



Sunday, October 6

Pre-conference Session: Oligonucleotide Therapeutics Education Workshop

Chair: Hassan Fakhri, Ph.D., *RNA Therapeutics Institute, UMass Chan Medical School*

RNA eh! The Leading Edge of the RNA Revolution at McGill, in Quebec and Across Canada

Thomas Duchaine, Ph.D., *McGill University*

Selecting a Platform for Novel Oligonucleotide Clinical Bioanalysis, Assessing Benefits and Challenges

Rebecca Lescarbeau, Ph.D., *Intellia Therapeutics*

The Evolving Landscape of Oligonucleotide Manufacturing: Meeting Future Demand with Ligation Technologies

David Butler, Ph.D., *Hongene Biotech Corp.*

Nonclinical Development of Nucleic Acid-Based Therapies

Jessica Grieves, DVM, Ph.D., DACVP, *Ionis Pharmaceuticals, Inc.*

Considerations on Oligonucleotide Therapeutics Development: A Non-Clinical and Clinical Regulatory Perspective

Kris Siezen, Ph.D., *Medicines Evaluation Board*

Pre-conference Session: Next-Gen Early Career Scientist Session

Co-Chairs: Eva-Maria Manz, Ph.D. Candidate, *ETH Zürich* & Jathavan Asohan, Ph.D. Candidate, *McGill University*

Session Keynote:

A Decade of Extrahepatic Oligonucleotide Delivery

Maire Fiona Jung, Ph.D. *Eli Lilly*

Establishment of Immune Competent In Vitro Models for Identification of ASOs with Inflammatory Profile

Mahnoush Bahjat, Ph.D., *AstraZeneca*

RES-010: a Therapeutic Antisense Oligonucleotide Targeting microRNA-22 for Treatment of Obesity and Metabolic Disorders to Increase GLP-1 Receptor Agonists Efficacy

Riccardo Panella, *Aalborg University*

RNAi Strategies Abolish Critical Toxicity in Allogeneic Cell Therapies

Reka Haraszti, Ph.D., *University Hospital Tübingen*

Toxicity of Antisense Oligonucleotides is Determined by the Synergistic Interplay of Chemical Modifications and Nucleotide Sequences, Not by Either Factor Alone

Sankha Pattanayak, Ph.D., *Creyon Bio*

Oxepane Nucleic Acid (ONA) Analogues: Synthesis and Application in Antisense and CRISPR-Cas9/12a Gene Editing Systems

Sunit Kumar Jana, Ph.D., *McGill University*

Stop-Codon Readthrough by R-ASO

Andrei Korostelev, Ph.D., *UMass Chan Medical School*



Annual Meeting Opening & Keynote Session

Keynote Presentation:

From Rare to Extremely Rare: Applying Lessons Learned from Duchenne Muscular Dystrophy Antisense Oligonucleotide Mediated Exon Skipping to Developing Individualized Treatment

Prof. Dr. Annemieke Aartsma-Rus, Ph.D., *Leiden University Medical Center*

Monday, October 7

Session I: Chemistry, Mechanism and Delivery I

Chair: Jonathan Hall, Ph.D., *ETH Zürich*

Modulating siRNA Activities Using Click Chemistry

Nathan Luedtke, Ph.D., *McGill University*

Engineering of Brainshuttle-Antisense Oligonucleotide Conjugates for Brain Delivery - a Field Guide

Felix Schumacher, Ph.D., *Roche*

Chemically Enhanced RNA Aptamers for Diagnostic and Therapeutic Applications

Jorgen Kjems, Ph.D., *University of Aarhus*

Enzyme (R)evolution in Nucleic Acid Synthesis

Stefan Lutz, Ph.D., *Codexis, Inc.*

2'-ALE chemistry enables the synthesis of RNA oligonucleotides over 100nt in length

Zidi Lyu, *McGill University*

Expanding the Toolbox of Chemical Reactions for the Generation and Application of ASO Conjugates

Laurent Knerr, Ph.D., *AstraZeneca*

Session II: Pre-Clinical I (Early Stage)

Chair: Maja Janas De Angelis, Ph.D, DABT, *Alnylam Pharmaceuticals*

Engineered RNA Targeting Systems in Neurodegeneration

Gene Yeo, Ph.D., MBA, *UCSD*

Preclinical Development of Personalized ASO Therapeutics Using Patient-Derived Organoid Systems

Scott Younger, Ph.D., *Genomic Medicine Center, Children's Mercy Kansas City*

Realizing the Promise of In Vivo CRISPR Therapies

Jonathan Phillips, Ph.D., *Intellia Therapeutics*

Oral Delivery of GalNAc-Conjugated siRNAs

Mikyung Yu, Ph.D., *Alnylam Pharmaceuticals*

Identification of Key Determinants for siRNA Therapeutics Using Quantitative Systems Pharmacology Modeling

Sukyung Woo, *University of Buffalo*



20th Annual Meeting of the Oligonucleotide Therapeutics Society
October 6-9, 2024, Centre Mont-Royal, Montreal, QC, Canada
Meeting Agenda

Towards a New Generation of Pulmonary Antisense Drugs

Alexey Revenko, Ph.D., *Ionis Pharmaceuticals*

Session III: Pre-clinical II (Late Stage)

Chair: Marie Wikström Lindholm, Ph.D., *Silence Therapeutics*

TfR1 Fab-Mediated Delivery of Oligonucleotides for the Treatment of Rare Neuromuscular Disorders

Oxana Beskrovnaya, Ph.D., *Dyne Therapeutics*

Translation of the Pharmacodynamics of Antibody-Oligonucleotide Conjugates for Neuromuscular Disease

Husam Younis, Ph.D., *Avidity Biosciences*

KRRO- 110, an RNA Editing Oligonucleotide for the Treatment of Alpha 1 Antitrypsin Deficiency (AATD)

Venkat Krishnamurthy, Ph.D., *Korro Bio, Inc.*

Expanding RNA Editing Applications to CNS Disorders Through Rational AIMER Chemistry Design

Michael Byrne, Ph.D. *Wave Life Sciences*

Tuesday, October 8

Session IV: DNA/RNA Editing

Chair: Laura Sepp-Lorenzino, Ph.D., *Intellia Therapeutics*

Guide and Template Engineering for Genome Editing

Erik Sontheimer, Ph.D., *UMass Chan Medical School*

Targeted Gene Insertion of Factor 9 as a Potential Durable Treatment for Hemophilia B

Leah Sabin, Ph.D., *Regeneron Genetic Medicines*

Writing DNA With RNA: Genome Engineering by Target Primed Reverse Transcription

Cecilia Cotta-Ramusino, Ph.D., *Tessera Therapeutics*

Enzymatically Controlled Release and Stabilization of mRNA Using a Multi-Layered Nucleic Acid Nanocapsule

Jessica Rouge, Ph.D., *University of Connecticut*

Development of a Selection Method for Evolution of Functional Variant Prime Editing Guide RNA Scaffolds

Jonathan Zhou, *Duke University*

Oligonucleotide Guided RNA Editing of SLC10A1 (NTCP) as a Therapeutic Approach to Lower Bile Acid Re-Uptake in Cholestatic Diseases

Gerard Platenburg, *ProQR Therapeutics*

Session V: Awards Session I - Lifetime Achievement Award

Introduction by **Mano Manoharan**, Ph.D., *Alnylam*

John Maraganore, Ph.D., *JMM Innovations*

2024 Lifetime Achievement Award



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Session VI: Chemistry, Mechanism, Delivery II

Chair: Steve Dowdy, Ph.D., *UCSD School of Medicine*

Development Of Selective Organ Targeting (SORT) Lipid Nanoparticles (LNPs) For Genome Correction Of Disease-Causing Mutations

Dan Siegwart, Ph.D., *UT Southwestern*

Expanding the Alphabet and Topology of Synthetic mRNAs

Xiao Wang, Ph.D., *MIT*

Delivery of RNA Therapeutics: Pulling Off the Great Endosomal Escape

Steve Dowdy, Ph.D., *UCSD School of Medicine*

Wednesday, October 9

Session VII: Awards Session II

Chair: Richard Geary, Ph.D., *OTS President, Ionis Pharmaceuticals*

Award Announcements

Poster Awards, Patient Advocacy Award, Paper of the Year Award – Basic Research

Dr. Alan M. Gewirtz Memorial Scholarship Award for Postdocs/Jr Professionals

Potent and Durable Gene Modulation in Heart and Muscle with Chemically Defined siRNAs

Hassan H. Fakhri, Ph.D., *Post-Doctoral Associate, Prof. Anastasia Khvorova Research Group, RNA Therapeutics Institute | UMASS Chan Medical School*

Dr. Alan M. Gewirtz Memorial Scholarship Award for Graduate Students

Modulation Of Somatic Repeat Expansion with siRNAs as a Therapeutic Approach to Huntington's Disease

Jillian Belgrad, M.D./Ph.D. Candidate, MS3/GS4, *Khvorova Lab, RNA Therapeutics Institute, UMass Chan Medical School*

Mary Ann Liebert publishers, Inc. Young Investigator Award

Advancing Antisense Oligonucleotides into Therapies for Rare Brain Disorders

Hélène Tran, Ph.D., *Research Director, Head of Antisense Oligonucleotide Therapeutics, Neurology Therapeutic Area, Servier Research Institute*

Session VIII: Clinical

Chair: Art Krieg, M.D., *RNA Therapeutics Institute, UMass Chan Medical School*

Identification and Early Clinical Development of Lepodisiran, a GalNAc-Conjugated siRNA Targeting Lipoprotein(a) Production

Laura Michael, Ph.D., *Eli Lilly and Co.*

Clinical Development of Zilebesiran, a Subcutaneous siRNA Targeting Angiotensinogen for Hypertension

Ishir Bhan, MD, *Alnylam Pharmaceuticals*

Lessons from a Phase One Trial of a Lipid-Conjugated siRNA, CBP-4888

Scott Johnson, *Comanche Biopharma*



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Individualized Neoantigen Therapy: mRNA Therapeutics Coming of Age in Cancer

Laureen Ojalvo, MD, Ph.D., *Moderna*

The OASIS-HAE Phase 3 Program of Donidalorsen for the Treatment of Hereditary Angioedema

Ken Newman, MD, *Ionis Pharmaceuticals*

Advancing N-of-1 Oligonucleotide Therapeutics: Progress, Lessons Learned, and Next Steps

Tim Yu, MD, Ph.D., *Boston Children's Hospital, N=1 Collaborative*

Session IX: Late Breaking Talks

Chair: Rebecca Miles, Ph.D., *ReiNA Consulting LLC*

Development of a Broad-Spectrum siRNA Therapy Against Human Coronaviruses

Julian Vogler, *LMU Munich*

Towards a Divalent siRNA Therapy for Prion Disease

Sonia Vallabh, *Broad Institute*

Non-clinical Development of Antisense Oligonucleotides and siRNAs: Evaluation of the Current Regulatory Perspective

Britt Duijndam, *Medicines Evaluation Board*

Antiviral miRNAs and Their Modulators: Regulation of IFN Response and Apoptosis in Virus-Infected Cells and Potential for Nucleic Acid Therapeutics

Tomoko Takahashi, Ph.D., *Saitama University*

Ultra-short 2'-O-Methyl-guanosine RNA Fragments Mediate Essential Natural TLR7/8 Antagonism and Provide a Backbone for Therapeutic Development

Olivier Laczka, Ph.D., *Noxopharm Ltd.*