 / Discounted Registration Deadline: February 5, 2019

SUNDAY, APRIL 7

Arrival and Registration

MONDAY, APRIL 8

Welcome and Keynote Address

Inder M. Verma, The Salk Institute, USA
History of Gene Therapy, Impediments Overcome, and Future Challenges

Design and Delivery

Paula T. Hammond†, Koch Institute for Integrative Cancer Research at MIT, USA
Nanoparticle Delivery of mRNA

Stephen L. Hart, UCL Institute of Child Health, UK
Nucleic Acid Delivery to the Lung

Christian Plank†, Technical University Munich, Germany
Delivery Systems for Nucleic Acids

Short Talk(s) Chosen from Abstracts

Workshop 1: Pathway to FDA Approval

Short Talks Chosen from Abstracts

Protein Replacement with mRNA

Thomas McCauley, Translate Bio, USA
Protein Replacement with mRNA

Michael Kormann†, University Children's Hospital, Germany
mRNA Therapy in the Lung

Christine Esau, Arcturus Therapeutics, USA
Protein Replacement with mRNA

Short Talk Chosen from Abstracts

Poster Session 1

TUESDAY, APRIL 9

Protein Replacement with Gene Therapy

Mark A. Kay, Stanford University, USA
Novel Recombinant AAVs for Classical and Genome Editing Applications

Luigi M. Naldini†, San Raffaele Telethon Institute, Italy
Application of Lentiviral Vectors for Treatment of Genetic Disease

Federico Mingozzi, Spark Therapeutics, Inc., USA
Liver Gene Transfer as Enzyme Replacement Therapy in Lysosomal Storage Diseases

Jean Bennett†, University of Pennsylvania, USA
Gene Therapy for Eye Disease

Short Talk(s) Chosen from Abstracts

Workshop 2: Delivery Systems for mRNA

Short Talks Chosen from Abstracts

Therapeutic Applications

Melissa J. Moore, Moderna Therapeutics, USA
Protein Replacement with mRNA

James M. Wilson†, University of Pennsylvania, USA
Application of AAV for Treatment of Genetic Disease

Adrian Krainer†, Cold Spring Harbor Laboratories, USA
Modulation of Protein Production through Splicing Modulation

Short Talk Chosen from Abstracts

Poster Session 2

WEDNESDAY, APRIL 10

Gene Correction

Michael C. Holmes†, Sangamo Therapeutics, Inc., USA
Gene Correction with Zinc Finger Proteins

Erik Sontheimer†, University of Massachusetts Medical School, USA
Optimizing Gene Editing Systems

Annemieke Aartsma-Rus†, Leiden University Medical Center, Netherlands
Correcting mRNA for Duchenne's Muscular Dystrophy

Patrick D. Hsu, The Salk Institute, USA
CRISPR Technologies for Targeting RNA

Short Talk(s) Chosen from Abstracts

Workshop 3: Viral Vector Design

Short Talks Chosen from Abstracts

Current Challenges

R. Jude Samulski†, University of North Carolina at Chapel Hill, USA
Design of AAV Vectors

Speaker to be Announced

Maria Limberis†, University of Pennsylvania, USA
Handling Immunogenicity Associated with Protein Replacement

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

THURSDAY, APRIL 11

Departure