

DAY 1 – SUNDAY, 11 OCTOBER 2026

8:00 AM **Registration Open** (Enjoy your breakfast at the hotel)

MORNING SESSIONS (running in parallel)

9:00 AM – 11:30 AM **Session I: Oligonucleotide Therapeutics Education Workshop**

Co-chairs: Alex Garanto, Ph.D., *Radboud Univ. Medical Center* & Chantal Ferguson, M.D./Ph.D., *Mass General Hospital*

9:00 – 9:10 **Opening remarks**

9:10 – 9:35 **Overview of the field, how we have come to where we are now**
 Laura Sepp-Lorenzino, Ph.D., *GNMmeds LLC*

9:35 – 10:00 **The Expanding Universe of Nucleic Acid Modifications: Origins, Applications and Frontiers**
 Martin Egli, Ph.D., *Vanderbilt University*

10:00 – 10:25 **Pharmacokinetics by Design: The Conjugate Toolkit**
 Vignesh Narayan Hariharan, Ph.D., *Comanche Biopharma*

10:25 – 10:50 **Models and controls: getting it right by learning from other people's mistakes and experience**
 Annemieke Aartsma-Rus, Ph.D., DACVP, *Leiden University Medical Center*

10:50 – 11:15 **Shaping the Oligonucleotide Field: The Industry-Academia Interface and What Comes Next**
 Art Levin, Ph.D., *Avidity Biosciences*

9:00 AM – 11:30 AM **Session II: IND – CMC Insights for Early-Stage Oligonucleotide Innovators**

Co-chairs: David Butler, Ph.D., *Advisor* & Marc Lemaitre, Ph.D., *ML_Consult*

9:00 – 9:30 **CMC workflow – a phase-appropriate strategy and execution**
 Hagen Cramer, Ph.D., *QurAlis*

9:30 – 10:00 **Translating Nucleic Acid Therapeutics from Discovery to the Clinic: Practical CMC and CMO Engagement Strategies**
 Eduardo Paredes, Ph.D., *Leal Therapeutics*

10:00 – 10:30 **Regulatory CMC Requirements for Oligonucleotide – A European Perspective**
 René Thürmer, Ph.D., *BfArM*

10:30 – 11:00 **USP Reference Standards and Impurity Analytical Reference Materials for Oligonucleotide Quality Across Modalities**
 Sarita Kattel, M.S., *US Pharmacopeia*

11:00 – 11:30 **Manufacturing for First in Human Clinical Trials: What Do You Really Need?**
 Kevin Fettes, Ph.D., *FTS Pharma Consulting*

CMC Session sponsor



11:30 AM – 1:30 PM *Hosted Lunch* (for all participants, served in the Mile High Ballroom)
& Mentorship Program Lunch (by invitation only, served in meeting room 501)

AFTERNOON SESSIONS (running in parallel)

1:30 PM – 3:05 PM **Session III: Next-Gen Early Career Scientist Session**
 Co-chairs: Daniel Knight, Ph.D. Candidate, *Carleton University* & Sarah Allen, Ph.D. Candidate, *UMass Chan Medical School/RTI*

1:30 – 1:50 **Session Keynote:**
 Strategic investments in RNA-based therapeutics at Novartis. Expanding therapeutic scope and clinical applicability
 Dr. Malgorzata Gonciarz, Ph.D., *Novartis*

Short Talks:

1:50 – 2:05 **Targeting HTLV-1 in the CNS: Antiviral siRNA Therapeutics for Glial Infection**
 Tiana Wang, Ph.D. Student, *The Kirby Institute, University of New South Wales (UNSW)*

2:05 – 2:20 **ERASOR: A platform to select anti-sense oligonucleotides for personalized treatment of genetic diseases**
 Emilio Harris-Mostert, Ph.D. Candidate, *Erasmus Medical*

2:20 – 2:35 **MSH3-Targeted Divalent siRNA Defines a Timing-Dependent Window for Somatic Repeat Expansion Suppression**
 Emily G. Knox, Postdoc, *UMass Chan Medical School*

2:35 – 2:50 **One Scaffold, Many Targets: Fully Modified RNA Nanostructures for Multi-Gene Silencing Beyond the Liver**
 Hassan H. Fakih, Ph.D., *UMass Chan Medical School*

2:50 – 3:05 **Preclinical Advancement of Antisense Oligonucleotide Therapy for DFNA9 Using Intracochlear and Intratympanic Delivery Approaches**
 Fien Aben, Ph.D. Fellow, *University of Antwerp*

1:30 PM – 3:00 PM **Session II: (cont.) IND – CMC Insights for Early-Stage Oligonucleotide Innovators**
 Co-chairs: David Butler, Ph.D., *Advisor* & Marc Lemaitre, Ph.D., *ML_Consumt*

1:30 – 2:00 **Learnings from Implementing CMC Analytical Control Strategies for Early-Stage Oligonucleotide Programs**
 Huijun Tian, Ph.D., *QurAlis*

2:00 – 2:30 **Chemoenzymatic Ligation for Complex Oligonucleotides**
 Chris (Xiaocen) Li, Ph.D., *Hongene Biotech*

2:30 – 3:00 **Control Strategies for Therapeutic Oligonucleotides: Key Insights and Regulatory Expectations**
 Timo Pöstges-Janotta, Ph.D., *BioSpring*

CMC Session sponsor



3:05 PM – 3:40 PM *Refreshment break*

AFTERNOON GENERAL SESSIONS

 3:40 PM – 4:45 PM **Career Event – Roundtable Discussions** (*Career Event will take place in Meeting Rooms 502-503*)

 Co-chairs: Emilio Harris-Mostert, Ph.D. Candidate, *Erasmus Medical Center*
 & Sarah Allen, Ph.D. Candidate, *UMass Chan Medical School/RTI*

Engage in roundtable discussions with moderators who have followed compelling career paths of their own. Designed to spark new connections, this session offers a personal, hands-on way to explore career opportunities and hear firsthand what different paths in the field can look like.

Roundtable topics:

Applying to jobs | Recognizing the values of your ideas | Building an expertise that matters | Building meaningful connections | Psychology of being a scientist | Crossing disciplines | Industry culture shock | Power of negotiation | Communicating your science | Work-life balance | Navigating the changing scientific landscape

 4:45 PM – 6:00 PM **Session IV: Opening Session and Keynote Address**

 Chair: Rebecca Miles, Ph.D., *BaseCure Therapeutics / President, OTS*
Opening by Rebecca Miles, Ph.D., *BaseCure Therapeutics / President, OTS*
Keynote Introduction: Anastasia Khvorova, Ph.D., *RNA Therapeutic Institute/UMass Chan Medical School*
Keynote Address: **My Journey across the RNA World: from Ribozymes to TMOs,**
 Thomas R. Cech, *Department of Biochemistry and BioFrontiers Institute, University of Colorado Boulder*

 6:00 PM – 7:00 PM **Book Signing**
The Catalyst: RNA and the Quest to Unlock Life's Deepest Secrets

 Thomas R. Cech, *Nobel Laureate*

Pre-ordered copies can be signed by the author during the Welcome Reception, Mile High Ballroom.

A limited number of copies will be available for purchase onsite — check with registration.

Book Signing Session Sponsor by:

 6:00 PM – 8:00 PM **Welcome Reception with Exhibitors (no Poster Hosting)**
Reception Sponsor:

 8:00 PM – 10:00 PM **Early Career Scientist Social Meet Up**
(Open to students, post-docs and/or trainees, pre-registration required)
LOCATION: *Lucky Strike Denver* | 500 16th St Mall #340, Denver, CO

Early Career Social Meet Up Sponsor:


DAY 2 – MONDAY, 12 OCTOBER 2026

8:00 AM **Registration Open** (*Enjoy your breakfast at the hotel*)

8:30 AM – 10:15 AM **Session V: Chemistry, Mechanism and Delivery (I)**

Co-chairs: Maja Janas De Angelis, Ph.D., DABT, *Alnylam Pharmaceuticals* & Ken Yamada, Ph.D., *UMass Chan Medical School*

8:30 – 8:50 **Targeting neurodegeneration with RNA-therapeutics**
Gene Yeo, Ph.D., *UCSD*

8:50 – 9:10 **Extending the Scope of Oligonucleotide Chemistry**
Tom Brown, Ph.D., *University of Oxford*

9:10 – 9:30 **Beyond the Duplex: RNA Hacking by Staple Oligomer Folds Target mRNA into a G-Quadruplex**
Yousuke Katsuda, Ph.D., *Kumamoto University*

Short Talks:

9:30 – 9:45 **Turning Cartilage Degeneration into a Targeting Cue for Intra-Articular RNA Therapy**
Mahima Dewani, Ph.D., *Brigham and Women's Hospital*

9:45 – 10:00 **A Subset of TRIM-Ago2 Drives mRNA Knockdown**
Chun-Che Tseng, Ph.D., *Arrowhead Pharmaceuticals*

10:00 – 10:15 **TXP1: A Next-Generation Brain-Selective Shuttle for Oligonucleotide Delivery to the CNS**
Pawel Stocki, Ph.D., *Ossianix*

10:15 AM – 10:45 AM **Refreshment break**

10:45 AM – 12:30 PM **Session VI: Pre-clinical (Early stage)**

Co-chairs: Helene Tran, Ph.D., *Servier Research & Development Institute* & Hassan H. Fakih, Ph.D., *UMass Chan Medical School/RTI*

10:45 – 11:05 **Preclinical development of a NAT8L-targeting antisense oligonucleotide to lower CNS N-acetylaspartate for the treatment of Canavan disease**
Peter Hagedorn, Ph.D., *Contera Pharma*

11:05 – 11:25 **Reactivating CEBPA in Acute Myeloid Leukemia using RNA-based approaches**
Maureen McKeague, Ph.D., *McGill University*

11:25 – 11:45 **Developing siRNA Conjugates for Arthritic Joint and Deep Brain Gene Silencing**
Craig Duvall, Ph.D., *Vanderbilt University*

Short Talks:

- 11:45 – 12:00 **RB042: The Journey of an Inhaled Splice-Switching Oligonucleotide**
Bao Le, Ph.D., *Atisama Therapeutics*
- 12:00 – 12:15 **Development of an allele-selective siRNA to treat PLN-R14del cardiomyopathy**
Annet Linders, Ph.D., *PLaN Therapeutics*
- 12:15 – 12:30 **Dual co-targeting of KRAS G12V and MYC by an EGFR-directed, inverted chimera**
Chad Pecot, M.D., *University of North Carolina*
- 12:30 PM – 2:00 PM **Hosted Lunch** (for all participants)
Lunch Sponsor: BACHEM
- 2:00 PM – 3:45 PM **Session VII: Pre-clinical (Late stage)**
Co-chairs: Husam Younis, Ph.D., *Atrium Therapeutics* & Beatriz Llamusi, Ph.D., *ARTHEX Biotech*
- 2:00 – 2:20 **Translational studies in non-human primate models that support the development of BC-006, an INHBE siRNA, for sustained weight management**
Jing Yang, Ph.D., *BaseCure Therapeutics*
- 2:20 – 2:40 **From Discovery toward the Clinic - Preclinical Development of an ASO Targeting a Cardiac Myofibroblast-specific lncRNA**
Denis Drygin, Ph.D., *Haya Therapeutics*
- 2:40 – 3:00 **INTENT LNPs for Cancer Vaccines: Mechanisms of Action, Early Clinical Results, and Advances in Point-of-Care Manufacturing**
Natalia Martin-Orozco, Ph.D., *Providence Therapeutics*

Short Talks:

- 3:00 – 3:15 **Comprehensive In Vivo Tolerability Assessment of 365 Antisense Oligonucleotides for Central Nervous System Targets: Implications for Lead Optimization**
Julie Douville, Ph.D., *N-Lorem Foundation*
- 3:15 – 3:30 **A mutation-agnostic ASO strategy demonstrates potent functional rescue and retinal preservation in RHO-linked retinitis pigmentosa**
Pietro De Angeli, Ph.D., *Centre for Ophthalmology, University Hospital Tübingen*
- 3:30 – 3:45 **Preclinical development of antisense oligonucleotide TRCN-1023 to restore UNC13A function in people living with Amyotrophic Lateral Sclerosis**
Terry Fang, Ph.D., *Trace Neuroscience*

3:45 PM – 4:15 PM POSTER FIRE SESSION IChair: Julia Alterman, Ph.D., *UMass Chan Medical School*

- **DeepRNAi: Toward Reducing Late-Stage Setbacks with a Frontier Model for siRNA Design,**
Scott Findlay, Ph.D., *Deepgenomics*
- **An LLM-orchestrated agent for autonomous antisense oligonucleotide design,**
Deepak Pant, *UCSD*
- **COMPASS Integrates AI-Based Docking and Cell Imaging to Overcome Biomarker Identification Bottlenecks in Aptamer Discovery,**
Hyeokjung Kim, Ph.D., *University of Minnesota*
- **miR-34a Exacerbates Ischemic Stroke Injury and Represents a Novel Therapeutic Target,**
Xuefang Ren, Ph.D., *UTHealth*
- **Endolysosomal Sequestration Limits Productive Delivery of Naked Antisense Oligonucleotides,**
Ewa Sitarska, Ph.D., *Harvard Medical School*
- **Phosphorothioate Chirality in Fully Chemically Modified CRISPR RNA Controls Cas9 Gene Editing,**
Ramadevi Chilamkurthy, Ph.D., *Wake Forest University School of Medicine*

4:30 PM – 7:30 PM *Poster Session – Reception I (Odd Numbered Posters Hosted)*

DAY 3 – TUESDAY, 13 OCTOBER 2026

8:30 AM – 10:15 AM **Session VIII: DNA/RNA Editing**

Co-chairs: Jonathan Watt, Ph.D., *UMass Chan Medical School*
 & Laura Sepp-Lorenzino, Ph.D., *GNMmeds LLC*

8:30 – 8:50 **Regulatable in Vivo gene Expression via Adaptamers**

Prashant Mali, Ph.D., *UCSD*

8:50 – 9:10 **Engineered prime editors with minimal genomic errors**

Vikash Pal Singh Chauhan, Ph.D., *Massachusetts Institute of Technology (MIT)*

9:10 – 9:30 **RNP Editing Cell Therapy**

Justin Eyquem, Ph.D., *UCSF*

Short Talks:

9:30 – 9:45 **RNA Engineering Towards CRISPR-Cas9 Treatment for Post-Traumatic Osteoarthritis**

Zach Lamantia, *Vanderbilt University*

9:45 – 10:00 **ASO-Responsive Catalytic RNA Switches for Tunable Control of Gene Therapies**

Guocai Zhong, Ph.D., *Department of Genetic and Cellular Medicine, UMass Chan Medical School*

10:00 – 10:15 **Clinical validation and next-generation EON discovery with Axiomer ADAR-mediated RNA editing platform**

Gerard Platenburg, *ProQR Therapeutics*

10:15 AM – 10:45 AM **Refreshment break**

10:45 AM – 12:30 PM **Session IX: Awards Session I | Awards Presentations and Lifetime Achievement Awards**

Chair: Rebecca Miles, Ph.D., *BaseCure Therapeutics / President, OTS*

Awards Presentations:

10:45 – 11:05 **Dr. Alan M. Gewirtz Memorial Scholarship – Graduate Student:**

Developing Next-Generation Peptide Nucleic Acids for Antigen Therapy

Sai Palavi Pradeep, Ph.D. Candidate, *University of Connecticut*

11:05 – 11:35 **Dr. Alan M. Gewirtz Memorial Scholarship – Postdoctoral fellow/Jr. Industrial Professional:**

Heteroduplex Oligonucleotides: A Platform for Enhanced Efficacy and Improved Safety of Antisense Oligonucleotides

Mitsugu Yanagidaira, M.D., Ph.D., *Harvard Medical School*

11:35 – 12:30 **Lifetime Achievement Award (I)**

Introduction by Mano Manoharan, Ph.D., *Alnylam*

Lifetime Achievement Award Presentation:

A 40+ Year Journey in RNA & Oligonucleotide Therapeutics

John Rossi, Ph.D., *City of Hope*

12:30 PM – 2:00 PM **Hosted Lunch** (for all participants)

- 2:00 PM – 3:45 PM** **BBB Panel: Beyond the Receptor: Defining the Rules for Productive Transvascular Delivery of Oligonucleotide Therapeutics**
 Chair and moderator: Anastasia Khvorova, Ph.D., *RNA Therapeutic Institute/UMass Chan Medical School*
- 2:00 – 2:05** **BBB Panel Introduction:**
 Anastasia Khvorova, Ph.D., *RNA Therapeutic Institute/UMass Chan Medical School*
- 2:05 – 2:55** **Intro Talks - Topics:**
- **Overview of approaches, molecular engineering, subcutaneous administration, dual shuttles**
 By James Gorman, Ph.D., *Wyss Institute*
 - **Antibody engineering, cargo engineering, charge neutralization, translational CNS delivery**
 By Felix Schumacher, Ph.D., *Roche*
 - **Aptamer-oligonucleotide conjugates, DAR, PK, quantitative pharmacology**
 By Thomas Ormsby, Ph.D., *RNA Therapeutics Institute, UMass Chan Medical School*
 - **CMC issues**
 By Firoz Antia, Ph.D., *Denali Therapeutics*
 - **Translational Pharmacology**
 By Christine Esau, Ph.D., *StealthCo*
- 2:55 – 3:30** **Panel Discussion**
- 3:30 – 3:45** **Audience Q&A**
- 3:45 PM – 4:15 PM** **POSTER FIRE SESSION II**
 Chairs: Aurelie Goyenvalle, Ph.D., *Université de Versailles*
- **Vehicle-Free PSMA-Targeted Delivery of Fully Modified miR-34a Enables Efficient Prostate Cancer Targeting and Extended Pharmacokinetics,**
 Digambar Waiker, Ph.D., *Purdue University*
 - **From Imbalance to Equilibrium: Antisense Oligonucleotide Therapy Targeting Allelic Expression of PEX6 in Zellweger Spectrum Disorder,**
 Evelyn Zavacky, Ph.D. Student, *McGill University*
 - **Preclinical development of antisense oligonucleotide therapy for rare kidney disease,**
 Sebastian Sjöström, Ph.D., *ETH Zürich*
 - **mRNA half-life is a key determinant of siRNA efficacy in vivo,**
 Kyle Weirether, *Alnylam Pharmaceuticals*
 - **Advancing ASO Safety Assessment Through CNS-Relevant In Vitro Models,**
 Laura Colar Zanjko, Ph.D. *Candidate, BioTalentum Ltd.*
 - **Internal cap-initiated translation provides efficient protein production from circular mRNA,**
 Kosuke Fukuchi, *Nagoya University*
- 4:30 PM – 7:30 PM** **Poster Session – Reception II (Even Numbered Posters Hosted)**

DAY 4 – WEDNESDAY, 14 OCTOBER 2026

8:30 AM – 10:10 AM **Session X: Chemistry, Mechanism and Delivery II**

Co-chairs: Anastasia Khvorova, Ph.D., *RNA Therapeutic Institute/UMass Chan Medical School*
& Philippe Barthelemy, Ph.D., *Inserm*

8:30 **Best of Both Worlds or Double-Edged Sword? Antibody-Mediated Delivery of Oligonucleotides**
Annemieke Madder, Ph.D., *Ghent University*
(PRE-RECORDED Presentation)

8:30 – 8:50 **Minimalist mRNA: Redefining Translation through Chemistry**
Masad J. Damha, Ph.D., *McGill University*

8:50 – 9:10 **Quantitative Imaging of Intrathecal ASO Distribution in Humans**
Brendon E. Cook, Ph.D., *Biogen*

Short Talks:

9:10 – 9:25 **ex-IM Chemistry: Toward Faster-Acting, More Potent siRNAs**
Zhi Ming Cheng, Ph.D., *RNA Therapeutics Institute, UMass Chan Medical School*

9:25 – 9:40 **DG9 Peptide Enables Systemic Delivery of Antisense Oligonucleotides to the Heart and Central Nervous System in Neuromuscular Disease Models**
Rika Maruyama, Ph.D., *University of Alberta*

9:40 – 9:55 **Sustained intra-articular delivery of antisense oligonucleotides using an injectable hydrophobic depot matrix**
Aleksandra Bednarz, Ph.D., *Hazel Therapeutics*

9:55 – 10:10 **Toward the Clinic: Targeted Oligonucleotide Conjugates with Built-in Endosomal Escape Enable Durable Pharmacology at Picomolar Tissue Exposure**
Miriam Bujny, Ph.D., *Sapreme Therapeutics*

10:10 AM – 10:45 AM **Refreshment break**

10:45 AM – 12:45 PM **Session XI: Awards Session II | Awards Presentations and Lifetime Achievement Award (II)**
Chair: Masad J. Damha, Ph.D., *McGill University*

Announcements:

10:45 – 10:50 **Posters awards | Travel grants | Paper of the year | Next-Gen Best Talk, People's Choice Poster Awards**

Awards Presentations:

10:50 – 11:20 **Nucleic Acid Therapeutics Journal Young Investigator Award**
Advancing oligonucleotide therapeutics for prion disease
Sonia Vallabh, Ph.D., & Eric Minikel, Ph.D., *Broad Institute*

11:20 – 11:50 **Dr. Fritz Eckstein Mid-career Achievement Award**
Tribute to Dr. Fritz Eckstein
Development of SORT lipid nanoparticles (LNPs) for genome correction of disease-causing mutations
Daniel J. Siegwart, Ph.D., & Eric Minikel, Ph.D., *UT Southwestern Medical Center*

11:50 – 12:45 **Lifetime Achievement Award (II):**
Introduction by Masad J. Damha, Ph.D., *McGill University*
Lifetime Achievement Award Presentation
 Pieter Cullis, Ph.D., *Professor, Department of Biochemistry and Molecular Biology, University of British Columbia.*

12:45 PM – 2:30 PM **Hosted Lunch** (for all participants)

2:30 PM – 4:25 PM **SESSION XII: Clinical Session and Patient Voice**
 Co-chairs: Annemieke Aartsma-Rus, *Leiden University Medical Center*
 & Scott Henry, Ph.D., *Ionis Pharmaceuticals*

2:30 – 2:50 **The development of tofersen for SOD1-ALS and lessons learned for future trial designs in ALS**
 Steve Garafalo, Ph.D., *Biogen*

2:50 – 3:10 **From Generation to Partnership: What Four Generations of SOD1 ALS Have Taught Me About the Future of RNA Therapeutics**
 Paula Trefiak, *Saskatchewan Workers' Compensation Board*

3:10 – 3:30 **The Parents' Journey Through Drug Development: Making the Impossible Possible for Angelman Syndrome**
 Allyson Berent, *DVM, Dipl. ACVIM, Foundation for Angelman Syndrome Therapeutics (FAST)*

3:30 – 3:35 **Awards Presentation: Patient Advocacy Award**
Presented by Annemieke Aartsma-Rus, Ph.D., *Leiden University Medical Center*

3:35 – 3:55 **Interventional genetics: who's in the waiting room?**
 Timothy Yu, MD, Ph.D., *Harvard/Boston Children's Hospital*

Short Talks:

3:55 – 4:10 **Clinical Safety of Individualized Antisense Oligonucleotides: Aggregate Experience from 22 Therapeutic Programs and 56 Patients**
 Laurence Mignon, Ph.D., *n-Lorem Foundation*

4:10 – 4:25 **Interim Pharmacokinetic and Pharmacodynamic Results of the Phase 1/2a AVANCE1 Trial on Tricyclo-DNA-Induced DMD Exon 51 Skipping in Duchenne Muscular Dystrophy**
 Helge Amthor, M.D., Ph.D., *Universite de Versailles*

4:25 PM – 4:45 PM **Closing Remarks**

7:00 PM – 12:00 AM **Annual Meeting Wrap Party:**

WHERE THE OTS COMMUNITY MEETS THE WILD WEST AT ALTITUDE! Get ready to saddle up for an unforgettable evening of line dancing, music, great food, and Western-style fun. Join colleagues and friends for a true Mile High celebration in a Wild West setting.



National Western Center – Stockyard Event Center



Shuttle buses depart 6:15 – 7:30 PM to the venue, looping back to the Convention Center throughout the evening.

Pick-up and drop-off location: Welton Drive or Colorado Convention Center Lobby B.

Wrap Party Sponsor:

