SAVE TODAY with Early Registration

11TH Annual

OPT Congress

Oligonucleotide, mRNA & Peptide Therapeutics

MARCH 18 – 19, 2026

BOSTON, MA + VIRTUAL

SEAPORT HOTEL

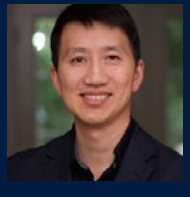
2026 CONFERENCE PROGRAMS

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

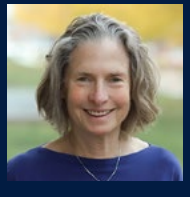
IN-PERSON SHORT COURSES

- 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

OPTCongress.com

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
Distinguished Professor, University of Colorado

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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

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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
Dushen Chetty, PhD, Vice President & Global Program Head, Prostate Cancer, Novartis Oncology

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: Thiomorpholino Oligonucleotides (TMOs) Useful for Exon Skipping, RNase H, and siRNA Applications

Marvin Caruthers, PhD, Distinguished Professor, University of Colorado

TMOs as cap/gapmers are very active in controlling the expression of glioblastomas, U4 noncoding RNA, allele specific knockdown of SLC6A1, and multicentric carpotarsal osteolysis. Similarly, TMOs control expression of DMD, STAT3 in head/neck tumors, inflammation via ITGA4, PKM, TUG 1 lncRNA, psoriasis, and recessive dystrophic epidermolysis bullosa. Recent research has focused on controlling expression of peroxiredoxin (PRDX) 6 via TMO modified siRNA and using TMOs to control PEG 10 translation.

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 to be Announced

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.

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: 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

BACHEM

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.

INNOVATIONS IN EXTRAHEPATIC DELIVERY AND TISSUE TARGETING

11:30 Targeted RNA Drug Delivery - Possible New Treatment Options

Thomas Frischmuth, PhD, CEO, rnatics

Oligomeric stabilized RNA therapeutics are delivered by attached targeting ligands. Particularly successful was the liver targeting via tri- GalNAc targeting the ASGPR receptor on liver cells. Baseclick has developed bioconjugation strategies to attach various ligands to oligomeric RNA therapeutics, for example branched tri-mannose was able to target specially the MRC1 receptor on macrophages and premature dendritic cells. One LNA molecule is entering clinical stage.

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, Director, CMC Policy and Advocacy, GSK

10:45 FEATURED PRESENTATION: A Platform Approach to Enzymatic Oligonucleotide Assembly—Technical and Regulatory Considerations
Benjamin Stevens, PhD, Director, CMC Policy and Advocacy, GSK

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 *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

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 Sponsored Presentation *(Opportunity Available)*

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

PLENARY KEYNOTE SESSION

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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.

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

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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 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: 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 BACHEM

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

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

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.

4:30 PANEL DISCUSSION: How to Manage Safety, Regulatory, and Manufacturing Challenges of Oligonucleotides and Antibody-Oligonucleotide Conjugates (AOCs)

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

Oligonucleotide therapeutics (e.g., siRNA, ASOs) and Antibody-Oligonucleotide Conjugates (AOCs) are rapidly evolving platforms for targeted gene modulation. The panel of subject matter experts will:

- Identify key safety concerns specific to oligonucleotides and AOCs
- Discuss evolving regulatory expectations from FDA, EMA, and other global authorities
- Share best practices for overcoming CMC and analytical challenges in AOC manufacturing
- Highlight strategies for de-risking development through case studies and expert insights

5:00 Close of Conference

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 Harnessing Circular RNA for *in vivo* CAR T Therapy

Paloma Giangrande, PhD, Senior Vice President, Discovery and Translational Biology, Orbital Therapeutics

At Orbital Therapeutics, we develop innovative circular RNA (circRNA) therapies delivered by targeted lipid nanoparticles (LNPs) to enable *in vivo* CAR T cell generation. This approach leverages circRNA's high stability and durable expression, combined with precise LNP delivery, to safely reprogram immune cells inside the body. The technology aims to simplify CAR T treatment, providing accessible, scalable immunotherapy options for cancer and autoimmune diseases.

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, Senior Vice President, Discovery and Translational Biology, Orbital Therapeutics

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

Moderator: Paloma Giangrande, PhD, Senior Vice President, Discovery and Translational Biology, Orbital Therapeutics

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

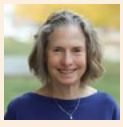
4:05 Chairperson's Remarks

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

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

mRNA & Emerging Oligonucleotide Modalities

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

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 xRNA Combination Therapy: A New Frontier for Cardio-Metabolic Disorders

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

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.

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.

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

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Debra Miller, Founder & CEO, CureDuchenne

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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 Leading the Circular RNA Revolution: Clinical Updates of RXRG001 and the Next Generation of *in vivo* CAR

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

This talk will highlight RiboX Therapeutics' proprietary plug-and-play circular RNA, ionizable lipids, and active targeting LNP platforms. It will discuss clinical updates on RXRG001, the first circular RNA therapeutic to enter clinical trials for the treatment of Radiation-Induced Xerostomia and the development of RXIM002, an *in vivo* generated circular RNA CAR delivered via actively targeted LNPs as an advanced therapeutic for autoimmune diseases.

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

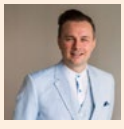
10:40 Chairperson's Remarks

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

Dushen Chetty, PhD, Vice President & Global Program Head, Prostate Cancer, Novartis Oncology

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 *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

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

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 Applying FORCE Platform for Targeted Oligonucleotide Delivery

Nicholas Yoder, PhD, Executive Director, Dyne Therapeutics

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

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

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.

Peptides & Emerging Drug Conjugates

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

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.

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 Nafiev, 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

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 3B010, a Novel GPC3-Targeting Peptide Radioligand with High Potency for Theranostic Applications in Treating Hepatocellular Carcinoma (HCC)

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

3B010, developed through our peptide discovery platform, exhibits excellent preclinical performance as a GPC3-targeting radioligand. Its high tumor retention and low kidney uptake in mouse models support its potential for clinical translation in both diagnostic imaging and radiotherapeutic applications. Human IIT studies demonstrate its high specificity and strong tumor retention in GPC3-overexpressing hepatocellular carcinoma (HCC).

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:
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- Discuss your research and collaborate with other attendees
- Your poster title and description will be published in our conference materials
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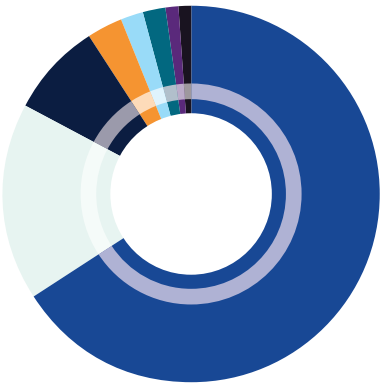

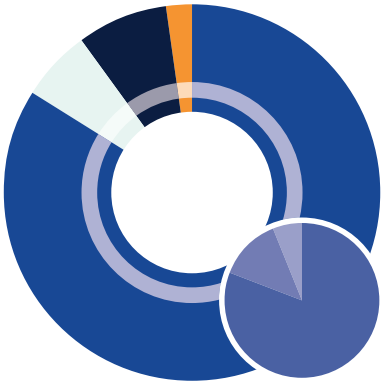

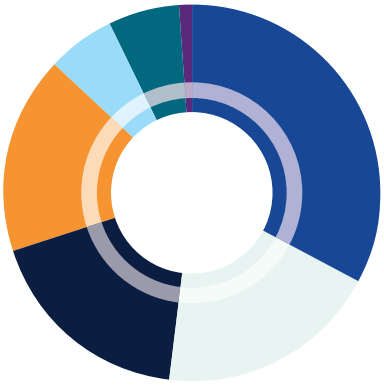

COMPANY TYPE

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%

2026 SPONSORS

CORPORATE SUPPORT SPONSORS

BACHEM

emp BIOTECH

oligo FACTORY

SynOligo BIOTECHNOLOGIES