Sunday, 11 October (Day ONE)

8.00 Registration Opens

9.00-12.00 Preconference Session: Oligonucleotide Therapeutics Education Workshop
Making Drugs Out of Oligonucleotides
sponsored by Korea Institute of Toxicology
optional, additional fee
Chair: Marc M. Lemaitre, PhD, M_L Consult

9.00-9.30 Chemistry of Therapeutic Oligonucleotides: A Brief Overview
Muthiah Manoharan, PhD, Alnylam Pharmaceuticals

9.30-10.00 Oligonucleotide routes of administration, in vivo distribution hurdles, what to consider
Brett Monia, PhD, Isis Pharmaceuticals

10.00-10.30 Drug development process - TOX, ADME, PK/PD, clinical POC and drug approval
Art Krieg, MD, Checkmate Pharmaceuticals

10.30-11.00 Refreshment Break

11.00-11.30 New Approach for Safety Evaluation of Oligonucleotide Therapeutics using RNAseq
Seokjoo Yoon, DVM, PhD, Korea Institute of Toxicology

11.30-12.00 Q&A: panel discussion with speakers

14.00 Meeting Starts: Welcome & Opening Remarks
Brett Monia, PhD, Isis Pharmaceuticals & OTS President
Annemieke Aartsma-Rus, PhD, Leiden University Medical Center & Event Chairperson

14.30-15.45 Keynote Presentation:
Tandem Repeats, RNA Diseases and Treatment
Charles A. Thornton, MD, University of Rochester Medical Center

15.45-16.15 Refreshment Break

16.15-18.30 Session I: Medicinal Chemistry and Novel Mechanisms of Action (non splicing)
Co-chairs: Mike Gait, PhD, MRC Laboratory
Jonathan Watts, PhD, RNA Therapeutics Institute, UMass Medical School

16.15-16.45 Antagonism of Toll-like receptors: Therapeutic approach for autoimmune and inflammatory diseases
Sudhir Agrawal, D. Phil., Idera Pharmaceuticals

16.45-17.15 Gene Upregulation by Targeting Long Noncoding RNAs
Claes Wahlestedt, MD, PhD, University of Miami Miller School of Medicine

17.15-17.30 A polyunsaturated fatty acid-siRNA conjugate targeting Huntingtin (Htt) mRNA shows prolonged efficacy, minimal toxicity, and broad distribution in mouse brain
Maire F. Osborn, PhD, RNA Therapeutics Institute, UMass Medical School

17.30-17.45 Evaluation of Novel Nucleotide Modifications for Improved Activity of Messenger RNA Therapeutics
Anton P. McCaffrey, PhD, TriLink BioTechnologies

17.45-18.00 Transcript Repair by Site-Directed RNA Editing
Thorsten Stafforst, PhD, Universität Tübingen

18.00-18.15 Potent and targeted activation of latent HIV-1 using small guide RNAs and the CRISPR/dCas9 activator complex
Sheena Saayman, PhD, The Scripps Research Institute
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18.15-18.30  Chemically-Modified crRNA:tracrRNA Complexes for CRISPR Gene Editing  
Mark Behlke, MD, PhD, Integrated DNA Technologies

18.30-20.30  Welcome Reception with Exhibitors and Open Poster Viewing

Monday, 12 October (Day TWO)

8.30 -12.30  Session II: Exon Skipping*  
Co-chairs: Annemieke Aartsma-Rus, PhD, Leiden University Medical Center  
Frank Rigo, PhD, Isis Pharmaceuticals

8.30-9.15  Introduction and Talk:  
Translating exon skipping for Duchenne from bench to bedside  
Annemieke Aartsma-Rus, PhD, Leiden University Medical Center

9.15-9.30  Cysteine Correction of NOTCH3: exon skipping as a potential therapeutic strategy for CADASIL  
Julie Rutten, MD, MSc, Leiden University Medical Center

9.30-9.45  Restoration of cftr Function by Splicing Modulation  
Batsheva Kerem, PhD, The Alexander Silberman Institute of Life Sciences

9.45-10.00  Antisense Oligonucleotide Delivery is an Effective Therapeutic Approach for CEP290-Associated LCA  
Alejandro Garanto, PhD, Radboud University Medical Center

10.00-10.15  Enhanced systemic exon skipping therapy for Duchenne muscular dystrophy: combined dystrophin restoration and myostatin inhibition in adult dystrophic mice  
Alberto Malerba, PhD, Royal Holloway-University of London

10.15-10.45  Refreshment Break

10.45-11.15  A microexon regulatory network associated with autism spectrum disorder  
Benjamin Blencowe, PhD, University of Toronto

11.15-11.45  Antisense Oligonucleotide Therapies for the Treatment of Neurodegenerative Diseases  
C. Frank Bennett, PhD, Isis Pharmaceuticals

11.45-12.00  Antisense-oligonucleotide-directed inhibition of nonsense-mediated mRNA decay  
Tomoki Nomakuchi, Graduate Student, Cold Spring Harbor Laboratory

12.00-12.15  In vivo restoration of type VII collagen expression in human-skin-graft mouse model upon antisense oligonucleotide-mediated exon skipping  
Jeroen Bremer, PhD Student, University of Groningen

12.15-12.30  Phosphorodiamidate Morpholino Oligomers (PMOs) in Development for the Treatment of Duchenne Muscular Dystrophy (DMD)  
Ryszard Kole, PhD, Sarepta Therapeutics

*Exon Skipping Session organized by COST Action BM1207, that is sponsored by the Cooperation of Science and Technology

12.30-14.30  Lunch Break with Meet the Experts tables

14.30-16.30  Session III: Emerging Concepts in RNA Biology, co-hosted by the RNA Society  
Chair: Tracy Johnson, PhD, University of California, Los Angeles

14.30-14.45  Introduction  
Tracy Johnson, PhD, University of California, Los Angeles

14.45-15.15  Immunomodulatory RNA molecules: A new class of potential antivirals and vaccine adjuvants  
Anna Marie Pyle, PhD, Yale University/Howard Hughes Medical Institute
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15.15-15.45 Catalyzing and Proofreading pre-mRNA Splicing  
Jonathan P. Staley, PhD, University of Chicago

15.45-16.00 Antisense oligonucleotide-mediated MAPT mRNA splicing reveals a toxic tau isoform in mouse models of tauopathy  
Kathleen M. Schoch, PhD, Washington University

16.00-16.15 miR-34a Regulates c-SRC and Shows Therapeutic Efficacy in Triple Negative Breast Cancer  
Brian D. Adams, PhD, Beth Israel Deaconess Medical Center

16.15-16.30 Non-canonical translation factors: in vivo functions and mRNA targets  
Kent E. Duncan, PhD, University Medical Center Hamburg-Eppendorf

16.30-18.30 Poster Session I  
ODD numbered posters hosted

18.30-20.00 Dinner on own

20.00-22.00 Early Stage Researchers Social Event

Tuesday, 13 October (Day THREE)

8.30-11.30 Session IV: Delivery  
Co-Chairs: David Blakey, PhD, MiNA Therapeutics  
Rudy Juliano, PhD, University of North Carolina

8.30-8.45 Introduction and Talk:  
Challenges and emerging solutions for oligonucleotides delivery  
David Blakey, PhD, MiNA Therapeutics

8.45-9.15 Tackling a Billion Year Old Delivery Problem with new siRNN Chemistry  
Steven F. Dowdy, PhD, University of California, San Diego

9.15-9.30 Targeted ECO/siRNA Nanoparticles for Treatment of Triple Negative Breast Cancer  
Zheng-Rong Lu, PhD, Case Western Reserve University

9.30-9.45 Photochemical internalisation (PCI) – an efficient technology for inducing endosomal escape and enhancing nucleic acid delivery in vitro and in vivo  
Anders Høgset, PhD, PCI Biotech AS

9.45-10.00 Development of a cell-penetrating peptide for the delivery of antisense oligonucleotides to spinal muscular atrophy mice  
Katharina E. Meijboom, PhD/DPhil student, University of Oxford

10.00-10.30 Refreshment Break

10.30-11.00 Enhancing cellular delivery of oligonucleotides via targeting approaches and small molecules  
Rudolph Juliano, PhD, University of North Carolina

11.00-11.15 DARPin-siRNA conjugates for receptor-specific targeting  
Johannes Winkler, PhD, University of Vienna, Department of Pharmaceutical Chemistry

11.15-11.30 Aptamer-Targeted Drug Delivery to Prostate Cancer  
Bethany Powell Gray, PhD, Duke University Medical Center

11.30-11.45 Targeting 4-1BB costimulation to the tumor stroma with bispecific aptamer conjugates enhances the therapeutic index of tumor immunotherapy  
Brett Schrand, PhD, University of Miami, MSOM

11.45 – 13.15 Lunch Break
13.15-15.15 Session V: Oligonucleotide Safety: Issues, Mechanisms, Mitigations  
Chair: David Corey, PhD, UT Southwestern Medical Center at Dallas

13.20-13.55 A mechanism of hepatotoxicity for high affinity antisense oligonucleotides  
Sebastien Burel, PhD, Isis Pharmaceuticals

13.55-14.30 Autoimmune disorders by aberrant activation of a cytoplasmic viral RNA sensor MDA5  
Hiroki Kato, PhD, Kyoto University

14.30-14.45 Signal Interrupted: How RNAs containing modified nucleotides suppress RIG-I activation  
Ann Feigen Durbin, Graduate Student, Harvard University and MIT

14.45-15.00 Structural requirements of oligonucleotide based TLR3 antagonists  
Peter Järver, PhD, Stockholm University

15.00-15.15 Reversirs for Rapid and Potent Reversal of siRNA Silencing Activity  
Ivan Zlatev, PhD, Alnylam Pharmaceuticals, Inc.

15.15-15.45 Refreshment Break

15.45-17.30 Session VI: Early Drug Discovery  
Co-chairs: Marc Abrams, PhD, Dicerna Pharmaceuticals  
Willeke van Roon-Mom, PhD, Leiden University Medical Center

15.45-16.15 Allele Selective Suppression of Mutant Huntingtin by SNP Targeting Antisense Oligonucleotides  
Punit Seth, PhD, Isis Pharmaceuticals

16.15-16.45 Short and long non coding RNA therapeutics for cardiac remodeling  
Thomas Thum, MD, PhD, Hannover Medical School

16.45-17.00 Therapeutic benefit of a HTT-lowering antisense oligonucleotide targeting the CAG-repeat in the R6/2 Huntington’s disease mouse model  
Nicole Datson, PhD, BioMarin

17.00-17.15 Anti-MicroRNA-132 oligonucleotide as a potent treatment for Non alcoholic Fatty liver disease  
David S. Greenberg, PhD, The Hebrew University of Jerusalem

17.15-17.30 Antisense oligonucleotides for SOD1 improve function and extend life of SOD1-G93A mice  
Alexander McCampbell, PhD, Biogen

17.30-19.30 Poster Session II  
EVEN numbered posters hosted

Wednesday, 14 October (Day FOUR)

8.30-10.30 Session VII: Late Drug Discovery  
Co-chairs: Rachel Meyers, PhD, Alnylam Pharmaceuticals  
Veit Hornung, MD, University Hospital Bonn

8.30-9.00 Triphosphate RNA oligonucleotides as selective RIG-I ligands for cancer immunotherapy  
Gunther Hartmann, MD, PhD, University Hospital Bonn

9.00-9.30 Antagonizing miR-103/107 to treat metabolic disease: RG-125 / AZD4076 is a first-in-modality clinical candidate  
Andy Tumbull, PhD, AstraZeneca

9.30-9.45 Evaluation of modified phosphorodiamidate morpholino oligomers (PMOs) for the treatment of patients with rare and infectious disease  
Bruce Wentworth, PhD, Sarepta Therapeutics

9.45-10.00 Enhanced Pharmacologic Activity and Durability Demonstrated with an ESC GalNAc-siRNA Targeting Transthyretin
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Rachel Meyers, PhD, Alnylam Pharmaceuticals

10.00-10.15 Pre-clinical evaluation of AAV5-miHTT gene therapy of Huntington’s disease
Pavlina Konstantinova, PhD, uniQure NV

10.15-10.30 A new therapeutic oligonucleotide to regulate the blood-brain barrier
Hiroya Kuwahara, MD, PhD, Tokyo Medical and Dental University

10.30-11.00 Refreshment Break

11.00-12.30 Session VIII: Awards Presentations & Talks
Chair: Masad Damha, PhD, McGill University

11.00-11.20 Dr. Alan M. Gewirtz Memorial Scholarship
Broad-spectrum, host-targeted miRNA therapeutics for the treatment of respiratory viral infections
Jana L. McCaskill, PhD, University of Edinburgh

11.20-11.40 Mary Ann Liebert, publishers Young Investigator Award
Antisense oligonucleotide therapy for ALS and frontal temporal dementia caused by a gain of toxicity from hexanucleotide expansion in the C9orf72 gene
Frank Rigo, PhD, Isis Pharmaceuticals

11.40-12.30 Lifetime Achievement Award
50 Years of Phosphorothioate Nucleic Acids
Fritz Eckstein, PhD, Max Planck Institute of Experimental Medicine

12.30-14.00 Lunch Break

14.00-16.30 Session IX: Clinical Studies
Co-chairs: Brett Monia, PhD, Isis Pharmaceuticals
          Art Krieg, MD, Checkmate Pharmaceuticals

14.00-14.30 Recent Progress with RNAi Therapeutics Addressing Hepatic Targets
Akshay Vaishnaw, MD, PhD, FRCP, Alnylam Pharmaceuticals

14.30-15.00 Developing Antisense Drugs for Cancer
Mark Edbrooke, PhD, AstraZeneca

15.00-15.30 Effective Antisense Targeting of ApoC-III in Familial Chylomicronemia Syndrome Reveals Novel Biology and Therapy
Joseph L. Witztum, MD, University of California, San Diego

15.30-15.45 Development of RNAi-based therapeutics using DPC technology
Bruce D. Given, MD, Arrowhead Research Corporation

15.45-16.00 SMAD7 Antisense for IBD
Gerald Horan, PhD, Celgene Corp

16.00-16.15 Mono and Combination Cancer Therapy Approaches for microRNA Mimics
Paul Lammers, MD, MSc, Mirna Therapeutics, Inc.

16.15-16.30 Individualized RNA-based cancer immunotherapy
Andreas N. Kuhn, PhD, BioNTech RNA Pharmaceuticals GmbH

16.30 Closing Remarks
Annemieke Aartsma-Rus, PhD, Event Chair

19.00 – 23.00 Closing Event
Additional fee required; reception from 19.00-20.00 and dinner, drinks, and dancing to follow.