



20<sup>th</sup> Annual Meeting of the Oligonucleotide Therapeutics Society  
October 6-9, 2024, Centre Mont-Royal, Montreal, QC, Canada  
**Meeting Agenda**  
As of August 23, 2024

## Sunday, October 6 (Day ONE)

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

<b>09:00-11:00</b> <b>Pre-conference Session - Oligonucleotide Therapeutics Education Workshop</b> Chair: Hassan Fakhri, 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 (reserved for program attendees only)*

<b>13:00-15:00</b> <b>Pre-conference Session Contd. - New-Gen Early Career Scientist Session</b> Co-Chairs: Eva-Maria Manz, Ph.D. Candidate, <i>ETH Zürich</i> & Jathavan Asohan, Ph.D. Candidate, <i>McGill University</i>
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13:00-13:05 Welcome & Introduction

13:05-13:30 Keynote Address:  
**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, *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*

14:00-14:15 **RNAi Strategies Abolish Critical Toxicity in Allogeneic Cell Therapies**

Reka Haraszti, *University Hospital Tubingen*



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- 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**  
Swagatam Mukhopadhyay, *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, *McGill University*
- 14:45-15:00 **Stop-codon readthrough by R-ASO**  
Andrei Korostelev., *UMass Chan Medical School*
- 15:00 Voting for Best Talk of Next Gen Session

#### 15:00-15:30 Refreshment Break

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

- 15:30-15:45 Welcome & Opening Remarks  
**David Corey**, Ph.D., *Organizing Chair, UT Southwestern*  
**Laura Sepp-Lorenzino**, Ph.D., *Organizing Chair, Intellia Therapeutics*  
**Masad J. Damha**, Ph.D., *Organizing Chair, McGill University*
- 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 (LUMC, the Netherlands)*

#### 16:45-19:30 Welcome Reception with Exhibitors (No host poster viewing)

#### 20:00-23:00 STUDENTS ONLY: Networking Event – Early Career Scientist Social Meet Up

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*



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09:20 – 09:45 **Chemically Enhanced RNA Aptamers For Diagnostic And Therapeutic Applications**  
Jorgen Kjems, Ph.D., *University of Aarhus*

09:45–10:00 **Enzyme (r)evolution in nucleic acid synthesis**  
Stefan Lutz, *Codexis, Inc.*

10:00-10:15 **The potential of gapmers to silence bacterial mRNAs**  
Linda Popella, *University of Wuerzburg*

10:15-10:30 **Expanding the toolbox of chemical reactions for the generation and application of ASO conjugates**  
Laurent Knerr, *AstraZeneca*

**10:30-11:00 Refreshment Break**

<b>11:00-13:00 Session II: Pre-Clinical I (Early Stage)</b> Chair: Maja Janas De Angelis, Ph.D, <i>DABT, Alnylam Pharmaceuticals</i>
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11:00 – 11:25 **Engineered RNA Targeting Systems In Neurodegeneration**  
Gene Yeo, Ph.D., MBA, *UCSD*

11:25 - 11:50 **Preclinical Development Of Personalized ASO Therapeutics Using Patient-Derived Organoid Systems**  
Scott Younger, Ph.D., *Genomic Medicine Center, Children's Mercy Kansas City*

11:50 – 12:15 **Realizing the Promise of In Vivo CRISPR Therapies**  
Jonathan Phillips, Ph.D., *Intellia Therapeutics*

12:15 – 12:30 **Oral delivery of GalNAc-conjugated siRNAs**  
Mikyung Yu, *Alnylam*

12:30 – 12:45 **Identification of Key Determinants for siRNA Therapeutics using Quantitative Systems Pharmacology Modeling**  
Sukyung Woo, *University of Buffalo*

12:45 – 13:00 **Towards A New Generation Of Pulmonary Antisense Drugs**  
Alexey Revenko, *Ionis*

**13:00-14:30 Hosted Lunch Break**

<b>14.30-16.30 Session III: Pre-clinical II (Late Stage)</b> Chair: Marie Wikström Lindholm, Ph.D., <i>Silence Therapeutics</i>
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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 **KRRO- 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*

16:10 – 16:25 **Antisense oligo targeting of GRN regulatory RNA upregulates progranulin: a potential therapeutic approach for granulin-related frontotemporal dementia**  
Yeliz Yuva-Aydemir, *Camp4 Therapeutics*

<b>16:30-17:00</b> <b>Session IV: Presentation of Awards I</b> Chair: Richard Geary, Ph.D., <i>Ionis Pharmaceuticals</i>
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Presentation of 2024 Society Advocacy Award  
Presentation of 2024 Society Paper of the Year Award – Basic Research Category

**17:00-20:00** **Poster Session I / Reception (drinks and bites)** (Viewing of ODD numbered posters)

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## Tuesday, October 8 (Day THREE)

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

<b>08:30-10:30</b> <b>Session V: DNA/RNA Editing</b> Chair: Laura Sepp-Lorenzino, Ph.D., <i>Intellia Therapeutics</i>
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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, *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**



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**11:00-12:30 Session VI: Awards Session / Lifetime Achievement Awards**

11:00 – 11:05 Introduction by **Mano Manoharan**, Ph.D., *Alynlam*

11:05 – 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 VII: 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 Siegwart, Ph.D., *UTSW*

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**

(Viewing of EVEN numbered posters)

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## Wednesday, October 9 (Day FOUR)

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

**08:30-10:30 Session VIII: Awards Session II**

Chair: David Corey, Ph.D., *UT Southwestern*

8:30-8:45 Presentation of Awards:

- Next Gen Session: Best Talk Announcement
- Announcement of Poster Awards
- Travel Grant Awards
- Paper of the Year Awardee – Late Discovery Category

8:45-9:05 Dr. Alan M. Gewirtz Memorial Scholarship Award for Graduate Students

9:05-9:30 Mary Ann Liebert publishers, Inc. Young Investigator Award

9:30-9:50 Dr. Alan M. Gewirtz Memorial Scholarship Award for Postdocs/Jr Professionals

9:50-10:30 Q&A

**10:30-11:00 Refreshment Break**



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**11:00-13:00 Session IX: Clinical**

Chair: Art Krieg, M.D., RTI, *UMass Chan Medical School*

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*

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**

**14:30-15:45 Session X: Late Breaking Talks**

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

14:30-14:55 **Development of a Broad-Spectrum siRNA Therapy Against Human Coronaviruses**  
Julian Vogler, *LMU Munich*

14:55-15:20 **Towards a divalent siRNA therapy for prion disease**  
Juliana Gentile, *Broad Institute*

15:20-15:45 **Non-clinical development of antisense oligonucleotides and siRNAs: evaluation of the current regulatory perspective**  
Britt Duijndam, *Medicines Evaluation Board*

15:45-16:10 **Antiviral miRNAs and Their Modulators: Regulation of IFN Response and Apoptosis in Virus-Infected Cells and Potential for Nucleic Acid Therapeutics**  
Tomoko Takahashi, *Saitama University*

16:10-16:35 **Ultra-short 2'-O-Methyl-guanosine RNA fragments mediate essential natural TLR7/8 antagonism and provide a backbone for therapeutic development**  
Olivier Laczka, *Noxopharm Ltd.*

**16:35-17:00 Closing Remarks**

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

Sponsored by: CHEMGENES