



15th Annual Meeting of the Oligonucleotide Therapeutics Society

13 October – 16 October 2019

[Holiday Inn Munich - City Centre](#)

Munich, Germany

Meeting Agenda

As of October 16, 2019

Sunday, October 13 (Day ONE)

9.00 Registration Opens

10.00-12.00 *Preconference Session:*

Next-Gen Early Career Scientist Meeting

Co-chairs: Ronald Buijsen, PhD, *Leiden University Medical Center*

Daniel O'Reilly, PhD, *McGill University*

10.00-10.05 **Welcome and Introduction**

10.05-10.35 **Session Keynote**

The First Few Bases: Career and Chemical Modification

Jonathan Watts, PhD, *RNA Therapeutics Institute, University of Massachusetts Medical School*

10.35-10.51 **Backbone-modified Locked Nucleic Acids for Therapeutic Antisense Applications**

Ysobel Baker, MChem, PhD, *University of Oxford*

10.51-11.07 **Deciphering the Interactive Network of the DLK1-DIO3 Locus in Hematopoiesis and Pediatric Leukemia**

Lonneke Verboon, *Universitätsklinikum Halle (Saale)*

11.07-11.23 **Artificial Exosomes for Oligonucleotide Delivery to the Brain**

Reka Haraszti, MD, PhD, *RNA Therapeutics Institute, UMass Medical School*

11.23-11.39 **Splice Modulation Therapy for a Variety of ABCA4 Mutations Underlying Stargardt Disease**

Alex Garanto, PhD, *Radboudumc*

11.39-11.55 **RNAi-suppression of Hepatitis B Virus Antigens Enables Therapeutic Vaccination to Induce Curative CD8 T-cell Responses in Mice**

Thomas Michler, MD, *Institute of Virology, Technische Universität München*

11.55-12.00 **Voting for Best Talk**

12.00-13.30 **Lunch Break**

Lunch on Own

13.30-17.00 *Preconference Session:*

Oligonucleotide Therapeutics Education Workshop:

Designing and Making Oligonucleotide Therapeutics, Understanding Their Development and CMC

RSVP and additional fee required

Chair: Marc Lemaitre, PhD, *M_L Consult*

13.30-13.35 **Welcome and Introduction**

13.35-14.05 **Oligonucleotide Manufacturing Process Planning, Development and Scale-up: CMC Strategy for Sustainable Manufacturing**

Eduardo PAREDES, PhD, *Nitto Denko Avecia*

14.05-14.35 **MiRNA Drug Development from Lead Selection to Phase 2**

Prof. Dr. Ekkehard Leberer, *Sanofi Deutschland GmbH*

14.35-15.05 **Oligonucleotide Therapeutics: Technology Evolution for Clinically Relevant Tissue Delivery**

Anastasia Khvorova, PhD, *RNA Therapeutics Institute, UMass Medical School*

15.05-15.30 **Refreshment Break**



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- 15.30-16.00 Current CMC and Regulatory Aspects for Oligonucleotide Therapeutics**
Thomas Rupp, PhD, *Thomas Rupp Consulting*
- 16.00-16.30 A Bioanalytical Toolkit to Support Oligonucleotide Therapeutics Throughout the Drug Development Pipeline**
Brooke Rock, PhD, *Amgen Research San Francisco*
- 16.30-17.00 Q&A**
- 17.00-18.00 Welcome Reception with Meet the Experts Networking**
- 18.00-19.30 Poster Session I**
ODD numbered posters hosted
- 19.30 Dinner On Own**
- 20.30 Early Career Scientist Social Event**
Location: Motel One – Lobby Bar
Sponsored by ChemGenes

Monday, October 14 (Day TWO)

- 8.00-8.30 Welcome Coffee**
- 8.30-8.45 Welcome & Opening Remarks**
Annemieke Aartsma-Rus, PhD, *Leiden University Medical Center*
Gunther Hartmann, MD, PhD, *University Hospital Bonn*
- 8.45-10.45 Session I: Nucleic Acid Chemistry**
Chair: Masad J. Damha, PhD, FCIC, *McGill University*
- 8.45-9.10 Sequence Selective Recognition of Double-Stranded RNA by Cationic Nucleobase and Backbone-Modified Peptide Nucleic Acids**
Eriks Rozners, PhD, *Binghamton University*
- 9.10-9.35 Synthesis and Properties of Oligonucleotides with Triazole, Carbamate and Amide Backbones**
Prof. Afaf El-Sagheer, *University of Oxford*
- 9.35-10.00 Generating Biologically Stable TNA Aptamers that Function with High Affinity and Thermal Stability**
John Chaput, PhD, *University of California, Irvine*
- 10.00-10.15 DNA Nanostructures for Cellular Delivery of Therapeutics**
Aurélien Lacroix, PhD Candidate, *McGill University*
- 10.15-10.30 Novel Mesyl Phosphoramidate Antisense Oligonucleotides as an Alternative to Phosphorothioates for Efficient Silencing of Oncogenic microRNA in vivo**
Marina A. Zenkova, Dr. Sc., *Institute of Chemical Biology and Fundamental Medicine SB RA*
- 10.30-10.45 Structurally Constraining of Inter-nucleotide Linkage Impacts on siRNA Potency and Allele Specificity**
Ken Yamada, PhD, *RNA Therapeutics Institute, UMass Medical School*
- 10.45-11.15 Refreshment Break**
- 11.15-12.15 Keynote Session:**
The Epigenetic Code in DNA and New Oligonucleotide Technologies
Prof. Dr. Thomas Carell, *Ludwig-Maximilians-Universität München*



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12.15-13.30 Lunch Break
hosted lunch buffet

13.30 -15.30 Session II: Nucleic Acid Functional Interference Techniques
Chair: Anastasia Khvorova, PhD, *RNA Therapeutics Institute, UMass Medical School*

13.30-13.55 ADAR Based Gene Editing Through ASO-based Recruitment
Prof. Dr. Thorsten Stafforst, *University of Tübingen*

13.55-14.20 Structure-Activity Correlations of Novel Backbone-Modified siRNA Analogs
Martin Egli, PhD, *Vanderbilt University School of Medicine*

14.20-14.45 Chemical Engineering for Extra-hepatic siRNA Delivery
Annabelle Biscans, PhD, *RNA Therapeutics Institute, UMass Medical School*

14.45-15.00 Cleavage of pre-mRNA in the Nucleus by RNase H1-dependent Antisense Oligonucleotides Causes RNA Polymerase II Transcription Termination
Frank Rigo, PhD, *Ionis Pharmaceuticals*

15.00-15.15 HTPPathwaySeq, a Novel Application for High-throughput RNAi Off-target Gene Identification
Prof. Pieter Mestdagh, *Biogazelle*

15.15-15.30 Deconstructing ASO-RNA Hybridization with Single-molecule Resolution
Sabrina Leslie, PhD, *McGill University*

15.30-16.00 Refreshment Break

16.00-17.35 Session III: Nucleic Acid Damage and Repair
Chair: Rachel Meyers, PhD, *Third Rock Ventures*

16.00-16.25 RNA or DNA: Unexpected Therapeutic Mechanism of a Classic Nucleoside Drug
Prof. Nathan W. Luedtke, *McGill University*

16.25-16.50 The DNA Damage Response - Friend or Foe?
Mattias Altmeyer, PhD, *University of Zurich*

16.50-17.05 Application of Genome Editing for Treatment of Retinal Diseases
Dong Hyun Jo, MD, PhD, *Seoul National University Hospital*

17.05-17.20 Small Nucleic Acid-Based Inhibitors of CRISPR-Cas9
Christopher Barkau, *Southern Illinois University School of Medicine*

17.20-17.35 Targeting DNA Repair Pathways in Cancer Using Oligonucleotides
Laura Croft, PhD, *Queensland University of Technology*

17.35-19.30 Poster Session II
EVEN numbered posters hosted

19.00 OTS Board of Directors Meeting

Dinner on Own

Tuesday, October 15 (Day THREE)

8.00-8.30 Welcome Coffee

8.30-10.35 Session IV: Nucleic Acid Immunity
Chair: Gunther Hartmann, MD, PhD, *University Hospital Bonn*



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8.30-8.55 RNA Decay Controls Inflammation

Shizuo Akira, MD, PhD, *Osaka University*

8.55-9.20 Nucleic Acid Sensing in Human Myeloid Cells

Veit Hornung, MD, *Ludwig-Maximilians-Universität München*

9.20-9.45 Circular RNA Immunity

Grace Chen, PhD, *Yale University School of Medicine*

9.45-10.05 Immune Sensing by TLR8 Requires Processing by Endosomal RNases

Eva Bartok, MD, *Institute of Clinical Chemistry and Clinical Pharmacology*

10.05-10.20 Responsive STING-activating Nanovaccines

Guizhi (Julian) Zhu, PhD, *Virginia Commonwealth University*

10.20-10.35 Development and Characterization of AZD8701, a High Affinity Antisense Oligonucleotide Targeting FOXP3 to Relieve Immunosuppression in Cancer

A. Robert MacLeod, PhD, *Ionis Pharmaceuticals*

10.35-11.00 Refreshment Break

11.00-13.00 Session V: Awards Presentations & Talks

Chair: Annemieke Aartsma-Rus, PhD, *Leiden University Medical Center*

Welcome & Announcements:

11.05-11.25 Dr. Alan M. Gewirtz Memorial Scholarship Award

Divalent siRNA Scaffold for Robust Gene Modulation in the Central Nervous System

Bruno M.D.C. Godinho, PhD, *RNA Therapeutics Institute, UMass Medical School*

11.25-11.45 Mary Ann Liebert publishers, Inc. Young Investigator Award

Antisense Oligonucleotides for the Treatment of Neurodegenerative Diseases

Holly Kordasiewicz, PhD, *Ionis Pharmaceuticals*

11.45-12.05 Paper of the Year Award

[Chemical Modification of PS-ASO Therapeutics Reduces Cellular Protein-binding and Improves the Therapeutic Index](#)

Xue-hai Liang, PhD, *Ionis Pharmaceuticals*

12.05-13.00 Lifetime Achievement Award

Living in the World of Oligonucleotide Therapeutics

Muthiah (Mano) Manoharan, PhD, *Alnylam Pharmaceuticals*

13.00-14.30 Lunch Break

Hosted lunch buffet

14.30-16.30 Session VI: Oligonucleotide Delivery and Targeting

Chair: Frank Rigo, PhD, *Ionis Pharmaceuticals*

14.30-14.55 Targeting Blood Clots and Tumors with Aptamers

Bruce Sullenger, PhD, *Duke University*

14.55-15.20 mRNA Therapy for Rare Diseases: Optimization of Payload and Delivery System

Peter Lutwyche, PhD, *Genevant Sciences Corp*

15.20-15.45 Cell Specific Centyrin Targeting of Oligonucleotides for Cancer

Karyn O'Neil, PhD, *Aro Biotherapeutics*



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15.45-16.00 Investigation of the Mechanism of Internalization of Fluorescently Labeled Cholesterol-modified siRNA Into Cultured Tumor Cells

Ivan Chernikov, *Institute of Chemical Biology and Fundamental Medicine
Siberian Branch of the Russian Academy of Sciences*

16.00-16.15 A Comprehensive RNAseq Analysis Shows That siRNA Based Modulation of Huntingtin in the Central Nervous System of Non-Human Primates is Highly Specific

Julia Alterman, PhD, *RNA Therapeutics Institute, UMass Medical School*

16.15-16.30 Unravelling the Mysteries of Endosomal Escape

Anders Wittrup, MD, PhD, *Lund University*

18.00-22.00 OTS Party at the [Münchner Künstlerhaus](#) (Munich Artist House)

sponsored by ChemGenes

Wednesday, 16 October (Day FOUR)

8.00-8.30 Welcome Coffee

8.30-10.35 Session VII: Oligonucleotide Preclinical Stage Discovery

Chair: Art Levin, PhD, *Avidity Biosciences*

Welcome & Announcements:

Poster Award Winners & Next-Gen Early Career Scientist Session - Best Talk Award

8.35-9.00 Clinical Transition of microRNA-based Therapeutic Concepts in Cardiovascular Disease
Thomas Thum, MD, PhD, *Hannover Medical School*

9.00-9.25 Development of MRG-110, an LNA-AntimiR Targeting miR-92a For Use in Tissue Repair and Revascularization

Rusty Montgomery, PhD, *miRagen*

9.25-9.50 Activation of Therapeutic RTK Variants by Splicing Interference

Luca Cartegni, PhD, *Rutgers University*

9.50-10.05 Heteroduplex-oligonucleotides for Highly Efficient Silencing of microRNA Through a Novel Intracellular Mechanism

Kotaro Yoshioka, MD, PhD, *Tokyo Medical and Dental University*

10.05-10.20 Oligo(ethylenamino)-Lipopeptide PMO Conjugates: Impact of Unsaturation and Particle Formation on Splice-Switching Activity

Dr. Ulrich Lächelt, *LMU Munich*

10.20-10.35 A Novel ASO Based Approach to Treat Peripheral Neuropathic Pain

Patrick Carroll, PhD, *INSERM, Institute for Neurosciences of Montpellier*

10.35-11.00 Refreshment Break

11.00-13.00 Session VIII: Oligonucleotide Preclinical Stage Development

Chair: Annemieke Aartsma-Rus, PhD, *Leiden University Medical Center*

11.00-11.25 Antisense Oligonucleotide Therapies for Rare Neurodegenerative Disorders

Daniel Curtis, PhD, *Amylon Therapeutics*

11.25-11.50 PNPLA3 Antisense Oligonucleotide, a Novel Precision Medicine Approach to Treat Non-alcoholic Steatohepatitis

Daniel Lindén, PhD, *AstraZeneca*



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- 11.50-12.15 The Role of Patient Organisations in Drug Development**
Elizabeth Vroom, *Duchenne Parent Project, World Duchenne Organization*
- 12.15-12.30 Metabolic and Energetic Benefits of microRNA-22 Inhibition**
Marc Thibonnier, MD, MS, *AptamiR Therapeutics, Inc.*
- 12.30-12.45 Gapmers Targeting DUX4 as a Therapeutic Strategy for Facioscapulohumeral Muscular Dystrophy**
Yi-Wen Chen, PhD, *Children's National Health System*

- 12.45-13.00 Development and Optimization of Antisense Therapy for Pompe Disease**
Atze Bergsma, PhD, *Erasmus MC*

- 13.00-14.30 Lunch Break**
hosted lunch buffet

- 14.30-17.10 Session IX: Oligonucleotide Clinical Studies**
Chair: Martin Maier, PhD, *Alnylam Pharmaceuticals*

- 14.30-14.55 mRNA Medicines Enable Intracellular, Membrane-bound, and Extracellular Secreted Therapeutics**
Tal Zaks, MD, PhD, *Moderna Therapeutics*
- 14.55-15.20 Patisiran in Clinical Practice – Experience from the UK National Amyloidosis Centre**
Julian Gillmore, PhD, *National Amyloidosis Centre, University College London*
- 15.20-15.45 Safety, PK, PD, and Exploratory Efficacy in Single and Multiple Dose Study of a SOD1 Antisense Oligonucleotide (BIIB067) in Participants with ALS**
Toby A. Ferguson, MD, PhD, *Biogen*
- 15.45-16.10 Accelerated Development of Antisense Oligonucleotides as Personalized Medicine**
Timothy M. Yu, MD, PhD, *Boston Children's Hospital, Harvard Medical School, Broad Institute*
- 16.10-16.25 Clinical Results from XCUR17, a Topically Applied Antisense Spherical Nucleic Acid in Patients with Psoriasis**
David A. Giljohann, PhD, *Excicure*
- 16.25-16.40 Late Breaking Talk:**
**Activation of CEBPA in Myeloid Cells by saRNA in HCC Patients:
The emergence of an Immunomodulatory Switch for Anti-cancer Therapy**
Prof Nagy Habib MD, ChM, FRCS, *Imperial College London*
- 16.40-16.55 Late Breaking Talk:**
ENVISION, a Phase 3 Study of Safety and Efficacy of Givosiran, an Investigational RNAi Therapeutic, in Acute Hepatic Porphyria Patients
Ilja Kubisch, MD, *Klinikum Chemnitz*
- 16.55-17.10 Late Breaking Talk:**
Further Progress with siRNA for Atherosclerotic Cardiovascular Disease: Update on the ORION Program Including Results from Recently Completed ORION-11 and ongoing ORION-1/3
T. Scott Johnson, MD, *The Medicines Company*
- 17.10-17.20 Closing Remarks**