

**Saturday, October 5<sup>th</sup>**

**15.00-17.00** Arrival and Early Registration

**Sunday, October 6<sup>th</sup>**

**07.00** Registration

**08.30** Welcome & Opening Remarks

*OTS President: Masad Damha, PhD, McGill University*

**08.45-10.30** Session I: Aptamers: Mechanisms & Applications

*Chair: Vittorio de Franciscis, PhD, National Council of Research, IEOS*

**08.45-09.15** **New Modes of Folding and Binding in Nucleic Acid Ligands with Enhanced Chemical Diversity -- Implications for Development of Diagnostics and Therapeutics**  
*Nebojsa Janjic, PhD, SomaLogic, Inc.*

**09.15-09.45** **MCP-1 Inhibitor NOX-E36. The long way from in vitro selection to clinical data**  
*Sven Klussmann, PhD, NOXXON Pharma AG*

**09.45-10.15** **Forward and Reverse Translation with Antithrombotic Aptamers and their Antidotes**  
*Bruce Sullenger, PhD, Duke University Medical Center*

**10.15-10.30** **Streamlined RNA Aptamer Selection with Microcolumn Devices and RAPID Method, and Identification with High-throughput RNA Interaction Assay**  
*Abdullah Ozer, PhD, Cornell University*

**10.30-11.00** Refreshment Break

**11.00-13.00** Session II: Immunomodulatory Oligonucleotides

*Chair: Gunther Hartmann, MD, PhD, University Hospital Bonn, Germany*

**11.00-11.30** **A Cyclic Di-nucleotide Signaling Pathway Mediates Innate Immune Responses to Cytosolic DNA in Mammalian Cells**  
*Lijun Sun, PhD, UT Southwestern Medical Center*

**11.30-12.00** **Structure-Function Studies of Cytosolic DNA Sensors Governing the Interferon Response**  
*Dinshaw J. Patel, PhD, Memorial Sloan-Kettering Cancer Center*

**12.00-12.30** **Nucleotide Starvation: a SAMHD1-dependent Strategy for Cell-intrinsic Anti-retroviral Defense**  
*Jan Rehwinkel, PhD, University of Oxford*

**12.30-12.45** **RNA<sup>Adjuvant</sup><sup>®</sup>: A Novel, Highly-potent, RNA-based Adjuvant for Prophylactic and Therapeutic Vaccines**  
*Karl-Josef Kallen, MD, PhD, CureVac GmbH*

**12.45-13.00** **cGAS Produces a 2'-5'-linked Cyclic Dinucleotide Second Messenger that Activates STING**  
*Marion Goldeck, MSc, University Hospital Bonn, Germany*

**13.00-14.00** Lunch Break

**14.00-16.00 Session III: Promoting RNA Degradation I**

*Chair: Muthiah Manoharan, PhD, Alnylam Pharmaceuticals*

**14.00-14.30 RNAi in Cell Nuclei**

*David Corey, PhD, UT Southwestern Medical Center*

**14.30-15.00 The Therapeutic Potential of Tricyclo-DNA**

*Christian Leumann, PhD, University of Bern*

**15.00-15.30 Assembly of the Catalytic Engine of RNAi, a RISCy Business**

*Qinghua Liu, PhD, UT Southwestern Medical Center*

**15.30-15.45 Atu027 Plus Gemcitabine in Advanced or Metastatic Pancreatic Cancer (Atu027-I-02)**

*Klaus Giese, PhD, Silence Therapeutics AG*

**15.45-16.00 Peptide-Conjugated Glucan Particles for Delivery of Therapeutic siRNA**

*Jessica L. Cohen, PhD, University of Massachusetts Medical School*

**16.00-16.30 Refreshment Break**

**16.30-17.30 Keynote Presentation I**

**Sequence-defined Carriers for Targeted Nucleic Acid Delivery**

*Ernst Wagner, PhD, Ludwig-Maximilians University (LMU) of Munich*

*Moderator: Mike Gait, PhD, MRC Laboratory*

**17.30-19.30 Welcome Reception and Poster Session I**

*ODD numbered posters hosted*

**Monday, October 7<sup>th</sup>**

**08.30-10.30 Session IV: Promoting RNA Degradation II**

*Chair: David Corey, PhD, UT Southwestern Medical Center*

**08.30-09.00 RNAi Therapeutics Using GalNAc-siRNA Conjugate Delivery Platform: New Era of Oligonucleotides**

*Muthiah Manoharan, PhD, Alnylam Pharmaceuticals*

**09.00-09.30 RNAi in Neurological Diseases**

*Beverly L. Davidson, PhD, University of Iowa*

**09.30-10.00 Can We Control the Phosphate Degradation in Nucleic Acids by Harnessing the Relative Hydration?**

*Jyoti Chattopadhyaya, DSc, PhD, University of Uppsala*

**10.00-10.15 Cocktails of Formulated Short Synthetic shRNAs Show Potent Inhibition of Hepatitis C Virus in HCV-Infected Chimeric Mice**

*Brian H. Johnston, PhD, Somagenics, Inc.*

**10.15-10.30 Hepatotoxic Potential of Therapeutic Oligonucleotides Can Be Predicted from Their Sequence and Modification Pattern**

*Peter H. Hagedorn, MSc, PhD, Santaris Pharma*

**10.30-11.00 Refreshment Break**

**11.00-13.00 Session V: Long Noncoding RNAs**

*Chair: Shuling Guo, PhD, Isis Pharmaceuticals*

**11.00-11.30 Long Noncoding RNAs as Drug Targets**

*Claes Wahlestedt, MD, PhD, University of Miami, Miller School of Medicine*

- 11.30-12.00 The Role of an HIV-encoded Antisense long non-coding RNA in Epigenetic Modulation of Viral Transcription and Latency**  
Kevin Morris, PhD, *The Scripps Research Institute, California*
- 12.00-12.15 Towards Therapy for Angelman Syndrome by Knockdown of a Repressive Long Non-coding RNA**  
Amanda J. Ward, PhD, *Isis Pharmaceuticals*
- 12.15-12.30 Selective Upregulation of Gene Expression by Blocking Specific PRC2-lncRNA Interactions**  
Arthur M. Krieg, MD, *RaNA Therapeutics, Inc.*
- 12.30-12.45 A Study of Twenty Mouse LincRNA Deletion Knockouts with LacZ Reporter Knock-Ins Reveals Unique Gene Expression Patterns and Subtle Phenotypes**  
David Friendewey, PhD, *Regeneron Pharmaceuticals, Inc.*
- 12.45-13.00 The Role of lncRNA in Small RNA-Triggered Gene Activation**  
John C. Burnett, PhD, *Beckman Research Institute at the City of Hope*
- 13.00-14.00 Lunch Break and Informal Poster Viewing**
- 14.00-15.00 Keynote Presentation II  
Micro RNAs and Cancer**  
Carlo M. Croce, MD, *The Ohio State University Comprehensive Cancer Center*  
Moderator: Vittorio de Franciscis, PhD, *National Council of Research, IEOS*
- 15.00-17.30 Session VI: Micro RNAs**  
*Chair: David Blakey, PhD, AstraZeneca*
- 15.00-15.30 MicroRNAs for Cardiac Regeneration**  
Mauro Giacca, MD, PhD, *International Centre for Genetic Engineering & Biotechnology (ICGEB)*
- 15.30-16.00 Transcriptional Gene Targeting by microRNA-Polycomb Complexes in Normal and Leukemic Haematopoiesis**  
Clara Nervi, MD, PhD, *University of Rome "La Sapienza"*
- 16.00-16.30 Refreshment Break**
- 16.30-17.00 Identification and Therapeutic Targeting of MicroRNAs Regulating Cholesterol/Lipid Metabolism**  
Anders Naar, PhD, *Harvard Medical School/Massachusetts General Hospital*
- 17.00-17.15 Silencing Gene Expression by Recruiting RISC**  
Jennifer A. Broderick, PhD, *University of Massachusetts Medical School*
- 17.15-17.30 Causes and Consequences of Dysregulated microRNAs in ALS**  
Eran Hornstein, MD, PhD, *Weizmann Institute of Science\**  
*\*Presenting for Dr. Hornstein will be Dr. Iddo Magen*
- 17.30-18.00 Special Session  
Business Aspects of Starting a Biotech Company**  
Mick McLean, PhD, *ABeterno Ltd and Link Technologies*  
Moderator: Marc Lemaitre, PhD, *Avecia*
- 18.00-20.00 Poster Session II**  
*EVEN numbered posters hosted*

**Tuesday, October 8<sup>th</sup>**

**08.30-11.00 Session VII: RNA Modulation**

*Chair: Frank Rigo, PhD, Isis Pharmaceuticals*

**08.30-09.00 Triple Repeat RNA Structure, Toxicity and Targeting**

*Wlodzimierz J. Krzyzosiak, PhD, Polish Academy of Sciences*

**09.00-09.30 Genome-wide Maps of RNA Binding Proteins Implicated in Neurodegeneration**

*Gene Yeo, PhD, University of California, San Diego*

**09.30-10.00 Antisense Approaches for Splicing Modulation in Disease**

*Michelle L. Hastings, PhD, Chicago Medical School, Rosalind Franklin University*

**10.00-10.30 High-resolution Xist Binding Maps Reveal 2-step Spreading During X-inactivation**

*Jeannie Lee, MD, PhD, Howard Hughes Medical Institute; Department of Molecular Biology, Massachusetts General Hospital; Department of Genetics, Harvard Medical School*

**10.30-10.45 Double-stranded Antisense Oligonucleotides (dsASO) for Highly Efficient Gene Silencing**

*Takanori Yokota, MD, PhD, Department of Neurology and Neuological Science, Tokyo Medical and Dental University*

**10.45-11.00 Synthesis and Biological Studies of New DNA Analogs Containing Lysine and Boranephosphonate Linkages**

*Luca Monfregola, PhD, University of Colorado at Boulder*

**11.00-11.30 Refreshment Break**

**11.30-13.15 Session VIII: Therapeutic Applications: Preclinical I**

*Chair: Rachel Meyers, PhD, Alnylam Pharmaceuticals*

**11.30-12.00 Antisense Oligonucleotide Technology Targeting Tmprss6 Ameliorates Hemochromatosis and Beta-Thalassemia**

*Stefano Rivella, PhD, Weill Cornell Medical College*

**12.00-12.30 Developing Gene Silencing Oligonucleotides and Delivery Technologies to Treat Brain Cancers**

*Kevin Petrecca, BSc, MD, PhD, FRCS(C), McGill University*

**12.30-12.45 A Selective RIG-I Agonist for Anti-melanoma Immunotherapy**

*Jasper G. van den Boorn, PhD, University Hospital Bonn, Germany*

**12.45-13.00 The Use of Antisense Oligonucleotides to Reduce Rhodopsin mRNA and Protein Levels in Photoreceptor Cells in the Mouse Retina**

*Sue Murray, BSc, Isis Pharmaceuticals*

**13.00-13.15 Preclinical Development of ALN-AS1, an RNAi therapeutic for the Treatment of Acute Intermittent Porphyria**

*William Querbes, PhD, Alnylam Pharmaceuticals*

**13.15-14.15 Lunch Break**

**14.15-15.45 Session IX: Therapeutic Applications: Preclinical II**

*Chair: Mike Gait, PhD, MRC Laboratory*

**14.15-14.45 Extracellular RNAs and PPMO Splice Switching Therapy for DMD**

*Matthew Wood, MD, PhD, University of Oxford*

**14.45-15.15 Ugimers – Antisense for Inflammation**

*Jürgen Soutschek, PhD, ugichem GmbH*

**15.15-15.30 SMN Rescue by Using Oligonucleotides of Tricyclo-DNA to Induce exon 7 Inclusion in SMN2 mRNA**

Valérie Robin, PhD, *University of Versailles Saint Quentin*

**15.30-15.45 Messenger RNA as a Novel Therapeutic Approach**

Joe Bolen, PhD, *Moderna Therapeutics*

**15.45-16.15 Refreshment Break**

**16.15-18.15 Session X: Therapeutic Applications: Clinical Development**

*Chair: Art Krieg, MD, RaNA Therapeutics, Inc.*

**16.15-16.45 Bench to Bedside Targeting of Hsp27: A Randomized Phase II Study of OGX-427 in Patients with Chemotherapy-Naive Metastatic Castration Resistant Prostate Cancer (mCRPC)**

Kim N. Chi, MD FRCPC, *BC Cancer Agency, Vancouver Prostate Centre, University of British Columbia*

**16.45-17.15 Antisense Oligonucleotide Therapies for the Treatment of Neurodegenerative Diseases**

C. Frank Bennett, PhD, *Isis Pharmaceuticals*

**17.15-17.45 RNA Interference Therapeutics for the Treatment of Transthyretin (TTR) Amyloidosis**

Rachel Meyers, PhD, *Alnylam Pharmaceuticals*

**17.45-18.15 Drisapersen Efficacy and Safety in Duchenne Muscular Dystrophy: Results of a Phase III, Randomized, Double-Blind, Placebo-Controlled Trial (Study DMD114044)**

John E. Kraus, MD, PhD, *GlaxoSmithKline*

**18.15-18.30 Progress in the Extension Study of Eteplirsen, a Phosphorodiamidate Morpholino Oligonucleotide**

Ryszard Kole, PhD, *Sarepta Therapeutics*

**18.30 Closing Remarks and Launch of the OTS 2014 Annual Meeting**

*OTS President: Masad Damha, PhD, McGill University*

**19.30 Post Meeting Social Event including Award Announcements**

*Additional RSVP and registration fee required.*