



Sunday, October 22	
8.00	<b>Registration Open</b> <i>Welcome Coffee – please enjoy breakfast at your hotel</i>
9.00 – 11.00	<b>Preconference Session</b> <b>Oligonucleotide Therapeutics Education Workshop:</b> <b>Innovating and Commercializing the Next Generation of Oligonucleotide Therapeutics</b> Co-Chairs: Alan Horsager, <i>PhD, Duet Therapeutics</i> Anna Pedrix Rosell, <i>PhD, Sixfold Bioscience</i>
9.00–9.05	<b>Introduction by Chairs</b>
9.05–9.20	<b>Wearing Two Hats: start up out of academia</b> Marcin Kortylewski, <i>PhD, City of Hope</i>
9.20–9.35	<b>Unlocking the Potential of Three-Stranded RNAi: A Journey into Switch Therapeutics</b> Dee Datta, <i>PhD, Switch Therapeutics</i>
9.35–9.50	<b>Venturing in RNA Entrepreneurship</b> Andrew Fraley, <i>PhD, Korro Bio</i>
9.50–10.05	<b>Pharma partnering to progress your oligonucleotide therapy</b> Rabia Ahmad, <i>Glaxo Smith Kline</i>
10.05–11.00	<b>Panel Discussion / Q&amp;A</b> <i>All presenters</i>
11.00 – 13.00	<b>Hosted Lunch Break</b>
13.00–15.00	<b>Preconference Session</b> <b>Next-Gen Early Career Scientist Session</b> Co-chairs: Jillian Belgrad, <i>MD/PhD Candidate, RTI, UMass Chan Medical School</i> Krystal Johnson, <i>PhD, UT Southwestern Medical Center, Corey Lab</i>
13.00–13.05	<b>Welcome &amp; Introduction</b>
13.05–13.30	<b>Session Keynote: Peptide-mediated delivery of RNA therapeutics</b> Taavi Lehto, <i>PhD, University of Tartu   Institute of Technology</i>
13.30–13.45	<b>Antisense oligonucleotides correct mis-splicing of neuronal microexons associated with autism spectrum disorders and schizophrenia: a new potential therapeutic strategy</b> Ainhoa Martinez, <i>Centro de Biología Molecular Severo Ochoa</i>
13.45–14.00	<b>Synthesis, Characterization, and Biological Evaluation of Novel 3-Component Lipid Nanoparticles for mRNA Delivery</b> Joshua Robinson, <i>University of Texas Southwestern Medical Center</i>
14.00–14.15	<b>TERA: A High-Throughput AI-Powered Platform for Optimizing the Durability of RNA Therapeutics</b> Cameron Thorpe, <i>ElevenTx</i>
14.15–14.30	<b>2'MOE and LNA gapmer ASOs are potent modulators of TLR7/8 sensing</b> Arwaf Alharbi, <i>Hudson Institute of Medical Research</i>



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14.30–14.45	<b>NATURA, a label-free and high-throughput assay platform to assess functional delivery of nucleic acid therapeutics from cell culture to animals</b> Tommaso Tabaglio, <i>Institute of Molecular and Cell Biology, A*STAR</i>
14.45–15.00	<b>miRNA disruption using Steric-Blocking Oligonucleotides as a novel therapeutic strategy for STXBP1-related disorders</b> Alex Felix, <i>PhD, University of Pennsylvania Perelman School of Medicine</i>
15.00	<i>Voting for Best Talk of Next Gen Session</i>
<b>15.00–15.30</b>	<b>Refreshment Break</b>
<b>15.30–16.45</b>	<b>Annual Meeting Opening &amp; Keynote Session</b>
15.30–15.45	<b>Welcome &amp; Opening Remarks</b> David Corey, <i>PhD, OTS President, UT Southwestern Medical Center</i> Alex Garanto, <i>PhD, Organizing Co-Chair, Radboud University Medical Center</i> Punit Seth, <i>PhD, Organizing Co-Chair, Alnylam Pharmaceuticals</i>
15.45–16.45	<b>Keynote Presentation</b> <i>Base Editing and Prime Editing: Correcting Mutations that Cause Genetic Disease in Cells, Animals, and Patients</i> David Liu, <i>PhD, Harvard, Broad Institute</i>
<b>16.45–19.30</b>	<b>Welcome Reception with Exhibitors</b> <i>No host Poster Viewing</i>
<b>20.00–23.00</b>	<b>STUDENTS ONLY – Networking Event   Early Career Scientist Social Meet Up</b> <i>Location: GATS Restaurant Barcelona (drinks &amp; bites)</i>  <i>(this event is limited to students ONLY &amp; RSVP was required / No Walk ins can be accommodated due to space limits)</i>  <b>Sponsored by</b>  



## Meeting Agenda

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Monday, October 23	
8.00	<b>Registration Open</b> <i>Welcome Coffee – please enjoy breakfast at your hotel</i>
8.30–10.30	<b>Session I: Chemistry and Delivery</b> Co-Chairs: Erin McConnell, <i>PhD, Carleton University</i> Vasant Jadhav, <i>PhD, Alnylam Pharmaceuticals</i>
8.35–9.00	<b>Targeting Transferrin Receptor to Transport ASOs over the BBB</b> Sarah Devos, <i>PhD, Denali Pharmaceuticals</i>
9.00–9.15	<b>Evaluating muscle homing peptides for improved uptake of phosphorodiamidate morpholino oligomers (PMOs) for Duchenne Muscular Dystrophy therapy</b> Anne-Fleur Schneider, <i>Leiden University Medical Centre</i>
9.15–9.40	<b>Advancing Novel Approaches Towards the Design of Therapeutic siRNAs</b> Mark Schlegal, <i>PhD, Alnylam Pharmaceuticals</i>
9.45–10.00	<b>Unprecedented, tissue- and modality-agnostic potency and durability improvement by targeted endosomal escape in vitro and in vivo: an update on Sapreme's unique endosomal escape enhancer platform</b> Miriam Bujny, <i>PhD, Sapreme Technologies</i>
10.00–10.15	<b>Novel thiomorpholino oligonucleotides (TMOs) as a robust next generation platform for splice switching antisense therapies</b> Rakesh Veedu, <i>PhD, Murdoch University</i>
10.15–10.30	<b>Chemical Evolution of a Highly Active DNAzyme with Allele-Specific Gene Silencing Activity</b> John Chaput, <i>University of California, Irvine</i>
10.30–11.00	<b>Refreshment Break</b>
11.00–13.00	<b>Session II: Pre-Clinical I (Early Stage)</b> Co-Chairs: Rob Collin, <i>PhD, Radboud University Medical Center</i> Holly Kordasiewicz, <i>PhD, Ionis Pharmaceuticals</i>
11.00–11.25	<b>Modulation of RNA splicing with antisense oligonucleotides for the treatment of rare disease</b> Michelle Hastings, <i>PhD, University of Michigan Medical School</i>
11.25–11.40	<b>Global rescue of TDP-43 mediated mis-splicing using (CA)<sub>n</sub> ASOs</b> Peter Hagedorn, <i>PhD, Roche Innovation Center Copenhagen</i>
11.40–12.05	<b>Targeting RNA-misprocessing in neurological diseases with RNA-targeting CRISPR-Cas systems</b> Lorea Blazquez, <i>PhD, Basque Foundation for Science</i>
12.05–12.20	<b>Correlation of single-cell CRISPR off-targets with the cellular chromatin and gene expression state</b> Alexandra Madsen, <i>AstraZeneca</i>



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12.20–12.45	<b>Prion protein-lowering ASOs: assessing single cell activity and efficacy in diverse models</b> Sonia Vallabh, <i>PhD, Broad Institute</i>
12.45–13.00	<b>Allele-selective small interfering RNA-mediated von Willebrand factor silencing in murine bleeding and thrombosis models</b> Yvonne Jongejan, <i>MSc, Leiden University Medical Center</i>
<b>13.00–14.30</b>	<b>Hosted Lunch Break</b>
<b>14.30–16.30</b>	<b>Session III: Pre Clinical II (Late Stage)</b> Co-Chairs: Bruno Godinho, <i>PhD, Atalanta Therapeutics</i> Aur�lie Goyenvalle, <i>PhD, DR, INSERM</i>
14.30–14.55	<b>RNA: a new frontier of precision medicine to reverse DNA methylation abnormalities in cancer and beyond</b> Annalisa Di Ruscio, <i>PhD, BIDMC</i>
14.55–15.20	<b>To the tumor and beyond: tales of a holistic microRNA delivery vehicle</b> Andrea Kasinski, <i>PhD, Purdue University</i>
15.20–15.45	<b>Development of a BNA modified ASO targeting alpha-synuclein for multiple system atrophy</b> Helene Tran, <i>PhD, Servier</i>
15.45–16.00	<b>Delivering siRNA to the skin: Novel chemistry and routes of administration for improved PK/PD</b> Julia Alterman, <i>PhD, University of Massachusetts Chan Medical School</i>
16.00–16.15	<b>anti-miR-22 therapy for NAFLD and obesity: from target discovery to clinical development</b> Riccardo Panella, <i>Center for RNA Medicine</i>
16.15–16.30	<b>Lipophilic conjugation ameliorates antimiR pharmacokinetic and pharmacodynamic (PK/PD) properties</b> Andrea Garc�a-Rey, <i>ARTHEX Biotech S.L</i>
<b>16.30–17.00</b>	<b>Session IV: Presentation of Awards   N of 1</b> Chair: David Corey, <i>PhD, OTS President, UT Southwestern Medical Center</i>
16.30–16.45	<b>Presentation of 2023 Society Advocacy Award</b> Julia Vitarello, <i>Mila's Mom / Mila's Miracle Foundation</i>  <b>Presentation of 2023 Society Paper of the Year Award – Basic Research Category</b> Sonia Vallabh, <i>Broad Institute</i>  <i>A single-cell mpa of antisense oligonucleotide activity in the brain</i> <a href="https://pubmed.ncbi.nlm.nih.gov/37188501/">https://pubmed.ncbi.nlm.nih.gov/37188501/</a>
16.45–17.00	<b>Accelerating Oligonucleotide Therapeutics Platform Solutions: From N of Many to N of 1</b> Sadik Kassim, <i>PhD, Danaher Corporation</i>
<b>17.00-20.00</b>	<b>Poster Session I Reception (drinks &amp; bites)</b> <i>ODD numbered posters hosted</i>



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Tuesday, October 24	
8.00	<b>Registration Open</b> <i>Welcome Coffee – please enjoy breakfast at your hotel</i>
8.30–10.30	<b>Session V: DNA/RNA Editing</b> Co-chairs: Keith Gagnon, <i>PhD, Wake Forest University School of Medicine</i> Pinar Akcakaya, <i>PhD, AstraZeneca</i>
8.30–8.55	<b>RNA Base Editing with endogenous ADAR</b> Thorsten Stafforst, <i>PhD, University of Tuebingen</i>
8.55–9.20	<b>Using Structure-Aware Machine Learning for Chemical Generalization in Guide RNA Design</b> Shannon Cosgrove, <i>PhD, Korro Bio</i>
9.20–9.45	<b>Molecular Technologies for Genome Editing and Cell Control</b> Omar Abudayyeh, <i>PhD, MIT</i>
9.45–10.00	<b>Enabling ADAR Editing at Disfavored Sites</b> Agya Karki, <i>PhD, University of California, Davis</i>
10.00–10.15	<b>Engineering an Improved Inhibitor of 53BP1 to Enhance CRISPR HDR Efficiency</b> Mark Behlke, <i>MD, PhD, Integrated DNA Technologies</i>
10.15–10.30	<b>Recent Advances in Non-Viral Delivery of Prime Editors Toward the Treatment of Patients with Glycogen Storage Disease Type 1b (GSD1b)</b> Seth Alexander, <i>PhD, Prime Medicine</i>
10.30–11.00	<b>Refreshment Break</b>
11.00–13.00	<b>Session VI: Awards Session I / Lifetime Achievement Awards</b> Chair: Masad Damha, <i>PhD, McGill University, Awards Chair for Board of Directors</i>
11.00–11.45	<b>Sudhir Agrawal, D.Phil</b> <b>2022 Lifetime Achievement Award</b> Evolution of Chemistry in the Development of RNA Therapeutics: Three decades later
11.45–12.30	<b>Cy Stein, PhD</b> <b>2023 Lifetime Achievement Award</b> Phosphorothioates and me: Reflections on a life time of experimentation
12.30–13.00	<b>Q&amp;A</b>
13.00–14.30	<b>Hosted Lunch Break</b>
14.30 - 15.30	<b>Session VII: Short Talks ONLY</b> Co-Chairs: Alex Garanto, <i>PhD, Radboud University Medical Center</i> Punit Seth, <i>PhD, Alnylam Pharmaceuticals</i>
14.30–14.45	<b>Generalizable anchor aptamer strategy for loading nucleic acid therapeutics on exosomes</b> Gang Han, <i>Tianjin Medical University</i>

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14.45 – 15.00	<b>Live cell imaging of LNP-mediated endosomal escape – towards mechanistic understanding of RNA delivery</b> Anders Wittrup, <i>Lund University</i>
15.00 – 15.15	<b>Engineered extracellular vesicles for delivery of therapeutic small RNA</b> Julia Rädler, <i>Karolinska Institute</i>
15.15 – 15.30	<b>TfR-targeted functional delivery of oligonucleotides to CNS tissues by local and systemic administration</b> Guillaume Jacquot, <i>PharmD, PhD, VECT-HORUS SAS</i>
<b>15.30 - 18.30</b>	<b>Poster Session II Reception</b> ( <i>drinks &amp; bites</i> ) <i>EVEN numbered posters hosted</i>



Wednesday, October 25	
8.00	<b>Registration Opens</b> <i>Welcome Coffee – please enjoy breakfast at your hotel</i>
8.30–10.30	<b>Session VIII: Awards Session II</b> Co-Chairs: David Corey, <i>PhD, UT Southwestern Medical Center, OTS President</i> Erin McConnell, <i>PhD, Carleton University</i>
8.30–8.45	<b>Presentation of Awards:</b> Next Gen Session: Best Talk Announcement Announcement of Poster Awards Travel Grant Awardees  <b>Presentation of the Paper of the Year Award – Late Discovery Category</b> Jinkuk Kim, <i>PhD, KAIST - Korea Advanced Institute of Science and Technology</i>  <i>A framework for individualized splice-switching oligonucleotide therapy</i> <a href="https://pubmed.ncbi.nlm.nih.gov/37438524/">https://pubmed.ncbi.nlm.nih.gov/37438524/</a>
8.45 – 9.05	<b>Dr. Alan M. Gewirtz Memorial Scholarship Award for Graduate Students</b> <b>Dynamic shuttling of AG02 to the nucleus may relieve microRNA repression</b> Krystal Johnson, <i>PhD, UT Southwestern Medical Center, Corey Lab</i>
9.10 – 9.30	<b>Mary Ann Liebert publishers, Inc. Young Investigator Award</b> <b>Developing next-generation synthetic nucleic acid-based therapeutics</b> Raman Bahal, <i>PhD, University of Connecticut</i>
9.30 – 9.50	<b>Dr. Alan M. Gewirtz Memorial Scholarship Award for Postdocs/Jr Professionals</b> <b>Chemical Engineering of Therapeutic siRNAs: the Role of Non-natural Backbone</b> Dan O'Reilly, <i>PhD, University of Massachusetts Chan Medical School</i>
9.50 –10.30	<b>Q&amp;A</b>
10.30 - 11.00	<b>Refreshment Break</b>
11.00-13.00	<b>Session IX: Clinical</b> Co-Chairs: Arthur Krieg, MD, RTI, <i>UMASS Chan Medical School</i> Takanori Yokota, MD, PhD, <i>Tokyo Medical and Dental University</i>
11.00–11.20	<b>Positive Results From a First-In-Human Study Support Continued Development of PGN-EDO51 for the Treatment of Duchenne Muscular Dystrophy (DMD)</b> Jaya Goyal, <i>PhD, PepGen</i>
11.20–11.40	<b>Antibody Oligonucleotide Conjugates (AOCs) Development for Muscle Disease</b> Mike Flanagan, <i>PhD, Avidity Biosciences</i>
11.40–12.00	<b>A first-in-human study of ARO_RAGE, and RNAi therapy designed to silence pulmonary RAGE expression</b> Mark O'Carroll, <i>PhD, Arrowhead Pharmaceuticals</i>
12.00–12.20	<b>ALN-APP, the first investigational RNAi therapeutic in development for Alzheimer's disease</b> Kirk Brown, <i>PhD, Alnylam Pharmaceuticals</i>



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12.20–12.40	<b>mRNA-3705 Therapy for Methylmalonic Acidemia: Interim Data From a Phase 1/2 Study</b> Patrick Burkett, MD, PhD, Moderna Therapeutics
12.40–13.00	<b>Long Term Safety and Efficacy of Antisense Oligonucleotide Therapy to Pre-kallikrein in Patients with Hereditary Angioedema (HAE)</b> Kenneth Newman, MD, Ionis Pharmaceuticals
13.00–14.30	<b>Hosted Lunch Break</b>
14.30–16.10	<b>Session X: Late Breaking Talks</b> Co-Chairs: Alex Garanto, PhD, Radboud University Medical Center Punit Seth, PhD, Alnylam Pharmaceuticals
14.30–14.55	<b>BigRNA: A giga-scale neural network for discovering RNA biology, investigating pathogenic variants and designing oligonucleotides</b> Brendan Frey, PhD, Deep Genomics
14.55–15.20	<b>RNase H1 Involvement in cEt ASO-Induced Toxicity in the CNS</b> Jill A Hsiao, Ionis Pharmaceuticals
15.20–15.45	<b>Deep learning-based design of cell-targeting ligands enables functional siRNA delivery in multiple extrahepatic tissues</b> Kyler Lugo, PhD, Nosis Bio
15.45–16.14	<b>Closing Remarks</b>
19.00–23.00	<b>Annual Meeting Wrap Party</b> Barcelona Maritime Museum separate RSVP & purchase required  Sponsored by  