Keystone Symposia in Steamboat Springs

Protein Replacement through Nucleic Acid Therapies

Steamboat Grand | Steamboat Springs, Colorado, USA | April 7–10, 2019

Scientific Organizers:
Padmanabh Chivukula, Arcturus Therapeutics, Inc, USA
Melissa J. Moore, Moderna Therapeutics, USA
Jean Bennett, University of Pennsylvania, USA

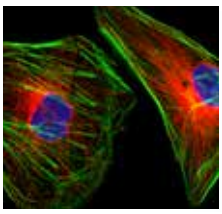
Protein replacement therapy based on recombinant protein has so far been limited to genetic diseases in which the mutated protein acts extracellularly. Nucleic acid therapies such as gene therapy and messenger RNA enable replacement of intracellular proteins, or extracellular proteins too complex to manufacture. This opens up the potential to treat many previously unapproachable diseases. After early setbacks in gene therapy, a new generation of therapeutics is showing progress in the clinic. Novel messenger RNA and RNA delivery technologies are also in development, the potential of which is only beginning to be demonstrated. These represent exciting areas of therapeutic development which also touch on fundamental questions about regulation of gene expression, protein production and immunity. This conference brings these communities together to discuss common challenges and complementarities, providing an opportunity for cross-fertilization.

Session Topics:
- Design and Delivery
- Protein Replacement with mRNA
- Protein Replacement with Gene Therapy
- Therapeutic Applications
- Gene Correction
- Current Challenges

Scholarship/Discounted Abstract Deadline: Dec 11, 2018; Abstract Deadline: Jan 8, 2019; Discounted Registration Deadline: Feb 5, 2019

Visit **www.keystonesymposia.org/19L3** for more details.





KEYSTONE SYMPOSIA™
on Molecular and Cellular Biology
Accelerating Life Science Discovery
a 501(c)(3) nonprofit educational organization

Left image of cytoskeletal proteins courtesy of National Cancer Institute, NIH

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Protein Replacement through Nucleic Acid Therapies (L3)

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

Scientific Organizers: Pad Chivukula, Jean Bennett and Paloma Giangrande

Sponsored by Moderna, Novo Nordisk A/S and Sarepta Therapeutics

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

SUNDAY, APRIL 7

Arrival and Registration

MONDAY, APRIL 8

Welcome and Keynote Address

*__Pad Chivukula__, Arcturus Therapeutics, Inc., USA

__Pieter Cullis__, University of British Columbia, Canada
Effective Non-Viral Vectors for Delivery of mRNA in vivo

Design and Delivery

*__Paul Burke__, Burke Bioventures LLC, USA
LNPs for Delivery of mRNA Therapeutics: Lessons from Translation

__Stephen L. Hart__, University College London, UK
Nucleic Acid Therapeutics for Cystic Fibrosis Delivered by Targeted Nanoparticles

__Manish Aneja__, ethris GmbH, Germany
mRNA Transcript-Activated Matrix for Bone Regeneration

__John H. Lockhart__, University of South Florida, USA
Short Talk: Effective Delivery of Therapeutic mRNA using Peptide-Based Nanoparticles

__Jeffrey Rubin__, Mayo Clinic Graduate School of Biomedical Sciences, USA
Short Talk: Enhanced Gene Delivery to the Kidney by Adenovirus and Adeno-Associated Virus

Protein Replacement with mRNA

*__Anton McCaffrey__, TriLink BioTechnologies, USA

__Pad Chivukula__, Arcturus Therapeutics, Inc., USA
Protein Replacement with mRNA

__Jeffrey R. Holt__, Boston Children's Hospital, USA
Improved Gene Therapy Restores Hearing, Balance, and Secondary Measures in Mice with Genetic Inner Ear Disorders

__Keiji Itaka__, Tokyo Medical and Dental University, Japan
mRNA Medicine for Introducing Therapeutic Transcription Factors

__Jia Tay__, Translate Bio, USA
Advancing the Development of mRNA Therapeutics to Restore Protein Function

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 University of Pennsylvania, USA
Considerations in Gene Therapy for the LSDs

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

__Jean Bennett__, University of Pennsylvania, USA
Seeing the Light with Retinal Gene Therapy: From Fantasy to Reality

Therapeutic Applications

__Paloma Giangrande__, Moderna, USA
Protein Replacement with mRNA for Inherited Metabolic Diseases

*__Drew Weissman__, University of Pennsylvania, USA
Talk Title to be Announced

__Adrian R. Krainer__, Cold Spring Harbor Laboratory, USA
Targeted Antisense Therapeutics for Modulation of Splicing or NMD

__Liangliang Hao__, Massachusetts Institute of Technology, USA
Short Talk: Tumor Penetrating RNA Delivery for Therapeutic Benefit of Pancreatic Cancer

WEDNESDAY, APRIL 10

Gene Correction

*__Erik Sontheimer__, University of Massachusetts Medical School, USA
Enhancing Genome Editing with New Cas9s and Chemically Engineered Guides and Homology-Dependent Repair Donors

__Annemieke Aartsma-Rus__, Leiden University Medical Center, Netherlands
Development of Antisense-Mediated Exon Skipping for Duchenne: It Takes More than an Antisense Oligonucleotide

__Patrick D. Hsu__, The Salk Institute for Biological Studies, USA
New Molecular Technologies for Transcriptome Engineering

__Ranjan Batra__, Locana Bio, USA
Reversal of Microsatellite Diseases using RNA-Targeting Proteins

Current Challenges

__Matthew Hirsch__, University of North Carolina at Chapel Hill, USA
AAV Gene Therapy Prevents and Reverses MPS1 Corneal Opacity

*__Luk H. Vandenberghe__, Harvard Medical School, USA
Development of Technology to Overcome Translational Hurdles

__Maria Limberis__, University of Pennsylvania, USA
Using Vectors as Vaccines

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

THURSDAY, APRIL 11

Departure