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

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
### 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

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<th>Time</th>
<th>Title</th>
<th>Speaker</th>
<th>Institution</th>
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<tr>
<td>8.30-9.00</td>
<td>Stability, Activity and Protein Interactions of Chemically Modified Nucleic Acids – The Structural View</td>
<td>Martin Egli, PhD, Vanderbilt University</td>
<td>Vanderbilt University</td>
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<td>9.00-9.30</td>
<td>New Approaches to Oligonucleotide Modification For Mechanism-Specific Applications</td>
<td>Jonathan Hall, PhD, ETH Zurich</td>
<td>ETH Zurich</td>
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<td>9.45-10.00</td>
<td>Complete Chemical Modification of siRNA, Lacking RNA, is Essential for Hydrophobic Conjugate Delivery <em>in vivo</em></td>
<td>Matthew Hassler, PhD, University of Massachusetts Medical School</td>
<td>University of Massachusetts Medical School</td>
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<td>10.00-10.15</td>
<td>Selective Targeting of Point Mutations by Third Generation Antisense (3GA) Oligonucleotides</td>
<td>Reina Improgo, PhD, Idera Pharmaceuticals</td>
<td>Idera Pharmaceuticals</td>
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<td>10.15-10.30</td>
<td>Effect of 2'-5' linkage modification on RNA interference efficiency</td>
<td>Maryam Habibian, PhD Candidate, McGill University</td>
<td>McGill University</td>
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<td>10.30-11.00</td>
<td>Refreshment Break – Level 3</td>
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#### 11.00-13.00  Session III: Delivery

**Chair:** Mano Manoharan, PhD, Alnylam Pharmaceuticals

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<tr>
<td>11.00-11.15</td>
<td>Antibody-siRNA Conjugates as a Novel Approach for the Delivery of Oligonucleotides</td>
<td>Art Levin, PhD, Avidity NanoMedicines</td>
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<td>11.15-11.45</td>
<td>Advances in Delivery of Investigational RNAi Therapeutics using Enhanced Stabilization Chemistry (ESC) siRNA-GalNAc Conjugates</td>
<td>Vasant Jadhav, PhD, Alnylam Pharmaceuticals</td>
<td>Alnylam Pharmaceuticals</td>
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<td>11.45-12.15</td>
<td>Lipid Nanoparticles (LNP) for mRNA Delivery: Therapeutic Applications and Challenges</td>
<td>Michael Hope, PhD, Acuitas Therapeutics</td>
<td>Acuitas Therapeutics</td>
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<td>12.15-12.45</td>
<td>Development of Smart Polymeric Nanocarriers for Systemic Nucleic Acid Delivery</td>
<td>Kanjiro Miyata, PhD, The University of Tokyo</td>
<td>The University of Tokyo</td>
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<td>12.45-13.00</td>
<td>CCR7 Aptamers-functionalized saRNA Conjugates for Targeted Cancer Therapy</td>
<td>Jiehua Zhou, PhD, Beckman Research Institute, City of Hope</td>
<td>Beckman Research Institute, City of Hope</td>
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#### 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*
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

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
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 Analogues 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 Gymnastically 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
Aurélie Goyenvalle, PhD, Université 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
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
Sergey 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, FRCPCH, 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
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