



12th Annual Meeting of the Oligonucleotide Therapeutics Society

September 25-28, 2016

Montreal, Quebec, Canada

Meeting Agenda - DRAFT

As of September 21, 2016

Sunday, 25 September (Day ONE)

8.00 Registration Opens

9.00-12.15 Preconference Session: Oligonucleotide Therapeutics Education Workshop

Nucleic Acid Therapeutics Development 101

sponsored by Korea Institute of Toxicology

optional, additional fee

Chair: Marc M. Lemaitre, PhD, *M_L Consult*

9.00-9.10 Welcome & Introduction

9.10-9.40 Manufacturing Oligonucleotides From Bench to Bed

Marc M. Lemaitre, PhD, *M_L Consult*

9.40-10.10 Aptamers and SELEX: The Past, The Present, and The Future

Paloma Giangrande, PhD, *University of Iowa*

10.10-10.40 Addressing Delivery Hurdles in the Translation of Oligonucleotide Therapeutics

Paul Burke, PhD, *Burke Bioventures*

10.40-11.05 Refreshment Break

11.05-11.35 Nonclinical Strategies for Oligonucleotide Development Programs

Jennifer Lockridge, PhD, *Dicerna Pharmaceuticals*

11.35-12.15 Q&A

14.00 Meeting Starts: Welcome & Opening Remarks

OTS President: Brett Monia, PhD, Ionis Pharmaceuticals

14.30-15.45 Keynote Presentation:

Noncoding RNA: Large, Small and Viral

Joan A. Steitz, PhD, *Sterling Professor of Molecular Biophysics and Biochemistry, Yale University*

Investigator, Howard Hughes Medical Institute

15.45-16.15 Refreshment Break – Mezzanine Level

16.15-18.15 Session I: Immune Effects of DNA and RNA

Chair: Veit Hornung, MD, *Ludwig-Maximilians-University Munich*

16.15-16.45 Structural Basis of CpG and Inhibitory DNA Recognition by Toll-like Receptor 9

Toshiyuki Shimizu, PhD, *University of Tokyo*

16.45-17.15 ATP hydrolysis by the viral RNA sensor RIG-I prevents unintentional recognition of self-RNA

Karl-Peter Hopfner, Dr. rer. nat., *Ludwig-Maximilians-University Munich*

17.15-17.45 Structural characterization of IFIT protein interaction with viral RNA

Bhushan Nagar, PhD, *McGill University*

17.45-18.00 Making “Cold” Tumors “Hot” With Intratumoral Injection of a Phosphodiester CpG-A Oligodeoxynucleotide (ODN) in a VLP (CMP-001): Potential To Increase The Efficacy of Anti-PD-1 For Cancer Immunotherapy

Arthur M. Krieg, MD, *Checkmate Pharmaceuticals*

18.00-18.15 Selective stimulation of RIG-I with a novel synthetic RNA induces strong anti-tumor immunity in mouse tumor models

Marcel Renn, PhD, *Rigontec GmbH*



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18.15-20.15 Welcome Reception with Exhibitors

Level 3, Foyer

19.15-20.15 Meet the Companies

Level 3, Salon Cartier

Monday, 26 September (Day TWO)

8.30 -10.30 Session II: Advances in Oligonucleotide Medicinal Chemistry

Joint session with International Society of Nucleosides, Nucleotides & Nucleic Acids

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

8.30-9.00 Stability, Activity and Protein Interactions of Chemically Modified Nucleic Acids – The Structural View

Martin Egli, PhD, *Vanderbilt University*

9.00-9.30 New Approaches to Oligonucleotide Modification For Mechanism-Specific Applications

Jonathan Hall, PhD, *ETH Zurich*

9.30-9.45 Recent Progress in the Development of Bridged Nucleic Acids – Design, Synthesis and Properties of GuNA and scpBNA

Satoshi Obika, PhD, *Osaka University*

9.45-10.00 Complete Chemical Modification of siRNA, Lacking RNA, is Essential for Hydrophobic Conjugate Delivery *in vivo*

Matthew Hassler, PhD, *University of Massachusetts Medical School*

10.00-10.15 Selective Targeting of Point Mutations by Third Generation Antisense (3GA) Oligonucleotides

Reina Improgo, PhD, *Idera Pharmaceuticals*

10.15-10.30 Effect of 2'-5' linkage modification on RNA interference efficiency

Maryam Habibian, PhD Candidate, *McGill University*

10.30-11.00 Refreshment Break – Level 3

11.00-13.00 Session III: Delivery

Chair: Mano Manoharan, PhD, *Alnylam Pharmaceuticals*

11.00-11.15 Antibody-siRNA Conjugates as a Novel Approach for the Delivery of Oligonucleotides

Art Levin, PhD, *Avidity NanoMedicines*

11.15-11.45 Advances in Delivery of Investigational RNAi Therapeutics using Enhanced Stabilization Chemistry (ESC) siRNA-GalNAc Conjugates

Vasant Jadhav, PhD, *Alnylam Pharmaceuticals*

11.45-12.15 Lipid Nanoparticles (LNP) for mRNA Delivery: Therapeutic Applications and Challenges

Michael Hope, PhD, *Acuitas Therapeutics*

12.15-12.45 Development of Smart Polymeric Nanocarriers for Systemic Nucleic Acid Delivery

Kanjiro Miyata, PhD, *The University of Tokyo*

12.45-13.00 CCR7 Aptamers-functionalized saRNA Conjugates for Targeted Cancer Therapy

Jiehua Zhou, PhD, *Beckman Research Institute, City of Hope*

13.00-15.00 Lunch Break with Meet the Experts Tables – Level 4

Please allow Meet the Experts tables seating for those who pre-registered



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15.00 -17.00 Session IV: Emerging Topics in RNA Biology

Joint session with RNA Society

Chair: Melissa J. Moore, PhD, *RNA Therapeutics Institute, University of Massachusetts Medical School*

15.00-15.30 3'UTR-mediated Protein-Protein Interactions Regulate Protein Functions

Christine Mayr, MD, PhD, *Memorial Sloan Kettering Cancer Center*

15.30-16.00 Dropping the Liquid: RNA-binding proteins and Their Functional Droplets

Jacob C. Schwartz, PhD, *University of Arizona Tucson*

16.00-16.30 Activating Frataxin Expression by Disrupting R-loop Formation

David Corey, PhD, *UT Southwestern*

16.30-16.45 Specialized microRNP and Translation Mechanisms in Quiescent Cancer Cells

Shobha Vasudevan, PhD, *Harvard Medical School*

16.45-17.00 Exosome-mediated Transfer of Immunomodulatory RNA between Helminths and their Mammalian Hosts

Amy H. Buck, PhD, *University of Edinburgh*

17.00-19.30 Poster Session I – Level 3

ODD numbered posters hosted

Tuesday, 27 September (Day THREE)

8.30-10.30 Session V: Preclinical Topics in Nucleic Acid Therapeutics I

Chair: Frank Rigo, PhD, *Ionis Pharmaceuticals*

8.30-9.00 Treatment Opportunities for Cystic Fibrosis Using Aerosolized Antisense Oligonucleotides

Shuling Guo, PhD, *Ionis Pharmaceuticals*

9.00-9.30 Antisense Oligonucleotide Therapy for ALS and Frontal Temporal Dementia Caused by a Gain of Toxicity from Repeat Expansion in the C9orf72 Gene

Clotilde Lagier-Tourenne, MD, PhD, *Massachusetts General Hospital*

9.30-10.00 A Nucleic Acid Target and a Nucleic Acid Drug: Interfering with Breast Cancer Progression

David L. Spector, PhD, *Cold Spring Harbor Laboratory*

10.00-10.15 Systemic administration of a novel development candidate, MTL-CEBPA, upregulates the liver-enriched transcription factor C/EBP- α and reverses CCl₄-induced liver failure in vivo

Nagy Habib, MD, ChM, FRCS, *Imperial College London*

10.15-10.30 Anti-MicroRNA-132 Oligonucleotide is a Highly Potent Treatment for Liver Steatosis

David S. Greenberg, PhD, *The Hebrew University of Jerusalem*

10.30-11.00 Refreshment Break – Level 3

11.00-13.00 Session VI: Preclinical Topics in Nucleic Acid Therapeutics II

Chair: Anastasia Khvorova, PhD, *RNA Therapeutics Institute, University of Massachusetts Medical School*

11.00-11.30 Toward Treating Preeclampsia by RNA Silencing

Melissa J. Moore, PhD, *RNA Therapeutics Institute, University of Massachusetts Medical School*

11.30-12.00 MicroRNA-based Therapeutics in Cancer



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Frank Slack, PhD, *Harvard Medical School*

- 12.00-12.30 Developing Oligonucleotide based Platforms for mRNA Up-regulation**
Balkrishen Bhat, PhD, *RaNA Therapeutics*
- 12.30-12.45 Targeting β -catenin by RNAi for cancer and chronic liver disease**
Marc Abrams, PhD, *Dicerna Pharmaceuticals*
- 12.45-13.00 Polyunsaturated Fatty Acid hsiRNA Conjugates Display Enhanced Distribution and Robust Huntingtin Gene Silencing in the Mouse Brain**
Bruno M.D.C. Godinho, PhD, *University of Massachusetts Medical School*
- 13.00-15.00 Lunch Break - Level 4**
- 15.00-17.00 Session VII: Advances in Aptamer Research and Development**
Chair: Paloma Giangrande, PhD, *University of Iowa*
- 15.00-15.30 Novel Aptamers Facilitate a Proteomics Explosion**
Larry Gold, PhD, *Somalogic*
- 15.30-16.00 Continuous, Real-time Detection of Biomolecules in Live Animals**
H. Tom Soh, PhD, *Stanford University*
- 16.00-16.30 Forward and Reverse Clinical Translation with Antithrombotic Aptamers**
Bruce Sullenger, PhD, *Duke University Medical Center*
- 16.30-16.45 Aptamer Drug Conjugates (ApDCs) of Active Metabolites of Nucleoside Analogs and Cytotoxic Agents in Pancreatic Ductal Adenocarcinoma**
Sorah Yoon, DVM, PhD, *Beckman Research Institute, City of Hope*
- 16.45-17.00 Neutralization of Extracellular Histones with Nucleic Acid Aptamers for the Treatment of Critical Illness**
Kevin Urak, MS, *University of Iowa*
- 17.00-19.30 Poster Session II – Level 3**
EVEN numbered posters hosted

Wednesday, 28 September (Day FOUR)

- 7.00 – 8.00 Ancillary Session – Meet the Professor**
Stanley T. Crooke, MD, PhD, *Ionis Pharmaceuticals*
Level 3, Salon International
- 8.30-10.30 Session VIII: Awards Presentations & Talks**
Chair: Art Krieg, MD, *Checkmate Pharmaceuticals*
- 8.30-8.50 Gewirtz Memorial Scholarship Award**
Inhibition of HIV-1 by Gymnotically Delivered 2'-Deoxy-2'-Fluoro-D-Arabinonucleic Acid Modified Antisense Oligonucleotides (2'-FANA ASOs)
Mayumi Takahashi, PhD, *Beckman Research Institute, City of Hope*
- 8.50-9.10 Young Investigator Award**
Preclinical Development of Tricyclo-DNA Antisense Oligonucleotides for the Treatment of Neuromuscular Diseases
Aur lie Goyenvalle, PhD, *Universit  de Versailles Saint Quentin en Yvelines*
- 9.10-9:30 Paper of the Year Award**
Single-Molecule Imaging Reveals that Argonaute Reshapes the Binding Properties of Its Nucleic Acid Guides
Victor Serebrov, PhD, *RNA Therapeutics Institute, UMass Medical Center*



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9.30-10.30 Lifetime Achievement Award
The Present and Future of Antisense Technology
Stanley T. Crooke, MD, PhD, *Ionis Pharmaceuticals*

10.30-11.00 Refreshment Break – Level 3

11.00-13.00 Session IX: Developments in Gene and RNA Editing

Chair: Charles A. Gersbach, PhD, *Duke University*

11.00-11.30 Advancing CRISPR Medicines: Challenges and Solutions
David Bumcrot, PhD, *Editas Medicine, Inc.*

11.30-12.00 Genome and Epigenome Editing for Gene Therapy and Programming Cell Phenotype
Charles A. Gersbach, PhD, *Duke University*

12.00-12.30 Genome Editing of Hematopoietic Stem and Progenitor Cells
Matt Porteus, MD, *Stanford University*

12.30-12.45 Harnessing Human ADAR2 for RNA Repair – Recoding a PINK1 Mutation Rescues Mitophagy
Philipp Reautschnig, *University of Tübingen*

12.45-13.00 Nucleotide Substitutions Tune CRISPR/Cas9 Cleavage Activity
Keith Gagnon, PhD, *Southern Illinois University*

13.00-14.30 Lunch Break – Level 4

14.30-17.00 Session X: Clinical Studies

Chair: David Blakey, PhD, *MiNA Therapeutics*

14.30-14.50 Advances in Ligand Conjugated Antisense Strategies (LICA) for Single Stranded Antisense Therapeutics
Brett Monia, PhD, *Ionis Pharmaceuticals*

14.50-15.10 Emerging Human Clinical Profile of siRNA-GalNAc Conjugates
Patrick Haslett, MB MRCP, *Alnylam Pharmaceuticals*

15.10-15.30 Clinical Results with DCR-MYC, and LNP-formulated DsiRNA Targeting the MYC Oncogene
Pankaj Bhargava, MD, *Dicerna Pharmaceuticals*

15.30-15.50 microRNA Targeted Therapies for Hematological Malignancies and Pathological Fibrosis: Translation from Basic Science to the Clinic
Anita Seto, PhD, *miRagen Therapeutics, Inc.*

15.50-16.10 Imetelstat - First in Class, First in Clinic Telomerase Inhibitor
Sergei Gryaznov, PhD, *Alios/Johnson & Johnson*

16.10-16.30 Correcting the Thrombin Defect and Bleeding Phenotype of Hemophilia A and B by Lowering Antithrombin with Fitusiran
K. John Pasi, MB, ChB, PhD, FRCP, FRCPath, FRCPC, *Royal London Hospital Hemophilia Center*

16.30-16.45 Phase I study of BP1001, a Drug Candidate Utilizing DNAbilize™ Antisense DNA Technology, in Patients with Hematologic Malignancies
Ana Tari Ashizawa, PhD, MBA, *Bio-Path Holdings, Inc.*

16.45-17.00 Clinical Pharmacokinetics of RG-101 Administered as a Single Dose to HCV and Non-HCV Infected End-Stage Renal Disease Subjects Undergoing Hemodialysis Compared to Normal Renal Function Subjects
John S. Grundy, PhD, *Regulus Therapeutics*



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17.00 Closing Remarks

19.00 – 23.00 Closing Event - Industria Italian Brasserie

100 Peel Avenue, upstairs Suite #112

additional fee and RSVP required by 12.00 Monday September 26