

DAY 1 – SUNDAY, 19 OCTOBER 2025

- 8.00** **Registration Open**
Welcome Coffee – please enjoy breakfast at your hotel
- 9.00 – 11.00** **Pre-Conference Session**
Oligonucleotide Therapeutics Education Workshop
Co-Chairs: Alex Garanto, Ph.D., *Radboud Univ. Medical Center* | Audrius Kilikevicius, Ph.D., *Eli Lilly and Company*
- 9.00 – 9.25** **Rewriting Genetic Information in RNA using Oligonucleotide-directed Adenosine Deamination**
Peter Beal, Ph.D., *UC Davis*
- 9.25 – 9.50** **Next-generation mRNA vaccines: New RNA modalities and delivery systems**
Camilla Foged, Ph.D., *University of Copenhagen*
- 9.50 – 10.15** **Improving the Safety and Specificity of RNAi Therapeutics**
Maja M. Janas de Angelis, Ph.D., DABT, *Alnylam Pharmaceuticals*
- 10.15 – 10.40** **Safety assessment of therapeutic oligonucleotides with focus on off-targets**
Patrik Andersson, Ph.D., *AstraZeneca*
- 10.40 – 11.05** **Early clinical development of nucleic acid therapeutics**
Art Krieg, M.D., *Zola Therapeutics*

This Education Workshop is Sponsored by:



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DATAPOINTS

- 11.15 – 13.00** **Hosted Lunch Break (for all participants)**
& Mentorship Program Lunch (by invitation)
- 13.00 – 14.40** **Pre-Conference Session:**
Next-Gen Early Career Scientist Session
Co-Chairs: Sarah Allen, Ph.D. Candidate, *UMass Chan Medical School* | Emilio Harris-Mostert, Ph.D. Candidate, *Erasmus MC*
- 13.05 – 13.25** **Session Keynote:**
From Molecules to Meaning: An Academic Journey to ASO Therapies for Duchenne Muscular Dystrophy
Aurelie Goyenvall, Ph.D., *UMR-1179- University of Versailles, INSERM*
- 13.25 – 13.40** **Accurate AI-Driven Prediction of Potent Therapeutic Fully Chemically Modified siRNAs**
Kathryn R. Monopoli, *UMass Chan Medical School*
- 13.40 – 13.55** **Ligand Valency, Placement and Positioning Do Matter: Programmable Surface Modification of Rectangular DNA Origami Modulates Its Biological Activity**
Natalia Navarro, *IQAC-CSIC, CIBER-BBN, Barcelona, Spain*
- 13.55 – 14.10** **Unraveling and controlling late-onset neurotoxicity of CNS-targeted antisense oligonucleotides through strategic chemical modifications**
Takayuki Kuroda M.D., *NucleoTIDE and Peptide Drug Discovery Center, Institute of Science Tokyo*
- 14.10 – 14.25** **Fatty acid mediated uptake system enables improved oligo delivery to muscle and brain**
Argimiro Mayoral-Olmos, *ARTHEX Biotech*
- 14.25 – 14.40** **Immunoengineering of light-inducible RNA agonists enables spatiotemporal control of RIG-I activation for cancer immunotherapy**
Sandra A. Lewash, *University Hospital Bonn*

14.40 – 15.30 **Refreshment Break**

15.30 – 16.45 **Opening Session and Keynote Address**

15.30 – 15.45 **Welcome and Opening Remarks**

Richard Geary, Ph.D., *Ionis Pharmaceuticals, OTS President*

15.45 – 16.45 **Keynote Presentation:**

The Remarkable Versatility of Splice-Switching ASOs

Adrian R. Krainer, Ph.D., *Professor, St. Giles Foundation – Co-Leader, Cancer Center Program*

16.45 – 18.15 **Career Event**

Co-Chairs: Eva-Maria Manz, Ph.D., *ETH Zürich* | Jathavan Asohan, Ph.D. Candidate, *McGill University*

Panel discussion on diverse career paths in academia and industry.

Panellists:

Reka Haraszti, Ph.D., *Universitätsklinikum Tübingen*

Keith Gagnon, Ph.D., *Wake Forest University*

Graham Parker, Ph.D., *Executive Editor, Mary Ann Liebert Inc.*

Marc Lemaitre, Ph.D., *ML_Consult LLC*

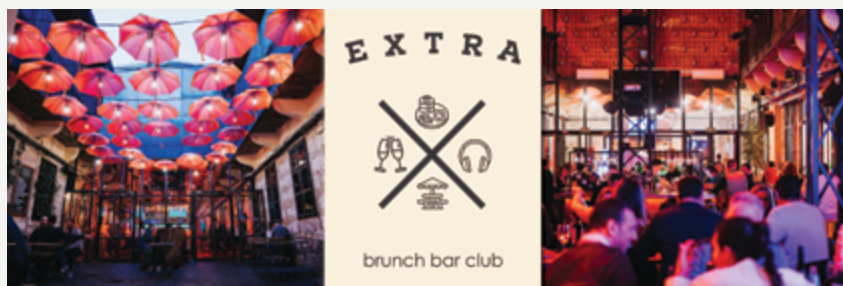
Krystal Johnson, Ph.D., *Alnylam Pharmaceuticals*

Annabelle Biscans, Ph.D., *AstraZeneca*

16.45 – 19.30 **Welcome Reception with Exhibitors (no Poster Hosting)**

20.00 – 22.00 **Early Career Scientist Social Meet Up (RSVP, for students only)**

Location: EXTRA Bar



Klauzál u. 15, 1072 Budapest | <https://extrabudapest.com/en>

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DAY 2 – MONDAY, 20 OCTOBER 2025

- 8.00 – 8.55 **Special Guest Talk – Familial Chylomicronemia, a Rare but Devastating Disease given Hope through Oligonucleotide Therapy**
Alan Brown, M.D., *Advocate Aurora Health*
- 9.00 – 10.50 **Session I: Chemistry, Mechanism and Delivery (I)**
Co-Chairs: Steven F. Dowdy, Ph.D., *UCSD School of Medicine* | Ian Huggins, Ph.D., *Ionis Pharmaceuticals*
- 9.00 – 9.25 **Click-based targeting and immunology of oligonucleotides**
Thomas Carell, Ph.D., *Ludwig-Maximilians-Universität (LMU) München*
- 9.25 – 9.50 **Life After Escape: Imaging Sorting, Action, and Degradation of Therapeutic RNAs in the Cytosol**
Anders Wittrup, Ph.D., *Lund University*
- 9.50 – 10.05 **Novel ligands for cell selective gene silencing**
Vadim Dudkin, *Souffle Therapeutics*
- 10.05 – 10.20 **The X-Zyme Platform: Expanding RNA silencing with precise & autonomous allele specific knockdown**
Joel Kaye Ph.D., *IE Therapeutics*
- 10.20 – 10.35 **Breaking the “2 -Hydroxyl Barrier” in CRISPR/Cas Editing Systems**
Abhishek Arora, *McGill University*
- 10.35 – 10.50 **Mechanism of Action and Nof1 Compassionate Use Case Reports of a Novel mRNA Cancer Vaccine Platform**
Bradley T. Sorenson, MBA, *Providence Therapeutics*
- 10.50 – 11.15 **Refreshment Break**
- 11.15 – 13.15 **Session II: Pre-Clinical I (Early Stage)**
Co-Chairs: Martin A. Maier, Ph.D., *Alnylam Pharmaceuticals* | Reka Haraszti, M.D., Ph.D., *Universitätsklinikum Tübingen*
- 11.15 – 11.40 **Targeting Regulatory RNAs with Antisense Oligonucleotides for the Potential Treatment of SYNGAP1-Related Disorders**
Dan Tardiff, Ph.D., *CAMP4*
- 11.40 – 12.05 **Targeting splicing factors in cancer and diseases**
Olga Anczukow, Ph.D., *The Jackson Laboratory for Genomic Medicine*
- 12.05 – 12.30 **Splicing modulation therapy for inherited retinal diseases**
Rob Collin, Ph.D., *Radboud University Medical Center*
- 12.30 – 12.45 **A Combinatorial siRNA Treatment for Fibrodysplasia Ossificans Progressiva**
Julia Alterman, Ph.D., *UMass Chan Medical School*
- 12.45 – 13.00 **Genotoxicity assessment of Oligonucleotide-based therapeutics – Will standard battery testing become redundant? - Evaluation of non-clinical test strategies from a regulatory perspective**
Clara Stock, *Dutch Medicines Evaluation Board*
- 13.00 – 13.15 **Engineering Selective Anti-Tumor Immunity with Fully Modified miRNA Mimics: Linking Chemical Structure to Functional Targetomes in T Cells**
Xavier Segarra Visent, *University Hospital Tübingen*
- 13.00 – 14.30 **Hosted Lunch Break (for all participants)**

14.30 – 16.30

Session III: Pre Clinical II (Late Stage)

Co-Chairs: Shalini Andersson, Ph.D., *AstraZeneca* | Virginia Arechavala-Gomez, Ph.D., *Biobizkaia Health Research Institute*

14.30 – 14.55

Answering Cajal's Challenge: Reactivating neurogenesis in the aged nervous system with ASOs
Don Cleveland, Ph.D., *UC San Diego*

14.55 – 15.20

Divalent siRNAs for Therapeutic Gene Silencing in the Central Nervous System
Corrie Gallant-Behm, Ph.D., *Atalanta Therapeutics*

15.20 – 15.45

RNA Therapeutic Approaches for Heart Failure: Targeting Phospholamban
Adam Mullick, Ph.D., *Ionis Pharmaceuticals*

15.45 – 16.00

VECTrans® platform achieves potent RNAi in the deep brain in NHP at minimal systemic dose
Guillaume Jacquot, *VECT-HORUS*

16.00 – 16.15

Development of LY3954068, an Intrathecally Administered Microtubule-Associated Protein Tau (MAPT) Small Interference RNA (siRNA) for Alzheimer's Disease
Kaushambi Roy Ph.D., *Eli Lilly*

16.15 – 16.30

Improved Preclinical and Clinical Performance of Complement Pathway-Targeting siRNA Conjugates
Marc Abrams Ph.D., *Sanogene Bio*

16.30 – 17.00

Poster – Fire Session I – Chair: Shuling Guo, Ph.D., *Vertex*

16.30 – 16.33

Time-resolved live-cell imaging of cytosolic RNA localization and fate
Johanna M. Johansson, *Lund University*

16.33 – 16.36

Chemical Modification of CRISPR-Cas12a Guide RNAs Towards Carrier-Free Delivery of CRISPR Therapeutics
Michael P. Cunningham, *McGill University*

16.36 – 16.39

Exon skipping peptide-conjugated morpholinos downregulate dynamin 2 to rescue centronuclear myopathy
Foteini Moschovaki-Filippidou, *Institut de Génétique et de Biologie Moléculaire et Cellulaire, IGBMC*

16.39 – 16.42

Binding to PDGFR receptor to enhance RNA-therapies targeted delivery in fibroblasts for collagen VI-related congenital muscular dystrophies
Sara Aguti Ph.D., *Queen Square Institute of Neurology, University College London*

16.42 – 16.45

Cardiac and skeletal muscle delivery of biotherapeutics with a blood vessel epicardial substance-targeting peptide
Biaobiao Wang, *Tianjin Medical University*

16.45 – 16.48

Dual-Peptide Strategy for Delivery of Therapeutic Oligonucleotides in Erythropoietic Protoporphyria
Angel Eduardo Santorelli Villamizar Ph.D., *ETH Zurich*

17.00 – 20.00

Poster Session – Reception I (ODD Numbered Posters Hosted)

DAY 3 – TUESDAY, 21 OCTOBER 2025

8.30 – 10.30

Session IV: Chemistry, Mechanism and Delivery (II)

Co-Chairs: Anastasia Khvorova, Ph.D., *UMass Chan Medical School/RTI* | Garth Kinberger, Ph.D., *Atalanta Therapeutics*

8.30 – 8.55

Oligonucleotide-barcoded library for in vivo screening of tissue-targeting peptide conjugates

Samuel Hildebrand, Graduate Student, *UMass Chan Medical School*

8.55 – 9.20

Decoding Argonaute: Structural Insights into Human Ago2 Catalysis

Ian J. MacRae, Ph.D., *The Scripps Research Institut*

9.20 – 9.45

The nucleobase guanine at the 3'-terminus of oligonucleotide RGLS4326 drives off-target AMPAR inhibition and CNS toxicity

Edmund Lee, Ph.D., *Regulus Therapeutics (a Novartis Company)*

9.45 – 10.00

SORT1-Mediated Delivery of siRNA to the Central Nervous System

Lucas Siow, Ph.D., *ProteinQure*

10.00 – 10.15

Enhancing CNS Delivery of Therapeutic Oligonucleotides with AQP4 Facilitator

Kotaro Yoshioka M.D., Ph.D., *Institution of Science Tokyo, Japan*

10.15 – 10.30

Optimizing High-DAR Antibody-siRNA Conjugates: Overcoming Clearance Liabilities to Enhance Extrahepatic Gene Silencing

Michael Cochran, *Avidity Biosciences*

10.30 – 11.00

Refreshment Break

11.00 – 12.30

Session V: Awards Session I | Lifetime Achievement Awards

Co-Chairs: Richard Geary, Ph.D., *Ionis Pharmaceuticals & President, OTS* | Mano Manoharan, Ph.D., *Alnylam Pharmaceuticals*

11.00 – 11.15

2025 OTS Lifetime Achievement Award (Posthumous)

Prof. Wojciech Stec

11.15 – 12.30

2025 OTS Lifetime Achievement Award

Pioneering RNA-Targeted Oligonucleotide Therapeutics: A 35-Year Journey

Brett Monia, Ph.D., CEO, *Ionis Pharmaceuticals*

12.30 – 14.45

Hosted Lunch Break (for all participants)

Lunch Sponsored by:



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12.45 – 14.25 **Session VI (Lunch Session): Advancing Oligonucleotide Manufacturing Towards a New Era of Therapeutics**

Chair: David Butler, Ph.D., *Hongene Biotech*

12.45 – 13.10 **Revolutionizing RNAi Therapeutics Manufacturing with Enzymes**

Stefan Lutz, Ph.D., *Codexis*

13.10 – 13.35 **Exploring Biocatalytic Syntheses of Oligonucleotides**

Roumen Radinov, Ph.D., *Alnylam Pharmaceuticals*

13.35 – 14.00 **Scalable Membrane Enabled One-Pot Liquid-Phase Oligonucleotide Synthesis**

Steven Ferguson, Ph.D., *University College Dublin*

14.00 – 14.25 **Advancing siRNA Manufacturing: From Conventional to Thermostable Enzymatic Ligation**

Chris Li, Ph.D., *Hongene Biotech*

14.45 – 16.35 **Session VII: DNA/RNA Editing**

Co-Chairs: Keith Gagnon, Ph.D., *Wake Forest University* | Blythe Sather, Ph.D., *Tune Therapeutics*

14.45 – 15.10 **Engineering insights into RNA trans-splicing platforms**

Aravind AsoKan, Ph.D., *Duke University*

15.10 – 15.35 **Discovery and development of CRISPR-associated transposases for RNA-guided gene insertion**

Sam Sternberg, Ph.D., *Columbia University*

15.35 – 15.50 **Improved RNA base editing with guide RNAs mimicking highly edited endogenous ADAR substrates**

Rui Zhang Ph.D., *Sun Yat-Sen University*

15.50 – 16.05 **Enhancing the Efficacy and Purity of Prime Editing using Chemically Modified Editing Templates and Modification-Tolerant Polymerases**

Jonathan Watts, *RNA Therapeutics Institute, UMass Chan Medical School*

16.05 – 16.20 **Developing AIMer-Based RNA Editing Technology to Correct a Nonsense Mutation in the Lung**

Jack D. Godfrey Ph.D., *Wave Life Sciences*

16.20 – 16.35 **Optimized RESTORE+ oligonucleotides for an efficacious and safe RNA base editing treatment for Alpha-1 antitrypsin deficiency**

Tobias Merkle, *AIRNA Bio Germany*

16.30 – 17.00 **Poster – Fire Session II – Chair:** Bruno Godinho, Ph.D., *Atalanta Therapeutics*

16.35 – 16.38 **Optimized Brain Delivery of RNA Therapeutics with Next-Generation Brainshuttle™ – antisense oligonucleotide conjugates for brain delivery**

Tatjana Sela, *Roche*

16.38 – 16.41 **Antisense Oligomers for Modulation of Enterococcus faecalis metabolism of L-Dopa in Parkinson's Disease**

Vincent Lau, *Helmholtz Centre for Infection Research*

16.41 – 16.44 **Developmental and epileptic encephalopathy 5 caused by a dominant-negative pathogenic SPTAN1 variant can be targeted with allele-specific antisense oligonucleotides**

Christiana Wang, *Baylor College of Medicine*

16.44 – 16.47 **Pharmacokinetics and endosomal escape of GalNAc-siRNAs,**

Fang-Ching Chao, *AstraZeneca*

16.47 – 16.50 **Oligonucleotide aptamers to improve ASO therapeutics**

Puri Fortes Ph.D., *CIMA/UNAV*

16.50 – 16.53 **Selection of DNA Aptamers for TDP-43 to Inhibit Protein Aggregation in Amyotrophic Lateral Sclerosis**

Daniel Knight, *Carleton University*

17.00 – 20.00 **Poster Session – Reception II (Even Numbered Posters Hosted)**

DAY 4 – WEDNESDAY, 22 OCTOBER 2025

8.30 – 10.30

Session VIII: Rare Diseases

Co-Chairs: Annemieke Aartsma-Rus, Ph.D., *Leiden University* | Richard Geary, Ph.D., *Ionis Pharmaceuticals & President, OTS*

8.30 – 8.55

Zebrokysen: Advancing a Personalized Antisense Therapy for CLN3 Batten Disease from Discovery to the Clinic

Michelle Hastings, Ph.D., *University of Michigan Medical School*

8.55 – 9.10

N-of-1 for N-of-Many: A Clinically Geared Platform Approach for Development of Scalable Patient-Customized Splice Modulation ASO Therapies for Ataxia Telangiectasia

Clemens Lochman, Ph.D. Student, *Hertie Institute for Clinical Brain Research*

9.10 – 9.25

Anti-FGF2 Aptamer Therapy in Achondroplasia

Yoshikazu Nakamura, Ph.D., *IMSUT University of Tokyo, RIBOMIC, Inc.*

9.25 – 9.40

N-of-1 personalized medicine for CMT2S: From patient-specific preclinical modeling to first-in-human dosing

Sandra P. Smieszek, Ph.D., *Vanda Pharmaceuticals*

9.40 – 9.55

Small binding RNAs (sbRNAs) as a novel therapeutic strategy for allele-selective silencing in Huntington's Disease

Megan Blewett, Ph.D., *Iris Medicine*

9.55 – 10.20

Patient Experience: Nusinersen

Steven Jones, *SMA Patient Advocate*

10.20 – 10.30

Q&A

10.30 – 11.00

Refreshment Break

11.00 – 13.00

Session IX: Awards Session II

Co-Chairs: Richard Geary, Ph.D., *Ionis Pharmaceuticals & President, OTS* | Masad J. Damha, Ph.D., *McGill University*

11.00 – 11.05

Award Announcements

Travel Grant Awardees, Poster Awards, Next Gen Session – Best Talk, People's Choice Poster Awards

11.05 – 11.15

President's Special Awards

David Corey, Ph.D., *UT Southwestern Medical Center*

Marc Lemaitre, Ph.D., *ML_Consumt Llc*

11.15 – 11.35

Award for Patient Advocacy

Strengthening Research, Advocacy, and Collaboration in ALS:
The Dual Perspective of a Scientist and Community Member

Daniel Knight, Ph.D. Candidate, *Carleton University*

11.35 – 11.55

Dr. Alan M. Gewirtz Memorial Scholarship Award – Graduate Students

Nuclear Argonaute: miRNA complexes recognize target sequences within chromatin-associated RNA and silence gene expression

Cristina Hofman, Ph.D. Candidate, *UT Southwestern Medical Center*

11.55 – 12.15

Dr. Alan M. Gewirtz Memorial Scholarship – Postdoctoral Fellows and Junior Industrial Professionals

An approach to disease-agnostic therapeutic genome editing

Sarah E. Pierce, Ph.D., *Broad Institute*

- 12.15 – 12.35 **Mary Ann Liebert publishers, Inc. Young Investigator Award**
Harnessing antisense oligonucleotide for the treatment of neurological disorders
Hien Tran Zhao, Ph.D., *Ionis Pharmaceuticals*
- 12.35 – 12.55 **Paper of the Year Award - Basic Research**
Enhancing siRNA efficacy in vivo with extended nucleic acid backbones
Ken Yamada, Ph.D., *UMass Chan Medical School*
<https://doi.org/10.1038/s41587-024-02336-7>
- 12.55 – 13.15 **Paper of the Year Award - Late Discovery**
Conjugation to a transferrin receptor 1-binding Bicycle peptide enhances ASO and siRNA potency in skeletal and cardiac muscles
Michele Carrer, Ph.D., *Ionis Pharmaceuticals*
<https://doi.org/10.1093/nar/gkaf270>
- 13.15 – 14.30 **Hosted Lunch (for all participants)**
- 14.30 – 16.10 **Session X: Clinical**
Co-Chairs: Art Krieg, M.D., *Zola Therapeutics* | Marie Wikström Lindholm, Ph.D., *Silence Therapeutics*
- 14.30 – 14.50 **Safety and immunogenicity of an optimized self-replicating RNA platform for low dose or single dose vaccine applications**
Andrew Geall, Ph.D., *Replicate Bioscience*
- 14.50 – 15.10 **ANQUR, the First-in-Human Phase 1 study of QRL-201 in ALS Advances to Dose-Range Finding using a Novel Population Pharmacokinetic Analysis**
Kasper Roet, Ph.D., *QurAlis*
- 15.10 – 15.30 **The Use of the Enhanced Delivery Oligonucleotide (EDO) Platform to Develop a Treatment of myotonic dystrophy 1 (DM1)- an Update on the FREEDOM Clinical Study**
James McArthur, Ph.D., *PepGen*
- 15.30 – 15.50 **Treating Dravet syndrome by upregulating SCN1A expression with zorevunersen (STK-001)**
Andreas Brunklaus, *University of Glasgow*
- 15.50 – 16.10 **Targeting APOC3 with Olezarsen: Results of Recent Phase 3 Trials**
Sotirios (Sam) Tsimikas M.D., *UCSD/Ionis Pharmaceuticals*
- 16.10 – 16.30 **RNAi mediated plasminogen lowering: Unlocking universal hemostasis**
Ali Murad, M.D., *Alnylam Pharmaceuticals*
- 16.30 – 17.00 **Closing Remarks**
- 19.00 – 23.00 **Annual Meeting Wrap Party**
(Pre-registration and additional fee required)

Location: Buda Castle Garden Bazaar

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