Capital Hilton

Oct 28, 2024 7:00 AM - Oct 30, 2024 7:00 PM

1001 16th Street NW, , Washington, DC 20036-5794 , USA

DIA/FDA Oligonucleotide-Based Therapeutics Conference

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Call for Posters and Roundtables Ends

58 DAYS 05 HOURS 29 MINUTES 00 SECONDS

Print Agenda

Day 1 Oct 28, 2024

8:30 AM — 4:45 PM

Registration

Speaker(s)

Sorcha McCrohan, MS, MSc

Specialist, Scientific Programs
DIA, United States

Sorcha McCrohan is a Specialist of Scientific Programs for the Americas Region 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 Meningococcal franchise. Sorcha holds a BA in Sociology from Mount Holyoke College and an MSc in Global Health, Disease Prevention & Control from Georgetown University.

Scott Henry, PhD

Vice President, Nonclinical Development
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.

Ronald Wange, PhD

Associate Director for Pharm/Tox, OND, CDER
FDA, 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 15 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 In addition, he was the primary author of the recently published draft guidance on Nonclinical Testing of Individualized ASOs for Severely Debilitating or Life-Threatening Diseases.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.

9:30 AM — 10:30 AM

Track: General Session

Session Chair(s)

Jeffrey Foy, PhD

Vice President, Toxicology
PepGen Inc., United States

Speaker(s)

Speaker

Representative Invited

FDA, United States

Dr. Peter Stein, M.D., is the Director of CDER's Office of New Drugs (OND). OND is responsible for the regulatory oversight of investigational studies during drug development and decisions regarding marketing approval for new (innovator or non-generic) drugs, including decisions related to changes to already marketed products. OND provides guidance to regulated industry on a wide variety of clinical, scientific, and regulatory matters.

10:30 AM — 11:00 AM

Refreshment and Networking Break

11:00 AM — 12:30 PM

Session 2 Track 1: Extra-Hepatic Delivery – Clinical Experience: Emerging

Track 1: Extra-Hepatic Delivery – Clinical Experience: Emerging

Xing Jing, PhD, MBA

Reviewer
FDA, United States

Dr. Xing Jing is an expert in oligonucleotide-based therapeutics. He possesses a broad range of experiences from biology to clinical trials. He was trained as a structural biologist in academia. After training, he worked in the field of R&D and bioinformatics in industry. Dr. Jing has been a clinical pharmacology reviewer at FDA since 2020. Initially, Dr. Jing was a reviewer in CDER at the FDA, where he reviewed oligonucleotide therapeutics and published a summary of clinical pharmacology for siRNA therapeutics. Currently, he is a reviewer of cell and gene therapies in CBER at the FDA. Dr. Jing's regulatory review experience covers a wide range of therapeutic areas including oncology, CNS, autoimmune, virology, etc.

Arthur A. Levin, PhD

Distinguished Scientist
Avidity Biosciences, United States

Speaker(s)

Pulmonary Delivery Platform and Clinical Programs

Representative Invited

Arrowhead Pharmaceuticals, United States

Dr. Hamilton currently serves as Vice President, Clinical Development at Arrowhead Pharmaceuticals and previously served as Medical Director at Arrowhead. In these roles, he has designed and managed multiple clinical studies with a wide range of siRNA compounds and has led clinical programs in various disease areas including hepatitis B, alpha-1 antitrypsin deficiency and dyslipidemia. He is the global medical lead on the AROAAT2001 (SEQUOIA) study evaluating the siRNA compound, ARO-AAT for the treatment of liver disease in the setting of alpha-1 antitrypsin deficiency. Dr. Hamilton holds an MD and an MBA from The Ohio State University and is board certified in Emergency Medicine.

Cardiac Delivery of Oligonucleotides

Thomas Thum

CSO
Cardior Pharmaceuticlas GmbH, Germany

Comanche Biopharma, United States

Delivery of Oligonucleotides to Muscle in Patients

Steve Hughes, MD, MBA

Chief Medical Officer
Avidity Biosciences, United States

Dr Hughes trained in general internal medicine and has been in the biotech/pharma sector for over 25 years. He has been involved in more than 60 clinical trials with more than 30 drugs across multiple therapeutic areas including multiple rare diseases, neurology, cardiac and oncology. Dr Hughes is currently Chief Medical Officer at Avidity BioSciences, an RNA focused company developing treatments for rare neuromuscular and cardac diseases. Prior to joining Avidity he has held senior drug development positions at several other RNA therapeutics companies.

11:00 AM — 12:30 PM

Session 2 Track 2: Pro-Arrhythmic Risk of Oligonucleotide Therapeutics: Is New Guidance Needed?

To date, the proarrhythmic assessment of oligonucleotide therapeutics has been influenced by existing International Conference on Harmonization (ICH) E14 and S7B guidance. However, oligonucleotide therapeutics exhibit distinctive physiochemical characteristics that may impact their proarrhythmic risk profile. Accumulating nonclinical and clinical data in this space may further inform future guidance development and regulatory decisions. This session will provide 1) a retrospective overview of the past practices used to conduct proarrhythmic assessment of oligonucleotide therapeutics in both nonclinical and clinical studies; 2) potential future recommendations to evaluate pro-arrhythmic risk of oligonucleotide therapeutics; and 3) FDAs perspective and experience with proarrhythmic risk assessment of oligonucleotide therapeutics.

Learning Objective :

- Recognize practices previously used to evaluate pro-arrhythmic risk both in nonclinical and clinical studies, and their correlation
- Debate the need for harmonized recommendations for evaluation of pro-arrhythmic risk of oligonucleotide therapeutics
- Discuss considerations for future recommendations to evaluate pro-arrhythmic risk of oligonucleotide therapeutics

Track: Track 2: Nonclinical

Session Chair(s)

Dr. Chi is a Pharmacology/Toxicology reviewer in the Division of Cardiovascular and Renal Products at the Office of New Drugs, CDER FDA. Prior to that, she was a clinical analyst and SME in the Federal Healthcare Practice at Deloitte Consulting LLP and her projects were focused on post-marketing drug safety and review modernization effort at OGD. 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 genetics and had postdoctoral training in molecular genetics and pathology.

Representative Invited

Amgen, United States

Speaker(s)

A Scientific Review of the Low Proarrhythmic Risk of Oligonucleotide Therapeutics

Hugo M Vargas, PhD

Executive Director
Amgen Inc., United States

I am an Executive Director at Amgen, and lead the Safety Pharmacology & Animal Research Center (SPARC) department. My team includes 60+ staff (Thousand Oaks, San Francisco, Burnaby BC) and we contribute actively and extensively to the entire drug discovery and development pipeline. My scientific responsibilities include preclinical safety pharmacology, profiling of preclinical candidates for treatment of cardiovascular disease, oversight for animal welfare, and strategic leadership responsibilities. I have a PhD (Pharmacology, Rutgers Grad Sch of Biomed Sci, 1983-88) and was a post-doctoral fellow (UCLA Sch of Med, 1988-91). I have held lead the Safety Pharmacology Society, and actively contribute to several drug safety consortia.

Future Recommendations: Update from ICH E14/S7B IWG

Representative Invited

Eli Lilly and Company, United States

11:00 AM — 12:30 PM

Session 2 Track 3: Commercial/Clinical Case Studies – Synthetic Oligos

Track: Track 3: CMC

Session Chair(s)

Firoz Antia, PhD

Head of Oligonucleotide Development
Biogen, 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

Speaker(s)

Oligonucleotide Solution API: Navigating the Regulatory Landscape

Chris Chorley

Associate Director, Regulatory Affairs CMC
Biogen, United States

Regulatory CMC Learnings and Perspectives from recent RNAi Filings

Representative Invited

Alnylam Pharmaceuticals, United States

Speaker

Representative Invited

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

Representative Invited

FDA, United States

12:30 PM — 1:30 PM

Networking Luncheon

1:30 PM — 3:00 PM

Session 3 Track 1: Extra Hepatic Delivery – Clinical Experience in CNS

Oligonucleotides are an emerging class of drugs with potential for the treatment of a wide range of central nervous system conditions. To date, there are two approved oligos for brain diseases and a large number of ongoing clinical trials. This session will review recent advances in chemical modifications and delivery techniques of oligonucleotides in clinical testing that are intended to enhance brain exposure and clinical efficacy.

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

Amy Kao

Medical Officer, Division of Neurology 2, OND, CDER
FDA, United States

Speaker(s)

C16 Conjugated to siRNA to Target Cells in the Central Nervous System

Representative Invited

Alnylam Pharmaceuticals, United States

Transferrin Receptor-Mediated Brain Delivery of Enzymes and Oligonucleotides Using Transport Vehicle Technology

Representative Invited

Denali Therapeutics, United States

Kirk serves as Head of Development Sciences at Denali Therapeutics. In this role he supports Pharmacokinetics, Quantitative Clinical Pharmacology, Safety Assessment, Multi-omics, and Bioanalytical teams to advance Denali's portfolio. Prior to Denali, Kirk was Director of DMPK and Project Team Lead at Assembly Biosciences, Principal Scientist and Group Leader at Amgen, and Principal Scientist at Pfizer. He has contributed to successful candidate identification, IND-enabling, and early clinical development activities for more than 20 molecules representing multiple modalities across therapeutic areas. Kirk has authored/co-authored >30 peer-reviewed publications. He obtained his Ph.D. in Medicinal Chemistry from the University of Washington.

1:30 PM — 3:00 PM

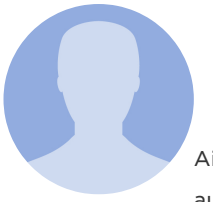
Session 3 Track 2: Translatability of CNS Safety and Pharmacology

This session will focus on current research efforts in the area of neurological diseases. The focus of the session will be

- Understand safety considerations for oligonucleotide therapeutics in the CNS

Track: Track 2: Nonclinical

Session Chair(s)



Representative Invited

Atalanta 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.

Representative Invited

FDA, United States

Lois Freed earned her undergraduate and Master's degrees from the University of Kansas and her Ph.D. from the University of Maryland. Prior to working at the FDA, Lois worked at the National Institute on Aging/NIH in the Laboratory of Neurosciences. Lois has been at the FDA since 1992, joining the Division of Neuropharmacological Drug Products as a nonclinical reviewer. She was the Supervisory Pharmacologist in this Division and then the Division of Neurology Products. Lois is currently the Director of the Division of Pharmacology/Toxicology in the Office of Neuroscience (OND/CDER).

Speaker(s)

Acute Transient Neurobehavioral Changes in NHPs

Hao Chen, PhD

Assistant Director/Toxicologist
Ionis Pharmaceuticals, Inc., United States

Rugonersen Pediatric Trial: How Did Nonclinical Studies (not) Translate?

Katharine is a pharmacologist and toxicologist with more than 30 years experience in nonclinical research and drug development. She has a proven track record in leading and empowering teams in large strategic initiatives e.g. immunogenicity. Her comprehensive experience spans various modalities (including large molecules, small molecules, and oligonucleotides), routes of administration, and disease areas including oncology. More recently she has been involved in the development of oligonucleotides in a number of neurological and rare diseases.

Safety and Biodistribution of both IT/ICV and Conjugated IV ASOs and siRNAs targeting CNS indications

Representative Invited

Retired, United States

Dr. Ken Frazier has a BS from WSU, DVM from KSU and PhD from the U of Miami. He did a residency in comparative pathology at UM Jackson Memorial Hosp and is board certified in pathology and toxicology, and is one of only a few veterinary nephrology experts in the world. He has over 100 scientific articles and book chapters and has extensive experience in the safety of antisense oligonucleotides as a faculty member at U of Georgia and 18 years as a sr. science fellow/safety liaison for GSK. Since retiring he has consulted with over 25 companies on ASO toxicology and lectures widely, including safety workshops for CDER, CBER & CFSAN. He has chaired many committees and meetings for STP, ESTP, ACVP and is incoming president of the IATP.

1:30 PM — 3:00 PM

Session 3 Track 3: Emerging Oligonucleotide CMC Guidance

This session will provide updates on emerging regulatory guidances from key global regulators such as FDA and EMA. Special emphasis will be placed on the forthcoming EMA guidance on synthetic oligonucleotides given the 2023 publication of the concept paper and feedback from industry. A panel discussion including both regulator and industry perspectives will follow to address audience questions and any future directions that will impact the global regulatory landscape for oligonucleotides.

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

- Identify key emerging global oligonucleotide guidance
- Apply concepts from the session to establishing regulatory strategies for meeting the requirements of global regulators for oligonucleotides

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.

Representative Invited

FDA, United States

Speaker(s)

Speaker

René Thürmer, PhD

Deputy Head of the Unit Pharmaceutical Biotechnology BfArM
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.

Speaker

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.

Speaker

Representative Invited

United States Pharmacopei, United States

Dr. Kevin Carrick is a Senior Director of Science & Standards in USP's Global Biologics Department. Dr. Carrick and his team work with the five USP Expert Committees and multiple Expert Panels in the area of biologics to develop standards that support biopharmaceutical quality assessment. These standards include documentary (monographs and general chapters) and physical reference standards for varied products from oligonucleotides to gene therapies.

Speaker

Representative Invited

Eli Lilly & Company, United States

3:00 PM — 3:30 PM

Refreshment and Networking Break

3:30 PM — 5:00 PM

Session 4 Track 1 and 2: Applying Toxicology Testing to the Clinic

Due to the unique pharmacokinetic properties of some oligonucleotide-based therapeutics, it can be challenging to compare the exposure achieved in animal pharmacology or toxicology studies to humans. Furthermore, understanding the relevant concentration of drug at the site of action (on and off target pharmacology and DDI) is key to the prediction of clinical outcomes. With a lack of clear guidance, several strategies have been employed to assess the relevance of nonclinical findings and predict clinical efficacy or safety. This session will share case examples of how programs have navigated from preclinical to clinical development including strategies used to calculate safety margins and DDI Liability with the goal of better predicting clinical outcomes.

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

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.

Andrew Slugg, MBA, MS

Senior Vice President, Global Head of Regulatory Affairs
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 great 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 three RNAi therapeutics. Andrew holds degrees from Bates College, Massachusetts College of Pharmacy and Health Sciences, and Babson College.

Speaker(s)

Refinement of PKPD Models and DDI Assessment 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.

Considerations for Determining Safety Margins for Oligonucleotides During Clinical Development

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

Speaker

Representative Invited

N-Lorem Foundation, United States

3:30 PM — 4:45 PM

Session 4 Track 3: Demonstrating Comparability for Oligonucleotides Therapeutics

Oligonucleotide therapeutics are larger and more complex than traditional small molecule drugs. Consequently, the task of demonstrating comparability of materials made by different manufacturing processes, may be more challenging for oligonucleotide therapeutics than for small molecule drugs. The session will include presentations from regulatory authorities, and industry scientists. The presentations will be followed by a panel discussion. Topics for discussion may include how to define comparability, the types of chemical and physicochemical characteristics that should be evaluated, analytical methods for assessing comparability, and the potential impact of observed differences in chemical and physicochemical properties.

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

- Define comparability requirements for oligonucleotide therapeutics
- Discuss how to evaluate the impact of manufacturing changes on oligonucleotide therapeutics
- Discuss how to demonstrate comparability between generic and brand name oligonucleotide therapeutics

Track: Track 3: CMC

Session Chair(s)

Daniel Capaldi, PhD

Vice President, Analytical and Process Development
Ionis Pharmaceuticals, Inc, United States

improvements and process optimization, analytical method development and validation, release and stability testing and impurity characterization

Fran Wincott, PhD

President
United States

Dr. Fran Wincott is President of Wincott & Associates, LLC, a consulting firm focused on providing assistance in the area of oligonucleotide manufacturing and development. Prior to founding Wincott & Associates, Dr. Wincott was Vice President of Oligonucleotide Manufacturing & Development at Eyetech Pharmaceuticals, Inc. (2002-2005). Prior to joining Eyetech Pharmaceuticals, Dr. Wincott served as Senior Director of Manufacturing Operations at Ribozyme Pharmaceuticals, Inc. From 1989-1993 she worked as a scientist at Merck, Inc. and Cortech, Inc. Dr. Wincott received her B.A. in Chemistry at the University of Pennsylvania in 1984 and a Ph.D. in Organic Chemistry in 1989 from Yale University

Speaker(s)

Speaker

Deyi Zhang, PhD, MS

Senior Chemist, Office of Generic Drugs
FDA, United States

Dr. Deyi Zhang is a senior chemist in the Office of Research and Standards (ORS), Office of Generic Drugs (OGD) at FDA specializing in complex active ingredients, including peptides, oligonucleotides and complex mixtures. He provides scientific inputs for regulatory policy and actively involves in pre-ANDA meetings, product-specific guidance development of such products, and manages related research activities. Dr. Zhang received his Ph.D. in organic chemistry from the University of Notre Dame. He had a two-year NIH postdoctoral fellowship training at the University of Pennsylvania before joining Eli Lilly in 2000. After 15 years in pharmaceutical industry, he joined FDA. He has over 50 publications and presentations.

Speaker

Representative Invited

Ionis Pharmaceuticals, United States

Speaker

with Novartis Oncology. Lawrence has been active in the areas of pharmaceutical regulations and medicinal chemistry, with his most notable work being the discovery and development of the oncology drug Farydak®.

5:00 PM — 6:00 PM

Poster Reception

Day 2 Oct 29, 2024

8:00 AM — 8:30 PM

Networking Breakfast

8:00 AM — 4:15 PM

Registration

8:30 AM — 9:30 AM

Welcome to Day Two and Session 5: Plenary Session: CRISPR Cures

CRISPR Cures: Actionable Nonclinical, CMC, and Regulatory Paths to Building Gene Editing Therapies for N=Many on a Platform of N=Rare

Track: General Session

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.

Speaker(s)

Speaker

Fyodor Urnov, PhD

Scientific Director
Innovative Genomics Institute , United States

9:30 AM — 10:00 AM

Refreshment and Networking Break

10:00 AM — 11:30 AM

Session 6 Track 1: Safety Observations in Late Clinic Development and Early Commercial Surveillance

Oligonucleotide therapeutics have become mainstream tools for regulation of rare and genetic diseases through their ability to modulate gene expression across a variety of processes. Generational advances in chemistry, delivery and targeting have positively impacted their safety profile. While challenges remain, cell-specific targeting has significantly decreased effective dose and reduced off-target effects, leading to improved safety. This session will address some of the strategies that have contributed to these improvements in safety.

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

- Understand the underlying advances that have contributed to the Safety of oligonucleotide-based therapeutics

Session Chair(s)

Louis O'Dea, MD

Chief Medical Officer and President
BIORCHESTRA (USA) Inc, United States

Representative Invited

FDA, United States

Anuradha Ramamoorthy, Ph.D. is a Policy Lead at the Office of Clinical Pharmacology (OCP), Food and Drug Administration (FDA). She received her Ph.D. in Medical and Molecular Genetics from Indiana University and was a postdoctoral fellow at the NIH and FDA. In her current role, she contributes to regulatory policy development, stakeholder engagement, and regulatory research focused on clinical pharmacology. Prior to this role, she was a Reviewer in the Genomics and Targeted Therapy, OCP contributing to the regulatory review of investigational new drug (IND), new drug application (NDA), and biologic licensing application (BLA).

Speaker(s)

The Evolution of Safety in Oligonucleotide-based Therapeutics; the Roles of Chemistry, Formulation and Targeting

Representative Invited

Ionis Pharmaceuticals, Inc., United States

Dr. Geary is Senior Vice President of Drug Development at Ionis Pharmaceuticals. He is responsible for preclinical and clinical development of antisense drugs. Since joining Ionis in 1995, Dr. Geary has been involved in discovery and development including the regulatory submission of more than thirty investigational new drug applications and two successful CTD/NDAs to U.S. and other regulatory agencies. Dr. Geary received his Ph.D. in Biopharmaceutics from the University of Texas, College of Pharmacy, Austin, Texas and his B.S. in Biology from Texas A&M University, College Station, Texas.

Clinical Case Study in Oligo Safety, TTR Amyloidosis

10:00 AM — 11:30 AM

Session 6 Track 2: Off Target Safety Assessment

Hybridization-dependent off-target effects are a potential safety concern for both oligonucleotide and gene editing therapeutics. This session will start with updated recommendations from Industry on identification, verification and risk assessment of off-target sites for oligonucleotides. This will be followed by a presentation with concrete examples illustrating how the recommendations could be used. The final presentation will focus on assessment of off-target editing for in vivo gene editing approaches.

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

- Describe the different steps in the recommended approach to identify and evaluate potential off-target effects
- Compare and contrast off-target assessments for oligonucleotides and gene editing applications
- Discuss specific considerations for different oligonucleotide and gene editing applications, classes, and delivery systems

Track: Track 2: Nonclinical

Session Chair(s)

Patrik Andersson, PhD

Senior Director, RNA Therapeutics Safety
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.

James Wild, PhD

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.

Discerning the Off-target Effects of RNase H-Dependent Antisense Oligonucleotides by Sequence Analysis and Transcriptomics

Representative Invited

Contera Pharma, Denmark

Peter Hagedorn is a Scientific Director and Head of Bioinformatics at Contera Pharma. Peter has worked with drug design and development of nucleic acid therapeutics for more than 15 years. He holds a master's degree in Biophysics from the Niels Bohr Institute, University of Copenhagen, Denmark, and a PhD in Molecular Biology and Bioinformatics from the University of Southern Denmark.

Assessing the Potential for Off-target Editing with in Vivo Liver-directed Base Editing Therapies

Representative Invited

Verve Therapeutics, United States

Joe Biedenkapp is Vice President of Editing Development at Verve Therapeutics, a clinical-stage genetic medicines company pioneering a new approach to the care of cardiovascular disease, potentially transforming treatment from chronic management to single-course gene editing medicines. In this role, Joe leads a team of scientists and computational biologists developing off-target data packages to support regulatory submissions for Verve's gene editing programs. Prior to joining Verve, Joe served in numerous cross-functional leadership positions at Shape Therapeutics, bluebird bio and Dyax, working across early and late-stage drug development, basic and clinical research, medical affairs, alliance management and product commercialization.

10:00 AM — 11:30 AM

Session 6 Track 3: CMC Considerations in Development of mRNA-based Therapeutics

The breadth of mRNA-based vaccines in clinical and commercial development has continued to mature beyond SARS-CoV-2 vaccines, with the platform nature of mRNA supporting common CMC principles between products. As the landscape of mRNA-based therapeutics in development expands and evolves, thoughtfully adapting principles from mRNA-based vaccines to meet the unique CMC requirements of therapeutic development across diverse therapeutic areas will be needed. This session will include industrial and regulatory perspectives on CMC challenges specific to

- Identify topics or challenges where collaboration between industry and regulators will be needed to successfully

Track: Track 3: CMC

Session Chair(s)

Brian Doyle

Director, LNP Process Development
Moderna, United States

Speaker(s)

Speaker

Representative Invited

Roche, China

Speaker

Eric Levenson, PhD, MS

Biological Reviewer
FDA, United States

Eric is a biological reviewer in the Office of Gene Therapy (OGT) / CBER/ FDA. He reviews a wide range of direct acting gene therapy products including DNA or RNA therapeutics delivered by nanoparticles carriers, including lipid nanoparticles. An interdisciplinary scientist, he has degrees in biochemistry (M.S., Ph.D. University of Delaware) with extensive training in virology, immunology, and chemical biology. His dissertation included developing novel polymer-oligonucleotide conjugates to study innate cellular response. Postdoctoral studies included whole genome siRNA and miRNA screens to find host gene targets of noroviral infection. Prior to joining OGT, Eric was a reviewer of allergenic products and studied host antiviral response.

Speaker

Representative Invited

Biontech SE, Germany

Andreas Kuhn, Senior Vice President RNA Biochemistry & Manufacturing, has worked in the field of

Speaker

Representative Invited

Paul-Ehrlich-Institute, Federal Agency of Vaccines and Biomedicines, Germany

11:30 AM — 12:45 PM

Networking Luncheon featuring Roundtable Discussions

12:45 PM — 2:15 PM

Session 7 Track 1 and 2: Gene Editing

This will be a joint clinical and non-clinical session on gene editing. The session will begin with an overview of the FDA guidance on human genome editing to provide an overview of the recommendations for sponsors developing such products. The subsequent talks will be from sponsors actively working in this space and will include two presentations on nonclinical development topics and one presentation on clinical development.

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

- Understand the latest guidance from FDA on the development of gene editing products
- Gain insight into unique nonclinical considerations for the development of gene editing products
- Understand how sponsors are approaching clinical development of gene editing products

Track: Track 1 and 2: Clinical/Nonclinical

Session Chair(s)

Scott Vafai

Vice President, Translational Medicine
Verve Therapeutics, United States

David Cantu

Speaker(s)

Speaker

Jonathan Phillips, PhD

Vice President, Pharmacology & Toxicology
Intellia Therapeutics, United States

Speaker

Laura Serwer

Senior Director, Head of Pharmacology & Toxicology
CRISPR Therapeutics, United States

Speaker

Representative Invited

Editas Medical, United States

12:45 PM — 2:15 PM

Session 7 Track 3: Challenges Around Oligoneucleotides Control Strategies

Close to a decade ago, pioneering papers were written on how to set up oligonucleotide control strategies, including proposed impurity grouping. Since then, industry and regulators have expanded their knowledge and their experience in this area. At the same time, the complexity of the oligonucleotide landscape is increasing, in particular because of the development of conjugates and new modifications. Because of the reached level of maturity, oligonucleotides are in scope of new or to-be-revised guidelines. The current session aims at exchanging on experiences and challenges pertaining to oligonucleotide control strategies in general and in view of the changing guideline landscape.

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

- Understand the current trends pertaining to oligonucleotide control strategies
- Understand the current challenges and how they could influence the content of new guidelines

Christian Wetter, PhD

Technical Regulatory Advisor
Roche, Switzerland

Christian Wetter is an organic chemist and holds a Ph.D. from the University of Marburg. He started his professional career at Roche in 2004 in Chemical Development before moving to Novartis to Regulatory CMC in 2009. Since 2020 he has been a Regulatory Advisor in Small Molecule Development at Roche. Christian has worked on small molecule, peptide, oligonucleotide and device development projects in various phases of development and commercial lifecycle. He is part of the European Pharma Oligonucleotide Consortium (EPOC) and is currently leading its regulatory subteam.

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.

Speaker(s)

Speaker

Lori Troup

Associate Director, Analytical Development
Dicerna Pharmaceuticals, United States

Speaker

Katharine Duncan

Senior Pharmaceutical Quality Assessor (SPQA), Division of New Drug API, Office
FDA, United States

2:15 PM — 2:45 PM

2:45 PM — 4:15 PM

Session 8: Hot Topics

Session 8: Hot Topics

Track: General Session

Session Chair(s)

Arthur A. Levin, PhD

Distinguished Scientist
Avidity Biosciences, United States

Ronald Wange, PhD

Associate Director for Pharm/Tox, OND, CDER
FDA, 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 15 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)

Speaker

Representative Invited

University of Manchester, United Kingdom

4:15 PM — 5:15 PM

Oligonucleotide Safety Working Group (OSWG) – Open Meeting

Oligonucleotide Safety Working Group (OSWG) – Open Meeting

Session Chair(s)

Jeffrey Foy, PhD

Vice President, Toxicology
PepGen Inc., United States

Day 3 Oct 30, 2024

7:30 AM — 8:00 AM

Networking Breakfast

7:30 AM — 12:40 PM

Registration

8:00 AM — 9:15 AM

Session 9 Track 1: Advantages & Challenges of Early Phase Clinical Studies with Oligos

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

- Recognize the opportunities and challenges of early phase clinical trials with oligonucleotides
- Formulate the designs of their own clinical programs to maximize those opportunities and address the challenges
- Interpret the findings of early phase oligonucleotide trials
- Identify the challenges associated with long oligo pharmacodynamic half-life and predicting optimal dose selection

Track: Track 1: Clinical

Session Chair(s)

Dan Swerdlow, MD, PhD

Senior Director, Early Clinical Development
GSK, United Kingdom

Sydney Stern, PhD, MS

Pharmacokineticist
FDA, 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)

Speaker

Representative Invited

Medpace, United States

Speaker

Representative Invited

Session 9 Track 2: Extra/Non-Hepatic Delivery

This session will describe strategies for targeting extra-hepatic organs and will feature data from sponsor programs. Delivery technologies, nonclinical study design considerations, and pharmacology, ADME, and toxicology data will be discussed.

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

- Recognize current efforts to deliver oligonucleotide-based products to cells or organs outside of the liver
- Have a greater understanding of the challenges associated with delivering oligos to muscle, lung, and brain

Track: Track 2: Nonclinical

Session Chair(s)

Jeffrey Foy, PhD

Vice President, Toxicology
PepGen Inc., United States

Representative Invited

FDA, United States

Speaker(s)

Development of a Novel Muscle-targeted Antibody Oligonucleotide Conjugate for the Treatment of Myotonic Dystrophy Type 1: Toxicology and Regulatory Approaches and Considerations

Representative Invited

Avidity Biosciences, United States

Eileen Blasi, Sr Director, Avidity Biosciences, San Diego, CA: Eileen leads toxicology and regulatory strategy of

Nonclinical Development of Inhaled Oligonucleotides: Nothing Good Comes Easily

Jessica Grieves, DVM, PhD

Director, Pathology and Nonclinical Development
Ionis Pharmaceuticals, United States

Jessica Grieves is a toxicologic pathologist at Ionis Pharmaceuticals where she is involved with the nonclinical development of ASOs, siRNAs, and gene editing modalities with a focus on pulmonary, neurology, and cardiometabolic therapeutic areas. Prior to Ionis, Jessica was a toxicologic pathologist at Takeda Pharmaceuticals.

Brain Delivery of Oligonucleotide-based Therapeutics: Challenges and Accomplishments

Jinhee Yang, PhD

Director
BIORCHESTRA Co., Ltd., Korea, Republic of

Dr. Jinhee Yang is a Director in the New Drug Development Division at BIORCHESTRA, contributing to the preclinical development of antisense therapeutics with CNS drug delivery systems and clinical biomarker studies. Prior to joining, she led a CNS pharmacology team at CKD Pharmaceutical Company, evaluating clinical candidates and lead molecules for the treatment of various CNS diseases. She received her Ph.D. in molecular neurobiology from KAIST, Korea, and had postdoctoral training in the pathophysiology of Alzheimer's disease at Yale University School of Medicine and Seoul National University School of Medicine.

8:00 AM — 9:15 AM

Session 9 Track 3: Innovative Manufacturing Approaches and Regulatory Implications

Continuous improvement in manufacturing of nucleic acid-based modalities requires innovative approaches, with the potential for introduction of new challenges, including the regulatory implications. This session will feature some of the recent innovative manufacturing approaches, along with a panel discussion to highlight the current regulatory landscape and identify prospects in closing potential gaps in understanding.

Track: Track 3: CMC

Session Chair(s)

Ramin Darvari, PhD, MS

Research Fellow
Pfizer Inc., United States

Ramin Darvari is an Associate 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

Senior Research Scientist, Global Regulatory Affairs-CMC
Eli Lilly & Company, United States

Rohit is a Senior Advisor at Eli Lilly & Company and is responsible for developing CMC regulatory strategies for small molecules, oligonucleotides and peptides. Previously, he was a senior CMC reviewer at FDA for 5 years where he reviewed small molecules, oligonucleotides and ADCs. Rohit obtained 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 learned about oligonucleotide chemistry.

Speaker(s)

Oscillating Fluidized Bed Oligonucleotide Synthesizer: Reagents and Solvent Reuse in Solid Phase Oligonucleotide Synthesis

Representative Invited

Eli Lilly, United States

A Ligation Platform Approach to Enzymatic Oligonucleotide Assembly

Representative Invited

Pfizer, Inc. , United States

Speaker

Representative Invited

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.

Speaker

Representative Invited

FDA, United States

9:25 AM — 10:40 AM

Session 10 Track 1: Clinical Pharmacology of Oligonucleotides

This session will explore the various clinical pharmacology aspects that are primarily unique to RNA-oligonucleotides. Topics will include: regulatory considerations regarding clinical pharmacology studies, transitioning to first-in-human studies, and the importance of pharmacodynamic biomarkers. Overall, this session will provide various viewpoints on the unique challenges encountered in the clinical development of oligonucleotides.

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

- Distinguish between EMA and FDA Clinical Pharmacology Guidelines in relation to oligonucleotides

Hobart Rogers, PharmD, PhD

Pharmacologist
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.

Speaker(s)

An Overview of EMA's Clinical Pharmacology Assessment of Oligonucleotides

Carolien Versantvoort, PhD

Senior Clinical Pharmacokinetics Assessor
Medicines Evaluation Board, Netherlands

Bio – Carolien Versantvoort I have over 20 years experience as senior clinical pharmacology assessor and scientific expert at Medicines Evaluation Board in the Netherlands for new medicines and generics. Since 2014, I am a member of the Pharmacokinetic Working Party / Product Specific Bioequivalence Guidance Drafting Group at EMA, currently as Chair. In addition, I am member of the leadership team for the Clinical Pharmacology Special Interest Area group at EMA's Methodology Working Party. Further, I was member of the ICH-M12 team as EU expert on the harmonisation of the drug interaction guideline.

9:25 AM — 10:40 AM

Session 10 Track 2: Non-Clinical Safety Assessment of Oligonucleotides

This session will provide updates on two areas of regulated nonclinical safety assessment (genotoxicity and developmental and reproductive toxicology (DART) evaluation) and experience of characterizing these hazards for therapeutic oligonucleotides (ONTs), as well as a novel application of Artificial Intelligence (AI) to improve the safety of this modality.

- Describe the structural determinants of therapeutic oligonucleotides that are amenable to employing AI/ML models

Track: Track 2: Nonclinical

Session Chair(s)

Representative Invited

GlaxoSmithKline R&D, United Kingdom

Paul C. Brown, PhD

Associate Director for Pharmacology and Toxicology, OND, CDER
FDA, United States

Dr. Brown's responsibilities include development and implementation of guidance and policy related to the nonclinical assessment of human pharmaceuticals. He has been at the FDA since 1996 when he joined the Division of Dermatology and Dental Drug Products as a Pharmacology/Toxicology reviewer. He was supervisor for Pharmacology/Toxicology in this Division from 2003 to 2008. Prior to coming to the FDA he was a Pharmacology Research and Training Fellow in the National Cancer Institute from 1991 to 1996. He worked on multidrug resistance gene structure and function in the Laboratory of Experimental Carcinogenesis. He received his Ph.D. in toxicology from the University of Maryland in 1991.

Speaker(s)

Key Considerations for DART Assessment of Oligonucleotides

Representative Invited

Eli Lilly, United States

Artificial Intelligence and Machine Learning Utility in Safety Assessments of Therapeutic Oligonucleotides

Chris Hart, PhD

CEO & Co-Founder
Creyon Bio, Inc. , United States

White House Office of Science and Technology Policy on biomedical research and health policy issues. Chris earned his PhD from Caltech and conducted post-doctoral training at Yale University.

9:25 AM — 10:40 AM

Session 10 Track 3: Streamlining Oligonucleotide Development with Platform Approaches

This session will discuss the benefits of using platform approaches in the development of therapeutic oligonucleotides, including streamlining processes and expediting regulatory submissions. The session will feature mini talks on platform strategies, where experts will share their insights and experiences. Following the talks, there will be a round table discussion with regulators and industry experts on the challenges and opportunities of implementing these strategies. The discussion will provide a forum for an open exchange of ideas and perspectives, and participants will have the opportunity to engage with the experts and learn from their experiences.

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

- Identify benefits of using platform approaches in the development of therapeutic oligonucleotides
- Recognize the regulatory challenges associated with using platform approaches
- Apply concepts from the session to enable the use of platform approaches

Track: Track 3: CMC

Session Chair(s)

Dominik Altevogt, PhD

Associate Director Regulatory Affairs CMC
Novartis, Switzerland

Dr. 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.

Speaker(s)

Speaker

Representative Invited

Alnylam Pharmaceuticals, United States

Speaker

Lawrence Perez, PhD

Senior Pharmaceutical Quality Assessor, CDER
FDA, United States

Lawrence Perez has been a CMC Reviewer for new drugs with the FDA since 2015 and in 2021 he became a Senior Pharmaceutical Quality Assessor for API New Drugs. Before that, Lawrence was a discovery chemist with Novartis Oncology. Lawrence has been active in the areas of pharmaceutical regulations and medicinal chemistry, with his most notable work being the discovery and development of the oncology drug Farydak®.

Speakers

Representative Invited

Biogen, United States

Speaker

Representative Invited

European Medicines Agency, Netherlands

Brian Dooley has worked as a quality specialist in the Pharmaceutical Quality Office of EMA since 2016, working mostly on centralised marketing authorisations and scientific advice, and supporting the development of scientific guidelines by the CHMP, QWP and BWP. From 2008 to 2016, Brian worked as a pharmaceutical assessor in the IMB/HPRA (Ireland). He holds a B.Sc. in Pharmacy (2005) and M.Sc. in Pharmaceutical Medicine (2015) both from Trinity College Dublin, Ireland. Areas of interest: lifecycle management, quality risk management, assessment-inspection interface, synthetic peptides, oligonucleotides, mRNA, sterilisation processes, radiopharmaceuticals.

10:40 AM — 11:10 AM

Session 11: Grand Q&A Panel

Speaker(s)

Session 11: Grand Q and A Panel

Ramesh Raghavachari, PhD

Chief, Branch I, DPMA1, OLDP, OPQ, CDER
FDA, United States

Ph.D - Temple University, Philadelphia, PA Currently a Chemist at FDA/CDER, has been with FDA since 2003.

12:25 PM — 12:40 PM

Closing Remarks

Speaker(s)

Sorcha McCrohan, MS, MSc

Specialist, Scientific Programs
DIA, United States

Sorcha McCrohan is a Specialist of Scientific Programs for the Americas Region 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 Meningococcal franchise. Sorcha holds a BA in Sociology from Mount Holyoke College and an MSc in Global Health, Disease Prevention & Control from Georgetown University.

Scott Henry, PhD

Vice President, Nonclinical Development
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

Ronald Wange, PhD

Associate Director for Pharm/Tox, OND, CDER
FDA, 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 15 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.

12:40 PM — 12:40 PM

Conference Adjourns