



13th Annual Meeting of the Oligonucleotide Therapeutics Society

September 24-27, 2017

Bordeaux, France

Meeting Agenda

As of September 20, 2017

Sunday, 24 September (Day ONE)

8.00 Registration Opens

9.00-12.00 Preconference Session: Oligonucleotide Therapeutics Education Workshop

Designing Oligo Therapeutics and Understanding Their PK
sponsored by Korea Institute of Toxicology

optional, additional fee

Chair: David Blakey, PhD, *MiNA Therapeutics*

9.00-9.20 Welcome & Introduction

PART ONE: Designing and Screening Oligonucleotides to Novel Targets for Different Approaches

9.20-9.50 Target Knock Down – ASOs and siRNA

Chris Hart, PhD, *Ionis Pharmaceuticals* and Martin Maier, PhD, *Alnylam Pharma*

9.50-10.05 CRISPR Approaches

Rachel Haurwitz, PhD, *Caribou Biosciences*

10.05-10.20 mRNA

Matthew Stanton, PhD, *Moderna Therapeutics*

10.20-10.35 Immune Stimulatory Oligos

Art Krieg, MD, *Checkmate Pharmaceuticals*

10:35-11.00 Refreshment Break

PART TWO: PK, Biodistribution, and Safety of Oligonucleotides

11.00-11.15 PK and Biodistribution

Richard Geary, PhD, *Ionis Pharmaceuticals*

11.15-11.30 AON Safety Screening - In vitro Assays Capturing Toxicity Risks

Sabine Sewing, PhD, *Hoffmann-La Roche, Roche Innovation Center*

11.30-12.00 Q&A

14.00 Meeting Starts: Welcome & Opening Remarks

OTS President: Arthur M. Krieg, MD, Checkmate Pharmaceuticals

14.15-15.00 Keynote Presentation:

A Worms Tale: Oligonucleotide-Guided Gene Regulation and Secrets of Inheritance and Immortality

Craig C. Mello, PhD, *RNA Therapeutics Institute, University of Massachusetts Medical School*

15.00-15.30 Refreshment Break

15.30-17.30 Session I: Nucleic Acid Chemistry

Chair: Jean-Jacques Toulmé, PhD, *Université de Bordeaux*

15.30-15.52 Modification and Multimerization of Therapeutic Oligonucleotides

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

15.52-16.15 Pyrrole-imidazole Polyamides: Artificial Genetic Switches for Therapeutic Gene Modulation

Hiroshi Sugiyama, PhD, *Kyoto University*



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- 16.15-16.37 Lipophilic LNAs to Improve Pharmacokinetics of Oligonucleotide Drugs**
Jesper Wengel, PhD, *University of Southern Denmark*
- 16.37-17.00 Thiomorpholinos, Morpholinos, Imidoamidates, and Other New DNA Analogues Useful for Biological Research**
Marvin H. Caruthers, PhD, *University of Colorado Boulder*
- 17.00-17.15 Stereodefined Phosphorothioate LNA Oligonucleotides**
Nanna Albaek, PhD, *Roche Innovation Center Copenhagen*
- 17.15-17.30 Triplex Forming Oligonucleotides (TFOs) LNA-containing: in silico and in vitro Studies**
Y. Vladimir Pabon-Martinez, MSc, PhD, *Karolinska Institutet*
- 17.30-18.30 Welcome Reception with Exhibitors**
- 18.30-20.30 Poster Session I**
ODD numbered posters hosted

Monday, 25 September (Day TWO)

- 8.30 -10.30 Session II: Delivery**
Chair: Laura Sepp-Lorenzino, PhD, *Vertex Pharmaceuticals, Inc.*
- 8.30-8.55 Advances in Targeted Delivery of Nucleotides Beyond the Liver**
Shalini Andersson, PhD, *AstraZeneca*
- 8.55-9.20 Cellular Dynamics Visualized from Molecules to Organisms at Increased Spatio-temporal Resolution**
Tomás Kirchhausen, PhD, *Boston Children's Hospital/Harvard Medical School*
- 9.20-9.45 Designed FN3 Domains for Extrahepatic Delivery of Oligonucleotides**
Vadim Dudkin, PhD, *The Janssen Pharmaceutical Companies of Johnson & Johnson*
- 9.45-10.00 Delivery of siRNA to the Lung by Lipid Peptide Nanoparticles as a Therapy for Cystic Fibrosis**
Stephen Hart, PhD, *University College London, Great Ormond Street Institute of Child Health*
- 10.00-10.15 Cyclic Peptides to Improve Delivery and Bio-activity of Antisense Oligonucleotides in Muscle Tissue**
Silvana Jirka, PhD, *Leiden University Medical Center*
- 10.15-10.30 Development of PPMO for the Treatment of DMD**
Marco A. Passini, PhD, *Sarepta Therapeutics*
- 10.30-11.00 Refreshment Break**
- 11.00-13.00 Session III: Immune Effects and Safety of Nucleic Acids**
Chair: Gunther Hartmann, MD, PhD, *University Hospital Bonn*
- 11.00-11.25 Nucleic Acid Immunity**
Gunther Hartmann, MD, PhD, *University Hospital Bonn*
- 11.25-11.50 Regulation of Inflammatory mRNAs via an Endonuclease Regnase-1**
Osamu Takeuchi, MD, PhD, *Kyoto University*
- 11.50-12.15 Development of CpG Oligonucleotides for the Immunotherapy of Cancer**
Robert L. Coffman, PhD, *Dynavax Technologies Corporation*
- 12.15-12.30 Investigation of Mortality Imbalance in Revusiran Phase 3 Study, ENDEAVOUR**
John Vest, MD, *Alnylam Pharmaceuticals*



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12.30-12.45 Immunologic Reshaping and Therapy of Cancer by Stimulation of Innate Nucleic Acid Sensor RIG-I

Christine Schuberth-Wagner, PhD, *Rigontec GmbH*

12.45-13.00 Bi-functional Oligonucleotides to Unleash TLR9-driven Antitumor Immune Responses

Marcin Kortylewski, PhD, *Beckman Research Institute, City of Hope National Medical Center*

13.00-15.00 Lunch Break with Meet the Experts Tables

15.00-17.00 Session IV: Emerging Topics in RNA Biology

Joint session with RNA Society

Chair: Lynne E. Maquat, PhD, *University of Rochester*

15.00-15.25 Tudor-SN-mediated Endonucleolytic Decay of Human-cell MicroRNAs Promotes G1/S Phase Transition

Lynne E. Maquat, PhD, *University of Rochester*

15.25-15.50 tRNA-Derived Fragments: From Biology to Technology and Therapeutics

Pavel Ivanov, PhD, *Brigham and Women's Hospital & Harvard Medical School*

15.50-16.15 Towards Translational Epigenetics: Non-coding RNAs and Junk Repeats in Normal Genome Regulation and Deregulation in Cancer

Jeanne B. Lawrence, PhD, *University of Massachusetts Medical School*

16.15-16.30 A 3' tRNA Derived Small RNA (tsRNA) Affects Ribosome Biogenesis and Translation in Rapidly Dividing Cells and a Target for Hepatocellular Carcinoma

Mark A. Kay, MD, PhD, *Stanford University*

16.30-16.45 Axiomer® Technology: Therapeutic Oligonucleotides for Directing Site-Specific A-to-I Editing by Endogenous ADAR Enzymes

Janne Turunen, PhD, *ProQR Therapeutics*

16.45-17.00 Hydrogel-assisted LNA Gapmer Delivery for in situ Gene Silencing in Spinal Cord Injury

Pedro Moreno, PhD, *i3S- Instituto de Investigação e Inovação em Saúde, Universidade do Porto*

17.00-19.30 Poster Session II

EVEN numbered posters hosted

Tuesday, 26 September (Day THREE)

8.30-10.30 Session V: Developments in Gene and RNA Editing

Chair: Rachel Haurwitz PhD, *Caribou Biosciences*

8.30-8.55 Targeting RNA with CRISPR/Cas9

Gene Yeo, PhD, *University of California, San Diego*

8.55-9.20 Robust In Vivo Gene Editing with Systemic Lipid Nanoparticle Delivery of CRISPR/Cas9 RNA Components

Amy Rhoden Smith, PhD, *Intellia Therapeutics, Inc.*

9.20-9.45 Messenger RNA as a Novel Therapeutic Approach

Matthew Stanton, PhD, *Moderna Therapeutics*

9.45-10.00 Significant Improvements in CRISPR Specificity Using Chemically Modified Single Guide RNAs

Douglas J. Dellinger, PhD, *Agilent Technologies, Inc.*



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10.00-10.15 Site-Directed RNA Editing for Correcting the Most Frequent Premature Termination Codon Mutations in CFTR

Maria Montiel, PhD, *The Marine Biological Laboratory*

10.15-10.30 Structure of Modified mRNAs Modulates Protein Expression

Iain McFadyen, PhD, *Moderna Therapeutics, Inc.*

10.30-11.00 Refreshment Break

11.00-13.00 Session VI: Preclinical Development

Chair: Frank Rigo, PhD, *Ionis Pharmaceuticals*

11.00-11.25 ATXN2 as a Therapeutic Target for SCA2 and ALS

Frank Rigo, PhD, *Ionis Pharmaceuticals*

11.25-11.50 Emerging microRNA Targets for Brain Diseases

Anna M. Krichevsky, PhD, *Harvard Medical School / Brigham & Women's Hospital*

11.50-12.15 Implementation of Customized NGS Technology for Screening of LNA Oligonucleotides

Mads Aaboe Jensen, MSc, PhD, *Roche Innovation Center Copenhagen A/S*

12.15-12.30 Upregulation of Nonsense Mutated CFTR with Antisense Oligonucleotides

Melissa Keenan, PhD, *Ionis Pharmaceuticals*

12.30-12.45 Acceleration of Diabetic Wound Healing by Local Delivery of PHD2-targeting sshRNAs

Anne Dallas, PhD, *SomaGenics, Inc.*

12.45-13.00 AAV5-miHTT Gene Therapy Demonstrates Broad Vector Distribution and Strong Mutant Huntingtin Lowering in a Huntington Disease Minipig Model

Melvin Evers, PhD, *uniQure biopharma B.V.*

13.00 Afternoon & Evening – Free Time to Explore Bordeaux

Optional: Junior Scientist Social Event

14.30 Cite du Vin Wine Museum

17.00 Cambridge Arms Pub

Come meet your fellow junior OTS members for an afternoon of wine exploration and networking. Please note that attendees are responsible for museum entry fee and food/drink costs. Feel free to come to one or both parts of this event!

Wednesday, 27 September (Day FOUR)

8.30-10.30 Session VII: Awards Presentations & Talks

Chair: Art Krieg, MD, *Checkmate Pharmaceuticals*

Welcome & Announcement of Ono Foundation Grant Program

8.40-9.00 Gewirtz Memorial Scholarship Award

Inhibiting the Androgen Receptor Interaction with the Long Non-Coding RNA SLNCR using 2'-FANA-Modified Oligonucleotides Decreases Melanoma Invasion and Proliferation

Karyn Schmidt, PhD, *Dana-Farber Cancer Institute*

9.00-9.20 Young Investigator Award

A 'Guided' Tour of an Early Career in RNA and Nucleic Acid Therapeutics

Keith Gagnon, PhD, *Southern Illinois University*



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- 9.20-9:40 Paper of the Year Award**
Treatment of Infantile-onset Spinal Muscular Atrophy with Nusinersen: a Phase 2, Open-label, Dose-escalation Study
Richard Finkel, MD, *Nemours Children's Hospital*
- 9.40-10.30 Lifetime Achievement Award**
A Life's Work in Oligonucleotides: From Chemical Synthesis to Peptide-PMO for Treatment of Neuromuscular Diseases
Michael J. Gait, PhD, *Medical Research Council, Laboratory of Molecular Biology*
- 10.30-11.00 Refreshment Break**
- 11.00-13.00 Session VIII: Preclinical Topics in Nucleic Acid Therapeutics**
Chair: Frank Slack, PhD, *BIDMC Cancer Center/Harvard Medical School*
- 11.00-11.25 MicroRNA-based Therapeutics in Cancer**
Frank Slack, PhD, *BIDMC Cancer Center/Harvard Medical School*
- 11.25-11.50 RNA-based Infection Research**
Jörg Vogel, PhD, *Helmholtz Institute for RNA-based Infection Research, Würzburg*
- 11.50-12.15 Exploiting the miRNA Biogenesis Pathway for Effective Oligonucleotide Therapeutics**
Richard I. Gregory, PhD, *Harvard Medical School/Boston Children's Hospital*
- 12.15-12.30 Exploring Chemical and Structural Diversity of Lipophilic Conjugates for Extrahepatic siRNA Delivery in vivo**
Annabelle Biscans, PhD, *University of Massachusetts Medical School*
- 12.30-12.45 INT-1B3 - a microRNA Mimic Acting as Tumor Suppressor and Inducing Apoptosis, Cell Cycle Arrest and Anti-Tumor Immunity – Shows Significant Activity in Experimental Tumor Models**
Michel Janicot, PhD, *InteRNA Technologies, BV*
- 12.45-13.00 Targeting Long Non-Coding RNAs with Nanoparticle-mediated RNAi for Breast Cancer Therapy**
Zheng-Rong Lu, PhD, *Case Western Reserve University*
- 13.00-14.30 Lunch Break**
- 14.30-16.30 Session IX: Clinical Studies**
Chair: Brett Monia, PhD, *Ionis Pharmaceuticals*
- 14.30-14.54 Antisense Platform Technology: Recent Phase 3 Experience and Beyond**
Richard Geary, PhD, *Ionis Pharmaceuticals*
- 14.54-15.18 Emerging Therapeutic Profile of RNA Interference Against Hepatic Targets**
Jae Kim, MD, *Alnylam Pharmaceuticals*
- 15.18-15.42 The Clinical Development of mRNA Drugs and Vaccines**
Tal Zaks, MD, PhD, *Moderna Therapeutics*
- 15.42-16.06 Nusinersen Treatment of Spinal Muscular Atrophy**
Richard Finkel, MD, *Nemours Children's Hospital*
- 16.06-16.30 Research and Development of a Novel Targeted HSP47 siRNA Lipid Nanoparticle for the Treatment of Fibrosis**
Roger Adami, PhD, *Nitto Biosciences*



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16.30 Closing Remarks

19.00 – 23.00 Closing Wrap Party at [Château Smith Haut Lafitte](#)

Limited tickets available; no onsite ticket sales.