Crystal Gateway Marriott

Sep 23, 2026 7:00 AM - Sep 25, 2026 3:00 PM

1700 Richmond Highway, Arlington, VA 22202, USA

Oligonucleotide-Based Therapeutics Conference

Convening industry and health authorities to inform, educate, and share advancements in oligonucleotide-based therapeutic product development.

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Early Bird Ends

Print Agenda

Day 1 Sep 23, 2026

8:15 AM — 4:45 PM

Registration

9:15 AM — 9:30 AM

Welcome and Opening Remarks

Welcome and Opening Remarks
Track: General Session

Session Chair(s)

Sorcha McCrohan, MS

Sr. Scientific Project Manager
DIA, United States

Sorcha McCrohan is a Senior Scientific Project Manager for Global Science at DIA. In her current role, she focuses on content development and strategy for DIA's meetings to improve and facilitate innovation in clinical research, drug development, and the fields of devices and diagnostics. Before joining DIA, she conducted COVID-19 research in Chiapas, Mexico, and worked in marketing within Pfizer's Global Vaccines franchise. Sorcha holds a BA in Sociology from Mount Holyoke College and an MSc in Global Health, Disease Prevention & Control from Georgetown University.

9:30 AM — 10:30 AM

Session 1: Keynote Address

Session 1: Keynote Address
Track: General Session

Session Chair(s)

Representative Invited

United States

10:30 AM — 11:00 AM

Refreshment and Networking Break

11:00 AM — 12:30 PM

Session 2 Track 1: The Brains Behind Delivery of Oligos to the CNS

The session will highlight the successes and challenges of delivering oligonucleotides to the brain, particularly overcoming the blood–brain barrier while achieving sufficient distribution and durability of effect in patients. Solution will focus on optimizing intrathecal dosing strategies, improving oligonucleotide chemistry for stability and potency, and exploring novel delivery platforms to enhance brain uptake. The discussion will emphasize balancing efficacy, safety, and patient burden to enable broader clinical adoption.
Learning Objective : At the conclusion of this session, participants should be able to:

- Compare various methods of delivery of oligos to the brain
- Understand the targets of new oligos for CNS diseases
- Evaluate the challenges and risks of clinical testing of oligos for brain diseases

Track: Track 1 Clinical

Session Chair(s)

Barry Ticho, MD, PHD

Chief Medical Officer
Stoke Therapeutics, United States

As Chief Medical Officer Dr. Ticho is responsible for Stoke's efforts to develop first-in-class RNA based disease-modifying medicines to treat severe genetic diseases. He is also co-founder and former CEO of Verve Therapeutics which is developing therapies to edit the genome and confer protection from cardiovascular disease. Prior to joining Stoke Barry was Head of R&D for Cardiovascular and Metabolic Diseases at Moderna Therapeutics. He was previously Head of External R&D Innovation for Cardiovascular and Metabolic Diseases at Pfizer and was Vice President of Clinical Development at Biogen. Barry obtained his MD and PhD degrees from the University of Chicago. He was on staff at Harvard Medical School and Massachusetts General Hospital

Speaker(s)

Speaker

Representative Invited

Alnylam Pharmaceuticals, United States

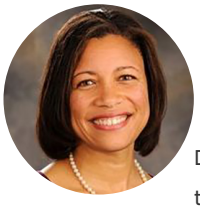
Toby is a neuromuscular neurologist and neuroscientist who joined Biogen in 2013. His professional experience has been focused on developing treatments for neuromuscular disease, with a focus on ALS. He is currently the Head of the Neuromuscular Development Unit at Biogen and leads the neuromuscular clinical development group. Toby plays a key role in developing ALS clinical trials, and in driving preclinical strategy within neurodegenerative and neuromuscular disease. Prior to Biogen, Toby had a clinical neuromuscular neurology practice and trained in neurology and neuromuscular disease at the University of Pennsylvania. He obtained an MD and PhD (Neuroscience) at the University of Florida.

11:00 AM — 12:30 PM

Session 2 Track 2: Extrahepatic Delivery 1: Engineering LNPs and Ligand Conjugates for Oligonucleotide Delivery to the Kidney and Lung Tumors

Session 2 Track 2: Extrahepatic Delivery 1: Engineering LNPs and Ligand Conjugates for Oligonucleotide Delivery to the Kidney and Lung Tumors
Track: Track 2 Nonclinical

Session Chair(s)



Elena Braithwaite, PHD

Toxicologist
FDA, United States

Dr. Elena Braithwaite is a toxicologist at the US Food and Drug Administration and a Diplomate of the American Board of Toxicology. She has a broad background in various aspects of basic research including DNA repair, mutagenesis and signal transduction.

Jeffrey Foy, PHD

Executive Director, Toxicology
Nimbus Therapeutics, United States

11:00 AM — 12:30 PM

Session 2 Track 3: Regulatory Considerations for Innovative and Sustainable Oligonucleotide Manufacturing Technology

The growing importance of oligonucleotide therapies in the therapeutic armamentarium requires a progressive approach to establishing robust global supply chains and streamlined regulatory pathways for commercialization and lifecycle management. Digitalization and sustainability are two areas of significant focus from the perspectives of innovators and regulators alike. In addition, new classes of oligonucleotide therapeutics continue to emerge alongside novel process chemistry and analytical technologies; oligonucleotide “longmers” are one such example. This session will address new developments in the rapidly evolving field of oligonucleotide development and manufacturing, with particular emphasis on where these technologies dovetail with emerging regulatory frameworks. Speakers from industry will present two unique cases in which such technologies are beginning to see broader application, along with the associated regulatory considerations. A joint panel of industry members and regulators will discuss the implications and the path forward to support the needs of patients.

Learning Objective : At the conclusion of this session, participants should be able to:

- Describe some of the emerging technologies for oligonucleotide CMC development and commercialization
- Explain what some of the potential regulatory hurdles may be for some of these innovative technologies
- Outline some potential opportunities for alignment between industry and regulators to facilitate successful implementation of these technologies

Track: Track 3 CMC

Session Chair(s)

Benjamin Stevens, PHD, MPH

Director CMC Policy and Advocacy
GlaxoSmithKline, United States

Ben Stevens is a Director of CMC Policy and Advocacy at GlaxoSmithKline and has nearly 15 years of drug discovery and regulatory experience. Prior to GSK, Ben was a Director of Regulatory Affairs CMC at Alnylam, a Principal Consultant at PAREXEL and an acting Branch Chief in the Office of New Drug Products (ONDP) at the FDA. Before FDA, Ben spent seven years in pharmaceutical R&D at Pfizer and Merck. Ben received a Ph. D. in Chemistry from the University of Pittsburgh, a M.P.H. from the Johns Hopkins and is a co-author of over 20 publications and patents.

Katherine Windsor, PHD

Senior Pharmaceutical Quality Assessor, CDER
Food and Drug Administration, United States

Dr. Katherine Windsor is a Senior Pharmaceutical Quality Assessor (Drug Substance Lead) in the Office of Pharmaceutical Quality within the Center for Drug Evaluation and Research (CDER) at FDA. Katherine has 10 years of experience assessing CMC aspects of drugs in several therapeutic areas, particularly anti-infectives and antivirals, and a wide variety of APIs, including oligonucleotides, peptides, antibody-drug conjugates, and small molecules. Katherine conducted postdoctoral research at Vanderbilt University and obtained her Ph.D. in Organic Chemistry from the University of Wisconsin-Madison and her B.S. in Chemistry from the University of Notre Dame.

12:30 PM — 1:30 PM

Networking Luncheon

1:30 PM — 3:00 PM

Session 3 Track 3: Advancing the Analytical Toolbox for mRNA-Based Medicines

As mRNA-based medicines expand into broader therapeutic and individualized applications, their unique analytical characterization requirements and challenges must be carefully considered. At the same time, emerging methodology and mRNA-specific regulatory guidance are enabling more sophisticated dialogue between developers and regulators regarding analytical control. In this session, perspectives on the current landscape of analytical capabilities for characterization of mRNA and lipid nanoparticles (LNPs) will be shared to motivate discussion on establishing appropriate and comprehensive control strategies.
Learning Objective : At the conclusion of this session, participants should be able to:

- Discuss critical quality attributes relevant to mRNA-based medicines and applicable regulatory guidance
- Compare emerging analytical technologies and approaches for characterization of mRNA and LNPs
- Apply key principles for developing modality- and application-appropriate analytical control strategies

Track: Track 3 CMC

Session Chair(s)

Brian Doyle

Senior Director, Technical Development
Moderna, United States

Speaker(s)

Sequencing-Based Strategies for Comprehensive Characterization of mRNA Therapeutics

Wayne Doyle, PHD, MBA

Head of Scientific Platforms and Strategy
Eclipsebio, United States

1:30 PM — 3:00 PM

Session 3 Tracks 1 and 2: Regulatory Considerations of Novel Conjugates: Translational and Clinical Pharmacology Perspectives

Session 3 Tracks 1 and 2: Regulatory Considerations of Novel Conjugates: Translational and Clinical Pharmacology Perspectives
Track: Track 1 Clinical

Session Chair(s)

Louis St. L. O'Dea, MD

Independent Consultant
United States

Patrik Andersson, PHD

Principal Scientist, Discovery Safety Specialist
AstraZeneca R&D, Sweden

I received my PhD in toxicology from Karolinska Institutet, Stockholm in 2003. Joined AstraZeneca R&D in Gothenburg in 2004 as a toxicologist supporting Cardiovascular and Metabolic drug projects in the Discovery phase. Since 2012 focusing on nucleotide drugs, including oligonucleotides and mRNA therapeutics. Currently leading the preclinical safety activities for oligonucleotides and targeted drug delivery in AstraZeneca as well as different mRNA applications.

3:00 PM — 3:30 PM

Refreshment and Networking Break

3:30 PM — 5:00 PM

Session 4 Track 1: Translational Safety of Genetically Targeted Oligonucleotides: Managing Mechanistic Risks, Immunogenicity, and AOC Platforms

This session will explore the evolving landscape of translational safety for genetically targeted oligonucleotide therapeutics, with a focus on understanding, assessing, and mitigating class-specific and emerging risks. We will begin by framing the key mechanistic safety considerations associated with oligonucleotides, including class-related adverse events such as liver function test (LFT) elevations, thrombocytopenia, and proteinuria. Building on this foundation, speakers will examine approaches to evaluating and de-risking immunogenicity and QTc liability, drawing on clinical and long-term data from GalNAc-siRNAs as a well-characterized platform. The session will then address safety challenges associated with newer modalities, including linker-containing antibody–oligonucleotide conjugates (AOCs), and discuss how lessons learned from established platforms can inform risk assessment for emerging chemistries, including CNS-targeted approaches. Overall, this session aims to foster shared learning and advance strategies to proactively manage safety across the next generation of oligonucleotide therapeutics.
Learning Objective : At the conclusion of this session, participants should be able to:

- Compare strategies to de-risk class-specific safety profiles
- Identify lessons learned from well-characterized GalNAc-siRNAs
- Apply how these insights can be applied to AOCs

Track: Track 1 Clinical

Session Chair(s)

Sydney Stern, PHD, MS

Associate Director
BeOne Medicines, United States

Dr. Sydney Stern is a clinical pharmacology reviewer in the Division of Translational and Precision Medicine (DTPM) in the Office of Clinical Pharmacology (OCP) at the FDA. She is a primary reviewer for oligonucleotide programs and rare diseases in OCP and she has extensive experience with in vitro/in vivo extrapolation. Dr. Stern has led several data projects in the rare disease space and research projects investigating strategies for selecting safe starting doses in oligonucleotide-based therapeutic. Her research interests are focused on the pharmacology of synthetic oligonucleotides and rare diseases. She received her Master of Science in Clinical Research and a Ph.D. in Pharmaceutical Sciences at University of Maryland Baltimore.

Speaker(s)

Comprehensive Immunogenicity Assessment of GalNAc-siRNAs: Clinical and Long-Term Data Insights

Chaejin Kim, PHARMD, PHD, MPH

Sr Scientist
Alnylam Pharmaceuticals, United States

3:30 PM — 5:00 PM

Session 4 Track 2: Optimizing Oligonucleotide Therapeutics: Translating PK/PD and Tissue Half-Life into Smarter Dose Selection and Risk Assessment

Session 4 Track 2: Optimizing Oligonucleotide Therapeutics: Translating PK/PD and Tissue Half-Life into Smarter Dose Selection and Risk Assessment
Track: Track 2 Nonclinical

Session Chair(s)

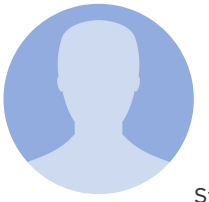
Tod Harper, PHD

Scientific Associate Director
Amgen, United States

Representative Invited

FDA, United States

Speaker(s)



Translational PK/PD Modeling Considerations

Representative Invited

GlaxoSmithKline, United Kingdom

Steve Hood received a PhD in Molecular Toxicology from the University of Surrey in 1993 and joined Glaxo Group Research as an Industrial Post doc. Steve is now a senior Scientific Director in Bioimaging, responsible for external imaging collaborations in the Bioimaging Expertise Network (BEN). As part of this network, Steve is also Co-Director of the GSK Centre for Molecular Imaging (COMI) at the University of Illinois at Urbana Champaign, where he works closely with Professor Stephen Boppart and his team. Steve has spent most of the last 2 decades working on GSK's diverse oligo portfolio and has supported projects ranging from inhaled SiRNAs, TLR antagonists, DMD exon skippers (Prosensa) and ASOs for TTR and HBV with Ionis.

Role of Tissue Half Life in Designing Clinical Studies

Representative Invited

Stoke Therapeutics, United States

Meena is the Senior Vice President of Translational DMPK and Clinical Pharmacology at Stoke Therapeutics. Prior to joining Stoke in 2018, Meena served as senior director of bioanalytical, pharmacology and biomarker development at Wave Life Sciences. She played a pivotal role in building Wave's stereopure oligonucleotide chemistry platform and in guiding the clinical entry of three antisense oligonucleotide programs. Earlier in her career, Meena worked at Alnylam Pharmaceuticals on siRNA chemistry and targeted siRNA delivery. Meena received her Ph.D. in chemistry with Dr. K.N. Ganesh at the National Chemical Laboratory in Pune, India, and did her postdoctoral research on nucleic acid analogs with Professor Larry W. McLaughlin at Boston Colle

3:30 PM — 5:00 PM

Session 4 Track 3: Advance Enzymatic Oligonucleotide Manufacturing: From Scalable Chemistry to Regulatory CMC Readiness

As therapeutic oligonucleotide modalities expand in complexity and scale, manufacturers are exploring enzymatic approaches to address limitations of conventional solid phase synthesis. This session provides a forward-looking discussion of emerging enzymatic manufacturing concepts, with a focus on how these technologies may impact impurity profiles, scalability, and overall product quality. Speakers will examine key scientific and CMC considerations relevant to enzymatic oligonucleotide production and discuss how potential benefits and risks could be evaluated within established

regulatory frameworks. The session aims to foster dialogue between industry and regulators on expectations, open questions, and areas where further alignment or data generation may be needed.
Learning Objective : At the conclusion of this session, participants should be able to:

- Evaluate, advantages and limitations of large scale enzymatic oligo synthesis vs. conventional solid phase synthesis
- Assess, readiness for regulatory acceptance of alternative synthesis routes
- Determine when transitioning synthesis technologies can improve long term scalability and manufacturing flexibility.

Track: Track 3 CMC

Session Chair(s)

Dominik Altevogt, PHD

Director Regulatory Affairs CMC
Novartis, Switzerland

Dominik Altevogt is an experienced professional in the pharmaceutical industry, with over 15 years of experience leading regulatory submissions and health authority interactions for small molecule drugs, with a special focus on synthetic peptides and oligonucleotides. He started his career in CMC regulatory affairs at Bachem AG and has since worked for F. Hoffmann-La Roche AG and Novartis AG. Dominik holds a Ph.D. in organic chemistry from the University of Freiburg, Germany, and is an active member of the European Pharma Oligonucleotide Consortium (EPOC), where he currently leads the platform strategies subteam.

5:00 PM — 6:00 PM

Networking and Poster Reception

Day 2 Sep 24, 2026

8:00 AM — 8:30 AM

Networking Breakfast

8:00 AM — 4:00 PM

Registration

8:30 AM — 9:30 AM

Welcome to Day Two and Session 5: Fireside Chat: Implementing the Plausible Mechanism Pathway: From Concept to Clinic

Welcome to Day Two and Session 5: Fireside Chat: Implementing the Plausible Mechanism Pathway: From Concept to Clinic

Track: General Session

Session Chair(s)

Hobart Rogers, PHARMD, PHD

Pharmacologist, CDER
FDA, United States

Dr. Bart Rogers is a reviewer in the Division of Translational and Precision Medicine in the Office of Clinical Pharmacology (OCP) at the FDA. Dr. Rogers also serves as an active duty officer with the United States Public Health Service. He serves as the lead for OCPs review of all synthetic oligonucleotides. His research interests are focused on the pharmacology of synthetic oligonucleotides, orphan disease drug development, and pharmacogenomics. Dr. Rogers completed his Pharm.D. degree from the University of Maryland, School of Pharmacy in 2004. He went on to obtain his Ph.D. in Clinical Pharmaceutical Sciences with a focus on cardiovascular pharmacogenomics from the same institution.

Dan Swerdlow, MD, PHD

Senior Director, Early Clinical Development
GSK, United Kingdom

Dan trained on the MD PhD programme at UCL, completing a PhD in genetic epidemiology. Thereafter he worked in London as a clinical academic in internal medicine and clinical pharmacology. In his academic research he led international human genetics consortia for drug target discovery in cardiometabolic disease, with findings published in the Lancet and Nature Communications. Before joining GSK in 2022, Dan led oligonucleotide clinical development programmes and a computational genomics group at Silence Therapeutics, prior to which he worked in AI-enabled translational and precision medicine at BenevolentAI. At GSK, Dan is a clinical lead on translational and early clinical development projects. He is an honorary associate professor at UCL.

9:30 AM — 10:00 AM

Refreshment and Networking Break

10:00 AM — 11:30 AM

Session 6 Track 1: Different Paths, Common Goals: Global Regulatory Perspectives on FIH Trials for Oligonucleotide Therapies

Applications for first-in-human (FiH)trials for oligonucleotide therapies present unique challenges, as sponsors must navigate evolving scientific considerations alongside differing regulatory expectations across regions. This session brings together senior leaders from global health agencies to share expectations on clinical trial and investigational new drug applications, assess risk, approach safety monitoring, guide engagement, and weigh platform understandings and prior knowledge in FIH applications. Through candid agency perspectives and cross-regional dialogue, attendees will gain practical insight into common application pitfalls and regulatory expectations. Participants will leave better equipped to design and strengthen FiH trial applications that are robust, aligned, and globally informed.
Learning Objective :

- Compare global regulatory expectations for first-in-human oligonucleotide trials, including key application content, approaches to dose selection and escalation, and safety monitoring
- Identify common deficiencies in FIH oligonucleotide applications and apply best practices to strengthen submissions across regions
- Apply health agency perspectives on use of platform knowledge and early regulatory engagement to improve FIH trial design and amendments

Track: Track 1 Clinical

Session Chair(s)

Andrew Slugg, MBA, MS

Senior Vice President, Global Head of Regulatory Sciences
Alnylam Pharmaceuticals, United States

Andrew began his career in industry over 20 years ago and has spent the last 17 years in Regulatory Affairs. He's had the fortune of being a part of many great teams who have brought seven novel therapies to market for a variety of conditions. This includes the first four RNAi therapeutics. Andrew holds degrees from Bates College, Massachusetts College of Pharmacy and Health Sciences, and Babson College.

10:00 AM — 11:30 AM

Session 6 Track 2: CNS

Session 6 Track 2: CNS
Track: Track 2 Nonclinical

Session Chair(s)

Aimee L. Jackson, PHD

Miragen Therapeutics, United States

Aimee has been working in the field of RNA interference and microRNAs for >15 years and has authored/co-authored >20 publications. She received her PhD from Univ of Colorado Health Science Cntr and performed post-doc research at Univ of Washington. Aimee joined Rosetta Inpharmatics/Merck, where she established the use of RNAi combined with expression profiling technologies for target identification, target validation, elucidation of drug mechanism-of-action, and patient stratification. She investigated the therapeutic application of siRNAs. Aimee leads the discovery/development of new therapeutic targets in diverse disease indications, pioneering the implementation of translational biomarkers for mechanistic proof-of-concept in patients.

10:00 AM — 11:30 AM

Session 6 Track 3: Navigating Change: Unlocking Opportunities and Overcoming Challenges in Recent US CMS Policy Developments

This session will provide an in-depth overview of the latest US FDA programs and policies including Pre-check, Platform designation, and CMC Development and Readiness Pilot Program (CDRP) which were designed to accelerate drug product manufacturing and may support emerging frameworks such as the Plausible Mechanism Pathway . Leading industry and FDA experts will also explore practical strategies for leveraging these regulatory flexibilities, address common hurdles, and clarify outstanding questions faced by oligonucleotide manufacturers. Real-world examples and actionable insights will equip participants to implement best practices in navigating this evolving regulatory landscape.
Learning Objective : At the conclusion of this session, participants should be able to:

- Describe the latest US FDA programs – Pre-check, Platform Designation, CDRP, CMC aspects of the Plausible Mechanism Pathway
- Understand industry challenges/opportunities with implementing these programs with specific considerations for oligos
- Determine next steps in formalizing these programs and best practices
- Focus on US (e.g., Platform Designation, CDRP, PreCheck)

Track: Track 3 CMC

Session Chair(s)

Rumi Raquel Young, MS

Director, Regulatory Policy
Novo Nordisk A/S, United States

Rumi Young is the Director of Regulatory Policy at Novo Nordisk, ensuring future growth and innovation by promoting effective biopharmaceutical policies. Previously, she led BD's Global Regulatory Policy team to shape future policies for medical devices, diagnostics, and combination products. As Assistant Director – Injection Devices at FDA, she managed reviewers and set technical and regulatory expectations for combination products and drug delivery devices. Before FDA, Rumi worked in R&D for a number of years at Genentech and AstraZeneca. She holds a Bachelor's in Chemistry and Chemical Biology and a Masters in Material Science Engineering from Cornell University.

Speaker(s)

Overview of Recent US FDA CMC Programs/Policies & Next Steps

Representative Invited

FDA, United States

Acting Director for the Division of Inspection Assessment within OPQ/OPF. His division is primarily responsible for the center level discipline review of facilities and inspections in support of drug applications. He joined the Agency in November 2009 after graduating with his PharmD from the University of Maryland and his MBA from the University of Baltimore. Prior to FDA, he had experience in the generic pharmaceuticals industry. He continues to practice pharmacy in the community setting.

Industry Insights on Recent US CMC Policy Changes: Navigating Opportunities and Challenges for Oligonucleotide Therapeutics

Representative Invited

GSK, United States

Katie Duncan is a Director of CMC Policy and Advocacy with Global Regulatory Affairs/CMC Excellence. Prior to joining GSK, Katie was a senior pharmaceutical quality assessor with the Office of Pharmaceutical Quality at the US Food and Drug Administration. She previously worked in small molecule drug discovery at a biotechnology company in San Diego, CA. She received her Ph.D. in organic chemistry from the Scripps Research Institute in La Jolla, CA and B.A. from Amherst College in Amherst, Massachusetts.

11:30 AM — 12:45 PM

Networking Luncheon with Roundtable Discussions

12:45 PM — 2:15 PM

Session 7 Track 3: Oligonucleotide Delivery: New Developments and Regulatory Strategies

This session will cover the regulatory strategies associated with any modifications made to oligonucleotides to overcome and/or supplement delivery. Conjugation of oligonucleotides with targeting entities has been explored to improve their pharmacokinetic and pharmacodynamic properties. Bridging considerations: Ease of use, improved adherence, reduced health care resource use are some of the advantages of drug-device combination products such as autoinjectors.

However, the introduction of any combination product late in the development brings regulatory considerations for bridging the early phase presentations with the commercial presentations.
Learning Objective :

- Describe some of the regulatory strategies for the control of oligonucleotide-linkers for the antibody-oligonucleotide conjugates
- Explain the potential regulatory hurdles for the bridging of the drug device combination products along with the science and risk-based solutions
- Outline some of the potential strategies and opportunities for alignment between industry and regulators to facilitate patient centric oligonucleotide drug development

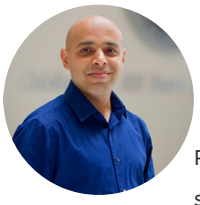
Track: Track 3 CMC

Session Chair(s)

Ramin Darvari, PHD, MS

Research Fellow
Pfizer Inc., United States

Ramin Darvari is a Research Fellow in Drug Product Design & Development group at Pfizer; contributing to the strategic and tactical planning for evaluation of external delivery technologies and internal delivery formulation & process development, with a focus on collaborative partner engagement. Ramin has lent his expertise in particle engineering and matrix-based drug delivery systems to evaluation and development of variety of applications, including his role as the drug product project lead for Pfizer-BioNTech Covid-19 Vaccine.



Rohit Tiwari, PHD

Director, Global Regulatory Affairs-CMC
Eli Lilly & Company, United States

Rohit is a Director at Eli Lilly & Company and is responsible for developing CMC regulatory strategies for oligonucleotides and oligonucleotide conjugates. Previously, he was a senior CMC reviewer at FDA for 5 years where he reviewed small molecules, oligonucleotides and ADCs. Rohit received his Ph.D. in Medicinal Chemistry from The Ohio State University working on the design and syntheses of nucleoside analogues. This was followed by a post-doctoral work at University of Notre Dame and ORISE research fellowship at FDA where he learned about oligonucleotide chemistry.

12:45 PM — 2:15 PM

Session 7 Tracks 1 and 2: E14/S&B

Session 7 Tracks 1 and 2: E14/S&B
Track: Track 1 Clinical

Session Chair(s)

Representative Invited

Eli Lilly, United States

2:15 PM — 2:45 PM

Refreshment and Networking Break

2:45 PM — 4:15 PM

Session 8: Hot Topics

The Hot Topics session will provide an overview of three areas that will provide clarity and likely influence the way of working with oligo projects. 1. An overview of the draft ICH S13 draft guideline on nonclinical safety of oligonucleotides that shortly after the meeting will be published for public consultation; 2. How will the regulatory roadmaps for New Approach Methodologies (NAMs) influence oligo R&D and 3. How can use of AI be applied to oligo therapeutics to increase throughput and success rate of oligo projects.
Learning Objective : At the conclusion of this session, participants should be able to:

- Mention key themes in the draft ICH S13 guideline on nonclinical safety for oligos
- Discuss specific situations where a NAMs approach could be applied to an oligo project
- Suggest potential areas in the oligo project Drug Discovery and Development value chain where application AI could add significant value

Track: General Session

Session Chair(s)

Patrik Andersson, PHD

Principal Scientist, Discovery Safety Specialist
AstraZeneca R&D, Sweden

I received my PhD in toxicology from Karolinska Institutet, Stockholm in 2003. Joined AstraZeneca R&D in Gothenburg in 2004 as a toxicologist supporting Cardiovascular and Metabolic drug projects in the Discovery phase. Since 2012 focusing on nucleotide drugs, including oligonucleotides and mRNA therapeutics. Currently leading the preclinical safety activities for oligonucleotides and targeted drug delivery in AstraZeneca as well as different mRNA applications.

Speaker(s)

ICH S13

Representative Invited

BfArM, Germany

• Head of unit Parallel Import/Distribution and Standard MA, German member SWP (EMA) at BfArM Since October 2011 • 2005–2011: Head of project management unit, licensing departm. Intense work in national/European regulatory affairs, all kinds of applications at BfArM. • Biology dipl., Univ. Bonn; PhD, Univ. Heidelberg

NAMs

Representative Invited

FDA, United States

Nakissa joined the FDA in 1996 as a pharmacology and toxicology reviewer. In 2002 she joined CDER OPQ as the Associate Director for Research Policy and Implementation. During that role, Nakissa worked on numerous projects focusing on research and policy development in drug quality, drug safety, bioequivalence of generic drugs, biopharmaceutics, nanotechnology, computational toxicology and environmental assessment. In 2021, she returned to CDER OND as the Senior Advisor for New Approach Methodologies, where she currently focuses on developing a path towards the regulatory acceptance of complex in vitro methods to support drug safety and meeting the 3Rs.

4:15 PM — 5:15 PM

DIA Oligonucleotide Safety Working Group (OSWG) – Open Meeting

DIA Oligonucleotide Safety Working Group (OSWG) – Open Meeting

Track: General Session

Session Chair(s)

Representative Invited

United States

Day 3 Sep 25, 2026

7:45 AM — 8:15 AM

Networking Breakfast

7:45 AM — 12:45 PM

Registration

8:15 AM — 9:30 AM

Session 9 Track 1: Targeting Muscle Diseases with Oligonucleotides

There is tremendous interest in developing effective and safe RNA-based therapeutics for the treatment of inherited muscle diseases. At least four RNA oligonucleotide drugs have received FDA accelerated approval; however, clinical benefit has yet to be determined. This session will navigate current and future landscapes of oligonucleotide-based therapeutic developments. The focus will be on defining hope, identifying challenges, and approaches to turn challenges into opportunities in treating disabling rare genetic muscle diseases. These diseases have presented with unprecedented pathogenesis and associated challenges for drug development. For example, exon skipping in Duchenne muscular dystrophy, nuclear RNA-protein aggregation in myotonic dystrophy, and inefficient repeat-mediated epigenetic repression of the DUX4 retrogene embedded in the D4Z4 repeat units in facioscapulohumeral dystrophy. Further, muscle as a target organ is tough to get the therapeutic RNA to go to when administered in bloodstream or subcutaneously. There are also challenges in the assessment of treatment response in these rare diseases necessitating development of biomarkers and endpoints, innovative clinical trial designs, and application in real world clinical practice.
Learning Objective : At the conclusion of this session, participants should be able to:

- Define muscle disease pathogenesis amenable to oligonucleotide therapy
- Recognize challenges and opportunities for developing safe and effective oligonucleotide drugs targeting muscle diseases
- Describe developments in assessment of treatment response in rare muscle diseases in clinical trials and clinical practice

Track: Track 1 Clinical

Session Chair(s)

Representative Invited

FDA, United States

8:15 AM — 9:30 AM

Session 9 Track 2: From Data to Decisions: Evolving the Regulatory Landscape for Oligonucleotide Therapeutics

Presentations in this session will review general toxicology and carcinogenicity data gathered and analyzed by regulatory agencies and industry for oligonucleotide therapeutics with well-characterized mechanisms and compositions. The aim is to inform and enhance regulatory decision-making by applying risk-based approaches that build on existing knowledge and determine best practices for evaluating new chemistries.
Learning Objective : At the conclusion of this session, participants should be able to:

- Understand the principles and examples of applying knowledge from “well characterized ONTs”, and discuss any concerns or challenges of this approach
- Identify data gaps for “well characterized ONTs” for certain endpoints
- Suggest innovative approaches to address the data gaps or challenges discussed above

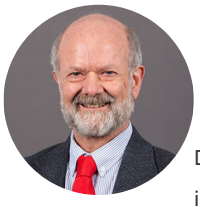
Track: Track 2 Nonclinical

Session Chair(s)

Xuan Chi, PHD

Supervisory Pharmacologist
CDER, FDA, United States

Dr. Chi is a Supervisory Pharmacologist in the Office of Cardiology, Hematology, Endocrinology and Nephrology, CDER FDA. Prior to that, she was a senior consultant in the Federal Healthcare Practice at Deloitte Consulting LLP and her projects were focused on post-marketing drug safety, data warehouse and review modernization effort. She had also worked as a staff fellow at Office of Blood Review and Research, CBER FDA. She is specialized in analyzing data from toxicological, pharmacological and clinical studies of original NDAs, BLAs, INDs, and 510(k)s. She had Ph.D. in molecular genetics from Baylor College of Medicine and had postdoctoral training in molecular genetics and pathology at Columbia University Medical Center.



Ronald Wange, PHD

Principal Consultant
Aclairo Pharmaceutical Development Group, Inc., United States

Dr. Wange is an Associate Director for Pharmacology & Toxicology within the Office of New Drugs in CDER at the FDA, and has over 19 years of experience reviewing small molecule drugs, biotherapeutic proteins and oligonucleotide-based therapeutics. He is a founding member of OND’s Pharmacology/Toxicology Oligonucleotide Subcommittee, which considers issues specifically related to the safety review of oligonucleotide-based therapeutics. In addition, he was the primary author of the recently published draft guidance on Nonclinical Testing of Individualized ASOs for Severely Debilitating or Life-Threatening Diseases. Prior to joining FDA, he was the head of the T-lymphocyte Signaling Unit at the National Institute on Aging at the NIH.

Speaker(s)

Comparative Analysis of Chronic vs. Sub-chronic Toxicity Study Findings with Well-characterized ONTs

Representative Invited

Dutch Medicines Evaluation Board, Netherlands

Carcinogenicity Assessment Strategies for ONTs: OSWG Perspectives and Key Considerations

Representative Invited

Ionis Pharmaceuticals, Inc., United States

Dr. Henry received a PhD in Biochemistry form North Dakota State University. He was a post-doc fellow at Parke Davis, Ann Arbor MI, depart. of toxicology. He joined Isis Pharmaceuticals, Inc. as a Sr Scientist in toxicology. He helped characterized and studied mechanisms of various toxicities e.g. the effects of oligonucleotide treatment on clotting time prolongation, alternative complement pathway activation, proinflammatory effects in rodents, platelet alterations and the effects related to the accumulation of oligonucleotide in kidney. As VP of Non-Clinical Development he has participated in the development of ~8 different phosphorothioate oligodeoxynucleotides and 30+ different 2'-MOE modified phosphorothioate oligonucleotides.

8:15 AM — 9:30 AM

Session 9 Track 3: Evolving Regulatory Landscape for Oligonucleotide Control Strategy and Comparability

Session 9 Track 3: Evolving Regulatory Landscape for Oligonucleotide Control Strategy and Comparability

Track: Track 3 CMC

Session Chair(s)

Claus Rentel, PHD

Vice President, Analytical Development and Quality Control
Ionis Pharmaceuticals, Inc., United States

Dr. Rentel is currently Vice President, Analytical Development and Quality Control at Ionis Pharmaceuticals, Inc., Carlsbad, California. Prior to joining Ionis in 2001 he worked in Quality Control and Special Analytics at CarboGen in Switzerland. He received his Ph.D. (summa cum laude) from the University of Tuebingen, Germany. Dr. Rentel has 20 years of experience in Quality Control. He has extensive expertise in the CMC development of oligonucleotide therapeutics and is an expert in mass spectrometric techniques. He has been responsible for IND filings of more than 60 oligonucleotides and participated in the NDA filings for KYNAMRO® (mipomersen), SPINRAZATM (nusinersen), WAYLIVRA® (volanesorsen), and TEGSEDITM (inotersen).

9:30 AM — 10:00 AM

Refreshment and Networking Break

10:00 AM — 11:15 AM

Session 10 Track 2: Extra-Hepatic Delivery

Session 10 Track 2: Extra-Hepatic Delivery
Track: Track 2 Nonclinical

Session Chair(s)

Tae-Won Kim

Executive Director, Toxicology
Ionis Pharmaceuticals, Inc., United States

James Wild, PHD, MS

Pharmacologist, CDER
FDA, United States

James Wild received a MS and PhD in Pharmacology and Toxicology at the University of California, Davis. Areas of study included idiopathic pulmonary fibrosis and characterization of a novel, ryanodine-sensitive receptor in the lung. Subsequently he completed two postdoctoral fellowships specializing in asthma research. In later career positions, James conducted discovery pulmonary disease research at EpiGenesis Pharmaceuticals, Schering-Plough Research Institute, and Johnson and Johnson PRDUS. Currently, James is a Senior Pharmacologist at the FDA supporting the Division of Anti-Infectives. Areas of interest include anti-infective drugs, oligonucleotide therapies, pulmonary research, and drug regulation.

10:00 AM — 11:15 AM

Session 10 Track 3: Real-World Case Studies in Oligonucleotide CMC Development and Commercialization

Session 10 Track 3: Real-World Case Studies in Oligonucleotide CMC Development and Commercialization
Track: Track 3 CMC

Session Chair(s)

Firoz Antia, PHD

Vice President, Oligonucleotide and Small Molecule CMC
Denali Therapeutics, United States

A PhD Chemical Engineer by training, Dr. Antia has spent over 30 years in the pharmaceutical industry carrying out process development with roles at Sandoz, J&J, Merck and Palatin Technologies, before joining Biogen in 2012, where he is now Head of Oligonucleotide Development

11:20 AM — 12:35 PM

Session 11: Closing Plenary

Session 11: Closing Plenary
Track: General Session

Session Chair(s)

René Thürmer, PHD

Quality Assessor
Federal Institute for Drugs and Medical Devices, Germany

Dr. René Thürmer received his diploma in chemistry and his Ph.D. in biochemistry from the University of Tübingen. He joined the BfArM (Federal Institute for Drugs and Medical Devices, Bonn, Germany) in 2000. He currently serves as a CMC reviewer and is Deputy Head of the Unit Pharmaceutical Biotechnology. His experience is in the field of formulation, manufacture and control of medicinal products, in particular in the field of peptides, proteins, liposomes, sustained release polymer drug products, depot formulations, polymer-conjugated drug products, natural and synthetic surfactants, nanomedicine and others. His special focus lies on oligonucleotide preparations.

12:45 PM — 12:45 PM

Closing Remarks

Closing Remarks
Track: General Session

Session Chair(s)

Sorcha McCrohan, MS

Sr. Scientific Project Manager
DIA, United States

Sorcha McCrohan is a Senior Scientific Project Manager for Global Science at DIA. In her current role, she focuses on content development and strategy for DIA's meetings to improve and facilitate innovation in clinical research, drug development, and the fields of devices and diagnostics. Before joining DIA, she conducted COVID-19 research in Chiapas, Mexico, and worked in marketing within Pfizer's Global Vaccines franchise. Sorcha holds a BA in Sociology from Mount Holyoke College and an MSc in Global Health, Disease Prevention & Control from Georgetown University.

12:45 PM — 12:45 PM

Conference Adjourns