20th Annual Meeting of the Oligonucleotide Therapeutics Society
October 6-9, 2024, Centre Mont-Royal, Montreal, QC, Canada
Meeting Agenda
As of July 25, 2024

Sunday, October 6 (Day ONE)

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

09:00-11:00	**Pre-conference Session - Oligonucleotide Therapeutics Education Workshop**
Chair:	Hassan Fakih, Ph.D., *Postdoctoral Associate, RNA Therapeutics Institute, UMass Chan Medical School*

09:00 – 09:05 Intro by chair

09:05 – 09:30 **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)

13:00-15:00	**Pre-conference Session Contd. - New-Gen Early Career Scientist Session**
Co-Chairs:	Eva-Maria Manz, Ph.D. Candidate, *ETH Zürich* & Jathavan Asohan, Ph.D. Candidate, *McGill University*

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*

13:30-15:00 Short Talks

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: ChemGenes

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*

09:45 – 10:30 Short Talks

10:30-11:00 Refreshment Break

11:00-13:00 Session II: Pre-Clinical I (Early Stage)
Chair: Maja Janas De Angelis, Ph,D, *DABT, Alnylam Pharmaceuticals*

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 – 13:00 Short Talks

13:00-14:30 Hosted Lunch Break

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

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.*

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 – 16:30 Q&A

16:30-17:00 Session IV: Presentation of Awards I
Chair: Richard Geary, Ph,D., *Ionis Pharmaceuticals*

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)

Tuesday, October 8 (Day THREE)

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

08:30-10:30 Session V: 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 – 10:30 Short talks

10:30-11:00 Refreshment Break

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

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

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)

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*
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 X: Late Breaking Talks**
Chair:	Laura Sepp-Lorenzino, Ph.D., *Intellia Therapeutics*

16:10-16:30 Closing Remarks

16:30 Closing of 20th Annual Meeting of the Oligonucleotide Therapeutics Society

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

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