



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

Meeting Agenda

As of October 4, 2024

Sunday, October 6 (Day ONE)

08:00-09:00 *Registration Open – Welcome Coffee*

09:00-11:10 <i>Pre-conference Session:</i> Oligonucleotide Therapeutics Education Workshop Chair: Hassan Fakh, Ph.D., <i>RNA Therapeutics Institute, UMass Chan Medical School</i>
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09:00-09:05 Intro by chair

09:05-09:30 **RNA eh! The Leading Edge of the RNA Revolution at McGill, in Quebec and Across Canada**
Thomas Duchaine, Ph.D., *McGill University*

09:30-09:55 **Selecting a Platform for Novel Oligonucleotide Clinical Bioanalysis, Assessing Benefits and Challenges**
Rebecca Lescarbeau, Ph.D., *Intellia Therapeutics*

09:55-10:20 **The Evolving Landscape of Oligonucleotide Manufacturing: Meeting Future Demand with Ligation Technologies**
David Butler, Ph.D., *Hongene Biotech Corp.*

10:20-10:45 **Nonclinical Development of Nucleic Acid-Based Therapies**
Jessica Grieves, DVM, Ph.D., DACVP, *Ionis Pharmaceuticals, Inc.*

10:45-11:10 **Considerations on Oligonucleotide Therapeutics Development: A Non-Clinical and Clinical Regulatory Perspective**
Kris Siezen, Ph.D., *Medicines Evaluation Board*

11:15-13:00 *Hosted Lunch Break (for all registered delegates)*
&
Mentorship Program Lunch (by invitation – reserved to Program Attendees only)

Location: LE GERMAIN Hotel - Room: Le Pavillon (2050 Mansfield Street.)

13:00-15:00 <i>Pre-conference Session:</i> Next-Gen Early Career Scientist Session Co-Chairs: Eva-Maria Manz, Ph.D. Candidate, <i>ETH Zürich</i> & Jathavan Asohan, Ph.D. Candidate, <i>McGill University</i>

13:00-13:05 Welcome & Introduction

13:05-13:30 *Session Keynote:*
A Decade of Extrahepatic Oligonucleotide Delivery
Maire Fiona Jung, Ph.D. *Eli Lilly*

13:30-13:45 **Establishment of Immune Competent In Vitro Models for Identification of ASOs with Inflammatory Profile**
Mahnoush Bahjat, Ph.D., *AstraZeneca*

13:45-14:00 **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*



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- 14:00-14:15 **RNAi Strategies Abolish Critical Toxicity in Allogeneic Cell Therapies**
Reka Haraszti, Ph.D., *University Hospital Tubingen*
- 14:15-14:30 **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*
- 14:30-14:45 **Oxepane Nucleic Acid (ONA) Analogues: Synthesis and Application in Antisense and CRISPR-Cas9/12a Gene Editing Systems**
Sunit Kumar Jana, Ph.D., *McGill University*
- 14:45-15:00 **Stop-Codon Readthrough by R-ASO**
Andrei Korostelev., Ph.D., *UMass Chan Medical School*
- 15:00-15:30 Refreshment Break**

15:30-16:45 Annual Meeting Opening & Keynote Session

- 15:30-15:45 Welcome & Opening Remarks
Richard Geary, Ph.D., *OTS President, Ionis Pharmaceuticals*
- 15:45-16:45 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*
- 16:45-19:30 Welcome Reception with Exhibitors - No poster hosting**
- 20:00-23:00 Early Career Scientist Social Meet Up (RSVP was required, no walk-ins, students only)**
Location: Messorém Bar (Rue Pitt 2233)

Sponsored by: 

Monday, October 7 (Day TWO)

08:00-08:30 Registration Open - Welcome Coffee

08:30-10:30 Session I: Chemistry, Mechanism and Delivery I

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

- 08:30-08:55 **Modulating siRNA Activities Using Click Chemistry**
Nathan Luedtke, Ph.D., *McGill University*
- 08:55-09:20 **Engineering of Brainshuttle-Antisense Oligonucleotide Conjugates for Brain Delivery - a Field Guide**
Felix Schumacher, Ph.D., *Roche*
- 09:20-09:45 **Chemically Enhanced RNA Aptamers for Diagnostic and Therapeutic Applications**
Jorgen Kjems, Ph.D., *University of Aarhus*



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- 09:45–10:00 **Enzyme (R)evolution in Nucleic Acid Synthesis**
Stefan Lutz, Ph.D., *Codexis, Inc.*
- 10:00-10:15 **2'-ALE Chemistry Enables the Synthesis of RNA Oligonucleotides Over 100 nt in Length**
Zidi Lyu, Ph.D. Candidate, *McGill University*
- 10:15-10:30 **Expanding the Toolbox of Chemical Reactions for the Generation and Application of ASO Conjugates**
Laurent Knerr, Ph.D., *AstraZeneca*

10:30-11:00 *Refreshment Break*

11:00-12.35	Session II: Pre-Clinical I (Early Stage)
Chair:	Maja Janas De Angelis, Ph.D, DABT, <i>Alnylam Pharmaceuticals</i>

- 11:00-11:25 **Preclinical Development of Personalized ASO Therapeutics Using Patient-Derived Organoid Systems**
Scott Younger, Ph.D., *Genomic Medicine Center, Children's Mercy Kansas City*
- 11:25-11.50 **Realizing the Promise of In Vivo CRISPR Therapies**
Jonathan Phillips, Ph.D., *Intellia Therapeutics*
- 11.50-12:05 **Oral Delivery of GalNAc-Conjugated siRNAs**
Mikyung Yu, Ph.D., *Alnylam Pharmaceuticals*
- 12:05-12:20 **Identification of Key Determinants for siRNA Therapeutics Using Quantitative Systems Pharmacology Modeling**
Sukyung Woo, *University of Buffalo*
- 12:20-12.35 **Towards a New Generation of Pulmonary Antisense Drugs**
Alexey Revenko, Ph.D., *Ionis Pharmaceuticals*

12.35-14:30 *Hosted Lunch Break*

14.30-16.10	Session III: Pre-clinical II (Late Stage)
Chair:	Marie Wikström Lindholm, Ph.D., <i>Silence Therapeutics</i>

- 14:30-14:55 **TfR1 Fab-Mediated Delivery of Oligonucleotides for the Treatment of Rare Neuromuscular Disorders**
Oxana Beskrovnaya, Ph.D., *Dyne Therapeutics*
- 14:55-15:20 **Translation of the Pharmacodynamics of Antibody-Oligonucleotide Conjugates for Neuromuscular Disease**
Husam Younis, Ph.D., *Avidity Biosciences*
- 15:20-15:45 **ex- 110, an RNA Editing Oligonucleotide for the Treatment of Alpha 1 Antitrypsin Deficiency (AATD)**
Venkat Krishnamurthy, Ph.D., *Korro Bio, Inc.*



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15:45-16:10 **Expanding RNA Editing Applications to CNS Disorders Through Rational Aimer Chemistry Design**
Michael Byrne, Ph.D. *Wave Life Sciences*

16:30-19:30 Poster Session I Reception - ODD numbered posters hosted

Tuesday, October 8 (Day THREE)

08:00-08:30 Registration Open - Welcome Coffee

08:30-10:30 Session IV: DNA/RNA Editing

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

08:30-08:55 **Guide and Template Engineering for Genome Editing**

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

08:55-09:20 **Targeted Gene Insertion of Factor 9 as a Potential Durable Treatment for Hemophilia B**

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

09:20-09:45 **Writing DNA With RNA: Genome Engineering by Target Primed Reverse Transcription**

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

09:45-10:00 **Enzymatically Controlled Release and Stabilization of mRNA Using a Multi-Layered Nucleic Acid Nanocapsule**

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

10:00-10:15 **Development of a Selection Method for Evolution of Functional Variant Prime Editing Guide RNA Scaffolds**

Jonathan Zhou, *Duke University*

10:15-10:30 **Oligonucleotide Guided RNA Editing of SLC10A1 (NTCP) as a Therapeutic Approach to Lower Bile Acid Re-Uptake in Cholestatic Diseases**

Gerard Platenburg, *ProQR Therapeutics*

10:30-11:00 Refreshment Break

11:00-12:30 Session V: Awards Session I - Lifetime Achievement Award

11:00-11:10 Introduction by **Mano Manoharan**, Ph.D., *Alnylam*

11:10-12:00 **John Maraganore**, Ph.D., *JMM Innovations*
2024 Lifetime Achievement Award

12:00-12:30 Q&A

12:30-14:30 Hosted Lunch Break

14:30-15:45 Session VI: Chemistry, Mechanism, Delivery II

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

14:30-14:55 **Development of Selective Organ Targeting (SORT) Lipid Nanoparticles (LNPs) for Genome Correction of Disease-Causing Mutations**

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



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- 14:55-15:20 **Expanding the Alphabet and Topology of Synthetic mRNAs**
Xiao Wang, Ph.D., *MIT*
- 15:20-15:45 **Delivery of RNA Therapeutics: Pulling Off the Great Endosomal Escape**
Steve Dowdy, Ph.D., *UCSD School of Medicine*
- 15.45-18.45 Poster Session II Reception - EVEN numbered posters hosted**
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Wednesday, October 9 (Day FOUR)

08:00-08:30 Registration Open - Welcome Coffee

08:30-10:15	Session VII: Awards Session II
Chair:	Richard Geary, Ph.D., <i>OTS President, Ionis Pharmaceuticals</i>

8:30-8:45 **Award Announcements**

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

8:45-9:15 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. Fakih, Ph.D., *Post-Doctoral Associate, Prof. Anastasia Khvorova Research Group, RNA Therapeutics Institute | UMASS Chan Medical School*

9:15-9:45 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*

9:45-10:15 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*

10:15-11:00 Refreshment Break

11:00-13:00	Session VIII: Clinical
Chair:	Art Krieg, M.D., <i>RNA Therapeutics Institute, UMass Chan Medical School</i>

11:00-11:20 **Identification and Early Clinical Development of Lepodisiran, a GalNAc-Conjugated siRNA Targeting Lipoprotein(a) Production**
Laura Michael, Ph.D., *Eli Lilly and Co.*

11:20-11:40 **Clinical Development of Zilebesiran, a Subcutaneous siRNA Targeting Angiotensinogen for Hypertension**
Ishir Bhan, MD, *Alnylam Pharmaceuticals*



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- 11:40-12:00 **Lessons from a Phase One Trial of a Lipid-Conjugated siRNA, CBP-4888**
Scott Johnson, *Comanche Biopharma*
- 12:00-12:20 **Individualized Neoantigen Therapy: mRNA Therapeutics Coming of Age in Cancer**
Laureen Ojalvo, MD, Ph.D., *Moderna*
- 12:20-12:40 **The OASIS-HAE Phase 3 Program of Donidalorsen for the Treatment of Hereditary Angioedema**
Ken Newman, MD, *Ionis Pharmaceuticals*
- 12:40-13:00 **Advancing N-of-1 Oligonucleotide Therapeutics: Progress, Lessons Learned, and Next Steps**
Tim Yu, MD, Ph.D., *Boston Children's Hospital, N=1 Collaborative*

13.00-14.30 *Hosted Lunch Break*

14:30-16:10 Session IX: Late Breaking Talks Chair: Rebecca Miles, Ph.D., <i>ReiNA Consulting LLC</i>

- 14:30-14:50 **Development of a Broad-Spectrum siRNA Therapy Against Human Coronaviruses**
Julian Vogler, *LMU Munich*
- 14:50-15:10 **Towards a Divalent siRNA Therapy for Prion Disease**
Sonia Vallabh, *Broad Institute*
- 15:10-15:30 **Non-Clinical Development of Antisense Oligonucleotides and siRNAs: Evaluation of the Current Regulatory Perspective**
Britt Duijndam, *Medicines Evaluation Board*
- 15:30-15:50 **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*
- 15:50-16:10 **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.*

16:10-16:30 Closing Remarks

19:00-23:00 *Annual Meeting Wrap Party at The Windsor Ballrooms*

Pre-registration and additional fee required. The party was sold out at the time of printing. Check with the registration team onsite for any cancellation if you still want to join.

LOCATION: Windsor Ballrooms, 1170 Rue Peel #110, Montreal QC H3B 0A9

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