## Sunday, 24 September (Day ONE)

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<tr>
<th>Time</th>
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<tr>
<td>8.00</td>
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| 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 |
| 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* |
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 Phosphorthioate 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 Revisiran Phase 3 Study, ENDEAVOUR  
John Vest, MD, Alnylam Pharmaceuticals
13th Annual Meeting of the Oligonucleotide Therapeutics Society
September 24-27, 2017
Bordeaux, France
Meeting Agenda
As of September 20, 2017

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 Rhode 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.
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*
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
16.30 Closing Remarks

19.00 – 23.00 Closing Wrap Party at Château Smith Haut Lafitte
Limited tickets available; no onsite ticket sales.