



18th Annual Meeting of the Oligonucleotide Therapeutics Society

October 2-5, 2022

Hilton Phoenix Resort at the Peak

Phoenix, Arizona, USA

Meeting Agenda

As of September 29, 2022

ALL TIMES LISTED IN MOUNTAIN STANDARD TIME

Sunday, October 2 (Day ONE)

8.00 Registration Opens / Welcome Coffee

9.00-11.00 Preconference Session: Educational Workshop

Nucleic Acid Therapeutics – The Basics

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

9.00-9.30 Oligonucleotides 101

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

9.30-9.55 mRNA Manufacturing and Analytics: An Overview of Current Methods

Ramsey Majzoub, PhD, *Intellia Therapeutics, Inc.*

9.55-10.20 Beyond Double Strand Breaks: Base Editing, Prime Editing and RNA Editing

Yi Yu, PhD, *Beam Therapeutics*

10.20-10.45 Toxicology and DMPK Assessment of siRNA Therapeutics

Joseph Dybowski, MPH, PhD, *Alnylam Pharmaceuticals*

10.45-11.00 Q&A Panel

11.00-13.00 Lunch Break

Lunch on Own

13.00-15.30 Preconference Session:

Next-Gen Early Career Scientist Meeting

Co-chairs:

Hassan Fakih, PhD, *RTI, UMass Chan Medical School*

Chantal Ferguson, MD/PhD Candidate, *RTI, UMass Chan Medical School*

13.00-13.05 Welcome & Introduction

13.05-13.35 Session Keynote Speaker:

Oligonucleotide Backbone Structure Modulation - A Beginning of my Journey in RNA Therapeutics World

Ken Yamada, PhD, *RNA Therapeutics Institute, UMass Chan Medical School*

13.35-14.05 Session Keynote Speaker:

Building a Biotech From Scratch

Anna Perdrix Rosell, PhD, *Sixfold Biosciences*

14.05-14.20 RIG-I adjuvanted mRNA vaccines

Christine Wuebben, PhD, *Institute of Clinical Chemistry and Clinical Pharmacology, University of Bonn*

14.20-14.35 Enhancing Delivery Efficiency of RNA Based Therapeutics via Repurposing of Cationic Amphiphilic Drugs

Cristina Muntean, *Ghent University*

14.35-14.50 Investigating consequences of nuclear AGO2 in colon cancer model systems

Krystal Johnson, *UT Southwestern Medical Center*

14.50-15.05 Origins of the increased affinity of phosphorothioate modified therapeutic nucleic acids for proteins



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Malwina Hyjek-Skladanowska, PhD, *International Institute of Molecular and Cell Biology*

15.05-15.20 Di-valent siRNA Mediated Silencing of MSH3 Blocks Somatic Repeat Expansion in Mouse Models of Huntington's Disease

Daniel O'Reilly, PhD, RNA Therapeutics Institute, *UMass Chan Medical School*

15.20-15.30 Voting for Best Talk

**15.30-17.30 Welcome Reception with Exhibitors
Open Poster Viewing**

17.30 Dinner On Own

**19.00-21.30 Early Career Scientist Social Event
Sponsored by ChemGenes**

Location: [Culinary Dropout](#)

5632 N 7th St, Phoenix, AZ 85014

Monday, October 3 (Day TWO)

7.30-8.30 Breakfast Served – Registration Open

8.40-9.00 Welcome & Opening Remarks

David Corey, PhD, *OTS President*

Keith T. Gagnon, PhD, *Meeting Co-Chair*

9.00-11.00 Session I: Nucleic Acid Chemistry - Dedicated to 100 Years of Gobind Khorana

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

9.00-9.25 Living in the World of RNA Therapeutics through Chemistry

Muthiah Manoharan, PhD, *Alnylam Pharmaceuticals*

9.25-9.50 Renaissance of P(V) Oligonucleotide Chemistry

Phil Baran, PhD, *Scripps Research Institute*

9.50-10.15 Photoresponsive Nucleic Acid Conjugates for RNA interference

Jean Paul Desaulniers, PhD, *Ontario Tech University*

10.15-10.30 Photocaged 5' cap analogs for optical control of mRNA translation in cells

Florian Peter Weissenböck, PhD, *Institute of Biochemistry, University of Münster*

10.30-10.45 Understanding Guide RNA 2'-Hydroxyl Ribose Requirements for CRISPR-Cas9 Gene Editing

Adrian A. Pater, *Southern Illinois University*

10.45-11.00 Reimagining the chemical space for nucleic acid therapeutics – Antisense oligonucleotides modified with stereoisomers of DNA

Graeme C. Freestone, PhD, *Ionis Pharmaceuticals*

11.00-11.30 Refreshment Break

11.30-13.00 Session II: Awards Session

Chair: David Corey, PhD, *UT Southwestern*

11.30-11.40 Award Presentations:

Best Talk Award – NextGen Session

Paper of the Year Awards



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Science Outreach and Patient Advocacy Awards

11.40-12.00 Dr. Alan M. Gewirtz Memorial Scholarship Award for Graduate Students
A Tale of Two Aptamers: SARS-CoV-2 binding DNA aptamers for diagnostics and therapeutics

Lucy F. Yang, PhD, *University of Washington*

12.00-13.00 OTS Lifetime Achievement Award 2020
From Thalassemia to Duchenne Muscular Dystrophy
Ryszard Kole, PhD, *University of North Carolina, Chapel Hill*

13.00-14.30 Lunch Break
Hosted lunch

14.30-16.00 Session III, Keynote: 20th Anniversary of OTS
Co-Chairs: Annemieke Aartsma-Rus, PhD, *LUMC*
Keith T. Gagnon, PhD, *Southern Illinois University*

Join us for this special session which will include founders of the OTS and past Board of Directors members who were there during the 'early days' of the society. Panelists include:

-Masad Damha, PhD, *McGill University*

-Gunther Hartmann, MD, PhD, *University Hospital Bonn*

-Anastasia Khvorova, PhD, *RTI, UMass Chan Medical School*

-Art Krieg, MD, *RTI, UMass Chan Medical School*

-Mano Manoharan, PhD, *Alnylam Pharmaceuticals*

-Brett Monia, PhD, *Ionis Pharmaceuticals*

-John Rossi, PhD, *City of Hope*

-Georg Sczakiel, PhD, *University of Luebeck*

-Hermona Soreq, PhD, *The Hebrew University of Jerusalem*

-Cy Stein, MD, PhD, *Retired*

16.00-18.30 Poster Session I with Lite Bites
ODD numbered posters hosted

Dinner On Own

Tuesday, October 4 (Day THREE)

7.30-8.30 Breakfast Served – Registration Open

8.30-10.30 Session IV: Preclinical
Co-chairs: Martin Maier, PhD, *Alnylam Pharmaceuticals*
Aurélien Goyenvalle, PhD, *DR Inserm, UMR 1179 INSERM-UVSQ*

8.30-8.55 Phosphoryl-guanidine backbone chemistry: understanding its impact on stereopure oligonucleotides

Chandra Vargeese, PhD, *Wave Life Sciences*

8.55-9.20 Gene Silencing in the Lung by Locally Delivered ASOs and siRNAs

Jonathan Watts, PhD, *RNA Therapeutics Institute, UMass Chan Medical School*

9.20-9.45 Centyrin-targeted Gys1 siRNA conjugates: A novel therapeutic modality for the treatment of Pompe disease

Karyn O'Neil, PhD, *Aro Biotherapeutics*



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9.45-10.00 Towards clinical application of antisense oligonucleotides for the treatment of Stargardt disease

Dyah Karjosukarso, PhD, *Dept. of Human Genetics, Radboud University Medical Center*

10.00-10.15 Antisense-oligonucleotide therapy targeting BRAF alleviates pediatric brain tumors with intractable epilepsy

Jeong Ho Lee, MD, PhD, *KAIST*

10.15-10.30 Therapeutic Efficacy of SNCA-targeting siRNAs in a Synucleinopathy Mouse Model

Pascaline Aimé-Wilson, PhD, *Regeneron Pharmaceuticals*

10.30-11.00 Refreshment Break

11.00-13.00 Session V: AI/Machine Learning

Co-chairs: Marie Wikström Lindholm, PhD, *Silence Therapeutics*

John Whittaker, PhD, *University of Cambridge*

11.00-11.15 Session Introduction: What's AI/ML Got to do With Oligonucleotide Therapeutics?

John Whittaker, PhD, *University of Cambridge*

11.15-11.40 The Power and Pitfalls of Machine Learning in Early Stage Drug Discovery

Charlotte Deane, PhD, *University of Oxford / Exscientia*

11.40-12.05 Human Genetics as an Enabler of RNAi Therapeutics

Paul Nioi, PhD, *Alnylam Pharmaceuticals*

12.05-12.30 eSkip-Finder: A Machine Learning-based Web Application and Database to Identify Efficient Antisense Oligonucleotide Sequences for Exon Skipping

Toshifumi Yokota, PhD, *University of Alberta*

12.30-12.45 Generative aptamer discovery using RaptGen

Tatsuo Adachi, PhD, *RIBOMIC Inc.*

12.45-13.00 RN.AI.Visions

Lykke Pedersen, PhD, *Abzu*

Morten Lindow, PhD, *Roche Innovation Center Copenhagen*

13.00-14.30 Hosted Lunch Break

14.30-16.30 FREE TIME

Optional: Early Career Scientist Meetup at the Lazy River

16.30-19.00 Poster Session II with Lite Bites

EVEN numbered posters hosted

19.00 Dinner On Own

Wednesday, October 5 (Day FOUR)

7.30-8.30 Breakfast Served – Registration Open

8.30-10.30 Session VI: Genome & RNA Editing

Co-Chairs: Laura Sepp-Lorenzino, PhD, *Intellia Therapeutics*

Patrick Finn, Ph.D., *Moderna*



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8.30-8.55 mRNA as Medicine

Patrick Finn, Ph.D., PhD, *Moderna*

8.55-9.20 Harnessing mobile genetic elements to write DNA sequences with RNA

Cecilia Cotta-Ramusino, PhD, *Tessera Therapeutics*

9.20-9.45 Special motif-carrying tRNA fragments cause Parkinson's disease neuronal death by translational arrest

Hermona Soreq, PhD, *The Hebrew University of Jerusalem*

9.45-10.00 Optimization strategies to achieve high efficiency gene editing in non-human primates using lipid nanoparticle delivery of mRNA

Cassie Gorsuch, PhD, *Precision BioSciences Inc.*

10.00-10.15 RNA base editing for the treatment of Alpha-1 antitrypsin deficiency

Prashant Monian, PhD, *Wave Life Sciences*

10.15-10.30 Evolution of Functional CRISPR Guide RNA Yields Variants with Distinct Activities

Korie Bush, *Duke University*

10.30-11.00 Refreshment Break

11.00-12.45 Session VII: Awards Session

Chair: David Corey, PhD, *UT Southwestern*

11.00-11.05 Announcement: Poster Award Winners

**11.05-11.25 Dr. Alan M. Gewirtz Memorial Scholarship Award for Postdocs
Optimizing ASO delivery in hiPSC-derived 2D and 3D neuronal models**

Ronald Buijsen, PhD, *Leiden University Medical Center*

**11.25-11.45 Mary Ann Liebert publishers, Inc. Young Investigator Award
Small circular mRNA vaccines**

Guizhi (Julian) Zhu, PhD, *Virginia Commonwealth University*

**11.45-12.45 OTS Lifetime Achievement Award 2021
30 Years of Making Sense Out of Antisense**

Frank Bennett, PhD, *Ionis Pharmaceuticals*

12.45-14.15 Lunch

14.15-16.15 Session VIII: Clinical

Co-Chairs: Art M. Krieg, MD, *RTI, UMass Chan Medical School*

Frank Bennett, PhD, *Ionis Pharmaceuticals*

14.15-14.35 Lessons Learned from ALS Clinical Trials

Toby Ferguson, MD, PhD, *Biogen*

14.35-14.55 Antisense oligonucleotide therapy to pre-kallikrein in the treatment of patients with hereditary angioedema (HAE)

Richard Geary, PhD, *Ionis Pharmaceuticals*

14.55-15.15 Phase 3 study, HELIOS-A, in hATTR patients evaluating a single dose regimen

Gabriel J. Robbie, *Alnylam Pharmaceuticals*

15.15-15.35 Clinical Advances in CRISPR Cas9 Therapeutics: In Vivo and Cell Therapy Applications



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Laura Sepp-Lorenzino, PhD, *Intellia Therapeutics*

15.35-15.55 SLN360 and Controlling Lp(a), The New Kid on the CVD Block

Curtis Rambaran, MD, *Silence Therapeutics*

15.55-16.15 Industrializing the non-profit discovery, development, and treatment for nano-rare patients

Tracy Cole, PhD, *n-Lorem Foundation*

16.15-16.30 Closing Remarks

**18.00-22.00 Annual Meeting Wrap Party - Poolside at the Hilton
sponsored by ChemGenes**

separate RSVP & Ticket required

Join friends and colleagues to celebrate the wrap of this year's annual meeting. Come prepared to splash in the pool or take a ride down the waterslide to cool off from the desert heat.

Dinner, drinks (2), and of course dancing included.