



# 16<sup>th</sup> Annual Meeting of the Oligonucleotide Therapeutics Society

September 27-30, 2020

Virtual Online Meeting

## Agenda

**All times listed in ET (Eastern Time)**

*As of September 25, 2020*

### Sunday, September 27 (Day ONE)

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

**10.00-12.00 Preconference Session**  
**Oligonucleotide Therapeutics Education Workshop:**  
**Nucleic Acid Therapeutics Development: How To...**  
Chair: Marc Lemaitre, PhD, *M\_L Consult*

**10.00-10.05 Welcome and Introduction**

**10.05-10.30 The ADME of siRNA GalNAc Conjugates**  
Chris MacLauchlin, PhD, *Alnylam Pharmaceuticals*

**10.30-10.55 CMC of Oligonucleotides: Categorization of Impurities**  
Thomas Rupp, *Thomas Rupp Consulting UG*

**10.55-11.20 Nonclinical Safety Evaluation Strategies for Antisense Oligonucleotide Therapeutics**  
Cathaline den Besten, PhD, *ProQR Therapeutics*

**11.20-11.45 Control of Impurities in Double Stranded siRNA Therapeutics**  
Adel Far, PhD, *Novartis Pharmaceuticals Canada*

**11.45-12.00 Q&A Panel**

**12.00-12.15 Break / Networking (15m)**

**12.15-12.50 Preconference Session**  
**Next-Gen Early Career Scientist Meeting**  
Co-chairs: Erin McConnell, PhD, *McMaster University*  
Joseph Ochaba, PhD, *Ionis Pharmaceuticals*

**12.15-12.20 Welcome & Introduction**

**12.20-12.50 Session Keynote:**  
**Peptides and Engineered EVs for Delivery of Biotherapeutics**  
Samir EL Andaloussi, PhD, *Karolinska Institut*

**On Demand Short Talks with Voting for Best Talk:**

**Therapeutic potential of antagomiR-23b for treating Myotonic Dystrophy**  
Beatriz Llamusi, PhD, *Arthex Biotech SL*

**Antisense oligonucleotide-mediated knockdown of mitochondrial fusion and fission factors modulates mitochondrial morphology and rescues mitochondrial dysfunction**  
Daniel Garcia, PhD, *Ionis Pharmaceuticals*

**Design and Enhanced Gene Silencing Activity of Spherical 2'-Fluoroarabinose Nucleic Acids (FANA-SNAs)**  
Hassan Fakih, PhD Student, *McGill University*

**Next Generation Small Nucleic Acid-Based Inhibitors of CRISPR-Cas9**  
Chris Barkau, BSc, *Southern Illinois University School of Medicine*

**Splice-Switching Antisense Oligonucleotides for the Treatment of Cystic Fibrosis**  
Wren E. Michaels, *Center for Genetic Diseases, Chicago Medical School, Rosalind Franklin University of Medicine and Science*



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### **Antisense oligonucleotide-mediated exon skipping for therapeutic reading frame correction in CLN3 Batten disease**

Jessica Centa, *Center for Genetic Diseases, Chicago Medical School, Rosalind Franklin University of Medicine and Science*

#### **13.00-14.30 Virtual Social Meetups:**

**#1 Early Career Scientist Social**  
*Sponsored by ChemGenes*

**#2 General Networking Meetup Social**

#### **Monday, September 28 (Day TWO)**

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

**10.00-11.00 Meeting Opening & Keynote Session**

#### **10.00-10.10 Welcome & Opening Remarks**

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

Jonathan Watts, PhD, *RNA Therapeutics Institute, University of Massachusetts Medical School*

#### **10.10-11.00 Keynote Presentation:**

**40 min talk/10min Knocking Down Disease – RNAi Therapeutics**

**Q&A What We Have Accomplished and Where We Need To Go**

Judy Lieberman, MD, PhD, *Harvard University & Boston Children's Hospital*

**11.00-11.15 Break / Networking (15m)**

#### **11.15-12.30 Session I: Nucleic Acid Chemistry**

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

#### **11.15-11.40 New Artificial Biomolecules For Oligonucleotide Therapeutics**

Takeshi Wada, PhD, *Tokyo University of Science*

#### **11.40-12.05 Generation of Nucleobase-modified Functional Nucleic Acids for Biosensing and Therapeutic Applications**

Marcel Hollenstein, PhD, *Institut Pasteur*

#### **12.05-12.30 Controlled Template-Independent Enzymatic Synthesis of RNA Oligonucleotides**

Jonathan Rittichier, PhD, *Harvard*

#### **On Demand Short Talks:**

#### **Small molecule-like oligonucleotides as novel antifungal reagents against tRNA 2'-phosphotransferase (Tpt1)**

Leonora Abdullahu, *McGill University*

#### **Fluorinated therapeutic agents to combat ALS**

Miguel Garavís, PhD, *Instituto de Química-Física Rocasolano, IQFR-CSIC*

#### **2'-O-methylation redistributes the RNA secondary structural ensemble**

Hala Abou Assi, PhD, *Duke University*

**12.30-12.45 Break / Networking (15m)**

#### **12.45-14.00 Session II: Delivery: New Tissues and Technologies**

Chair: Laura Sepp-Lorenzino, PhD, *Intellia Therapeutics*



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### 12.45-13.10 Recent Progress with Antibody Oligonucleotide Conjugates

Art Levin, PhD, *Avidity BioSciences*

### 13.10-13.35 Selective Organ Targeting (SORT): A Strategy for Effective Tissue Specific mRNA Delivery and CRISPR/Cas Gene Editing

Daniel Siegwart, PhD, *UT Southwestern*

### 13.35-14.00 Vaccinating Against Neoantigens Induced in Tumors and Pathogen Infected Cells

Eli Gilboa, PhD, *University of Miami, Miller School of Medicine*

### On Demand **Short Talks:**

**Bioengineering bacterial outer membrane vesicles as delivery systems for RNA therapeutics targeted to lung epithelial cytosols**

Elena Eftychiou, *Northumbria University*

**The morphology and formation of lipid nanoparticles entrapping nucleic acids**

Jayesh Kulkarni, PhD, *University of British Columbia*

### 14.00-14.15 Break / Networking Zoom Rooms (15m)

### 14.15-15.05 Session III: Cellular Delivery and Endosomal Escape

Chair: Steve Dowdy, PhD, *UCSD School of Medicine*

### 14.15-14.40 An Intracellular Depot Supports the Extended Duration of GalNAc-siRNA Conjugates

Christopher Brown, PhD, *Alnylam Pharmaceuticals*

### 14.40-15.05 Design of Lipid Nanoparticle (LNP) Systems to Enable Gene Therapies

Pieter Cullis, PhD, FRSC, *The University of British Columbia*

### On Demand **Short Talks:**

**Efficient, targeted cytoplasmic delivery of oligonucleotides via Sapreme's endosomal escape enhancers**

Guy Hermans, PhD, *Sapreme Technologies BV*

**Overhanging duplex oligonucleotides for enhanced systemic delivery**

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

**Improved Functional Delivery of RNA therapeutics in the Eye Using Fatty-Acid Conjugation**

Charles Allerson, PhD, *DTx Pharma, Inc.*

**Visualizing endosomal escape of ligand-conjugated siRNA**

Hampus Du Rietz, *Department of Clinical Sciences, Oncology, Faculty of Medicine, Lund University*

### 15.15-16.45 Virtual Social Meetup

## Tuesday, September 29 (Day THREE)

### 9.00-9.50 Networking Meetup – Virtual Coffee Talk

### 10.00-10.50 Session IV: Genome Editing

Chair: Ross Wilson, PhD, *University of California, Berkeley*

### 10.00-10.25 A Modular CRISPR/Cas9 Genome Editing Platform for Durable Therapeutic Knockout and Targeted Gene Insertion Application

Anthony Forget, PhD, *Intellia Therapeutics*



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**10.25-10.50 Systemic Delivery of PNA Nanoparticles to Correct F508del CFTR *In Vivo***

Alexandra Piotrowski-Daspi, PhD, *Yale University*

**On Demand Short Talks:**

**Development of receptor-targeted nanocomplexes for in vivo delivery of CRISPR/Cas9 as a potential therapy for Cystic Fibrosis**

Amy Walker, PhD, *UCL GOS Institute of Child Health*

**5' Modifications Improve Efficacy of DNA Donors for Precision Genome Editing**

Zexiang Chen, *RNA Therapeutics Institute, University of Massachusetts Medical School*

**Gene Editing and Cis-Trans Activity Effects with Heavily Modified Cas12a 5' Pseudoknots**

Ramadevi Chilamkurthy, *Department of Biochemistry and Molecular Biology, School of Medicine, Southern Illinois University*

**10.50-11.00 Break / Networking (10m)**

**11.00-12.15 Session V: Big Data, AI and Machine Learning in Oligonucleotide Therapeutic Development**

Chair: Peter Hagedorn, PhD, *Roche*

**11.00-11.25 Fireside Chat with Brendan Frey, CEO & Founder of Deep Genomics**

Brendan Frey, PhD, FRSC, *Deep Genomics*

**11.25-11.50 High-throughput Analysis Reveals Rules for Target Binding by AGO1–4 RISCs**

Karina Jouravleva, PhD, *University of Massachusetts Medical School*

**11.50-12.15 Acute Neurotoxicity of Antisense Oligonucleotides After Intracerebroventricular Injection into Mouse Brain Can Be Predicted from Sequence Features**

Peter Hagedorn, PhD, *Roche*

**12.15-12.30 Break / Networking (15m)**

**12.30-13.40 Session VI: Awards Presentations & Talks**

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

**12.30-12.40 Welcome & Award Announcements**

Next-Gen Early Career Scientist Session - Best Talk Award

Paper of the Year Awards

Lifetime Achievement Award Announcement

**12.40-13.00 Dr. Alan M. Gewirtz Memorial Scholarship Award for Graduate Students**

**RNAi modulation of APOE as a potential therapeutic for Alzheimer Disease**

Chantal M. Ferguson, *RNA Therapeutics Institute, University of Massachusetts Medical School*

**13.00-13.20 Dr. Alan M. Gewirtz Memorial Scholarship Award for Postdoctoral Fellows and Junior Industrial Professionals**

**Designing New Chemically Engineered siRNAs for Extrahepatic Delivery**

Annabelle Biscans, PhD, *AstraZeneca*

**13.20-13.40 Mary Ann Liebert publishers, Inc. Young Investigator Award**

**Advancing the Safety and Reach of RNAi Therapeutics**

Maja M. Janas De Angelis, PhD, *Alnylam Pharmaceuticals, Inc.*

**On Demand Short Talks:**

***Paper of the Year Award - Basic***



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**Antisense-mediated transcript knockdown triggers premature transcription termination**

Jong-Sun Lee, PhD, *UT Southwestern*

**Paper of the Year Award – Late Discovery**

**Lowering PCSK9 through siRNA based therapeutics: A revolution in the management of a common disease**

Frederick J. Raal, PhD, *University Of The Witwatersrand, Johannesburg*

Kausik K Ray, MD, MPhil, *Dept of Primary Care & Public Health Imperial Clinical Trials Unit, UK*

**13.40-14.00 Break / Networking (20m)**

**14.00-15.15 Session VII: Early Stage Technology & Novel Mechanisms**

Chair: Isabel Aznarez, PhD, *Stoke Therapeutics*

**14.00-14.25 Understanding the function of RNA-binding proteins by integration of in vitro and vivo data**

Christopher B. Burge, PhD, *Massachusetts Institute of Technology*

**14.25-14.50 Counteracting the Chromatin Effects of a Splicing-correcting ASO Enhances Its Therapeutic Efficacy in SMA Mice**

Adrian R. Krainer, PhD, *Cold Spring Harbor Laboratory*

**14.50-15.15 Antisense Approaches for the Treatment of Cystic Fibrosis**

Shuling Guo, PhD, *Ionis Pharmaceuticals*

**On Demand Short Talks:**

**Rational Design of Aptamer Switches with Tunable pH Response**

Ian Thompson, BSc, *Stanford University*

**Looping in phase separation: Controlling RNA:DNA hybrids during transcription**

Jacob Schwartz, PhD, *University of Arizona*

**15.15-16.45 Networking Social Meetup**

**Wednesday, September 30 (Day FOUR)**

**9.00-9.50 Networking Meetup Session – “Coffee Talk”**

**10.00-11.00 Session VIII: Turning RNA Against an RNA Virus: Oligonucleotide Therapeutics in the Fight Against COVID-19**

Chair: Jonathan Watts, PhD, *RNA Therapeutics Institute, University of Massachusetts Medical School*

**10.00-10.20 ALN-COV: an Investigational RNAi Therapeutic for COVID-19**

Akin Akinc, PhD, *Alnylam Pharmaceuticals*

**10.20-10.40 Antisense oligonucleotides as therapeutic agents for SARS-CoV-2**

Lisa Henderson, PhD, *NINDS*

**10.40-11.00 Fighting SARS-CoV-2 with systemically and locally delivered oligonucleotide cocktails**

Jonathan Watts, PhD, *RNA Therapeutics Institute, University of Massachusetts Medical School*

**11.00-11.15 Break / Networking (15m)**

**11.15-12.30 Session IX: Preclinical Results**

Chair: Martin Maier, PhD, *Alnylam Pharmaceuticals*

**11.15-11.40 Preclinical Development of Antisense Oligonucleotides for Inherited Retinal Diseases**

Jim Swildens, PhD, *ProQR Therapeutics*



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**11.40-12.05 mRNA Chemistry and Manufacturing Process Modulates Innate Immune Activation**

John Joyal, PhD, *Moderna*

**12.05-12.30 Preclinical Efficacy of a Precision Medicine Approach Using a TANGO ASO in the Dravet Mouse Model**

Anne Christiansen, PhD, *Stoke Therapeutics*

**On Demand Short Talks:**

**ASO-mediated selective inhibition of DNA damage response activation at telomeres improves aging phenotypes and extends lifespan in different animal models and species**

Fabrizio d'Adda di Fagagna, *IFOM – the FIRC Institute for Molecular Oncology Foundation*; *IGM-CNR – Istituto di Genetica Molecolare-Consiglio Nazionale delle Ricerche*

**Next Generation miR-29 Mimic as a Therapy for Pulmonary Fibrosis**

Rusty Montgomery, PhD, *miRagen Therapeutics, Inc.*

**Stereopure oligonucleotides support efficient ADAR-mediated RNA editing in non-human primates**

Chikdu Shivalila, PhD, *Wave Life Sciences*

**12.30-12.45 Break/ Networking (15m)**

**12.45-14.15 Session X: N=1 and Rare Diseases / Patients as Investigators**

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

**12.45-12.55 Facilitating The Development Of Oligonucleotide Therapeutics For N-Of-1+ Diseases**

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

**12.55-13.20 ASOs For Primary Prevention of Prion Disease: A Patient-led Initiative**

Sonia Vallabh, PhD and Eric Minikel, PhD, *The Broad Institute*

**13.20-13.45 Models and Mechanisms of FUS-ALS: In Vivo Studies in Mouse and Man**

Neil A. Shneider, MD, PhD, *Columbia University*

**13.45-14.15 Early Forays into Individualized Therapeutics: Ethical Considerations**

Alison Bateman-House, PhD, MPH, MA, *Grossman School of Medicine, NYU Langone Health*

**On Demand Short Talk:**

**Patient-Specific Antisense Oligonucleotide Therapeutic Development for a Girl with Mucopolipidosis**

Margot Cousin, PhD, *Mayo Clinic*

**14.15-14.30 Break / Networking (15m)**

**14.30-16.10 Session XI: Oligonucleotide Clinical Studies**

Co-Chairs: Arthur M. Krieg, MD, *Checkmate Pharmaceuticals*

James Hamilton, MD, MBA, BS, *Arrowhead Pharmaceuticals*

**14.30-14.50 Antisense Therapies for Lipid Disorders**

Sam Tsimikas, MD, FACC, FAHA, FSCAI, *Ionis Pharmaceuticals*

**14.50-15.10 Human Translation of GalNAc-siRNA Conjugates with Improved Specificity**

Vasant Jadhav, PhD, *Alnylam Pharmaceuticals*

**15.10-15.30 VIR-2218, an X-targeted siRNA for the Functional Cure of HBV**

Phillip S Pang, MD, PhD, *Vir Biotechnology, Inc.*



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**15.30-15.50 Chronic Liver-Specific LDHA Suppression in the Treatment of Primary Hyperoxaluria: Evaluating the Safety and Efficacy of the Investigational Therapy, Nedosiran**

Kerry S. Russell, MD, PhD, *Dicerna* and Bob Brown, PhD, *Dicerna*

**15.50-16.10 TRiM Platform Derived siRNA Therapeutics: Targeting Hepatocytes and Beyond**

James Hamilton, MD, MBA, BS, *Arrowhead Pharmaceuticals*

**16.10-16.20 Closing Remarks**

**16.30-18.00 Annual Meeting Wrap Party – Virtual Edition**

***sponsored by ChemGenes***

A big shout out to ChemGenes, our party sponsor! They continue to champion the OTS Annual Meeting and the Wrap Party. So be sure to grab a refreshment and snacks (on you this year!) and join us on Zoom for a virtual party you will LOVE. We'll be laughing together, puzzling together and.....well, we can't share every detail. Don't miss out!!