DAY 1 - SUNDAY, 19 OCTOBER 2025

0900-1105: PRE-CONFERENCE SESSION: Oligonucleotide Therapeutics Education Workshop
Co-chairs: Alex Garanto, Ph.D., Radboud Univ. Medical Center & Audrius Kilikevicius, Ph.D., Eli Lilly and Co.

0900-0925: **Rewriting Genetic Information in RNA using Oligonucleotide-directed Adenosine Deamination**, Peter Beal, Ph.D., UC Davis

0925-0950: **Next-generation mRNA vaccines: New RNA modalities and delivery systems,** Camilla Foged, Ph.D., University of Copenhagen

0950-1015: **Improving the Safety and Specificity of RNAi Therapeutics,** Maja M. Janas de Angelis, Ph.D., DABT, Alnylam Pharmaceuticals

1015-1040: **Safety assessment of therapeutic oligonucleotides with focus on off-targets,** Patrik Andersson, Ph.D., AstraZeneca

1040-1105: **Early clinical development of nucleic acid therapeutics,** Art Krieg, MD, Zola Therapeutics

This Education Workshop is sponsored by:

GINKGO DATAPOINTS

1115-1300 ***Hosted Lunch (for all participants)***
&
Mentorship Program Lunch (by invitation)

1300-1440: 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

1300-1325: Session Keynote:
From Molecules to Meaning: An Academic Journey to ASO Therapies for Duchenne Muscular Dystrophy, Aurelie Goyenvalle, Ph.D**.,** UMR-1179- University of Versailles, INSERM

1325-1340 **Accurate AI-Driven Prediction of Potent Therapeutic Fully Chemically Modified siRNAs,** Kathryn R. Monopoli, UMass Chan Medical School

1340-1355 **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

1355-1410 **Unraveling and controlling late-onset neurotoxicity of CNS-targeted antisense oligonucleotides through strategic chemical modifications**, Takayuki Kuroda MD, NucleoTIDE and PepTIDE Drug Discovery Center, Institute of Science Tokyo

1410-1425 **Fatty acid mediated uptake system enables improved oligo delivery to muscle and brain**, Argimiro Mayoral-Olmos, ARTHEx Biotech

1425-1440 **Immunoengineering of light-inducible RNA agonists enables spatiotemporal control of RIG-I activation for cancer immunotherapy,** Sandra A. Lewash**,** University Hospital Bonn

1440-1530 ***Refreshment Break***

1530-1645 OPENING SESSION AND KEYNOTE ADDRESS
Chair: Richard Geary, Ph.D., Ionis Pharmaceuticals, OTS President

1530-1545 Welcome and Opening Remarks
Richard Geary, Ph.D., Ionis Pharmaceuticals, OTS President

1545-1645 Keynote Presentation:
The Remarkable Versatility of Splice-Switching ASOs
Adrian R. Krainer, Ph.D., Professor, St. Giles Foundation - Co-Leader, Cancer Center Program

1645-1815: 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

1645-1930 ***Welcome Reception with Exhibitors (no Poster Hosting)***

2000-2200 ***Early Career Scientist Social Meet Up (RSVP, for students only)***

Location: EXTRA Bar - Klauzál u. 15, 1072 Budapest | https://extrabudapest.com/en

Sponsored by: CHEMGENES

DAY 2 - MONDAY, 20 OCTOBER 2025

0830-0855: Special Guest Talk - **Familial Chylomicronemia, a Rare but Devastating Disease given Hope through Oligonucleotide Therapy,** Alan Brown, MD, Advocate Aurora Health

0900-1050: SESSION I: Chemistry, Mechanism and Delivery (I)
Co-chairs: Steven F. Dowdy, Ph.D., UCSD School of Medicine & Ian Huggins, Ph.D., Ionis Pharmaceuticals

0900-0925: Click-based targeting and immunology of oligonucleotides, Thomas Carell, Ph.D., Ludwig-Maximilians-Universität (LMU) München

0925-0950: Life After Escape: Imaging Sorting, Action, and Degradation of Therapeutic RNAs in the Cytosol, Anders Wittrup, Ph.D., Lund University

0950-1005: **Novel ligands for cell selective gene silencing**, Vadim Dudkin, Souffle Therapeutics

1005-1020: **The X-Zyme Platform: Expanding RNA silencing with precise & autonomous allele specific knockdown,** Joel Kaye Ph.D., 1E Therapeutics

1020-1035: **Breaking the "2'-Hydroxyl Barrier" in CRISPR/Cas Editing Systems**, Abhishek Arora, McGill University

1035-1050: **Mechanism of Action and Nof1 Compassionate Use Case Reports of a Novel mRNA Cancer Vaccine Platform**, Bradley T. Sorenson, MBA, Providence Therapeutics

1050-1115 ***Refreshment break***

1115-1315 SESSION II: Pre-clinical (early stage)
Co-chairs: Martin A. Maier, Ph.D., Alnylam Pharmaceuticals & Reka Haraszti, MD, Ph.D., Universitätsklinikum Tübingen

1115-1140: Targeting Regulatory RNAs with Antisense Oligonucleotides for the Potential Treatment of SYNGAP1-Related Disorders, Dan Tardiff, Ph.D., CAMP4

1140-1205: Targeting splicing factors in cancer and diseases, Olga Anczukow, Ph.D., The Jackson Laboratory for Genomic Medicine

1205-1230: Splicing modulation therapy for inherited retinal diseases, Rob Collin, Ph.D., Radboud University Medical Center

1230-1245: **A Combinatorial siRNA Treatment for Fibrodysplasia Ossificans Progressiva,** Julia Alterman, Ph.D., UMass Chan Medical School

1245-1300: **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

1300-1315: **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

1315-1430 Hosted Lunch (for all participants)

1430-1630: SESSION III: Pre-clinical (late stage)
Co-chairs: Shalini Andersson, Ph.D., AstraZeneca & Virginia Arechavala-Gomeza, Ph.D., Biobizkaia Health Research Institute

1430-1455: Answering Cajal's Challenge: Reactivating neurogenesis in the aged nervous system with ASOs, Don Cleveland, Ph.D., UC San Diego

1455-1520: Divalent siRNAs for Therapeutic Gene Silencing in the Central Nervous System, Corrie Gallant-Behm, Ph.D., Atalanta Therapeutics

1520-1545: RNA Therapeutic Approaches for Heart Failure: Targeting Phospholamban, Adam Mullick, Ph.D., Ionis Pharmaceuticals

1545-1600: **VECTrans® platform achieves potent RNAi in the deep brain in NHP at minimal systemic dose,** Guillaume Jacquot, VECT-HORUS

1600-1615: **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

1615-1630: **Improved Preclinical and Clinical Performance of Complement Pathway-Targeting siRNA Conjugates,** Marc Abrams Ph.D., Sanegene Bio

1630-1700 POSTER-FIRE SESSION I
Chair: Shuling Guo, Ph.D., Vertex

1630-1633: **Time-resolved live-cell imaging of cytosolic RNA localization and fate**, Johanna M. Johansson, Lund University

1633-1636: **Chemical Modification of CRISPR-Cas12a Guide RNAs Towards Carrier-Free Delivery of CRISPR Therapeutics**, Michael P. Cunningham, McGill University

1636-1639: **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

1639-1642: **Binding to PDGFRα 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

1642-1645: **Cardiac and skeletal muscle delivery of biotherapeutics with a blood vessel epicardial substance-targeting peptide**, Biaobiao Wang, Tianjin Medical University

1645-1648: **Dual-Peptide Strategy for Delivery of Therapeutic Oligonucleotides in Erythropoietic Protoporphyria**, Angel Eduardo Santorelli Villamizar Ph.D., ETH Zurich

1700-2000 Poster Session – Reception I (Odd Numbered Posters Hosted)

DAY 3 - TUESDAY, 21 OCTOBER 2025

0830-1030: SESSION IV: Chemistry, Mechanism and Delivery (II)
Co-chairs Anastasia Khvorova, Ph.D., UMASS Chan Medical School/RTI & Garth Kinberger, Ph.D., Atalanta Therapeutics

0830-0855: Oligonucleotide-barcoded library for in vivo screening of tissue-targeting peptide conjugates, Samuel Hildebrand, Graduate Student, UMass Chan Medical School

0855-0920: Decoding Argonaute: Structural Insights into Human Ago2 Catalysis, Ian J. MacRae, Ph.D., The Scripps Research Institute

0920-0945: 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)

0945-1000: **SORT1-Mediated Delivery of siRNA to the Central Nervous System**, Lucas Siow, Ph.D., ProteinQure

1000-1015: **Enhancing CNS Delivery of Therapeutic Oligonucleotides with AQP4 Facilitator**, Kotaro Yoshioka MD, Ph.D., Institution of Science Tokyo, Japan

1015-1030: **Optimizing High-DAR Antibody-siRNA Conjugates: Overcoming Clearance Liabilities to Enhance Extrahepatic Gene Silencing,** Michael Cochran, Avidity Biosciences

1030-1100 Refreshment break

1100 – 1230: 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:
Prof. Wojciech Stec
Lifetime Achievement Award 2025 – Posthumous Award

11.15-12.30:
Brett Monia, Ph.D., CEO, *Ionis Pharmaceuticals*
Lifetime Achievement Award 2025
Pioneering RNA-Targeted Oligonucleotide Therapeutics: A 35-Year Journey

1230-1445 Hosted Lunch (for all participants)

Lunch Sponsored by:

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1245-1425: SESSION VI (Lunch Session): Advancing Oligonucleotide Manufacturing Towards a New Era of Therapeutics
Chair: David Butler, Ph.D., Hongene Biotech

1245-1310: **Revolutionizing RNAi Therapeutics Manufacturing with Enzymes,** Stefan Lutz, Ph.D., Codexis

1310-1335: **Exploring Biocatalytic Syntheses of Oligonucleotides**, Roumen Radinov, Ph.D., Alnylam Pharmaceuticals

1335-1400: **Scalable Membrane Enabled One-Pot Liquid-Phase Oligonucleotide Synthesis**, Steven Ferguson, Ph.D., University College Dublin

1400-1425: **Advancing siRNA Manufacturing: From Conventional to Thermostable Enzymatic Ligation**, Chris Li, Ph.D., Hongene Biotech

1445 – 1635: **SESSION VII:** DNA/RNA Editing
Co-Chairs: Keith Gagnon, Ph.D., Wake Forest University & Blythe Sather, Ph.D., Tune Therapeutics

1445-1510: **Engineering insights into RNA trans-splicing platforms,** Aravind AsoKan, Ph.D., Duke University

1510-1535: **Discovery and development of CRISPR-associated transposases for RNA-guided gene insertion,** Sam Sternberg, Ph.D., Columbia University

1535-1550: **Improved RNA base editing with guide RNAs mimicking highly edited endogenous ADAR substrates,** Rui Zhang Ph.D., Sun Yat-Sen University

1550-1605: **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

1605-1620: **Developing AIMer-Based RNA Editing Technology to Correct a Nonsense Mutation in the Lung,** Jack D. Godfrey Ph.D., Wave Life Sciences

1620-1635: **Optimized RESTORE+ oligonucleotides for an efficacious and safe RNA base editing treatment for Alpha-1 antitrypsin deficiency,** Tobias Merkle, AIRNA Bio Germany

1635-1700: **POSTER-FIRE SESSION II**
Chair: Bruno Godinho, Ph.D., Atalanta Therapeutics

1635-1638: **Diligent design of Brainshuttle™-antisense oligonucleotide conjugates for brain delivery,** Tatjana Sela, Roche

1638-1641: **Antisense Oligomers for Modulation of Enterococcus faecalis metabolism of L-Dopa in Parkinson's Disease**, Vincent Lau, Helmholtz Centre for Infection Research

1641-1644: **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

1644-1647: **Pharmacokinetics and endosomal escape of GalNAc-siRNAs**, Fang-Ching Chao, AstraZeneca

1647-1650: **Oligonucleotide aptamers to improve ASO therapeutics,** Puri Fortes Ph.D., CIMA/UNAV

1650-1653: **Selection of DNA Aptamers for TDP-43 to Inhibit Protein Aggregation in Amyotrophic Lateral Sclerosis,** Daniel Knight, Carleton University

1700-2000 ***Poster Session – Reception II (Even Numbered Posters Hosted)***

DAY 4 - WEDNESDAY, 22 OCTOBER 2025

0830-1030: SESSION VIII: Rare Diseases
Chair: Annemieke Aartsma-Rus, Ph.D., Leiden University & Richard Geary, Ph.D., Ionis Pharmaceuticals & President, OTS

0830-0855: Zebronkysen: Advancing a Personalized Antisense Therapy for CLN3 Batten Disease from Discovery to the Clinic, Michelle Hastings, Ph.D., University of Michigan Medical School

0855-0910: **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

0910-0925: **Anti-FGF2 Aptamer Therapy in Achondroplasia,** Yoshikazu Nakamura, Ph.D., IMSUT University of Tokyo, RIBOMIC, Inc.

0925-0940: **N-of-1 personalized medicine for CMT2S: From patient-specific preclinical modeling to first-in-human dosing,** Sandra P. Smieszek, Ph.D., Vanda Pharmaceuticals

0940-0955: **Small binding RNAs (sbRNAs) as a novel therapeutic strategy for allele-selective silencing in Huntington's Disease,** Megan Blewett, Ph.D., Iris Medicine

0955-1020 Patient Experience: Nusinersen, Steven Jones, SMA Patient Advocate

1020-1030 Q&A

1030-1100 Refreshment break

1100-1315: SESSION IX: Awards Session II
Co-Chair: Richard Geary, Ph.D., Ionis Pharmaceuticals & President, OTS & Masad J. Damha, Ph.D., McGill University

1100 - 1105 ***Award Announcements***
Travel Grant Awardees, Poster Awards, Next Gen Session - Best Talk

1105 - 1115 ***President's Special Awards***
David Corey, Ph.D., UT Southwestern
Marc Lemaitre, Ph.D., ML_Consulting

1115-1135 ***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

1135-1155 ***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, PhD Candidate, UT Southwestern Medical Center

1155-1215 ***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

1215-1235 ***Mary Ann Liebert publishers Inc Young Investigator Award***

Harnessing antisense oligonucleotide for the treatment of neurological disorders, Hien Tran Zhao, Ph.D., Ionis Pharmaceuticals, Inc.

1235-1255 ***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

1255-1315 ***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

1315-1430 ***Hosted Lunch (for all participants)***

1430-1630 **SESSION X:** Clinical
Co-chairs: Art Krieg, MD, Zola Therapeutics & Marie Wikström Lindholm, Ph.D., Silence Therapeutics

1430-1450: **Safety and immunogenicity of an optimized self-replicating RNA platform for low dose or single dose vaccine applications,** Andrew Geall, Ph.D., Replicate Bioscience

1450-1510: **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

1510-1530: **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

1530-1550: **Treating Dravet syndrome by upregulating SCN1A expression with zorevunersen (STK-001),** Andreas Brunklaus, University of Glasgow

1550-1610: **Targeting APOC3 with Olezarsen: Results of Recent Phase 3 Trials**, Sotirios (Sam) Tsimikas MD, UCSD/Ionis Pharmaceuticals

1610-1630: **RNAi mediated plasminogen lowering: Unlocking universal hemostasis, Ali Murad, MD**, Alnylam Pharmaceuticals

1630-1700 Closing remarks

1900-2400 **Annual Meeting Wrap Party (***Pre-registration and additional fee required)***:**

Location: **Buda Castle Garden Bazaar**

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