11th Annual Meeting of the Oligonucleotide Therapeutics Society
October 11 – 14, 2015
Leiden, The Netherlands
Meeting Agenda *as of September 24, 2015*

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*

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*

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 initernalisation (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 Fiegen 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 Turnbull, 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

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.