



**10th Annual Meeting of the
Oligonucleotide Therapeutics Society
October 12 – 15, 2014 ~ San Diego, CA
Meeting Agenda**

Updated: October 12, 2014

Sunday, October 12th

1:00pm Arrival and Early Registration

4:00pm-5:00pm Keynote Presentation:
Gene silencing therapy for neurodegenerative diseases
Don W. Cleveland, PhD, *University of California, San Diego*

5:00pm-7:00pm Welcome Reception with Exhibitors & Poster Session I
ODD numbered posters hosted

Monday, October 13th

7:30am-8:30am Breakfast

8:30am Welcome & Opening Remarks
OTS President: Masad J. Damha, PhD, McGill University

8:45am-10:30am Session I: Aptamers
*Co-Chairs: Bruce Sullenger, PhD, Duke University Medical Center
Matthew Levy, PhD, Albert Einstein College of Medicine*

8:45am-9:15am Biomarker discovery using DNA aptamers
Weihong Tan, PhD, University of Florida, Hunan University

9:15am-9:45am Immunostimulatory CpG-siRNA conjugates: from intracellular processing to antitumor activity
Marcin Kortylewski, PhD, Beckman Research Institute at City of Hope National Medical Center

9:45am-10:15am Therapeutic journey in retinal disease. Enhancing efficacy by targeting aptamers to VEGF, PDGF and Complement Pathways
Samir Patel, MD, Ophthotech Corporation

10:15am-10:30am Agonistic CD200R1 DNA Aptamers are Potent Immunosuppressants In Vitro and In Vivo and Prolong Allogeneic Skin Graft Survival
Jean Gariépy, PhD, University of Toronto

10:30am-11:00am Refreshment Break

11:00am-1:00pm Session II: Immunomodulatory Oligonucleotides
Chair: Gunther Hartmann, MD, PhD, University Hospital Bonn, Germany

11:00am-11:30am Structural Basis for Viral RNA Recognition by Human IFIT Proteins
Bhushan Nagar, PhD, McGill University

11:30am-12:00pm Intracellular DNA sensing
Veit Hornung, MD, University Hospital, University of Bonn

12:00pm-12:30pm RNA sensing by innate immune receptors
Stefan Bauer, PhD, Philipps-Universität Marburg

12:30pm-12:45pm Spherical Nucleic Acids (SNAs): A New Class of ImmunoModulatory Agents
Sergei M. Gryaznov, PhD, AuraSense Therapeutics

12:45pm-1:00pm EnanDIM: A new class of enantiomeric oligodeoxynucleotides for TLR-9 activation
Kerstin Kapp, PhD, MOLOGEN AG

1:00pm-2:30pm Lunch Break



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2:30pm-4:30pm Session III: RNA Modulation I

*Co-Chairs: Rachel Meyers, PhD, Alnylam Pharmaceuticals
Gunter Meister, PhD, University of Regensburg*

2:30pm-3:00pm The Neisseria meningitidis CRISPR-CAS9 System for Genome Editing

Erik J. Sontheimer, PhD, University of Massachusetts Medical School

3:00pm-3:30pm Unexpected insights and opportunities linking RNA biology and metabolism

Matthias W. Hentze, MD, European Molecular Biology Laboratory (EMBL)

3:30pm-4:00pm Slicer and the Argonautes

Leemor Joshua-Tor, PhD, HHMI/Cold Spring Harbor Laboratory

4:00pm-4:15pm It's all about the Design: Mechanistic Insights and Progress on the GalNAc Conjugate Platform for Targeted Delivery of RNAi Therapeutics to the Liver

Martin A. Maier, PhD, Alnylam Pharmaceuticals

4:15pm-4:30pm Guide strand loading of human Argonaute-2 involves target RNA

Georg Sczakiel, PhD, University of Lübeck

4:30pm-6:30pm Poster Session II

EVEN numbered posters hosted

7:00pm Meet the Experts

Optional event, additional RSVP required

Tuesday, October 14th

7:30am-8:30am Breakfast

8:30am-10:15am Session IV: RNA Modulation II

*Co-Chairs: Marc Lemaitre, PhD, M_L Consult
Jonathan Watts, PhD, University of Southampton*

8:30am-9:00am Controlling siRNA Properties with Nucleobase Modifications

Peter A. Beal, PhD, University of California, Davis

9:00am-9:30am DNA Cages and Nanotubes: Design and Biological Properties

Hanadi Sleiman, PhD, McGill University

9:30am-9:45am Optimization of Antisense Drugs by P-Stereochemistry Control

Meena, PhD, WaVe Life Sciences

9:45am-10:00am 2'-OMe-Phosphorodithioate-modified siRNAs Show Increased Loading Into the RISC Complex and Enhanced Gene Silencing Activity

Xianbin Yang, PhD, AM Biotechnologies

10:00am-10:15am Evaluation of Exonic and Intronic Off-Target Effects Associated with LNA-modified Antisense Oligonucleotides

Piotr J. Kamola, PhD Candidate, GlaxoSmithKline

10:15am-10:45am Refreshment Break

10:45am-12:30pm Session V: Long Noncoding RNAs

*Co-Chairs: Tariq Rana, PhD, University of California, San Diego
Michael McManus, PhD, University of California, San Francisco*

10:45am-11:15am The Dark Matter of the Genome

Michael McManus, PhD, University of California, San Francisco



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- 11:15am-11:45am** **lncRNAs: Function and Mechanism in Controlling Cellular Identity**
Mitchell Guttman, PhD, *California Institute of Technology*
- 11:45am-12:15pm** **Imaging RNA and RNA biology using RNA mimics of green fluorescent protein**
Samie R. Jaffrey, MD, PhD, *Weill-Cornell Medical College, Cornell University*
- 12:15pm-12:30pm** **Effective and long lasting KD of nuclear retained lncRNA in a broad range of mouse tissues achieved with single stranded LNA enhanced antisense oligonucleotides**
Niels M. Frandsen, PhD, *Exiqon A/S*

12:30pm-1:45pm **Lunch Break**

1:45pm-3:45pm **Session VI: miRNAs**

Chair: Deirdre MacKenna, PhD, Regulus Therapeutics

- 1:45pm-2:15pm** **MicroRNA-based therapeutics in cancer**
Frank J. Slack, PhD, *BIDMC Cancer Center/Harvard Medical School*
- 2:15pm-2:45pm** **Systems-Level Insights into the Roles of MicroRNAs in Pulmonary Hypertension**
Stephen Y. Chan, MD, PhD, *Harvard Medical School/Brigham and Women's Hospital*
- 2:45pm-3:15pm** **Identification and therapeutic exploitation of microRNAs that regulate cancer metastasis**
Sohail F. Tavazoie, MD, PhD, *Rockefeller University*
- 3:15pm-3:30pm** **A combined strategy for the selective targeting of glioma by aptamer-miRNA/antimiR conjugates**
Vittorio de Franciscis, PhD, *National Council of Research, IEOS*
- 3:30pm-3:45pm** **Identification of the ESCRT-I complex as an important modulator of anti-miR uptake by cancer cells**
Tim Wagenaar, PhD, *SANOFI Oncology*

3:45pm-4:15pm **Refreshment Break**

4:15pm-6:00pm **Session VII: RNA Modulation**

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

- 4:15pm-4:45pm** **Tricyclo-DNA: highly promising antisense oligonucleotides for exon-skipping approaches in Duchenne Muscular Dystrophy**
Aurélie Goyenvallé, PhD, *Université de Versailles Saint Quentin*
- 4:45pm-5:15pm** **Targeted antisense modulation of Pre-mRNA Splicing**
Adrian R. Krainer, PhD, *Cold Spring Harbor Laboratory*
- 5:15pm-5:45pm** **Mechanisms and Programs of Neuronal Splicing Regulation**
Douglas L. Black, PhD, *University of California, Los Angeles*
- 5:45pm-6:00pm** **Personalized therapy for X-linked agammaglobulinemia (XLA) by oligonucleotide-induced splice-correction**
Burcu Bestas, PhD Candidate, *Karolinska Institutet*

6:30pm **OTS Board of Directors Meeting**

Wednesday, October 15th

7:30am-8:30am **Breakfast**

8:30am-10:30am **Session VIII: Preclinical/Clinical I**

Chair: Brett Monia, PhD, ISIS Pharmaceuticals, Inc.



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- 8:30am-9:00am Therapeutic opportunities of antisense oligonucleotides in the kidney**
Adam E. Mullick, PhD, *ISIS Pharmaceuticals, Inc.*
- 9:00am-9:30am Next generation SMART DRUGS: aptamer-based cell-targeted therapies**
Paloma H. Giangrande, PhD, *University of Iowa*
- 9:30am-10:00am Antibody Containing Nanoparticles: Multifunctional Cancer Therapeutics**
Mark E. Davis, PhD, *California Institute of Technology*
- 10:00am-10:15am Development of Exosomes-Mediated Oligonucleotides Delivery for Gene Silencing in vivo and in vitro**
Marie Didiot, PhD, *University of Massachusetts Medical School*
- 10:15am-10:30am Novel formulation enhances oligonucleotides delivery to muscle**
Gang Han, PhD Candidate, *Tianjin Medical University*
- 10:30am-11:00am Refreshment Break**
- 11:00am-1:00pm Session IX: Preclinical/Clinical II**
Chair: Mark A. Kay, MD, PhD, Stanford University
- 11:00am-11:30am Regulation of miRNA-mediated gene silencing by microRNA precursors**
Mark A. Kay, MD, PhD, *Stanford University*
- 11:30am-12:00pm Development of RNAi-based Therapeutics Using Dynamic Polyconjugate (DPC) Delivery Technology**
David L. Lewis, PhD, *Arrowhead Research Corporation*
- 12:00pm-12:30pm Update on the Preclinical Development of LNP-Based RNA Products**
Ian MacLachlan, PhD, *Tekmira Pharmaceuticals Corporation*
- 12:30pm-12:45pm Influence of intracellular localization on lncRNA knockdown**
Mark Behlke, MD, PhD, *Integrated DNA Technologies, Inc.*
- 12:45pm-1:00pm Cystic Fibrosis Transmembrane Regulator expression is regulated by long antisense non-coding RNAs**
Sheena Saayman, PhD, *The Scripps Research Institute*
- 1:00pm-2:30pm Lunch Break**
- 2:30pm – 2:45pm Awards Presentation**
- 2:45pm-4:45pm Session X: Preclinical/Clinical III**
Chair: Muthiah Manoharan, PhD, Alnylam Pharmaceuticals
- 2:45pm-3:15pm RNAi Therapeutic Development for the Treatment of TTR Amyloidosis**
Tracy S. Zimmermann, PhD, *Alnylam Pharmaceuticals*
- 3:15pm-3:45pm Developing Antisense Drugs for Cardiometabolic Diseases: Clinical Proof of Concept & Beyond**
Sanjay Bhanot, MD, PhD, *Isis Pharmaceuticals*
- 3:45pm-4:15pm The Treatment of Human Disease via inhibition of microRNAs: RG-101 and RG-012**
Neil W. Gibson, PhD, *Regulus Therapeutics, Inc.*
- 4:15pm-4:30pm Delivery of Single and Double-Stranded Oligonucleotides: The Clinical Experience with SMARTICLES®**
Richard T. Ho, MD, PhD, *Marina Biotech, Inc.*
- 4:30pm-4:45pm Nucleic Acid Polymers for the Treatment of Chronic HBV: A new therapeutic alternative**
Andrew Vaillant, PhD, *REPLICor Inc.*



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- 4:45pm** **Closing Remarks**
- 6:00pm** **Closing Banquet at the [USS Midway Museum](#)**
Additional RSVP and registration fee required