

17th Annual Meeting of the Oligonucleotide Therapeutics Society

September 26-29, 2021

VIRTUAL

Meeting Agenda

All Times Listed in Eastern Time (ET)

As of September 25, 2021



Sunday, September 26 (Day ONE)

9.00-9.50 Networking – Virtual Coffee Meetup

10.00-12.00 Preconference Session

Oligonucleotide Therapeutics Education Workshop: Oligo Therapeutics – The Basics

Chair: Marc Lemaitre, PhD, *M_L Consult*

10.00-10.05 Welcome and Introduction

10.05-10.30 Successes and Challenges of Current Technologies for Developing Therapies for Genetic Diseases

Meena, PhD, *Stoke Therapeutics*

10.30-10.55 Method Validation Approaches for Oligonucleotides and Raw Materials

Heidi G. Heck, MS, *Nitto Denko Avecia*

10.55-11.20 Oligonucleotide Synthesis: Reminders of the Classics and Exploring the New

Masad J. Damha, PhD, FCIC, *McGill University*

11.20-11.45 Antisense Drug Development Guideposts: An Ionis View

Richard Geary, PhD, *Ionis Pharmaceuticals*

11.45-12.00 Q&A Panel

12.00-12.15 Break / Networking

12.15-14.00 Preconference Session

Next-Gen Early Career Scientist Meeting

Co-chairs: Leonora Abdullahu, *McGill University*

Aur lie Lacroix, PhD, *Sixfold Biosciences*

12.15-12.45 Session Keynote:

Chemical Biology, CRISPR, RNA Therapeutics, and More – Just Love What You Do!

Keith Gagnon, PhD, *Southern Illinois University*

12.45-13.00 Discovery And Characterization Of Spike N-terminal Domain-binding Aptamers For Rapid SARS-CoV-2 Detection

Lucy Yang, *University of Washington*

13.00-13.15 Scalable Synthesis Of DNA And RNA Oligomers Using Mechanochemistry

James Thorpe, *McGill University*

13.15-13.30 Differential Uptake Of Antisense Oligonucleotides In Mouse Hepatocytes And Macrophages Revealed By Simultaneous Two-photon Excited Fluorescence And Coherent Raman Imaging

Prabuddha Mukerjee, *University of Illinois at Urbana Champaign*

13.30-13.45 Simultaneous, Single-particle Measurements Of Size And Loading Give New Insights Into The Structure Of Drug-delivery Nanoparticles

Albert Kamanzi, PhD, *University of British Columbia*

13.45-14.00 Investigating The Impact Of Epitranscriptomic Modifications On RNA Ensembles

Hala Abou Assi, PhD, *Duke University*

Session Closing & Voting for Best Talk

14.00-15.00 Virtual Social Meetups:

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#1 General Networking Meetup Social

#2 Next-Gen Early Career Scientist Social

Monday, September 27 (Day TWO)

9.00-9.50 Networking – Virtual Coffee Meetup

10.00-10.15 Welcome & Opening Remarks

Annemieke Aartsma-Rus, PhD, *Leiden University Medical Center*

Daniel O'Reilly, PhD, *RNA Therapeutics Institute, University of Massachusetts Medical School*

10.15-11.30 Session I: Nucleic Acid Chemistry

Chair: Masad J. Damha, PhD, FCIC, *McGill University*

10.15-10.40 **Catalytic Asymmetric Synthesis of P-Containing Scaffolds with P(III) and P(V) Transfers**
Scott Miller, PhD, *Yale University*

10.40-11.05 **A New Class of Oligonucleotide Building Blocks**
Maureen McKeague, PhD, *McGill University*

11.05-11.30 **Sequence-based Design of Small Molecules Targeting RNA Structure**
Matthew Disney, PhD, *The Scripps Research Institute*

On Demand: 5' Phosphorothioate Modifications Impact siRNA Activity Profiles

Andrew Varley, BSc, MSc, *Ontario Tech University*

Backbone Hydrocarbon-constrained Nucleic Acids Modulate Hybridization Kinetics For RNA

Graeme Freestone, PhD, *Ionis Pharmaceuticals*

Aminoxy Click Chemistry (aocc): A Versatile Bioconjugation Approach

Dhrubajyoti Datta, PhD, *Alnylam Pharmaceuticals Inc.*

11.30-11.45 Break / Networking

11.45-13.45 Session II: Rare Diseases and the Role of Patient Advocates

Chair: Timothy Yu, MD, PhD, *Harvard/Boston Children's Hospital*

11.45-12.05 **n-Lorem, a Charitable Solution for Patients with Ultra-Rare Mutations**
Stanley T. Croke, MD, PhD, *n-Lorem Foundation*

12.05-12.25 **Antisense Oligonucleotide Therapies for ALS**
Robert H. Brown, MD, DPhil, *UMass Medical School*

12.25-12.45 **Preliminary Studies on Use of ASOs to Reverse Imprinting and Treat Angelman Syndrome**
Elizabeth M. Berry Kravis, MD, PhD, *Rush University Medical Center*

12.45-13.05 **Development of Lumasiran for the Treatment of Primary Hyperoxaluria Type 1**
Pushkal Garg, MD, *Alnylam Pharmaceuticals*

13.05-13.25 **The N=1 Collaborative**
Timothy Yu, MD, PhD, *Harvard/Boston Children's Hospital*

13.25-13.45 **Panel Discussion**
with Maarika Kimbrell, JD, *USFDA*

On Demand: Exon-skipping Antisense Oligonucleotide Therapy For A Patient With Nieman Pick Type C

Haiyan Zhou, MD, PhD, *University College London*

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13.45-14.00 Break / Networking

14.00-15.15 **Session III: 20th Anniversary of Mammalian RNAi**
Chair: John Maraganore, PhD, *Alnylam Pharmaceuticals*

14.00-14.25 **Intronless genes trigger Argonaute surveillance and a novel mode of co-translational silencing**

Craig Mello, PhD, *UMass Medical School*

14.25-14.50 **A Journey with the Argonautes**

Leemor Joshua-Tor, PhD, *Cold Spring Harbor Laboratory*

14.50-15.15 **Harnessing RNAi for a New Class of Medicine**

John Maraganore, PhD, *Alnylam Pharmaceuticals*

On Demand: A Genome-wide Screen Of Sirna Molecules Against Sars-cov-2

Omer Weissbrod, *Eleven TX*

Identification Of Small Interfering RNAs For Allele-selective Silencing Of Murine Von Willebrand Factor

Yvonne Jongejan, MSc, *Leiden University Medical Center*

Imaging Huntingtin Messenger RNA For Diagnosis And Therapy

Eric Wickstrom, PhD, *Thomas Jefferson University*

15.15-16.15 Virtual Social Meetups

Tuesday, September 28 (Day THREE)

9.00-9.50 **Networking – Virtual Coffee Meetup**

10.00-11.15 **Session IV: Delivery**

Chair: Annabelle Biscans, PhD, *AstraZeneca*

10.00-10.25 **Combinatorial Development of RNA Delivery Systems**

Daniel G. Anderson, PhD, *Massachusetts Institute of Technology*

10.25-10.50 **Extra-hepatic delivery of Centyrin-targeted siRNA conjugates**

Karyn T. O'Neil, PhD, *Aro Biotherapeutics*

10.50-11.15 **Novel Technologies for Enhancing Oligonucleotide Delivery in Neuromuscular Disease**

Matthew Wood, PhD, *University of Oxford*

On Demand: Unlocking the Potential of Oligonucleotide Therapeutics for Duchenne Muscular Dystrophy Through Enhanced Delivery

James McArthur, PhD, *PepGen*

Endosomal Escape Vehicle Conjugation Enhances Functional Delivery Of Oligonucleotides

Ziqing "Leo" Qian, PhD, *Entrada Therapeutics*

Improved Delivery And Therapeutic Efficacy Of Oligonucleotide Therapeutics With The Use Of Optimizer Delivery Vehicles

Edward Barnes, PhD, *Aptamer Group*

11.15-11.30 Break / Networking

11.30-12.35 **Session V: Awards Session**

Chair: Annemieke Aartsma-Rus, PhD, *Leiden University Medical Center*

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11.30-11.35 **Welcome**

Best Talk Award – NextGen Session
Paper of the Year Award Presentation

11.35-11.55 **Dr. Alan M. Gewirtz Memorial Scholarship Award for Graduate Students**
Sequence Controlled DNA-Polymer Conjugates for The Delivery of Nucleic Acid Therapeutics

Hassan Fakhri, *McGill University*

11.55-12.15 **Dr. Alan M. Gewirtz Memorial Scholarship Award for Postdoctoral Fellows and Junior Industrial Professionals**

Heteroduplex-structure Reduces CNS-toxicity Of ASO With Intrathecal Administration And Enhances Therapeutic Index In Rodents And Non-human Primates

Kotaro Yoshioka, MD, PhD, *Tokyo Medical and Dental University*

12.15-12.35 **Mary Ann Liebert publishers, Inc. Young Investigator Award**
Antisense Oligonucleotides to treat Inherited Retinal Diseases

Alex Garanto, PhD, *Radboudumc*

On Demand: Paper of the Year Award, Basic Research Category:
Investigating the pharmacodynamic durability of GalNAc-siRNA conjugates
Vasant Jadhav, PhD, *Alnylam Pharmaceuticals*

12.35-12.45 **Break / Networking**

12.45-14.00 **Session VI: Genome and RNA Editing**

Chair: Keith Gagnon, PhD, *University of Southern Illinois*

12.45-13.10 **Programmable RNA Editing: New Approaches, New Challenges**

Prashant Mali, PhD, *UCSD*

13.10-13.35 **In Vivo Epigenome Editing with CRISPR Systems**

Charles Gersbach, PhD, *Duke University*

13.35-14.00 **Prime Editing: Precise and Versatile Genome Editing without Double-Strand Breaks or Donor DNA**

Andrew Anzalone, MD, PhD, *Prime Medicine*

On Demand: CLUSTER Guide RNAs Enable Precise And Efficient RNA Editing In Cell Culture And In Vivo

Philipp Reautschnig, PhD, *University of Tübingen*

Chemically Optimized Stereopure Oligonucleotides Direct ADAR-mediated RNA Editing Of SERPINA1 Transcripts, Yielding Functional Alpha-1 Antitrypsin Protein In A Mouse Model For Alpha-1 Antitrypsin Deficiency

Prashant Monian, PhD, *Wave Life Sciences*

Iterative Optimization of a Universal Chemically Modified Anti-CRISPR-Cas9 Oligonucleotide: Toward In Vivo Proof-of-Concept

Christopher L. Barkau, *Southern Illinois University School of Medicine*

14.00-14.15 **Break / Networking**

14.15-16.45 **Session VII: Bob Letsinger, PhD – 100 Years of History**

Co-chairs: Mano Manoharan, PhD, *Alnylam Pharmaceuticals*

Masad J. Damha, PhD, FCIC, *McGill University*

14.15-14.20 **Welcome and Introduction**

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Masad J. Damha, PhD, FCIC, *McGill University*

- 14.20-14.35 Robert Letsinger: The Father of Synthetic DNA Chemistry**
Marvin H. Caruthers, PhD, *University of Colorado Boulder*
- 14.35-14.50 Bob Letsinger: Inspiration, Mentor, Motivating Genius, and Life-long Friend**
Kelvin K. Ogilvie, PhD, *Acadia University*
- 14.50-15.05 Bob Letsinger: "Well, let's see what you can do."**
Paul Miller, PhD, *Johns Hopkins Bloomberg School of Public Health*
- 15.05-15.20 Robert Lewis Letsinger: Early Years**
Margaret Schott, PhD, *Northwestern University*
- 15.20-15.35 Bob Letsinger – True Pioneer of Nucleic Acid Chemistry, Mimetics and Conjugates**
Sergei M. Gryaznov, PhD, *MAIA Biotechnology, Inc.*
- 15.35-15.50 Business With Bob: Navigating the Start-up World with an Oligonucleotide Giant**
Chad Mirkin, PhD, *Northwestern University*
- 15.50-16.05 Meetings with Bob**
Martín Egli, PhD, *Vanderbilt University*
- 16.05-16.20 Hundred Years of Bob Letsinger and Early Years of Oligonucleotide Therapeutics**
Muthiah Manoharan, PhD, *Alnylam Pharmaceuticals*
- 16.20-16.40 Family Tribute**
Reed Letsinger
- 16.40-16.45 Concluding Remarks**

16.45-17.45 Virtual Social Meetups

Wednesday, September 29 (Day FOUR)

9.00-9.50 Networking – Virtual Coffee Meetup

10.00-11.45 Session VIII: Oligonucleotide Preclinical I
Co-chairs: Shalini Andersson, PhD, *AstraZeneca*
Jimmy Weterings, PhD, *AstraZeneca*

10.00-10.05 Poster Award Winners Announcement

10.05-10.30 PCSK9 ASO Story including Phase 1 Data (Dyslipidemia/ Liver targeting)
Björn Carlsson, PhD, *AstraZeneca*

10.30-10.55 SLN360 - A Case Study for Overcoming the Challenge of a Species-restricted mRNA Target
Marie Wikström Lindholm, PhD, *Silence Therapeutics*

10.55-11.20 RNAi-based ApoE Targeting in Alzheimer's Disease: Innate Immune Response Activation for Plaque Clearance Modulation
Anastasia Khvorova, PhD, *RNA Therapeutics Institute, UMass Medical School*

11.20-11.45 ALN-APP for the Treatment of Autosomal Dominant Alzheimer's Disease
Kirk Brown, PhD, *Alnylam Pharmaceuticals*

On Demand: Guanidine-bridged Nucleic Acid Enhances Potency Of Antisense Oligonucleotides In Heart

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Tadashi Umemoto, PhD, *Luxna Biotech Co. Ltd.*

Efficient Exon 53 Skipping Of The Human Dystrophin Transcript In A Mouse Model For Duchenne Muscular Dystrophy

Sarah Engelbeen, *Leiden University Medical Center*

Structure-based Optimization Of Lipophilic siRNA Conjugates For Albumin Binding Yields Improved Pharmacokinetics And Carrier-free Tumor Gene Silencing

Ella Hoogenboezem, *Vanderbilt University*

DUET-01: Bispecific Oligonucleotide Targeting TLR9 and STAT3 Signaling for B Cell Lymphoma Immunotherapy

Alan Horsager, PhD, *Duet Therapeutics*

11.45-12.00 **Break / Networking**

12.00-13.15 **Session IX: Oligonucleotide Preclinical II**

Co-chairs: Holly Kordasiewicz, PhD, *Ionis Pharmaceuticals*

Bruno Godinho, PhD, *Atalanta Therapeutics*

12.00-12.25 **SSRI-conjugated oligonucleotides for the treatment of major depressive disorder and Parkinson's disease**

Analia Bortolozzi Biassoni, PhD, *Institute of Biomedical Research of Barcelona*

12.25-12.50 **High-resolution visualization of nucleic acid-based therapeutics in cells and tissues using NanoSIMS**

Haibo Jiang, PhD, *The University of Hong Kong/The University of Western Australia*

12.50-13.15 **Using Nuclear Imaging to Understand Delivery and Distribution of Intrathecal ASOs in Humans**

Jenna Sullivan, PhD, *Biogen*

On Demand: Stealth Fluorescence Labeling For Live Microscopy Imaging Of Nucleic Acid Therapeutics

Tom Baladi, PhD, *AstraZeneca*

Targeting 3' And 5' Untranslated Regions With Antisense Oligonucleotides To Stabilize Frataxin mRNA And Increase Protein Expression

Marek Napierala, *UAB*

Systemically Administered DNA/RNA Heteroduplex Oligonucleotides Achieve Blood To Brain Delivery And Efficient Gene Knockdown In The CNS

Takanori Yokota, MD, PhD, *Tokyo Medical and Dental University*

13.15-13.30 **Break / Networking**

13.30-15.10 **Session X: Oligonucleotide Clinical Studies**

Co-chairs: Martin Maier, PhD, *Alnylam Pharmaceuticals*

Richard Geary, PhD, *Ionis Pharmaceuticals*

13.30-13.55 **Development of the Moderna COVID-19 Vaccine**

Randy Hyer, MD, PhD, MPH, *Moderna*

13.55-14.20 **Overview on Pulmonary Program IONIS-ENAC-2.5Rx**

Ken Newman, MD, *Ionis Pharmaceuticals*

14.20-14.45 **Harnessing the Promise of Liver-Directed CRISPR Gene Editing for Rare Diseases**

Laura Sepp-Lorenzino, PhD, *Intellia Therapeutics*

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14.45-15.10 Dose-related and prolonged reductions in blood pressure with a RNAi therapeutic targeting angiotensinogen in hypertensive patients: Interim results from a Phase 1 study with ALN-AGT01

Stephen Huang, MD, *Anylam Pharmaceuticals*

On Demand: Evaluation Of DMD Transcripts Imbalance Pre And Post Golodirsen Treatment In Myod-converted Fibroblasts From 4053-101 Clinical Trial Patients

Rachele Rossi, PhD, *UCL*

15.10-15.20 Closing Remarks

David Corey, PhD, *Incoming OTS President*

Daniel O'Reilly, PhD, *Organizing Co-Chair*

15.30-17.00 Annual Meeting Wrap Party

Sponsored by ChemGenes

Grab a refreshment and snacks and join us for a virtual party you will LOVE. We'll reveal the winners of the Talent Show, laugh together at [comedian Don McMillian](#) (back in 2021 by popular demand), followed by [BarNone Trivia](#) a live team trivia game! Prizes will be awarded to the winning teams. Don't miss out!

A big shout out to [ChemGenes](#), our party sponsor - they continue to champion the OTS Annual Meeting and the Wrap Party for 2021.