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| **Sunday, October 2(Day ONE)** | | | | | |
| **8.00** | | **Registration Opens / Welcome Coffee** | | | |
| **9.00-11.00** | | ***Preconference Session: Educational Workshop***  **Nucleic Acid Therapeutics – The Basics**  Chair: Annemieke Aartsma-Rus, PhD, *Leiden University Medical Center* | | | |
| **9.00-9.30** | | | | **Oligonucleotides 101**  Annemieke Aartsma-Rus, PhD, *Leiden University Medical Center* | |
| **9.30-9.55** | | | | **mRNA Manufacturing and Analytics: An Overview of Current Methods** Ramsey Majzoub, PhD, *Intellia Therapeutics, Inc.* | |
| **9.55-10.20** | | | | **Beyond Double Strand Breaks: Base Editing, Prime Editing and RNA Editing** Yi Yu, PhD, *Beam Therapeutics* | |
| **10.20-10.45** | | | | **Toxicology and DMPK Assessment of siRNA Therapeutics**  Joseph Dybowski, MPH, PhD, *Alnylam Pharmaceuticals* | |
| **10.45-11.00** | | | | **Q&A Panel** | |
| **11.00-13.00** | | **Lunch Break** Lunch on Own | | | |
| **13.00-15.30** | | ***Preconference Session:* Next-Gen Early Career Scientist Meeting**  Co-chairs:  Hassan Fakih, PhD, *RTI, UMass Chan Medical School* Chantal Ferguson, MD/PhD Candidate, *RTI, UMass Chan Medical School* | | | |
| **13.00-13.05** | | | | **Welcome & Introduction** | |
| **13.05-13.35**  **13.35-14.05** | | | | ***Session Keynote Speaker:***  **Oligonucleotide Backbone Structure Modulation - A Beginning of my Journey in RNA Therapeutics World** Ken Yamada, PhD, *RNA Therapeutics Institute, UMass Chan Medical School*  ***Session Keynote Speaker:***  **Building a Biotech From Scratch** Anna Perdrix Rosell, PhD, *Sixfold Biosciences* | |
| **14.05-14.20** | | | | **RIG-I adjuvanted mRNA vaccines**  Christine Wuebben, PhD, *Institute of Clinical Chemistry and Clinical Pharmacology, University of Bonn* | |
| **14.20-14.35** | | | | **Enhancing Delivery Efficiency of RNA Based Therapeutics via Repurposing of Cationic Amphiphilic Drugs**  Cristina Muntean, *Ghent University* | |
| **14.35-14.50** | | | | **Investigating consequences of nuclear AGO2 in colon cancer model systems**  Krystal Johnson, *UT Southwestern Medical Center* | |
| **14.50-15.05** | | | | **Origins of the increased affinity of phosphorothioate modified therapeutic nucleic acids for proteins**  Malwina Hyjek-Skladanowska, PhD, *International Institute of Molecular and Cell Biology* | |
| **15.05-15.20** | | | | **Di-valent siRNA Mediated Silencing of MSH3 Blocks Somatic Repeat Expansion in Mouse Models of Huntington’s Disease**  Daniel O'Reilly, PhD, RNA Therapeutics Institute, *UMass Chan Medical School* | |
| **15.20-15.30** | | | | **Voting for Best Talk** | |
| **15.30-17.30** | | **Welcome Reception with Exhibitors *Open Poster Viewing*** | | | |
| **17.30**  **19.00-21.30** | | **Dinner On Own**  **Early Career Scientist Social Event *Sponsored by ChemGenes***  Location: [Culinary Dropout](https://www.culinarydropout.com/locations/phoenix-az/) 5632 N 7th St, Phoenix, AZ 85014 | | | |
| **Monday, October 3 (Day TWO)** | | | | | |
| **7.30-8.30** | | **Breakfast Served – Registration Open** | | | |
| **8.40-9.00** | | **Welcome & Opening Remarks**  David Corey, PhD, *OTS President*Keith T. Gagnon, PhD, *Meeting Co-Chair* | | | |
| **9.00-11.00** | | **Session I: Nucleic Acid Chemistry - Dedicated to 100 Years of Gobind Khorana** Masad J. Damha, PhD, FCIC, *McGill University* | | | |
| **9.00-9.25** | | | | **Living in the World of RNA Therapeutics through Chemistry** Muthiah Manoharan, PhD, *Alnylam Pharmaceuticals* | |
| **9.25-9.50** | | | | **Renaissance of P(V) Oligonucleotide Chemistry** Phil Baran, PhD, *Scripps* *Research Institute* | |
| **9.50-10.15** | | | | **Photoresponsive Nucleic Acid Conjugates for RNA interference**  Jean Paul Desaulniers, PhD, *Ontario Tech University* | |
| **10.15-10.30** | | | | **Photocaged 5′ cap analogs for optical control of mRNA translation in cells**  Florian Peter Weissenböck, PhD, *Institute of Biochemistry,* *University of Münster* | |
| **10.30-10.45** | | | | **Understanding Guide RNA 2'-Hydroxyl Ribose Requirements for CRISPR-Cas9 Gene Editing**  Adrian A. Pater, *Southern Illinois University* | |
| **10.45-11.00** | | | | **Reimagining the chemical space for nucleic acid therapeutics – Antisense oligonucleotides modified with stereoisomers of DNA**  Graeme C. Freestone, PhD, *Ionis Pharmaceuticals* | |
| **11.00-11.30** | | **Refreshment Break** | | | |
| **11.30-13.00** | | **Session II: Awards Session**  Chair: David Corey, PhD, *UT Southwestern* | | | |
| **11.30-11.40** | | | | ***Award Presentations:***  Best Talk Award – NextGen Session   Paper of the Year Awards  Science Outreach and Patient Advocacy Awards | |
| **11.40-12.00** | | | | ***Dr. Alan M. Gewirtz Memorial Scholarship Award for Graduate Students* A Tale of Two Aptamers: SARS-CoV-2 binding DNA aptamers for diagnostics and therapeutics** Lucy F. Yang, PhD, *University of Washington* | |
| **12.00-13.00** | | | | ***OTS Lifetime Achievement Award 2020***  **From Thalassemia to Duchenne Muscular Dystrophy** Ryszard Kole, PhD, *University of North Carolina, Chapel Hill* | |
| **13.00-14.30** | | **Lunch Break**  *Hosted lunch* | | | |
| **14.30-16.00** | | **Session III, Keynote: 20th Anniversary of OTS**  Co-Chairs: Annemieke Aartsma-Rus, PhD, *LUMC* Keith T. Gagnon, PhD, *Southern Illinois University* | | | |
|  | | | | Join us for this special session which will include founders of the OTS and past Board of Directors members who were there during the ‘early days’ of the society. Panelists include: | |
|  | | | | -Masad Damha, PhD, *McGill University*  -Gunther Hartmann, MD, PhD, *University Hospital Bonn*  -Anastasia Khvorova, PhD, *RTI, UMass Chan Medical School*  -Art Krieg, MD, *RTI, UMass Chan Medical School*  -Mano Manoharan, PhD, *Alnylam Pharmaceuticals* | -Brett Monia, PhD*, Ionis Pharmaceuticals*  -John Rossi, PhD, *City of Hope*  -Georg Sczakiel, PhD, *University of Luebeck*  -Hermona Soreq, PhD, *The Hebrew University of Jerusalem*  -Cy Stein, MD, PhD, *Retired* |
| **16.00-18.30** | | **Poster Session I with Lite Bites**  *ODD numbered posters hosted* | | | |
|  | | ***Dinner On Own*** | | | |
| **Tuesday, October 4 (Day THREE)** | | | | | |
| **7.30-8.30** | | **Breakfast Served – Registration Open** | | | |
| **8.30-10.30** | | **Session IV: Preclinical** Co-chairs: Martin Maier, PhD, *Alnylam Pharmaceuticals*  Aurélie Goyenvalle, PhD, DR *Inserm*, *UMR 1179 INSERM-UVSQ* | | | |
| **8.30-8.55** | | | | **Phosphoryl-guanidine backbone chemistry: understanding its impact on stereopure oligonucleotides** Chandra Vargeese, PhD, *Wave Life Sciences* | |
| **8.55-9.20** | | | | **Gene Silencing in the Lung by Locally Delivered ASOs and siRNAs** Jonathan Watts, PhD, *RNA Therapeutics Institute, UMass Chan Medical School* | |
| **9.20-9.45** | | | | **Centyrin-targeted Gys1 siRNA conjugates: A novel therapeutic modality for the treatment of Pompe disease** Karyn O'Neil, PhD, *Aro Biotherapeutics* | |
| **9.45-10.00** | | | | **Towards clinical application of antisense oligonucleotides for the treatment of Stargardt disease**  Dyah Karjosukarso, PhD, *Dept. of Human Genetics,* *Radboud University Medical Center* | |
| **10.00-10.15** | | | | **Antisense-oligonucleotide therapy targeting BRAF alleviates pediatric brain tumors with intractable epilepsy**  Jeong Ho Lee, MD, PhD, *KAIST* | |
| **10.15-10.30** | | | | **Therapeutic Efficacy of SNCA-targeting siRNAs in a Synucleinopathy Mouse Model**  Pascaline Aimé-Wilson, PhD, *Regeneron Pharmaceuticals* | |
| **10.30-11.00** | | **Refreshment Break** | | | |
| **11.00-13.00** | | **Session V: AI/Machine Learning**  Co-chairs: Marie Wikström Lindholm, PhD, *Silence Therapeutics*  John Whittaker, PhD, *University of Cambridge* | | | |
| **11.00-11.15** | | | | **Session Introduction: What’s AI/ML Got to do With Oligonucleotide Therapeutics?** John Whittaker, PhD, *University of Cambridge* | |
| **11.15-11.40** | | | | **The Power and Pitfalls of Machine Learning in Early Stage Drug Discovery** Charlotte Deane, PhD,*University of Oxford / Exscientia* | |
| **11.40-12.05** | | | | **Human Genetics as an Enabler of RNAi Therapeutics** Paul Nioi, PhD, *Alnylam Pharmaceuticals* | |
| **12.05-12.30** | | | | **eSkip-Finder: A Machine Learning-based Web Application and Database to Identify Efficient Antisense Oligonucleotide Sequences for Exon Skipping** Toshifumi Yokota, PhD, *University of Alberta* | |
| **12.30-12.45** | | | | **Generative aptamer discovery using RaptGen**  Tatsuo Adachi, PhD, *RIBOMIC Inc.* | |
| **12.45-13.00** | | | | **RN.AI.Visions**  Lykke Pedersen, PhD, *Abzu*  Morten Lindow, PhD, *Roche Innovation Center Copenhagen* | |
| **13.00-14.30** | | **Hosted Lunch Break** | | | |
| **14.30-16.30** | | **FREE TIME**  ***Optional: Early Career Scientist Meetup at the Lazy River*** | | | |
| **16.30-19.00** | | **Poster Session II with Lite Bites**  *EVEN numbered posters hosted* | | | |
| **19.00** | | ***Dinner On Own*** | | | |
| **Wednesday, October 5 (Day FOUR)** | | | | | |
| **7.30-8.30** | | **Breakfast Served – Registration Open** | | | |
| **8.30-10.30** | | **Session VI: Genome & RNA Editing** Co-Chairs: Laura Sepp-Lorenzino, PhD, *Intellia Therapeutics*  Patrick Finn, Ph.D., *Moderna* | | | |
| **8.30-8.55** | | | | **mRNA as Medicine**  Patrick Finn, Ph.D., PhD, *Moderna* | |
| **8.55-9.20** | | | | **Harnessing mobile genetic elements to write DNA sequences with RNA** Cecilia Cotta-Ramusino, PhD, *Tessera Therapeutics* | |
| **9.20-9.45** | | | | **Special motif-carrying tRNA fragments cause Parkinson’s disease neuronal death by translational arrest** Hermona Soreq, PhD, *The Hebrew University of Jerusalem* | |
| **9.45-10.00** | | | | **Optimization strategies to achieve high efficiency gene editing in non-human primates using lipid nanoparticle delivery of mRNA**  Cassie Gorsuch, PhD, *Precision BioSciences Inc.* | |
| **10.00-10.15** | | | | **RNA base editing for the treatment of Alpha-1 antitrypsin deficiency**  Prashant Monian, PhD, *Wave Life Sciences* | |
| **10.15-10.30** | | | | **Evolution of Functional CRISPR Guide RNA Yields Variants with Distinct Activities**  Korie Bush, *Duke University* | |
| **10.30-11.00** | | **Refreshment Break** | | | |
| **11.00-12.45** | | **Session VII: Awards Session**  Chair: David Corey, PhD, *UT Southwestern* | | | |
| **11.00-11.05** | | **Announcement: Poster Award Winners** | | | |
| **11.05-11.25** | | | | ***Dr. Alan M. Gewirtz Memorial Scholarship Award for Postdocs* Optimizing ASO delivery in hiPSC-derived 2D and 3D neuronal models** Ronald Buijsen, PhD, *Leiden University Medical Center* | |
| **11.25-11.45** | | | | ***Mary Ann Liebert publishers, Inc. Young Investigator Award***  ***Small circular mRNA vaccines***  Guizhi (Julian) Zhu, PhD, *Virginia Commonwealth University* | |
| **11.45-12.45** | | | | ***OTS Lifetime Achievement Award 2021***  **30 Years of Making Sense Out of Antisense** Frank Bennett, PhD, *Ionis Pharmaceuticals* | |
| **12.45-14.15** | | **Lunch** | | | |
| **14.15-16.15** | **Session VIII: Clinical** Co-Chairs: Art M. Krieg, MD, *RTI, UMass Chan Medical School*  Frank Bennett, PhD, *Ionis Pharmaceuticals* | | | | |
| **14.15-14.35** | | | | **Lessons Learned from ALS Clinical Trials**  Toby Ferguson, MD, PhD, *Biogen* | |
| **14.35-14.55** | | | | **Antisense oligonucleotide therapy to pre-kallakrein in the treatment of patients with hereditary angioedema (HAE)** Richard Geary, PhD, *Ionis Pharmaceuticals* | |
| **14.55-15.15** | | | | **Phase 3 study, HELIOS-A, in hATTR patients evaluating a single dose regimen**  Gabriel J. Robbie, *Alnylam Pharmaceuticals* | |
| **15.15-15.35** | | | | **Clinical Advances in CRISPR Cas9 Therapeutics: In Vivo and Cell Therapy Applications**  Laura Sepp-Lorenzino, PhD, *Intellia Therapeutics* | |
| **15.35-15.55** | | | | **SLN360 and Controlling Lp(a), The New Kid on the CVD Block**  Curtis Rambaran, MD, *Silence Therapeutics* | |
| **15.55-16.15** | | | | **Industrializing the non-profit discovery, development, and treatment for nano-rare patients**  Tracy Cole, PhD, *n-Lorem Foundation* | |
| **16.15-16.30** | | | **Closing Remarks** | | |
| **18.00-22.00** | | | **Annual Meeting Wrap Party - Poolside at the Hilton sponsored by ChemGenes** *separate RSVP & Ticket required*  Join friends and colleagues to celebrate the wrap of this year’s annual meeting. Come prepared to splash in the pool or take a ride down the waterslide to cool off from the desert heat.  Dinner, drinks (2), and of course dancing included. | | |