

KEYSTONE SYMPOSIA

on Molecular and Cellular Biology

Protein Replacement through Nucleic Acid Therapies (L3)

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

Scientific Organizers: Pad Chivukula, Melissa J. Moore and Jean Bennett

Sponsored by Moderna and Sarepta Therapeutics

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

Speaker to be Announced

Design and Delivery

Speaker to be Announced

Stephen L. Hart, UCL Institute of Child Health, UK

Nucleic Acid Delivery to the Lung

Christian Plank, Technical University Munich, Germany

mRNA Transcript-Activated Matrix for Bone Regeneration

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

Beverly L. Davidson, Children's Hospital of Philadelphia and The University of Pennsylvania, USA

Talk Title to be Announced

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 R. Krainer, Cold Spring Harbor Laboratory, USA

Restoring Protein Production through Splicing Modulation

Short Talk Chosen from Abstracts

Poster Session 2

WEDNESDAY, APRIL 10

Gene Correction

Michael C. Holmes, 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

Restoring Dystrophin Expression for Duchenne 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

Using Vectors as Vaccines

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

THURSDAY, APRIL 11

Departure