**SAVE TODAY** with Early Registration

11TH Annual

# OPT Congress

## Oligonucleotide, mRNA & Peptide Therapeutics

**MARCH 18 – 19, 2026**

**BOSTON, MA + VIRTUAL**

SEAPORT HOTEL

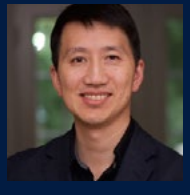
## 2026 CONFERENCE PROGRAMS

- Oligonucleotide Discovery & Delivery
- Oligonucleotide CMC & Manufacturing
- mRNA & Emerging Oligonucleotide Modalities
- Peptides & Emerging Drug Conjugates

## IN-PERSON SHORT COURSES

- Safety & Toxicity of Nucleic Acids
- Successful Late Phase Regulatory Submission for a Complex Oligonucleotide
- Next Gen ADCs & Advanced Linkers & Conjugates: Mastering Design, Linker Optimization & Stability

## PLENARY KEYNOTE SPEAKERS



**Timothy Yu, PhD**
*Associate Professor Pediatrics, Genetics & Genomics, Boston Children's Hospital*



**Brenda Bass, PhD**
*Distinguished Professor, Biochemistry, University of Utah*



**Debra Miller**
*Founder & CEO, CureDuchenne*



**Weimin Wang, PhD**
*Founder & CEO, Sanegene Bio*



#OPTBoston

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# WELCOME TO OPT CONGRESS:

## Oligonucleotide, mRNA & Peptide Therapeutics

**OPT Congress 2026** is the leading industry event for scientists, directors, and technology providers driving innovation across oligonucleotide, mRNA, and peptide therapeutics. Now in its 11th year, this event provides a unique forum for translating cutting-edge science into transformative medicines, with expanded 2026 programming that includes the brand-new Peptides & Emerging Drug Conjugates conference. This year's agenda brings together experts from discovery through development, with dedicated content spanning AI-enabled design, extrahepatic delivery, scalable manufacturing, and next-gen modalities including tRNA, circular RNA, and radioligand peptide conjugates.

Across four focused conferences—Oligonucleotide Discovery & Delivery, CMC & Manufacturing, mRNA & Emerging Modalities, and Peptides & Emerging Conjugates—attendees will explore 60+ scientific presentations, collaborative case studies, and real-world implementation strategies. From novel linkers and chemistries to regulatory insights, tech transfer, and delivery breakthroughs, OPT Congress fosters dialogue among pharma, biotech, academia, and technology providers. Join us for two days of actionable insights, short courses, and customizable content designed to support your therapeutic programs from bench to bedside.

## EVENT-AT-A-GLANCE

| WEDNESDAY, MARCH 18 | THURSDAY, MARCH 19 |
|---|---|
| Short Courses | Conference Programs |
| Safety & Toxicity of Nucleic Acids | Oligonucleotide Discovery & Delivery |
| Successful Late Phase Regulatory Submission for a Complex Oligonucleotide | Oligonucleotide CMC & Manufacturing |
| Next Gen ADCs & Advanced Linkers & Conjugates: Mastering Design, Linker Optimization & Stability | mRNA & Emerging Oligonucleotide Modalities |
| | Peptides & Emerging Drug Conjugates |

With Thanks to Our

## EXECUTIVE ADVISORY BOARD

**Marvin Caruthers, PhD**
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**Ekkehard Leberer, PhD**
*Senior Life Sciences Consultant, ELBIOCON*

# PLENARY KEYNOTE PRESENTATIONS



### N-of-1 Therapeutics: Progress, Pitfalls, and Prospects for Future Individualized Medicines

**Timothy Yu, PhD**
*Associate Professor Pediatrics, Genetics & Genomics, Boston Children's Hospital*



### ADAR RNA Editing: Applying Current Knowledge to Future Applications

**Brenda Bass, PhD**
*Distinguished Professor, Biochemistry, University of Utah*



### Venture Philanthropy in Drug Development from a Rare-Disease Patient-Advocacy Perspective

**Debra Miller**
*Founder & CEO, CureDuchenne*



### Oligonucleotide and Delivery Chemistry for siRNA Conjugates: Past Innovations and Future Opportunities

**Weimin Wang, PhD**
*Founder & CEO, Sanegene Bio*

"Truly inspiring space to immerse oneself in novel and emerging technologies with collegial, supportive, approachable leaders in our community!"

**-Nigel Danielson-Ewin,** *Pharma Innovation Researcher, Shimadzu Scientific Instruments*

# PROGRAM KEYNOTE & FEATURED PRESENTATIONS



**Thiomorpholino Oligonucleotides (TMOs) Useful for Exon Skipping, RNase H, and siRNA Applications**
*Marvin Caruthers, PhD, Distinguished Professor, University of Colorado*



**Recent Advancements of Oligo-Conjugates Revolutionizing the Field**
*Mano Manoharan, PhD, Distinguished Scientist & Senior Vice President, Innovation Chemistry, Alnylam Pharmaceuticals*



**A Platform Approach to Enzymatic Oligonucleotide Assembly—Technical and Regulatory Considerations**
*Benjamin Stevens, PhD, Director, CMC Policy and Advocacy, GSK*



**Simplifying the Synthesis of Oligonucleotides**
*Phil Baran, PhD, Chair & Professor, Department of Chemistry, Scripps Research Institute*



**Preclinical Development of an RNA/DNA Hybrid TLR7/8/9 Agonist for Cancer Immunotherapy**
*Arthur Krieg, MD, Founder, President and Acting CEO/CSO, Zola Therapeutics*



**The Use of Therapeutic tRNAs for the Treatment of Duchenne Muscular Dystrophy and Dilated Cardiomyopathy**
*Elisabeth Gardiner, PhD, CSO, Tevard Biosciences*



**The Infinite Loop—Machine Learning for Discovery, Delivery, and Rapid Manufacturing of Potential Medicines**
*Bradley L. Pentelute, PhD, Professor, Department of Chemistry, Massachusetts Institute of Technology*



**Leveraging Radical Enzymology Towards New Peptide Architectures**
*Vahe Bandarian, PhD, Professor, Biological Chemistry, University of Utah; Co-Founder, Sethera Therapeutics*



**Advancing Targeted Radioligand Therapies—Clinical Perspectives on Lutetium- and Actinium-Based PSMA-617 Conjugates**
*Mark Rutstein, MD, Global Head, Oncology Development, Novartis Pharma SAS*

# TOP REASONS to Attend

### BE INSPIRED BY SCIENCE-DRIVEN PLENARY KEYNOTE SESSIONS

Gain insights from world-renowned academics and industry leaders as they share the latest breakthroughs driving therapeutic innovation.

### CHOOSE FROM FOUR EXPERTLY CURATED CONFERENCES—INCLUDING NEW PEPTIDE COVERAGE

Tailor your experience across focused tracks covering oligonucleotide discovery and delivery, CMC and manufacturing, mRNA and emerging modalities, and peptides and novel drug conjugates.

### LEARN FROM CASE STUDIES DRIVING REAL-WORLD IMPACT

Explore how experts are solving challenges in developing new drug modalities, tissue-specific delivery, extrahepatic targeting, oral bioavailability, AI-enabled design, and regulatory compliance.

### STAY AHEAD OF EMERGING SCIENTIFIC AND TECHNOLOGICAL TRENDS

Delve into the latest in GalNAc and lipid-based delivery, peptide and RNA modalities, conjugate chemistries, linker design, and AI/ML tools reshaping oligo and peptide-based drug development.

### ENHANCE YOUR EXPERTISE WITH INTERACTIVE SHORT COURSES

Join in-depth, hands-on sessions that tackle critical areas including understanding the unique safety and toxicity profiles of nucleic acid therapeutics, developing next-gen ADCs, linkers and conjugates, and navigating successful regulatory submissions for complex oligonucleotides.

### NETWORK WITH THE PEOPLE WHO MATTER

Engage with leading scientists, executive directors, regulatory strategists, formulation experts, and business development leaders in breakout groups, curated networking sessions, and informal meetups.

### DISCOVER TECHNOLOGIES ADVANCING THERAPEUTIC PIPELINES

Explore the exhibit hall and poster sessions to connect with innovative platform developers, CROs, CDMOs, and analytics providers offering next-gen solutions to accelerate your development timelines.



## UNPARALLELED NETWORKING OPPORTUNITIES

- **NETWORK** onsite during the Welcome Reception, Refreshment Breaks, Luncheon, and Closing Reception.
- **CONTINUE** your discussions during our Breakout Sessions.
- **ENGAGE** with our industry-leading sponsors.
- **TAKE PART** in live Q&A with speakers and participants following each presentation.
- **PARTICIPATE** in 1-on-1 networking with an easy-to-navigate profile search, filter, and scheduling platform.
- **IDENTIFY** and establish meetings with participants who have similar initiatives and challenges within minutes.

# SHORT COURSES

**WEDNESDAY, MARCH 18 | 8:00 – 10:00 AM | IN-PERSON ONLY**
7:30am Short Course Registration and Morning Coffee

## SC1: Safety & Toxicity of Nucleic Acids

Nucleic acid drugs continue to deliver on their promise to become a third therapeutic modality, in addition to small molecules and biologics. Several antisense oligonucleotide drugs have been on the market for some time, while the first RNAi approval was granted in 2018. Despite the common "nucleic acid" component, the mechanisms of action and non-specific effects differ for each of these drug types.

**KEY TOPICS INCLUDE:**

- Different types of nucleic acid-based drugs
- Mechanisms of action and non-specific effects
- Current approaches to address non-specific and potentially toxic effects
- Findings secondary to class-effect of oligonucleotides

**INSTRUCTORS:**

***Sarah Lamore, PhD, Senior Director, Toxicology, Wave Life Sciences***
***Kristy Szretter, PhD, Scientific Director, Takeda Pharmaceutical***
***Fengjiao Zhang, PhD, DABT, Director, Toxicology, Preclinical & Clinical Discovery & Development Team, Wave Life Sciences***
***Xiao Shelley Hu, PhD, President and Founder, Translational Consulting LLC***

## SC2: Successful Late Phase Regulatory Submission for a Complex Oligonucleotide

ICH guidelines have established clear expectations for the control strategy for synthetically manufactured medicines. Oligonucleotides fall into the synthetic category and yet their manufacture and control are very different compared to small molecules. In this short course we will look at the requirements for a control strategy combining starting material control, process understanding and final drug substance specifications and methods. With a common understanding in mind we will discuss how to apply the control principles to therapeutic oligonucleotides from early development to registration.

**KEY TOPICS INCLUDE:**

- The principles of a control strategy from ICH guidance and its practical application
- Unique challenges (and advantages) in applying the principles to oligonucleotides
- Early development strategies for oligonucleotides
- Achieving a successful commercial control strategy for oligonucleotides

**INSTRUCTORS:**

***Mike Webb, PhD, Founder & CEO, MikeWebbPharma Ltd.***
***Chris Oswald, Founder, Owner, and Principal Consultant, Coswald Consulting LLC***






## SC3: Next Gen ADCs & Advanced Linkers & Conjugates: Mastering Design, Linker Optimization & Stability

Conjugated modalities such as antibody-drug conjugates (ADCs), oligonucleotide conjugates, and peptide-drug conjugates are revolutionizing precision medicine. However, their success relies on smart linker strategies that ensure stability, controlled payload release, and manufacturability. This intensive 2-hour course will explore the latest innovations in linker chemistry, site-specific conjugation, formulation and delivery considerations, and scalable manufacturing approaches. Participants will gain practical insights into optimizing linker design for enhanced efficacy, reduced toxicity, and regulatory compliance.

**KEY TOPICS INCLUDE:**

- Choosing the right modality to pursue a drug target
- Interplay of antibody, linker and payload
- "Rational" approach for designing next-generation ADCs
- Linker design and optimization
- Manufacturing and scalability
- Clinical updates
- Case studies and key learnings

**INSTRUCTORS:**

***Sunny Zhou, PhD, Professor, Chemistry & Chemical Biology, Northeastern University***
***Amit Nayyar, PhD, General Manager, Cohance***

*Premium or separate registration required for Short Courses.*

# Oligonucleotide Discovery & Delivery

Driving Innovation in Design, Development, and Performance for the Next Wave of Oligonucleotide Therapeutics

## WEDNESDAY, MARCH 18

**7:30 am Short Course Registration and Morning Coffee**

**8:00 Recommended Short Course***

SC1: Safety & Toxicity of Nucleic Acids

*Premium Registration or separate registration required. See Short Courses page for details.

**9:30 Main Conference Registration and Morning Coffee**

**10:30 Welcome Remarks by Conference Director**

## INNOVATION IN DISCOVERY, DELIVERY, AND PERFORMANCE

**10:40 Chairperson's Remarks**

*Aimee Jackson, PhD, Principal, Jackson Biosciences*

**10:45 FEATURED PRESENTATION: The Zorevunersen Story: Insights from the Development of an Investigational ASO for Dravet Syndrome**



*Isabel Aznarez, PhD, Vice President, Discovery Research, Stoke Therapeutics*

Zorevunersen is an investigational antisense oligonucleotide designed to address the underlying genetic cause of Dravet syndrome. Clinical and natural-history data highlight the persistent burden of seizures and developmental challenges despite best-available standard of care. We present safety and effectiveness results from early studies of the effect of zorevunersen on seizure frequency and non-seizure outcomes. A global Phase 3 trial is underway to confirm these findings.

**11:15 Antisense Oligonucleotide Therapies for Neurological Disorders**

*Adrian Krainer, PhD, St. Giles Foundation Professor, Cold Spring Harbor Laboratory, CSHL Cancer Center*

We previously developed nusinersen, an ASO that modulates alternative splicing of *SMN2* exon 7 in the context of spinal muscular atrophy. The long-lasting effects of CNS-administered ASOs allow infrequent intrathecal dosing, providing an effective approach to treat neurological disorders. Consequently, many ASOs are being developed against neurology and neuro-oncology targets. Splice-switching ASOs, in particular, are highly versatile, reflecting the pervasiveness of splicing. I will describe selected applications of this technology.

**11:45 RNAi to Engineer Immune Cells for Therapy**

*Reka Haraszti, PhD, Resident & Group Leader, Hematology & Oncology, University Hospital Tuebingen*

Therapeutic cells exhibit distinct transcriptomic profiles in on-tumor versus off-tumor immune responses, independent of receptor clonality. To deliberately modulate these states, we integrate RNAi therapies into adoptive cell-therapy manufacturing, achieving durable effects in proliferating cells. In an allogeneic stem cell transplant and CAR T cell models, we are exploring multiplex siRNA cocktails and miRNA mimics that selectively reduce off-tumor activity while preserving anti-tumor responses through transcriptomic reprogramming of T cells.

**12:15 pm Transition to Lunch**

**12:25 LUNCHEON PRESENTATION: Bridging the Manufacturing Chemistries between Drug Research and Development for Morpholino Oligos**

*Yongfu Li, PhD, Head Chemist, Gene Tools LLC*

Phosphorodiamidate Morpholino Oligonucleotides (PMOs) are a unique class of antisense therapeutics for the treatment of genetic diseases. Variability in synthetic methodologies across manufacturers can result in inconsistent structural entities, potentially complicating regulatory approval for drug development. We present an optimized synthetic process for Morpholino oligonucleotide production and advocate the importance of a standardized structural framework to facilitate efficient translation from preclinical development to clinical-scale manufacturing in the Morpholino therapeutic industry.

**12:55 Session Break**

**1:35 Chairperson's Remarks**

*Adrian Krainer, PhD, St. Giles Foundation Professor, Cold Spring Harbor Laboratory, CSHL Cancer Center*

**1:40 Why Oligotherapy Has Not Reached Its Potential and How New Chemistry Can Change That**

*David Tabatadze, PhD, President, ZATA Pharmaceuticals, Inc.*

It has been 46 years since ZATA's co-founder, Dr. Paul Zamecnik (1911–2009), pioneered the field of oligotherapy (ASO on 1978). And yet, despite decades of research and investment, oligotherapy has not reached anything close to its full potential. The reason? The chemistry toolbox we use to build oligonucleotides is still too limited. Current synthesis platforms lack the versatility needed to design and fine-tune truly effective ON-based drugs.

**2:10 Patient-Scientist Led Development of a Divalent siRNA Therapy for Prion Disease**

*Sonia Vallabh, PhD, Prion Scientist, Broad Institute*

Prion disease is a rapidly progressive neurodegenerative disease that is universally fatal and currently untreatable. Pathogenesis is driven by the misfolding of a single causal protein, the prion protein (PrP), into a self-propagating conformer. This talk will describe the academic-led advancement of a PrP-lowering divalent siRNA through preclinical and IND enabling studies, as well as regulatory and clinical strategy.

**2:40 Sponsored Presentation** *(Opportunity Available)*

**3:10 Grand Opening Refreshment Break in the Exhibit Hall with Poster Viewing**

## PLENARY KEYNOTE SESSION

**4:00 Welcome Remarks by Conference Director**

*Gemma Smith, Senior Conference Director, Production, Cambridge Healthtech Institute*

**4:05 Chairperson's Remarks**

*Adrian Krainer, PhD, St. Giles Foundation Professor, Cold Spring Harbor Laboratory, CSHL Cancer Center*



**4:10 N-of-1 Therapeutics: Progress, Pitfalls, and Prospects for Future Individualized Medicines**

*Timothy Yu, PhD, Associate Professor Pediatrics, Genetics & Genomics, Boston Children's Hospital*

Successes in oligonucleotide therapeutics have spurred the creation of bespoke therapies for rare genetic conditions, even for single patients. This talk will review lessons, challenges, and opportunities stemming from these pioneering efforts, and offer perspectives on the ethical and regulatory hurdles to be overcome to realize a future of individualized medicines, whether as proof of concept or provision of care.



**4:50 ADAR RNA Editing: Applying Current Knowledge to Future Applications**

*Brenda Bass, PhD, Distinguished Professor, Biochemistry, University of Utah*

Much is known about biochemical properties of ADAR RNA editing enzymes from decades of *in vitro* studies, but how these properties correlate with *in vivo* editing is not always clear. Properties established *in vitro* will be compared with observations made *in vivo*, with a focus on properties relevant to therapeutic applications, such as guided RNA editing. Recent progress on how inosine precludes activation of an immune response will be presented.

# Oligonucleotide Discovery & Delivery

Driving Innovation in Design, Development, and Performance for the Next Wave of Oligonucleotide Therapeutics

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

**6:30 Close of Day**

## THURSDAY, MARCH 19

**7:30 am Registration and Morning Coffee**

## DRIVING CLINICAL TRANSLATION AND DEVELOPMENT

**8:00 Chairperson's Remarks**

*David Corey, PhD, Professor, Department of Pharmacology, UT Southwestern*

**8:05 Clinical Translation of the Pharmacological Properties of Phosphoryl Guanidine-Containing Stereopure Oligonucleotides**

*Elizabeth Wagner, PhD, Director of Translational Medicine, Biology, Wave Life Sciences*

Wave's PRISM platform enables the generation of chimeric phosphoryl guanidine (PN) backbone-containing stereopure oligonucleotides with position-controlled chemistry and stereochemical configuration. We will show examples where stereopure design and incorporation of PN linkages improve the pharmacological properties of oligonucleotides designed for distinct genetic targets, modalities, and tissues. Data from our ongoing clinical trials suggests that the improved pharmacological properties of investigational PN-containing oligonucleotides are translating into the clinic.

**8:35 miRNAs: The Case for Renewed Focus on Basic Science and Clinical Development**

*David Corey, PhD, Professor, Department of Pharmacology, UT Southwestern*

The importance of miRNAs for basic science has always been clear, but the application to therapy has lagged far behind. Now, however, our understanding of the mechanism of action for miRNAs and tools for identifying miRNA has advanced. Together, these advances provide the foundation for understanding how miRNAs affect disease and improve drug development.

**9:05 Sponsored Presentation** *(Opportunity Available)*

## IN-PERSON BREAKOUT DISCUSSIONS

**9:35 In-Person Breakout Discussions**

Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussion page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON BREAKOUT DISCUSSION: Interfacing New Research with the Biotech Arena**

*Marvin Caruthers, PhD, Distinguished Professor, University of Colorado*

**IN-PERSON ONLY BREAKOUT: Industry Perspectives on Platform Development and Pipeline Evolution**

*Chandra Vargeese, PhD, CTO & Head, Platform Discovery Sciences, Wave Life Sciences*

**10:20 Coffee Break in the Exhibit Hall with Poster Viewing**

## ADVANCES WITH OLIGO-CONJUGATES



**11:00 KEYNOTE PRESENTATION: Recent Advancements of Oligo-Conjugates Revolutionizing the Field**

*Mano Manoharan, PhD, Distinguished Scientist & Senior Vice President, Innovation Chemistry, Alnylam Pharmaceuticals*

Breakthroughs in LNPs formulation and trivalent GalNAc conjugation of chemically modified oligonucleotides have paved the way to efficient delivery of oligonucleotide therapeutics to the liver. Additional ligands and delivery platforms are on the horizon for delivery to extrahepatic tissues. Lipid-conjugated siRNAs for CNS delivery and antibody-conjugated siRNAs for muscle delivery have entered clinical studies. An overview of chemical modifications, linkers, and targeting ligands for efficient delivery will be presented.

## BREAKTHROUGHS IN DELIVERY SCIENCE

**11:30 Engineering Delivery Vehicles and RNA-Design to Develop Potent Vaccines and Immunotherapies**

*Akash Gupta, PhD, Postdoctoral Researcher, Massachusetts Institute of Technology*

This talk will describe how to enhance immune activation and therapeutic benefit. Using combinatorial and medicinal chemistry, we developed ionizable lipids that drive strong antibody and T-cell responses, achieving protection comparable to FDA-approved vaccines at 100-fold lower doses. We also created adjuvant-like mRNAs (ADJ-mRNAs) that directly reprogram innate immune pathways, yielding durable anti-tumor immunity and improved responses against influenza and SARS-CoV-2. Together, these strategies advance next-generation personalized vaccines and immunotherapies.

**12:00 pm Transition to Lunch**

**12:10 Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:40 Session Break**

## PLENARY KEYNOTE SESSION

**1:20 Chairperson's Remarks**

*David Corey, PhD, Professor, Department of Pharmacology, UT Southwestern*



**1:25 Venture Philanthropy in Drug Development from a Rare-Disease Patient-Advocacy Perspective**

*Debra Miller, Founder & CEO, CureDuchenne*

CureDuchenne, the leading Duchenne patient-advocacy organization, will discuss its initiatives to accelerate the development and regulatory approval of the first drugs to treat Duchenne muscular dystrophy, in addition to its recent efforts supporting the next generation of improved therapeutic products. The presentation will outline existing gaps and strategic opportunities within the development pipeline, focusing on efforts to establish effective treatment options for all Duchenne patients, regardless of their genetic mutation.



**2:05 Oligonucleotide and Delivery Chemistry for siRNA Conjugates: Past Innovations and Future Opportunities**

*Weimin Wang, PhD, Founder & CEO, Sanegene Bio*

RNAi technology is at a crossroads; the focus is shifting from rare genetic disease applications to common chronic indications and general medicine. In this presentation, we will review key medicinal-chemistry concepts used in current RNAi medicines and describe next-generation approaches to enable this powerful modality to become a central component of standard of care for obesity and cardiovascular therapy.

**2:45 Refreshment Break in the Exhibit Hall and Last Chance for Poster Viewing**

## NEXT-GENERATION EDITING THERAPIES

**3:25 Chairperson's Remarks**

***Jonathan Watts, PhD, Professor, RNA Therapeutics Institute, University of Massachusetts Chan Medical School***

**3:30 Prime Editing with Chemically Modified Templates and Modification-Tolerant Polymerases**

***Jonathan Watts, PhD, Professor, RNA Therapeutics Institute, University of Massachusetts Chan Medical School***

We show that evolved and engineered polymerases can efficiently read 2'-O-Me-modified editing templates in both traditional fused-PE and split PE formats. Using these polymerases to carry out PE with modified templates, we show significantly improved editing efficiency when compared to MMLV RT and unmodified templates. We also show that the inclusion of non-nucleotide blockers into editing templates can improve editing purity by reducing readthrough into the scaffold.

**4:00 Advances in Therapeutic Genome Editing with Cas9 mRNA**

***Weijun Chen, PhD, Director, RNA Technologies Lead, Intellia Therapeutics***

Intellia is developing potentially curative gene editing treatments to transform the lives of patients. Cas9 mRNA has been applied to our pipelines of *in vivo* and *ex vivo* CRISPR-based therapies for life-threatening diseases with high unmet need. Our lead clinical programs, NTLA-2001 for ATTR amyloidosis and NTLA-2002 for hereditary angioedema, have entered Phase 3 trials.

**4:30 Development of a Versatile Platform for ADAR-Mediated RNA Editing across Tissues**

***Jack Godfrey, PhD, Senior Scientist, Biology, Wave Life Sciences***

Wave's AIMers are short, chemically modified oligonucleotides that direct A-to-I RNA editing via endogenous ADAR enzymes. Our optimized AIMer design supports efficient RNA editing in extrahepatic tissues including the central nervous system, kidney, and lung. We will show that AIMers support RNA editing and functional protein restoration of disease-relevant targets in multiple tissues.

**5:00 Close of Conference**

# Oligonucleotide CMC & Manufacturing

Advancing Analytical, Regulatory, and Manufacturing Strategies for Scalable, Compliant Oligonucleotide Therapeutics

## WEDNESDAY, MARCH 18

**7:30 am Short Course Registration and Morning Coffee**

**8:00 Recommended Short Course***

SC1: Safety & Toxicity of Nucleic Acids
OR
SC2: Successful Late Phase Regulatory Submission for a Complex Oligonucleotide
*Premium Registration or separate registration required. See Short Courses page for details.

**9:30 Main Conference Registration and Morning Coffee**

**10:30 Welcome Remarks by Conference Director**

## REGULATORY INSIGHTS AND APPROACHES

**10:40 Chairperson's Remarks**

*Benjamin Stevens, PhD, Senior Director, Regulatory Affairs CMC Policy and Advocacy, Alnylam*



**10:45 FEATURED PRESENTATION: A Platform Approach to Enzymatic Oligonucleotide Assembly—Technical and Regulatory Considerations**

*Benjamin Stevens, PhD, Senior Director, Regulatory Affairs CMC Policy and Advocacy, Alnylam*

This presentation introduces a versatile platform for enzymatic oligonucleotide assembly that offers improved efficiency, sustainability, and scalability. The approach relies on engineered ligase enzymes and templated purification, and supports rapid, cost effective, high-purity oligonucleotide API synthesis. The platform supports a range of potential API classes. The discussion will explore regulatory challenges, addressing flexibility for parallel manufacturing and regulatory alignment on specifications and reuse of reagents.

**11:15 CMC Regulatory Strategies: Comparison of Recently Approved US and Ex-US Commercial Applications for Antisense Oligonucleotides (ASOs)**

*Stephanie Nelson, Director, CMC Regulatory, Ionis Pharmaceuticals*

This presentation will focus on similarities and differences between recently approved US and ex-US commercial applications for ASOs. High-level information covering the CMC regulatory strategies and submission content among countries will be discussed, along with similarities and differences between health authority requests.

**11:45 AstraZeneca CMC Regulatory Experience on Clinical and Marketed Products**

*Thimma Rawalpally, PhD, CMC Director, AstraZeneca*

AstraZeneca's global CMC regulatory experience across clinical and marketed oligonucleotide therapeutics addresses starting material designation, stereochemistry and impurity control strategy, identity testing strategy, terminal sterilization feasibility assessment, specification setting under ICH frameworks. We share platform efficiencies in manufacturing, method validation, stability consolidation, and container-closure integrity. We also outline regulatory review priorities—engagement and harmonized established conditions enable approvals and lifecycle management worldwide.

**12:15 pm Transition to Lunch**

**12:25 Luncheon Presentation to be Announced**

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**12:55 Session Break**

## INNOVATIONS IN CMC AND ANALYTICAL DEVELOPMENT

**1:35 Chairperson's Remarks**

*Robert Dream, PhD, Managing Director, HDR Co. LLC*

**1:40 Methods to Establish Stereochemical Comparability in Phosphorothioated Oligos: Analytical Approaches & Development Strategies**

*Adebowale Shoroye, Scientist, Biogen*

Substitution of a nonbridging oxygen for sulfur on the phosphodiester backbone of an oligonucleotide creates a chiral center at the phosphorus atom of the linkage. Oligonucleotides with >10 phosphorothioate linkages are therefore mixtures of up to hundreds of thousands of diastereomers. Here, we evaluated three orthogonal methods—CD, 31P-NMR, and NP1 digestion followed by LCMS—for their sensitivity to detect change and their ability to monitor batch-to-batch stereochemical comparability.

**2:10 Identifying the Best Analytical Methods for Characterizing Impurities**

*Mike Webb, PhD, Founder & CEO, MikeWebbPharma Ltd.*

Chemically modified therapeutic oligonucleotides are less pure and contain many more complex impurities than typical molecules. Current chromatography methods struggle to resolve all the impurities. For this reason, either a mixture of UV and MS detection is used or, alternatively, a second orthogonal chromatography method. In this presentation we will discuss the current strategies for characterizing impurities and some ideas about how we can improve the toolbox in the future.

**2:40 Control Strategy for Antibody Oligonucleotide Conjugates (AOCs)**

*Katherine Stapleton Arie, PhD, Senior Scientist, Avidity*

Avidity Biosciences is developing Antibody Oligonucleotide Conjugates (AOCs), combining monoclonal antibodies with oligonucleotide therapies to treat rare genetic diseases, such as Myotonic Dystrophy Type 1 and Duchenne Muscular Dystrophy. This talk will review approaches to the analytical control strategy that take into account the complexities associated with the different intermediates that ultimately make up an AOC product.

**3:10 Grand Opening Refreshment Break in the Exhibit Hall with Poster Viewing**

emp BIOTECH

## PLENARY KEYNOTE SESSION

**4:00 Welcome Remarks by Conference Director**

*Gemma Smith, Senior Conference Director, Production, Cambridge Healthtech Institute*

**4:05 Chairperson's Remarks**

*Adrian Krainer, PhD, St. Giles Foundation Professor, Cold Spring Harbor Laboratory, CSHL Cancer Center*



**4:10 N-of-1 Therapeutics: Progress, Pitfalls, and Prospects for Future Individualized Medicines**

*Timothy Yu, PhD, Associate Professor Pediatrics, Genetics & Genomics, Boston Children's Hospital*

Successes in oligonucleotide therapeutics have spurred the creation of bespoke therapies for rare genetic conditions, even for single patients. This talk will review lessons, challenges, and opportunities stemming from these pioneering efforts, and offer perspectives on the ethical and regulatory hurdles to be overcome to realize a future of individualized medicines, whether as proof of concept or provision of care.



**4:50 ADAR RNA Editing: Applying Current Knowledge to Future Applications**

*Brenda Bass, PhD, Distinguished Professor, Biochemistry, University of Utah*

# Oligonucleotide CMC & Manufacturing

Advancing Analytical, Regulatory, and Manufacturing Strategies for Scalable, Compliant Oligonucleotide Therapeutics

Much is known about biochemical properties of ADAR RNA editing enzymes from decades of *in vitro* studies, but how these properties correlate with *in vivo* editing is not always clear. Properties established *in vitro* will be compared with observations made *in vivo*, with a focus on properties relevant to therapeutic applications, such as guided RNA editing. Recent progress on how inosine precludes activation of an immune response will be presented.

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

**6:30 Close of Day**

## THURSDAY, MARCH 19

**7:30 am Registration and Morning Coffee**

## ADVANCING SCALABLE AND SUSTAINABLE MANUFACTURING

**8:00 Chairperson's Remarks**

***Stephany Standley, PhD, Vice President Process Development, Wave Life Sciences***



**8:05 KEYNOTE PRESENTATION: Simplifying the Synthesis of Oligonucleotides**

***Phil Baran, PhD, Chair & Professor, Department of Chemistry, Scripps Research Institute***

This talk will discuss the development of new tools to synthesize and potentially manufacture small (cyclic dinucleotides & CDNs) and large oligonucleotides using the naturally occurring oxidation state of phosphorus: P(V). A particular emphasis will be placed on the invention of new methods to rapidly access internucleotide linkages beyond canonical phosphodiesters and phosphorothioates, such as the mesyl-phosphoramidate backbone, without recourse to toxic reagents.

**8:35 Recent Experiences with siRNA Manufacturing**

***Isaiah Cedillo, Executive Director, Manufacturing & Operations, Ionis Pharmaceuticals***

Ionis Pharmaceuticals has recently completed several large-scale manufacturing campaigns of conjugated small interfering RNA (siRNA) drug substances, including both peptide and GalNAc conjugates. These efforts have provided valuable insights into the unique challenges of siRNA manufacturing at commercial scale. This presentation will highlight observations related to process impurities encountered during synthesis and conjugation and discuss strategies employed to optimize purification and isolation operations.

**9:05 Innovating Oligonucleotide Production: Enzymatic Fragment Ligation for siRNA**

***Wuming Yan, PhD, Chief Scientist: Oligonucleotide, Technology, Asymchem Boston Corp.***

Asymchem offers a comprehensive platform for oligonucleotide development from early research to commercial production. This presentation introduces Enzymatic Fragment Ligation (EFL) as a next-generation solution for siRNA synthesis. Unlike traditional solid-phase methods, EFL assembles short RNA fragments under mild aqueous conditions, reducing impurities and improving yield, scalability, and cost efficiency. By optimizing fragment design, ligation parameters, and purification strategies, EFL enables high-purity siRNA manufacturing with enhanced reproducibility.

## IN-PERSON BREAKOUT DISCUSSIONS

**9:35 In-Person Breakout Discussions**

Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussion page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON BREAKOUT DISCUSSION: New NMPA Oligonucleotide Guideline: Considerations for Oligonucleotide Development and Regulatory Submissions**

***Benjamin Stevens, PhD, Senior Director, Regulatory Affairs CMC Policy and Advocacy, Alnylam***

**IN-PERSON ONLY BREAKOUT: Strategies for Outsourcing Manufacturing**

***Khaled Yamout, Analytical Sciences, Quality and Manufacturing Consultant, Y-Chem Consulting LLC***

**10:20 Coffee Break in the Exhibit Hall with Poster Viewing**

**11:00 Phase-Appropriate Development of Control Strategy for New Therapeutics Oligonucleotides**

***Marc Lemaitre, PhD, Oligonucleotide Therapeutics CMC/Regulatory Consultant, ML_Consult LLC***

The development of new therapeutics oligonucleotides can be quite different depending on purpose: N=1, rare disease, small or large indication, and other considerations. Presence of new modifications or usage of well-known starting materials can also impact the process. We will present some different situations to help attendees who are new to such endeavors.

**11:30 Synthesis and Characterization of Stereopure Chimeric Oligonucleotides**

***Stephany Standley, PhD, Vice President Process Development, Wave Life Sciences***

At Wave Life Sciences, the PRISM platform enables synthesis of stereopure oligonucleotides containing chimeric phosphorothioate (PS), phosphoryl guanidine (PN), and phosphodiester (PO) backbones that are rationally designed to optimize pharmacology and efficacy. We will describe approaches, to synthesize and characterize stereopure oligonucleotides at multiple scales, which enable detailed structure-activity relationship analysis.

**12:00 pm Transition to Lunch**

**12:10 Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:40 Session Break**

# Oligonucleotide CMC & Manufacturing

Advancing Analytical, Regulatory, and Manufacturing Strategies for Scalable, Compliant Oligonucleotide Therapeutics

## PLENARY KEYNOTE SESSION

**1:20 Chairperson's Remarks**
*David Corey, PhD, Professor, Department of Pharmacology, UT Southwestern*



**1:25 Venture Philanthropy in Drug Development from a Rare-Disease Patient-Advocacy Perspective**
*Debra Miller, Founder & CEO, CureDuchenne*
CureDuchenne, the leading Duchenne patient-advocacy organization, will discuss its initiatives to accelerate the development and regulatory approval of the first drugs to treat Duchenne muscular dystrophy, in addition to its recent efforts supporting the next generation of improved therapeutic products. The presentation will outline existing gaps and strategic opportunities within the development pipeline, focusing on efforts to establish effective treatment options for all Duchenne patients, regardless of their genetic mutation.



**2:05 Oligonucleotide and Delivery Chemistry for siRNA Conjugates: Past Innovations and Future Opportunities**
*Weimin Wang, PhD, Founder & CEO, Sanegene Bio*
RNAi technology is at a crossroads; the focus is shifting from rare genetic disease applications to common chronic indications and general medicine. In this presentation, we will review key medicinal-chemistry concepts used in current RNAi medicines and describe next-generation approaches to enable this powerful modality to become a central component of standard of care for obesity and cardiovascular therapy.

**2:45 Refreshment Break in the Exhibit Hall and Last Chance for Poster Viewing**

WuXi TIDES

## ADVANCING SCALABLE AND SUSTAINABLE MANUFACTURING

**3:25 Chairperson's Remarks**
*Rakesh Dixit, PhD, DABT, CEO & President, Bionavigen Oncology, LLC; CSO, TMAB Therapeutics, Regio Biosciences*

**3:30 Accelerating CMC Development for Extra-Hepatic siRNA Delivery: A Biotech Perspective**
*Sibo Jiang, PhD, Vice President, CMC Development, Sanegene Bio*
SanegeneBio has built a strong hepatic siRNA pipeline and extended its LEAD (Ligand and Enhancer Assisted Delivery) platform across several extrahepatic programs. Extrahepatic delivery presents unique CMC challenges across process development, analytics, manufacturing, and scale-up. This presentation will share a case study on siRNA CMC development, highlighting practical strategies that enabled robust, scalable manufacturing to support IND-enabling activities for extrahepatic programs.

**4:00 Novel Approaches to the Manufacturing of AOCs and Oligo-Peptide Conjugates**
*Robert Dream, PhD, Managing Director, HDR Co. LLC*
The manufacturing of AOCs has emerged as a critical area of innovation in the development of targeted therapeutics and diagnostic tools. This novel approach is aimed at improving the efficiency, specificity, and scalability of conjugation strategies, addressing key challenges such as site-selective modification, linker stability, and product homogeneity by integrating advanced bioconjugation chemistries, optimized purification protocols, and automation-friendly workflows.

# mRNA & Emerging Oligonucleotide Modalities

Next-Generation RNA Therapies for Increased Efficacy, Stability, and Targeted Delivery

## WEDNESDAY, MARCH 18

**7:30 am Short Course Registration and Morning Coffee**

**8:00 Recommended Short Course***

SC1: Safety & Toxicity of Nucleic Acids

*Premium Registration or separate registration required. See Short Courses page for details.

**9:30 Main Conference Registration and Morning Coffee**

**10:30 Welcome Remarks by Conference Director**

### INNOVATIONS IN OLIGO MODALITIES

**10:40 Chairperson's Remarks**

*Dan Peer, PhD, Professor & Director, Laboratory of Precision Nanomedicine; Vice President for Research, Tel Aviv University*

**10:45 KEYNOTE PRESENTATION: Preclinical Development of an RNA/DNA Hybrid TLR7/8/9 Agonist for Cancer Immunotherapy**



*Arthur Krieg, MD, Founder, President and Acting CEO/CSO, Zola Therapeutics*

Viral nucleic acids detected by TLR7/8 (RNA) and TLR9 (DNA) induce CD8+ T cells. We have developed first-in-class TLR7/8/9 agonists in lipid nanoparticles that induce unprecedented IFN-a without activating inflammatory cytokine responses. Our development candidate, Z-007, activates human tumor-associated immune cells *ex vivo*, can be delivered IV with excellent safety in mice and nonhuman primates, and induces tumor regression in murine models and in spontaneous canine tumors without apparent toxicity.

**11:15 Optimized RESTORE+ Oligonucleotides for an Efficacious and Safe RNA Base Editing Treatment for Alpha-1 Antitrypsin Deficiency**

*Namita Bisaria, Head, Research Strategy and Operations, AIRNA*

We developed a fully chemically modified, GalNAc-conjugated oligonucleotide platform that recruits endogenous ADAR for precise A-to-I RNA editing. Targeting SERPINA1 to correct the PiZZ mutation in AATD, we achieved >90% *in vitro* and >50% *in vivo* editing, restoring therapeutic M-AAT levels with subcutaneous dosing. NHP studies showed extended half-life and no toxicity, supporting a safe, durable, disease-modifying therapy and rapid expansion to other genetic diseases.

**11:45 Talk Title to be Announced**

*Daniel Glazier, PhD, Scientist II, Medicinal Chemistry, Alltrna*

**12:15 pm Transition to Lunch**

**12:25 Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:55 Session Break**

### TRENDS IN OLIGO THERAPEUTICS

**1:35 Chairperson's Remarks**

*Paloma Giangrande, PhD, President & Founder Biologic Insights LLC; CSO & Founder, Program Therapeutics Inc.*

**1:40 PANEL DISCUSSION: Emerging Modalities and Technologies for Developing Oligo Therapeutics**

*Moderator: Paloma Giangrande, PhD, President & Founder Biologic Insights LLC; CSO & Founder, Program Therapeutics Inc.*

This panel brings together leaders from industry, academia, and investment to discuss state-of-the-art oligonucleotide strategies, including circRNA, RNA editing, siRNAs, and engineered tRNAs. Presentations will span from discovery and design to delivery and real-world case studies, offering a platform to share translational advances and future directions for oligo-based therapeutics.

*Panelists:*

*Alan Horsager, PhD, Managing Partner, Concept Bio*

*Neil Kubica, PhD, Therapeutics Division Lead, General Inception*

*Tiziana Rossetti, PhD, Principal, Sofinnova Partners*

*Laura Sepp-Lorenzino, PhD, Biotech Executive, Board Member, Advisor, former CSO of Intellia Therapeutics, Inc.*

*Sebastian Trousil, PhD, Co-Founder & COO, City Therapeutics*

**2:40 Sponsored Presentation** *(Opportunity Available)*

**3:10 Grand Opening Refreshment Break in the Exhibit Hall with Poster Viewing**

### PLENARY KEYNOTE SESSION

**4:00 Welcome Remarks by Conference Director**

*Gemma Smith, Senior Conference Director, Production, Cambridge Healthtech Institute*

**4:05 Chairperson's Remarks**

*Adrian Krainer, PhD, St. Giles Foundation Professor, Cold Spring Harbor Laboratory, CSHL Cancer Center*



**4:10 N-of-1 Therapeutics: Progress, Pitfalls, and Prospects for Future Individualized Medicines**

*Timothy Yu, PhD, Associate Professor Pediatrics, Genetics & Genomics, Boston Children's Hospital*

Successes in oligonucleotide therapeutics have spurred the creation of bespoke therapies for rare genetic conditions, even for single patients. This talk will review lessons, challenges, and opportunities stemming from these pioneering efforts, and offer perspectives on the ethical and regulatory hurdles to be overcome to realize a future of individualized medicines, whether as proof of concept or provision of care.



**4:50 ADAR RNA Editing: Applying Current Knowledge to Future Applications**

*Brenda Bass, PhD, Distinguished Professor, Biochemistry, University of Utah*

Much is known about biochemical properties of ADAR RNA editing enzymes from decades of *in vitro* studies, but how these properties correlate with *in vivo* editing is not always clear. Properties established *in vitro* will be compared with observations made *in vivo*, with a focus on properties relevant to therapeutic applications, such as guided RNA editing. Recent progress on how inosine precludes activation of an immune response will be presented.

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

**6:30 Close of Day**

## THURSDAY, MARCH 19

**7:30 am Registration and Morning Coffee**

### OPTIMIZING mRNA DESIGN & DELIVERY

**8:00 Chairperson's Remarks**

*Dmitry Samarsky, PhD, CSO and Board Member, ARNAgen Therapeutics*

**8:05 mRNA Design as it Relates to Physical and Functional Characterization**

*Khaled Yamout, Analytical Sciences, Quality and Manufacturing Consultant, Y-Chem Consulting LLC*

# mRNA & Emerging Oligonucleotide Modalities

Next-Generation RNA Therapies for Increased Efficacy, Stability, and Targeted Delivery

The emergence of mRNA modality as a therapeutic alternative for treatment of viruses, infectious disease, and more has highlighted the need for understanding how the design of mRNA impacts functionality of mRNA. To understanding how mRNA design impacts translation one needs to correlate the physical characterization to functional characterization of mRNA. We will discuss various strategies for assessing the impact of mRNA design on physical and functional characterization of mRNA.

**8:35 Advancing Cancer Immunotherapy with Synthetic mRNA Biology: Clinical insights from STX-001 and Platform innovations from STX-003**

***Jaspreet Khurana, PhD, Senior Director, mRNA Programming, Strand Therapeutics, Inc.***

mRNA therapeutics have redefined vaccine development, yet their transformative potential in cancer gene therapy remains largely unrealized. At Strand Therapeutics, we are pioneering a programmable mRNA platform designed to expand the therapeutic window of cytokine-based immunotherapies by combining the potency of self-replicating mRNA with sophisticated genetic circuit engineering. This approach aims to deliver precise and controlled immune activation with improved safety across a range of solid tumors.

**9:05 Transformer-Based Models for mRNA Sequence Understanding and *de novo* Sequence Generation**

***Sizhen Li, PhD, Computational Scientist Lead, Digital R&D, Sanofi***

This presentation introduces mRNA-LM and mRNA-GPT, language models for comprehensive mRNA sequence analysis and rational design. mRNA-LM extracts sequence representations, predicts biophysical and biological properties, and defines reward functions for optimization. mRNA-GPT, a decoder-based model, generates *de novo* mRNA sequences with improved stability and translational efficiency. Together, these models enable systematic exploration of the mRNA design space and accelerate early-stage vaccine and therapeutic development.

## IN-PERSON BREAKOUT DISCUSSIONS

**9:35 In-Person Breakout Discussions**

Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussion page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON BREAKOUT DISCUSSION: Design and Optimization of mRNA Drugs**

***Iris Grossman, PhD, Chief Therapeutics Officer, R&D, Eleven Therapeutics US, Inc.***

***Jaspreet Khurana, PhD, Senior Director, mRNA Programming, Strand Therapeutics, Inc.***

***Sizhen Li, PhD, Computational Scientist Lead, Digital R&D, Sanofi***

***Alex Zinoviev, PhD, Director of mRNA Platform, Gene Therapy, Eli Lilly & Co.***

**IN-PERSON ONLY BREAKOUT: Tackling Challenges with mRNA Delivery**

***Ekkehard Leberer, PhD, Professor of Biochemistry, Technical University of Munich; Senior Consultant, ELBIOCON; Advisor, Neuway Pharma***

***Dan Peer, PhD, Professor & Director, Laboratory of Precision Nanomedicine; Vice President for Research, Tel Aviv University***

**IN-PERSON ONLY BREAKOUT: Emerging Oligo Modalities for Diverse Therapeutic Applications**

***Namita Bisaria, Head, Research Strategy and Operations, AIRNA***

***Sushma Gurumurthy, PhD, Former Senior Director, Oncology Research, Moderna, Inc.***

***Edo Kon, PhD, Director of Business Development, RiboX Therapeutics***

***Arthur Krieg, MD, Founder, President and Acting CEO/CSO, Zola Therapeutics***

**10:20 Coffee Break in the Exhibit Hall with Poster Viewing**

BACHEM

**11:00 Next-Generation Payloads and Delivery Technologies**

***Dan Peer, PhD, Professor & Director, Laboratory of Precision Nanomedicine; Vice President for Research, Tel Aviv University***

Active cellular targeting using lipid nanoparticles (LNPs) represents the next generation of RNA delivery vehicles. In my presentation, I will detail four payloads: locked nucleic acids (LNA), siRNA, mRNA, CRISPR mRNA, and sgRNAs in a single LNP. I will highlight several studies, including novel therapeutics for IBD, for hematological malignancies, for expressing a toxin protein in cancer, and for therapeutic genome editing. Special emphasis will be made on CMC challenges.

**11:30 PANEL DISCUSSION: Challenges and Successes Funding RNA Therapeutics R&D**

***Moderator: Dmitry Samarsky, PhD, CSO and Board Member, ARNAgen Therapeutics***

Prominent entrepreneurs from academia, biotech, pharma and venture capitalists share their views on existing opportunities and limitations when it comes to funding innovation in early-stage research for developing RNA-based therapies.

***Panelists:***

***Anastasia Khvorova, PhD, Professor, RNA Therapeutic Institute, University of Massachusetts Medical School***

***Alex Zinoviev, PhD, Director of mRNA Platform, Gene Therapy, Eli Lilly & Co.***

**12:00 pm Transition to Lunch**

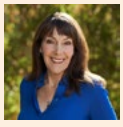
**12:10 Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:40 Session Break**

## PLENARY KEYNOTE SESSION

**1:20 Chairperson's Remarks**

***David Corey, PhD, Professor, Department of Pharmacology, UT Southwestern***



**1:25 Venture Philanthropy in Drug Development from a Rare-Disease Patient-Advocacy Perspective**

***Debra Miller, Founder & CEO, CureDuchenne***

CureDuchenne, the leading Duchenne patient-advocacy organization, will discuss its initiatives to accelerate the development and regulatory approval of the first drugs to treat Duchenne muscular dystrophy, in addition to its recent efforts supporting the next generation of improved therapeutic products. The presentation will outline existing gaps and strategic opportunities within the development pipeline, focusing on efforts to establish effective treatment options for all Duchenne patients, regardless of their genetic mutation.



**2:05 Oligonucleotide and Delivery Chemistry for siRNA Conjugates: Past Innovations and Future Opportunities**

***Weimin Wang, PhD, Founder & CEO, Sanegene Bio***

RNAi technology is at a crossroads; the focus is shifting from rare genetic disease applications to common chronic indications and general medicine. In this presentation, we will review key medicinal-chemistry concepts used in current RNAi medicines and describe

next-generation approaches to enable this powerful modality to become a central component of standard of care for obesity and cardiovascular therapy.

**2:45 Refreshment Break in the Exhibit Hall and Last Chance for Poster Viewing**

## TARGETING DIVERSE INDICATIONS

**3:25 Chairperson's Remarks**

*Alex Zinoviev, PhD, Director of mRNA Platform, Gene Therapy, Eli Lilly & Co.*

**3:30 FEATURED PRESENTATION: The Use of Therapeutic tRNAs for the Treatment of Duchenne Muscular Dystrophy and Dilated Cardiomyopathy**

*Elisabeth Gardiner, PhD, CSO, Tevard Biosciences*

Premature termination codons (PTCs) represent 10-15% of the genetic cause of disease. Therapeutic tRNAs enable ribosomes to read through PTCs and produce full-length, functional protein. Therapeutic tRNAs have been designed for all three stop codons (TAA, TAG, and TGA) and validated in translationally relevant preclinical models. Unlike traditional gene-specific approaches that address individual proteins, tRNAs offer a unified therapeutic platform for diverse genetic conditions that are mediated by PTCs.

**4:00 xRNA: A Chemically Modified RNA Platform Enabling Sustained Therapeutic Protein Expression with Cross-Species Durability**

*Adi Gilboa-Geffen, PhD, CTO, Eleven Therapeutics*

Utilizing combinatorial screens we can optimize mRNA molecules to improve both durability and translation of their payloads. The easily programable nature of xRNA enables us to combine multiple payloads, generating a highly modular single backbone, that synchronizes the expression of multiple therapeutic proteins.

**4:30 Developing mRNA Therapeutics for Cardiovascular Diseases**

*Ajit Magadum, PhD, Assistant Professor, Center for Regenerative Medicine, Department of Internal Medicine, Heart Institute, University of South Florida*

mRNA therapeutics is rapidly emerging as a groundbreaking strategy for treating cardiovascular diseases (CVD), which claimed 20 million lives globally in 2022 and affects 650 million people. In my presentation, I will share a decade of our work on mRNA therapies that promote cardiac function and repair, combat fibrosis, cell death, and hypertrophy in CVD models. Additionally, we introduce novel cell-specific mRNA expression platforms, advancing the field of CVD therapeutics.

**5:00 Close of Conference**

# Peptides & Emerging Drug Conjugates

Design and Delivery for Developing Novel Peptide, Antibody and Radioligand Modalities

## WEDNESDAY, MARCH 18

**7:30 am Short Course Registration and Morning Coffee**

**8:00 Recommended Short Course***

SC3: Next Gen ADCs & Advanced Linkers & Conjugates: Mastering Design, Linker Optimization & Stability

*Premium Registration or separate registration required. See Short Courses page for details.

**9:30 Main Conference Registration and Morning Coffee**

**10:30 Welcome Remarks by Conference Director**

## KEYNOTE SESSION: ADVANCES IN CONJUGATED THERAPIES

**10:40 Chairperson's Remarks**

*Vadim Dudkin, PhD, Founding CTO, Souffle Therapeutics*



**10:45 KEYNOTE PRESENTATION: Advancing Targeted Radioligand Therapies—Clinical Perspectives on Lutetium- and Actinium-Based PSMA-617 Conjugates**

*Mark Rutstein, MD, Global Head, Oncology Development, Novartis Pharma SAS*

We will review efficacy and safety outcomes from pivotal and ongoing studies, explore mechanisms driving therapeutic benefit, and discuss strategies to optimize patient selection and sequencing. Additionally, the talk will address the evolving role of alpha-emitting conjugates in overcoming resistance and enhancing durability of response. These insights underscore the potential of peptide-based radioligand therapies to redefine precision oncology and improve outcomes for patients with advanced prostate cancer.



**11:30 KEYNOTE PRESENTATION: The Infinite Loop—Machine Learning for Discovery, Delivery, and Rapid Manufacturing of Potential Medicines**

*Bradley L. Pentelute, PhD, Professor, Department of Chemistry, Massachusetts Institute of Technology*

Lack of data is slowing down how we can use machine learning to create powerful new medicines. We are solving this problem by creating data highways from millions of small molecules, peptides and small proteins. We are now able to use machine learning to discover and create new functional molecules quickly. Our next step is to create an infinite loop where we automatically design, build, and test potential new medicines.

**12:15 pm Transition to Lunch**

**12:25 Luncheon Presentation to be Announced**

PROIMMUNE www.proimmune.com

**12:55 Session Break**

## TARGET-SPECIFIC DELIVERY

**1:35 Chairperson's Remarks**

*Vadim Dudkin, PhD, Founding CTO, Souffle Therapeutics*

**1:40 Cell and Tissue Specific Delivery of Therapeutic Payloads**

*Vadim Dudkin, PhD, Founding CTO, Souffle Therapeutics*

Delivery of therapeutic payloads in a cell and tissue specific manner has given rise to several classes of novel medicines – from ADCs to oligonucleotide conjugates to targeted radiopharmaceuticals. The presentation will provide an overview of concepts and methods for selection and characterization of ligands enabling these delivery approaches.

**2:10 Exploring Different Targeting Modalities for the Delivery of siRNA**

*Justin Murray, PhD, Senior Director, Research, Amgen Inc.*

**2:40 Sponsored Presentation** *(Opportunity Available)*

**3:10 Grand Opening Refreshment Break in the Exhibit Hall with Poster Viewing**

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## NOVEL PAYLOADS & LINKERS

**4:05 A Clinically Validated NMT Inhibitor as an ADC Payload**

*Michael Weickert, PhD, CEO, Pacylex Pharmaceuticals*

Zelenirstat is the first N-myristoyltransferase (NMT) inhibitor to be tested in patients. NMT inhibitors work by multiple mechanisms to kill cancer cells. Phase 1 results with oral zelenirstat showed good safety and extended survival in solid tumor patients. As an ADC payload, zelenirstat was significantly more potent against solid tumor cancer cells using several different targeting antibodies and linkers. Dozens of related NMTis may also serve as ADC payloads.

**4:35 The FORCE™ Platform Leverages TfR1 to Deliver Therapeutic Oligonucleotides to the Central Nervous System**

*Amit Luthra, PhD, Associate Director, Dyne Therapeutics Inc.*

Dyne's FORCE Platform leverages a transferrin receptor-1 targeting Fab to enable targeted intracellular delivery of therapeutic payloads and is advancing in clinical development for neuromuscular disorders, including Duchenne muscular dystrophy and myotonic dystrophy type 1. In mouse models, the platform demonstrated the ability to deliver oligonucleotide therapeutics to the central nervous system with high efficiency. It also enables robust delivery of oligonucleotides to the primate brain, with widespread tissue distribution.

**5:00 Safety and CMC Challenges with Antibody Oligonucleotide Conjugate (AOC) and Peptide Drug Conjugates (PDC)**

*Rakesh Dixit, PhD, DABT, CEO & President, Bionavigen Oncology, LLC; CSO, TMAB Therapeutics, Regio Biosciences*

Antibody–oligonucleotide conjugates (AOCs) and peptide–drug conjugates (PDCs) are novel therapies that combine targeted delivery with strong intracellular effects but introduce unique safety risks. AOCs may cause immunogenicity from new conjugation sites, activate innate immunity via toll-like receptors, or induce unintended gene modulation through off-target hybridization. For PDCs, risks include proteolytic breakdown, fast renal clearance, off-target binding, and payload leakage leading to toxicity. Both face aggregation, heterogeneity, and narrow therapeutic windows.

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

oligo YAMASA

**6:30 Close of Day**

## THURSDAY, MARCH 19

**7:30 am Registration and Morning Coffee**

## NEW PEPTIDE & PROTEIN CHEMISTRIES

**8:00 Chairperson's Remarks**

*Sunny Zhou, PhD, Professor, Chemistry & Chemical Biology, Northeastern University*

**8:05 Photoactivation of Conjugates as Novel Therapeutics with Spatio-Temporal Control**

*Sunny Zhou, PhD, Professor, Chemistry & Chemical Biology, Northeastern University*

# Peptides & Emerging Drug Conjugates

Design and Delivery for Developing Novel Peptide, Antibody and Radioligand Modalities

I will present novel protein conjugates that can be activated by light. One example is photoimmunotherapy (PIT), in which the photosensitizer payloads generate reactive species (ROS) that kills cells with light and oxygen. Another example is photo-caging or photo-activation. The precise spatial and temporal control offered by photomedicine not only significantly minimizes off-site toxicities and enhances the therapeutic index, but also markedly expand the range of druggable targets.

**8:35 FEATURED PRESENTATION: Leveraging Radical Enzymology Towards New Peptide Architectures**

***Vahe Bandarian, PhD, Professor, Biological Chemistry, University of Utah; Co-Founder, Sethera Therapeutics***

Cyclic and polycyclic peptides are of considerable interest as therapeutic agents because the conformational restriction resulting from macrocyclization ensure specificity and resistance to proteolytic degradation. However, assembly of polymacrocyclic structures is often challenging because of the necessity for multi-step syntheses involving orthogonal protecting groups. In this presentation, a scalable, radical-mediated enzyme process for catalyzing formation of thioether crosslinks across a broad spectrum of peptide substrates is discussed.

**9:05 Sponsored Presentation** *(Opportunity Available)*

## IN-PERSON BREAKOUT DISCUSSIONS

**9:35 In-Person Breakout Discussions**

Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussion page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON BREAKOUT DISCUSSION: Use of AI-ML to Accelerate Drug Development**

***Ruben Abagyan, PhD, Professor, Skaggs School of Pharmacy and Pharmaceutical Sciences, University of California, San Diego***

***Alan Nafiiev, PhD, CEO & Founder, Receptor.AI***

**10:20 Coffee Break in the Exhibit Hall with Poster Viewing**

BACHEM

**11:00 Chemically Enhanced Phage Display**

***Jianmin Gao, PhD, Professor of Chemistry, Boston College***

Phage display is a powerful platform for screening peptide libraries. However, this popular technology has been largely limited to natural peptides. This presentation will discuss several chemical methods for the facile modification of phage displayed peptides to enable multicyclization and the introduction of designer functionalities. Screening of such chemically enhanced phage libraries allows quick identification of peptide natural product like inhibitors for various challenging targets including protein-protein interactions.

**11:30 Diversity-Generating Amino Acids for Peptide Macrocylization**

***Terry Moore, PhD, Associate Professor, Pharmaceutical Sciences, University of Illinois Chicago***

We present ring-closing amino acids bearing linkers with functional groups that enable further derivatization. This strategy facilitates efficient peptide macrocyclization and introduces a point of diversification, expanding the structural and chemical diversity of cyclic peptides. Applications include targeting wild-type and mutant estrogen receptors, as well as other therapeutically relevant proteins. The approach supports the generation of cyclic peptide libraries from a common sequence for drug discovery and chemical biology.

**12:00 pm Transition to Lunch**

**12:10 Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:40 Session Break**

## GENERATIVE MODELS FOR PEPTIDE DESIGN

**1:20 Chairperson's Remarks**

***Ruben Abagyan, PhD, Professor, Skaggs School of Pharmacy and Pharmaceutical Sciences, University of California, San Diego***

**1:25 Generating Large Binders and Constrained Oligomers to Target Challenging Pockets and Interfaces**

***Ruben Abagyan, PhD, Professor, Skaggs School of Pharmacy and Pharmaceutical Sciences, University of California, San Diego***

Disease associated molecular profiles reveals new targets together with their molecular partners, interfaces, mutations, and therapeutic objectives. Some of the interfaces are not suitable for small molecules and larger oligomers, with unusual monomers and attachments needed. Constrained backbone helps to make sampling in both chemical space and conformational space feasible. We present benchmarks and optimized structure prediction algorithms to enable optimization of these binders and some success stories.

**1:50 Generative Sequence Models to Design Programmable Biologics**

***Pranam Chatterjee, PhD, Assistant Professor, Department of Bioengineering, University of Pennsylvania***

We develop discrete generative models to design functional biologics directly from sequence. Our newest models, PepTune, AReUReDi, and TR2-D2 extend discrete diffusion and flow-based generation to produce noncanonical and cyclic peptides optimized for high affinity, solubility, and half-life, as well as low toxicity and non-fouling properties. We finally show that these algorithmic frameworks now generalize across modalities, supporting the design of mRNA, membrane proteins, and nanobody therapeutics.

**2:20 AI-Guided Multi-Objective Optimization of Peptides: Balancing Target Affinity & Membrane Permeability**

***Alan Nafiiev, PhD, CEO & Founder, Receptor.AI***

In this talk we will discuss the development of predictive models to evaluate peptide target binding and passive diffusion across cell membranes. Application of AI-driven multi-objective optimization strategies to enhance both affinity and permeability simultaneously and case examples demonstrating how these approaches accelerate peptide drug discovery will also be highlighted.

**2:45 Refreshment Break in the Exhibit Hall and Last Chance for Poster Viewing**

WuXi TIDES

## OVERCOMING TRANSLATIONAL CHALLENGES

**3:30 Targeting the Untargeted via Peptides: A New Approach to Treat Cancer**

***Martin Ulmschneider, PhD, Professor, Department of Chemistry, Kings College London***

At the cellular level cancer cells remain similar to their environment and the often subtle changes have proved hard to target and vary widely by stage and type of cancer. Many properties common to tumors have remained untargeted primarily because they are difficult to modulate via small molecules. Transformative changes in peptide technology are now paving the way to target more complex collective cellular properties.

**4:00 2K015, a Novel Nectin4-Targeting Peptide Radioligand with High Tumor Retention: Preclinical and Human IIT Study Evaluation**

***Weiliang (Timo) Xu, PhD, Associate Director, Business Development, Zonsen Peplib Biotech***

2K015 is a novel Nectin-4-targeting peptide radioligand with pM binding affinity, demonstrating strong tumor uptake and an improved biodistribution profile versus 68Ga-BT8009 from Bicycle Therapeutics in SW780-Nectin4 CDX models. Importantly, in a human IIT (n=3), 68Ga-2K015 achieved clear tumor imaging with low liver and kidney uptake, supporting strong translational potential for clinical development.

**4:30 Antibody–Peptide Fusions for Precise, Durable GPCR Modulation**

***Arjan Hada, PhD, Senior Scientist, Machine Learning Bioinformatics, iBio Inc.***

Peptide drugs are potent but often short-lived. We introduce antibody–peptide fusions delivering selective GPCR agonism with potential for extended exposure. Screening leverages machine-learning–designed soluble GPCR analogs that present native-like epitopes in stable, screening-ready surrogates. These designs are structurally validated and show specific ligand binding, providing reliable tools for discovery and characterization.

**5:00 Close of Conference**



# HOTEL AND TRAVEL

**Conference Venue and Hotel:**
Seaport Hotel Boston
One Seaport Lane
Boston, MA 02110

**Discounted Room Rate:** $269 s/d
**Discounted Room Rate Cut-off Date:** February 18, 2026

**For Reservations and Additional Information:**
**Visit optcongress.com/hotel-travel**




# POSTER INFORMATION

***Present a Poster and Save $50!***

Cambridge Healthtech Institute encourages attendees to gain further exposure by presenting their work in the poster session. To secure an onsite poster board and/or ensure your poster is included in the conference materials, your submission must be received, and your registration paid in full by **February 6, 2026.**

**Reasons you should present your research poster at this conference:**

- Your research will be seen by our international delegation, representing leaders from top pharmaceutical, biotech, academic and government institutions
- Discuss your research and collaborate with other attendees
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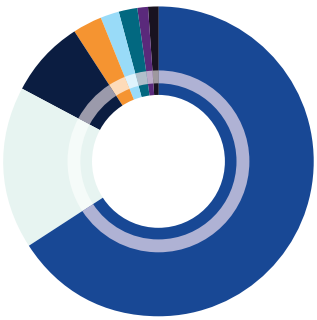
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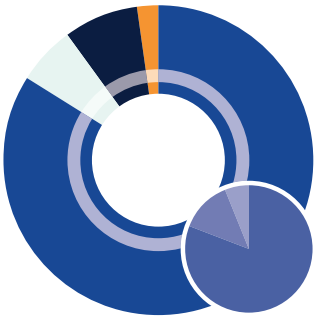

**Kristin Skahan**
Senior Business Development Manager
781.972.5431
kskahan@healthtech.com


## 2025 ATTENDEE DEMOGRAPHICS



### COMPANY TYPE

| | |
|---|---|
| Biotech | 66% |
| Pharma | 17% |
| Academic | 8% |
| Services | 3% |
| Healthcare | 2% |
| Other | 2% |
| CRO | 1% |
| Societies | 1% |

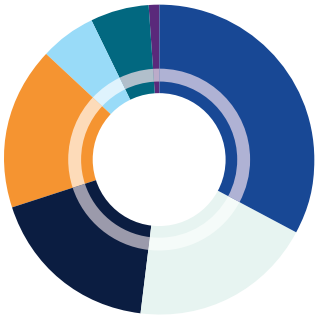


### GEOGRAPHIC LOCATION

| | |
|---|---|
| USA | 84% |
| Asia | 6% |
| Europe | 8% |
| Rest of World | 2% |

**US Breakdown**

| | |
|---|---|
| East Coast | 81% |
| West Coast | 13% |
| Midwest | 6% |



### DELEGATE TITLE

| | |
|---|---|
| Scientist/Technologist | 33% |
| Director | 19% |
| Executive | 18% |
| Sales & Marketing | 17% |
| Manager | 6% |
| Professor | 6% |
| Assistant | 1% |

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### CONFERENCE PRICING

| **PREMIUM PACKAGE: IN-PERSON ONLY**<br>**(Includes access to 4 conferences and on-demand for one year. Includes 1 in-person short course.)** | Commercial | Academic, Government, Hospital-affiliated |
|---|---|---|
| Early Bird Registration until December 5 | $1999 | $949 |
| Early Registration until January 9 | $2099 | $999 |
| Advance Registration until February 6 | $2299 | $1099 |
| After February 6 | $2499 | $1199 |
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| Early Bird Registration until December 5 | $1599 | $699 |
| Early Registration until January 9 | $1799 | $749 |
| Advance Registration until February 6 | $1999 | $849 |
| After February 6 | $2199 | $949 |

### SHORT COURSE PRICING | IN-PERSON ONLY

| | | |
|---|---|---|
| One short course | $699 | $399 |

> **The conference was very valuable. The presentations and information were immensely useful. The networking opportunities were endless**
>
> **Mariana Bravo Hernandez**
> Senior Scientist, IONIS

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**POSTER SUBMISSION:** Poster materials are due by **February 6, 2026.** Once your registration has been fully processed, we will send an email containing a unique link allowing you to submit your poster abstract and other materials. If you do not receive your link within 5 business days, please contact jring@healthtech.com.

*CHI reserves the right to publish your poster materials in various marketing materials and products.*

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