DIA/FDA Oligonucleotide-Based Therapeutics Conference

Arlington, VA | September 23-25

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Federal Institute for Drugs and Medical Devices

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GSK

PROGRAM ADVISORS

Scott Henry, DrPH
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Ionis Pharmaceuticals, Inc.

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Avidity Biosciences

James Wild, MS
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FDA

Overview

The *DIA/FDA Oligonucleotide-Based Therapeutics Conference* brings together leading experts to inform, educate, and share advancements in oligonucleotide-based therapeutic product development. Developed collaboratively by regulators, industry professionals, and academics, the program covers a wide range of topics from the nonclinical, CMC, and clinical areas, including emerging CMC guidance's and considerations, learnings from recent regulatory filings, extra-hepatic and CNS delivery of oligonucleotides, toxicology testing, gene editing, and safety assessments. The conference offers a unique three-day experience with multiple perspectives presented, and the opportunity to interface with regulators from around the globe.

Learning objectives

At the conclusion of this activity, participants should be able to:

- Analyze the latest strategies for clinical use of oligonucleotide therapies and explain the specific challenges of developing RNA-based therapeutics
- Describe the chemistry, manufacturing, and controls challenges associated with the development of oligonucleotides, including formulation and specification issues
- Describe the technology landscape, CMC challenges, and regulatory considerations associated with novel oligonucleotide delivery approaches
- Explain the latest global regulatory updates in oligonucleotide therapeutic developments

Why You Can't Miss It

- Learn about the latest advancements, regulatory updates, and emerging trends in oligonucleotide therapies directly from leading industry experts and global regulators
- Our three tracks (Clinical, Nonclinical, and CMC) will comprehensively cover a wide range of aspects of oligonucleotide therapeutics—from development to regulatory collaboration—and provide practical insights into current challenges and successful strategies for overcoming hurdles in clinical use, safety assessments, and CMC
- This conference offers a networking opportunity to engage with peers, innovators, and regulators in sessions designed to foster meaningful collaboration and knowledge exchange

Who Should Attend

Conference Designed for:

Professionals involved in the following areas of oligonucleotide science:

- Drug Discovery
- Clinical
- Quality Assurance
- Vaccines
- Delivery Technologies
- Safety and Toxicology
- Diagnostics
- Preclinical
- CMC
- RNAi
- Biotechnology
- Clinical Pharmacology/Research
- Regulatory

Track Descriptions

Track 1: Clinical
There are more than a dozen oligonucleotide-based products approved for therapeutic use and nearly 100 currently in clinical testing. Track 1 will explore the lessons learned and the ongoing challenges to clinical use of oligonucleotide therapies

Track 2: Nonclinical
As oligonucleotide-based therapeutics advance, understanding their nonclinical development and safety is critical for their success in clinical and commercial stages. This track offers an in-depth look at the latest safety assessments, preclinical models, and regulatory expectations for nonclinical data.

Track 3: CMC
This track provides a collaborative forum for innovators and regulators to present and discuss cutting edge topics in oligonucleotide chemistry, manufacturing, and controls (CMC). Emphasizing emerging topics that require ongoing global regulatory collaboration, attendees will gain a deeper understanding of key insights into current challenges and successful strategies for navigating this evolving field.

Schedule At-A-Glance (All times listed are Eastern Time)

DAY ONE | WEDNESDAY, SEPTEMBER 23		ROOM
8:15AM-4:45PM	Registration	Grand Ballroom Foyer
8:15-9:15AM	Networking Breakfast	**Grand Ballroom ABJK**
9:15-9:30AM	Welcome and Opening Remarks	**Grand Ballroom ABJK**
9:30-10:30AM	Session 1: Keynote Address	**Grand Ballroom ABJK**
10:30AM-11:15AM	Refreshment and Networking Break	Grand Ballroom Foyer
10:35AM-11:05AM	Case Study Sponsored by Agilent: Analytical Validation Approaches at Every Phase of Oligonucleotide Development	
11:15-12:45PM	Session 2: CONCURRENT SESSIONS Track 1: The Brains Behind Delivery of Oligos to the CNS Track 2: Extrahepatic Delivery 1: Engineering LNPs and Ligand Conjugates for Oligonucleotide Delivery to the Kidney and Lung Tumors Track 3: Unlocking Faster, Sustainable Oligonucleotide Manufacturing Through Regulatory Innovation and Excellence	**Grand Ballroom CDE** **Grand Ballroom EFG** **Grand Ballroom ABJK**
12:45-1:45PM	Networking Luncheon	**Grand Ballroom ABJK**
1:45-3:15PM	Session 3: CONCURRENT SESSIONS Tracks 1 and 2: Regulatory Considerations of Novel Conjugates: Translational and Clinical Pharmacology Perspectives Track 3: Advancing the Analytical Toolbox for mRNA-Based Medicines	**Grand Ballroom EFG** **Grand Ballroom ABJK**
3:15-3:45PM	Refreshment and Networking Break	**Grand Ballroom Foyer**
3:45-5:15PM	Session 4: CONCURRENT SESSIONS Track 1: Translational Safety of Genetically Targeted Oligonucleotides: Managing Mechanistic Risks, Immunogenicity, and AOC Platforms Track 2: Optimizing Oligonucleotide Therapeutics: Translating PK/PD and Tissue Half-Life into Smarter Dose Selection and Risk Assessment Track 3: Advance Enzymatic Oligonucleotide Manufacturing: From Scalable Chemistry to Regulatory CMC Readiness	**Grand Ballroom CDE** **Grand Ballroom EFG** **Gand Ballroom ABJK**
5:15-6:15PM	Networking Reception	Grand Ballroom Foyer

DAY TWO | THURSDAY, SEPTEMBER 24		ROOM
8:00AM-4:15PM	Registration	Grand Ballroom Foyer
8:00-8:30AM	Networking Breakfast	Grand Ballroom ABJK
8:30-9:30AM	Welcome to Day Two and Session 5: Implementing the Plausible Mechanism Pathway: From Concept to Clinic	**Grand Ballroom ABJK**
9:30-10:00AM	Refreshment and Networking Break	Grand Ballroom Foyer
10:00-11:30AM	Session 6: CONCURRENT SESSIONS Track 1: Strengthen Applications for Oligonucleotide FIH Trials Through Global Regulatory Agency Leader Perspectives Track 2: Mind Over Matter: Cracking the Code of CNS Delivery Track 3: Navigating Change: Unlocking Opportunities and Overcoming Challenges in Recent US CMC Policy Developments	**Grand Ballroom CDE** **Grand Ballroom EFG** **Grand Ballroom ABJK**
11:30AM-12:45PM	Networking Luncheon	**Grand Ballroom ABJK**
12:45-2:15PM	Session 7: CONCURRENT SESSIONS Tracks 1 and 2: Navigating the Testing Strategy for ONTs based on the Updated Q&A's of ICH E14 and S7B Track 3: Oligonucleotide Delivery: New Developments and Regulatory Strategies	**Grand Ballroom EFG** **Grand Ballroom ABJK**
2:15-2:45PM	Refreshment and Networking Break	Grand Ballroom Foyer
2:45-4:15PM	Session 8: Hot Topics	**Grand Ballroom ABJK**
4:15-5:15PM	Oligonucleotide Safety Working Group (OSWG) – Open Meeting	**Grand Ballroom CDE**

DAY THREE | FRIDAY, SEPTEMBER 25		ROOM
7:45AM-12:45PM	Registration	Grand Ballroom Foyer
7:45-8:15AM	Networking Breakfast	Grand Ballroom ABJK
8:15-9:30AM	Session 9: CONCURRENT SESSIONS Track 1: Muscling in on RNA: The Future of Oligonucleotide Therapy for Heart and Muscle Disease Track 2: From Data to Decisions: Evolving the Regulatory Landscape for Oligonucleotide Therapeutics Track 3: Evolving Regulatory Landscape for Oligonucleotide Control Strategy and Comparability	**Grand Ballroom CDE** **Grand Ballroom EFG** **Grand Ballroom ABJK**
9:30-10:00AM	Refreshment and Networking Break	
10:00-11:15AM	Session 10: CONCURRENT SESSIONS Track 1: Safety of Oligonucleotide Therapeutics: Lessons from Clinical Development and Post-Marketing Experience Track 2: Extra-Hepatic Delivery Strategies to Optimize Distribution and Exposure for Improved Efficacy and Safety in Translational Models Track 3: Real-World Regulatory Experiences with Clinical/Commercial Oligonucleotides	**Grand Ballroom EFG** **Grand Ballroom ABJK**
11:20AM-12:35PM	Session 11: Grand Q&A Panel	**Grand Ballroom ABJK**

12:35-12:45PM	Closing Remarks	Grand Ballroom ABJK
12:45PM	Conference Adjourns	

Continuing Education Credit Allocation

This program is eligible for Attendance Credit. No Continuing Education will be offered.

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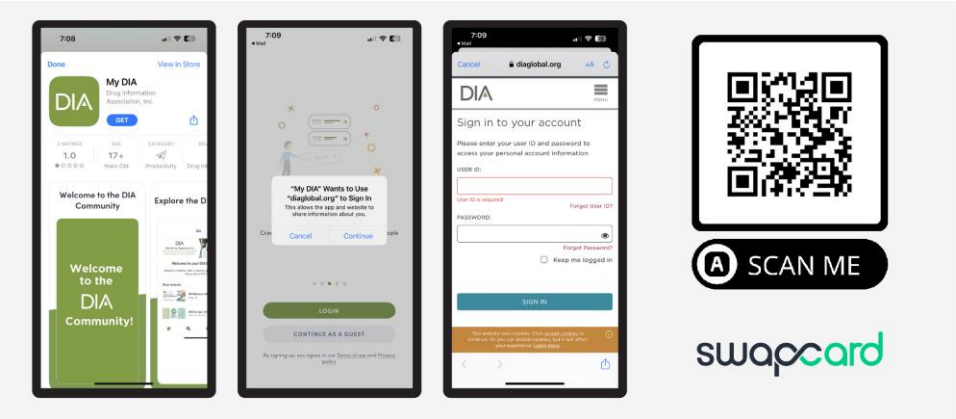

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