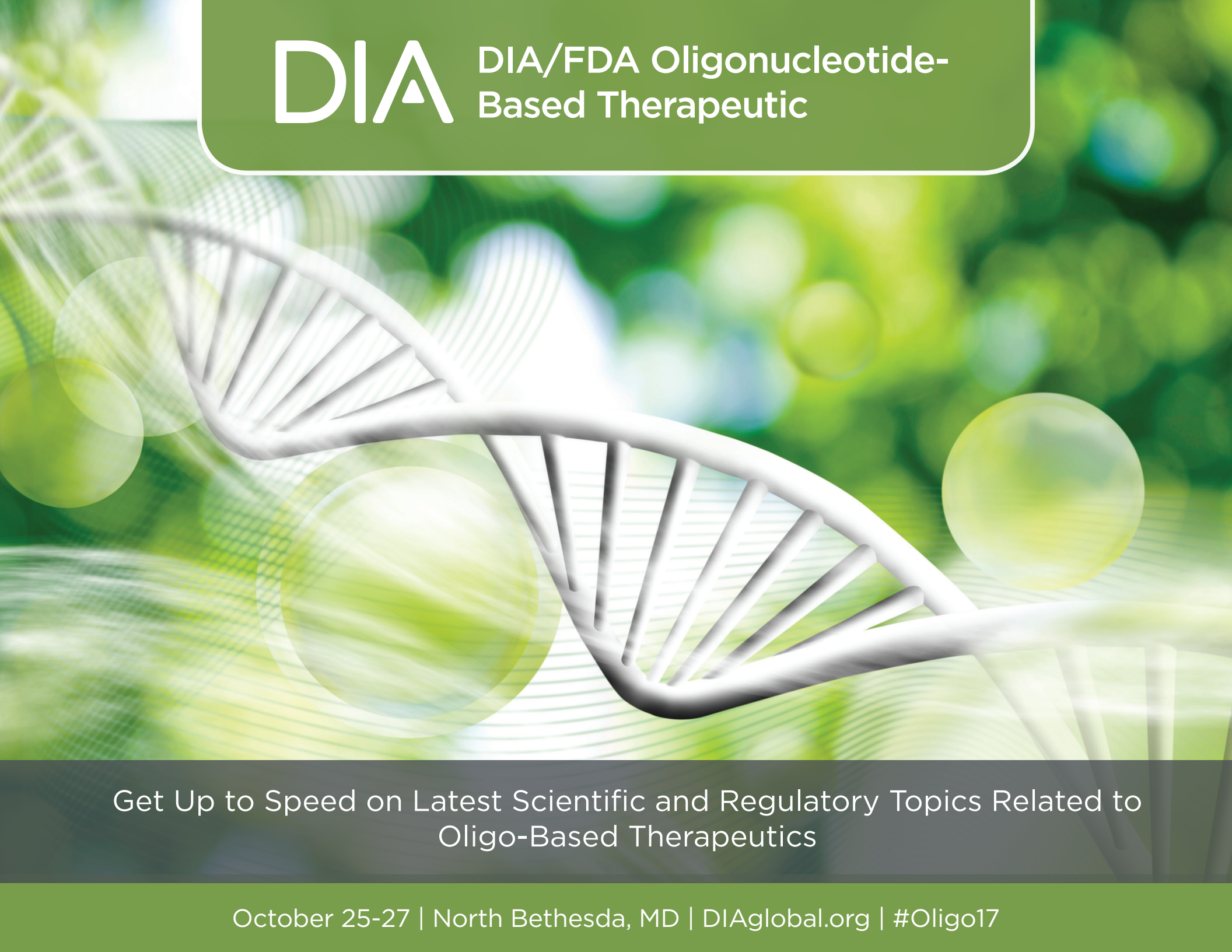
DIA
DIA/FDA Oligonucleotide-Based Therapeutic
Get Up to Speed on Latest Scientific and Regulatory Topics Related to Oligo-Based Therapeutics
October 25-27 | North Bethesda, MD | DIAglobal.org | #Oligo17

DIA/FDA Oligonucleotide-Based Therapeutic Conference

Oligonucleotide therapeutics are approaching clinical productivity as the result of advances in oligonucleotide chemistry combined with new knowledge of the fundamental principles that define the in vivo behavior of oligonucleotides.

Representing a diverse class of drugs, including small interfering RNAs (siRNAs), antisense oligonucleotides (ASOs), microRNAs, aptamers, and others, oligo-based therapies offer a broad pipeline to treat multiple disease states.

Understanding the issues in nonclinical and clinical development of oligonucleotides as well as the CMC challenges are critical to the success of industry and regulatory agencies to appropriately address the unmet medical needs of patients.

From gene editing to immune stimulatory oligonucleotides, DIA's conference covers the latest scientific and regulatory topics related to oligo-based therapeutics.

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Oligonucleotide drugs are an inherently flexible vehicle for therapeutic design, given their molecular features. Similar to other treatment modalities, early success for oligo-based therapeutics is measured by preclinical studies which examine their pharmacological profile. The pharmacokinetics—absorption, distribution, metabolism, and excretion (ADME)—of oligonucleotide drugs are dynamic, and depend on the chemical modifications and structural architecture of the drug.

Determining the pharmacokinetics of an oligonucleotide-based therapeutic is essential to understanding the trafficking, cellular uptake, and other behaviors of the drug.

Questions to be addressed:

What are the patterns of absorption and distribution for oligonucleotide drugs?

How do target- and tissue-specific differences in cellular uptake affect the pharmacokinetics of oligonucleotides when administered systemically and intrathecally?

Given the complexity and variability of these drugs, it is important to consider their pharmacokinetics and pharmacodynamics when readying a product for market or for preclinical study.[1] For those in the preclinical phase of development, the *DIA/FDA Oligonucleotide-Based Therapeutics Conference* provides information on **relevant biomarkers**, **oligonucleotide cellular uptake patterns**, distribution patterns, **safety concerns**, and **methods to integrate novel mechanisms of RNA processing**. Information about the barriers to successful synthesis, as well as **how to overcome these barriers** will also be provided.

Questions to be addressed:

What biomarkers are promising for oligonucleotide therapy?

How would an investigator use biomarkers to interpret pharmacokinetic and pharmacodynamic parameters and dose-response relationships?

[1] *Moschos, Sterghios A., Louise Usher, and Mark A. Lindsay. "Clinical potential of oligonucleotide-based therapeutics in the respiratory system." Pharmacology & therapeutics 169 (2017): 83-103.*

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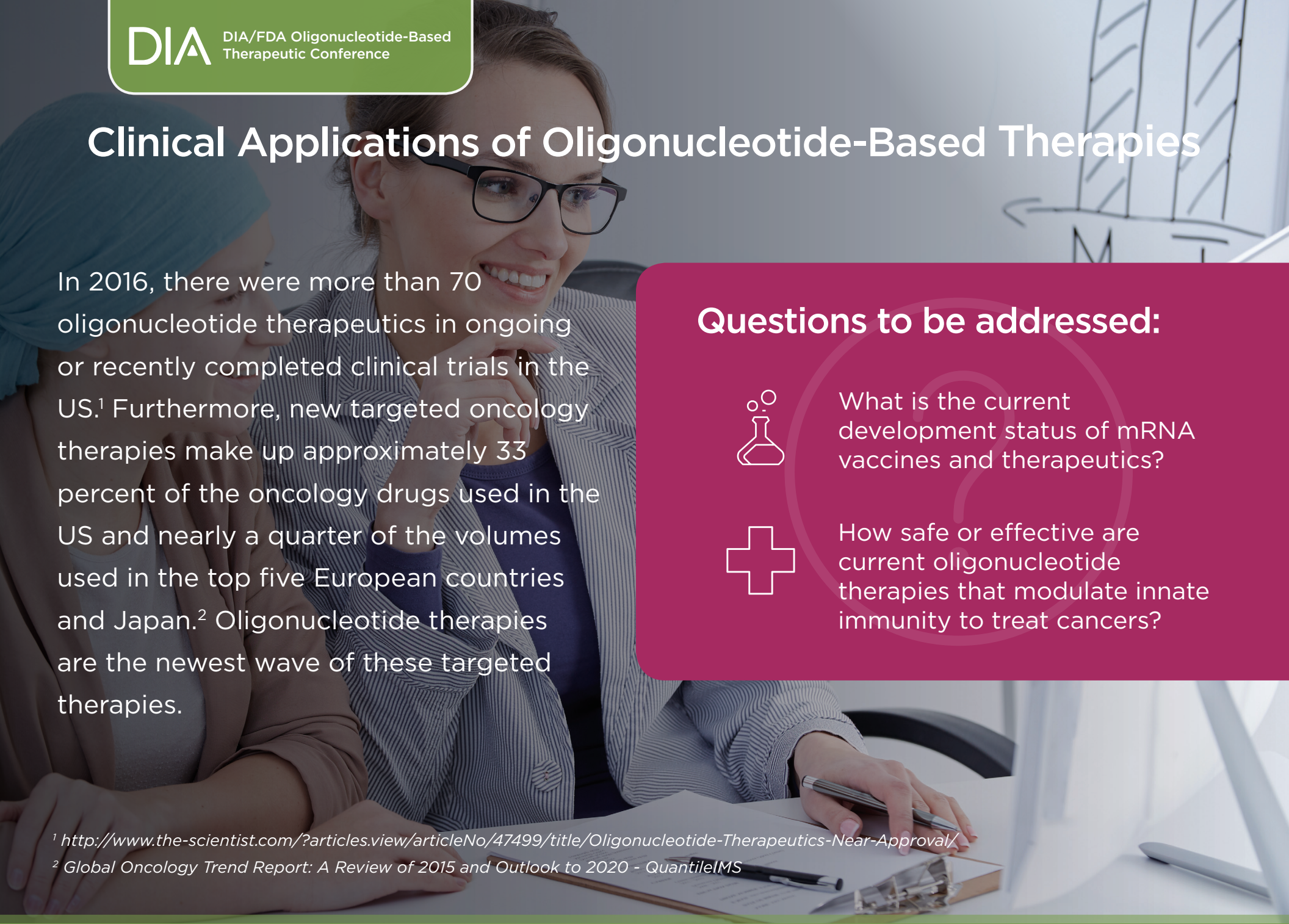
Clinical Applications of Oligonucleotide-Based Therapies



In 2016, there were more than 70 oligonucleotide therapeutics in ongoing or recently completed clinical trials in the US.[1] Furthermore, new targeted oncology therapies make up approximately 33 percent of the oncology drugs used in the US and nearly a quarter of the volumes used in the top five European countries and Japan.[2] Oligonucleotide therapies are the newest wave of these targeted therapies.

Questions to be addressed:

- What is the current development status of mRNA vaccines and therapeutics?
- How safe or effective are current oligonucleotide therapies that modulate innate immunity to treat cancers?

[1] *http://www.the-scientist.com/?articles.view/articleNo/47499/title/Oligonucleotide-Therapeutics-Near-Approval/*

[2] *Global Oncology Trend Report: A Review of 2015 and Outlook to 2020 - QuantileIMS*

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Currently there are several synthetic nucleic acids that modulate immune pathways and trigger immune responses against tumor antigens in subjects with cancer in development. mRNA-based oligonucleotides provide an interesting, multi-faceted basis for therapeutics, and oligonucleotides are able to serve a wide range of functions as antigens and cell-signaling factors in a single molecule.

Unlike some oligonucleotide treatments, mRNAs promote the expression of a protein, rather than inhibit it, leading to different nuances in regulation compared with other nucleic acid-based therapies. As vaccines or as immunotherapies, mRNA-based oligonucleotides are able to serve a wide range of functions as antigens and cell-signaling factors in a single molecule.

Questions to be addressed:

How are regulatory bodies approaching differentiation and approval of these therapies?

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A recent analysis predicts that the global antisense and RNAi therapeutics market will reach $4.58 billion by 2022.[3]

Interested in bringing an oligonucleotide product to market? The *DIA/FDA Oligonucleotide-Based Therapeutics Conference* will provide regulatory perspectives and describe the development status of some current oligonucleotide therapies for the following disease states:

1. **Oncology, including immunotherapies**
2. **Neuromuscular diseases**
3. **Cardiovascular and metabolic diseases**

Questions to be addressed:

What are the challenges faced in designing for neurologic or other diseases?

How does an investigator make sense of endpoints, their clinical meaningfulness, and evaluation in trials?

[3] *Antisense And RNAi Therapeutics Market Worth $4.58 Billion By 2022." Grand View Research. Press release. http://www.grandviewresearch.com/press-release/antisense-rnai-therapeutics-market*

DIA/FDA Oligonucleotide-Based Therapeutic Conference

There has been a dramatic increase in the complexity of drug candidates being promoted into preclinical and clinical development, and this has created new challenges for CMC professionals working with oligo-based therapeutics. Additonal strategies for defining and sourcing non-standard oligonucleotide-specific starting materials are needed, as well as different approaches in characterizing and controlling product quality.

The *DIA/FDA Oligonucleotide-Based Therapeutics Conference* will provide best practices to address the CMC challenges noted above, as well as explain strategies for qualifying oligonucleotide impurities.

Questions to be addressed at #Oligo17:

How are the recent communications from regulatory authorities regarding the selection and justification of starting materials impacting oligo-based therauputics CMC?

What are the unique challenges associated with starting material selection for oligonucleotides?

What are appropriate characterization expectations for oligonucleotide impurities?

What are appropriate reporting, identification, and qualification thresholds for oligonucleotide therapeutics?

How should oligonucleotide impurities be qualified?

Can platform data be used to help characterize and qualify oligonucleotide impurities?

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Oligonucleotide Drug-Device Combination Products are an emerging trend, yet there are various issues that must be considered during the development of drug-device combination products for oligonucleotide therapeutics. From design factors to validation processes, industry experts will describe major technical and regulatory considerations for developing oligonucleotide drug-device combination products.

Questions to be addressed:

How are human factors studies incorporated into the development of oligonucleotide drug-device combination products?

What are the promises and challenges in using drug-device combination approach for targeted oligonucleotide drug delivery?

What are some strategies for ensuring product quality, including uniformity of dose delivery, under conditions of use?

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Conference Highlights

- Poster Session
- Hot topics including mRNA, CRISPR, Immune stimulatory oligonucleotides, and updates on recently approved and late-stage oligo drugs
- Luncheon Round Table Discussions on cutting-edge topics with key thought leaders
- Oligo Safety Working Group (OSWG) Meeting-Open to all Oligo17 attendees! Not a DIA Member yet? Join this discussion to learn more!

This is a very targeted meeting and a "must attend" for those developing drugs based on oligonucleotides. It is a multidisciplinary meeting that is well organized and has several new and challenging topics where industry and regulatory agency attendees participate with a view to enabling getting drugs to patients.

- Previous Attendee

Keynote Speakers

Philip J. Brooks, PhD

Program Director, Division of Clinical Innovation National Center for Advancing Translational Sciences (NCATS), National Institutes of Health

and

Arthur M. Krieg, MD

President and CEO
Checkmate Pharmaceuticals

What You Can Expect

- FDA's current thinking about the regulatory science governing oligo-based therapeutics
- Open, engaging discussions with senior-level regulatory and industry leaders on accepted new concepts and approaches for oligo-based therapeutics
- Exploration of emerging ideas with key thought leaders, FDA speakers, and attendees
- Opportunities for cross-functional communication with related biopharmaceutical disciplines, such as toxicology, pharmacology, clinical research, pharmacovigilance, chemistry, delivery technologies, and many more

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What's happening at DIA in 2017?

Download the DIA Events and Training Calendar to see where and when DIA will be hosting conferences, meetings, short courses, and training opportunities for you and your team.

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