FINAL WEEKS **TO REGISTER**

5th ANNUAL | **OCTOBER 20 - 21, 2020**

OPT Congress

Oligonucleotide & Precision Therapeutics

VIRTUAL

DISCOVERY ■ DEVELOPMENT ■ DELIVERY

CONFERENCE PROGRAMS:

Oligonucleotide Discovery & Delivery

Oligonucleotide CMC and Regulatory Strategies

SHORT COURSE:

Examining the Safety and Toxicity of Nucleic Acid Therapeutics

FEATURED SPEAKERS



Brett Monia, PhD
CEO, Ionis Pharmaceuticals



Edward Kaye, MD
CEO, Stoke Therapeutics



Ekkehard Leberer, PhD
Senior Director, R&D Alliance Management, Sanofi



Marvin Caruthers, PhD
Distinguished Professor, University of Colorado



Phil Baran, PhD
Professor, Department of Chemistry, Scripps Research

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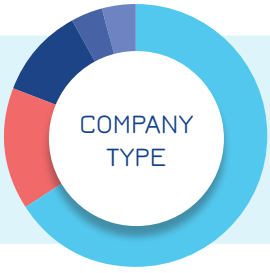
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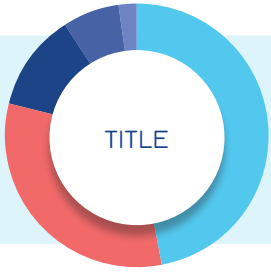
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Shannon Kresge | Business Development Manager | 781-972-1343 | skresge@healthtech.com

2019 Attendee Demographics



- Biotech **66%**
- Pharmaceutical **15%**
- Academic **11%**
- Hospital/Healthcare **4%**
- Other **4%**



- Executive/Director **47%**
- Scientist **32%**
- Sales & Marketing **12%**
- Manager **7%**
- Other **2%**



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Ekkehard Leberer, PhD
Senior Director, R&D Alliance Management, Sanofi

TUESDAY, OCTOBER 20, 2020

Oligonucleotide Discovery & Delivery	Oligonucleotide CMC & Regulatory Strategies
Plenary Session	
Oligonucleotide Discovery & Delivery	Oligonucleotide CMC & Regulatory Strategies
Short Course	

WEDNESDAY, OCTOBER 21, 2020

Oligonucleotide Discovery & Delivery	Oligonucleotide CMC & Regulatory Strategies
Closing Plenary Session	

SHORT COURSE

Tuesday, October 20 | 3:30 - 5:00pm

SC1: Examining the Safety and Toxicity of Nucleic Acid Therapeutics

Nucleic acid drugs continue to deliver on their promise to become a third therapeutic modality, in addition to small molecules and biologics. Several antisense oligonucleotide drugs have been on the market for some time, while the first RNAi approval was granted in 2018. In addition, numerous mRNA and CRISPR therapeutic programs have entered clinical stages. Despite the common "nucleic acid" component, the mechanisms of action and of non-specific effects differ for each of these drug types.

Topics to be discussed include:

- Different types of nucleic acid-based drugs
- Mechanisms of actions and non-specific effects
- Current approaches to address non-specific and potentially toxic effects

Instructors:

Jeffrey Foy, PhD, Senior Director, Toxicology, Dicerna Pharmaceuticals

John Davis II, PhD, Vice President, Pre-Clinical Development, Wave Life Sciences

Xiao Shelley Hu, Senior Director, Head of DMPK and Clinical Pharmacology, Wave Life Sciences

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Oligonucleotide Discovery & Delivery

Optimizing Design, Delivery and Performance

TUESDAY, OCTOBER 20

OPTIMIZATION DESIGN, DELIVERY AND PERFORMANCE



10:05 am FEATURED PRESENTATION: Controlling Chirality of Phosphorothioates in Antisense Oligonucleotides Does Not Enhance Potency or Duration of Effect in the CNS

Brad Wan, PhD, Principal Scientist, Medicinal Chemistry, Ionis Pharmaceuticals

Bicyclic oxazaphospholidine (OAP) monomers were used to prepare a series of phosphorothioate (PS)-modified gapmer antisense oligonucleotides (ASOs) targeting Malat-1 or Lrrk2 mRNA. We found that controlling PS chirality in the MOE wings or in the DNA gap did not enhance ASO potency or duration of effect in the CNS relative to their stereo-random counterparts. Complete details, including sequence, design of ASOs and lessons learned, will be presented.

10:25 Control of Backbone Stereochemistry Provides a New Dimension for the Optimization of Oligonucleotide Drug Candidates

Muthu Kandasamy, PhD, Dir, WAVE Life Sciences

Wave Life Sciences has developed PRISM™, a discovery and drug development platform, which enables us to generate stereopure oligonucleotides to target genetically defined diseases. Using PRISM, we optimize stereopure oligonucleotides to meet pre-defined product profiles. We illustrate by example that optimized, stereopure oligonucleotides exhibit the desired activity across multiple modalities We demonstrate that the potencies of stereopure oligonucleotides in cellular models under free-uptake conditions help predict their potencies in animal models.

10:45 Session Break

11:05 PANEL DISCUSSION: Live Q&A with Session Speakers

Moderator: Mano Manoharan, PhD, Distinguished Scientist & Sr VP Innovation Chem, Innovation Chemistry, Alnylam Pharmaceuticals

Panelists:

Brad Wan, PhD, Principal Scientist, Medicinal Chemistry, Ionis Pharmaceuticals

Muthu Kandasamy, PhD, Dir, WAVE Life Sciences

11:25 Coffee Break - View Our Virtual Exhibit Hall

PLENARY



11:55 Recent Advances in Antisense Technology at Ionis Pharmaceuticals

Brett P. Monia, PhD, CEO, Ionis Pharmaceuticals, Inc.

The development of antisense technology to treat a broad range of rare and common diseases is now validated. Antisense medicines offer tremendous hope for patients afflicted with serious diseases that cannot be addressed with traditional drug discovery platforms. With numerous antisense medicines recently receiving market authorization, along with a large and diverse pipeline of medicines on the horizon, antisense is poised to revolutionize the practice of medicine for generations.



12:20 pm Using ASOs to Upregulate Protein Expression

Edward M. Kaye, MD, CEO, Stoke Therapeutics

Stoke has developed an RNA platform that efficiently upregulates protein expression. This is accomplished by removing retained non-coding elements at the pre-mRNA level, and thereby increasing the amount of full-length mRNA. This increase in full-length mRNA from a normal copy of the gene results in an increase of full-length protein, which we are using to address haplo-insufficient diseases.

12:40 LIVE FIRESIDE CHAT: The Future Outlook of RNA Targeted Therapeutics



Moderator: Dmitry Samarsky, PhD, CTO, Sirnaomics

- Recent breakthroughs and advancements in RNA therapeutics and gene editing/expression
- Opportunities of neurological diseases in drug discovery, development and delivery
- Challenges that lie ahead

Panelists:

Brett P. Monia, PhD, CEO, Ionis Pharmaceuticals, Inc.

Edward M. Kaye, MD, CEO, Stoke Therapeutics

1:00 Lunch Break - View Our Virtual Exhibit Hall

OPTIMIZING DESIGN, DELIVERY AND PERFORMANCE

1:40 Learning from Failures: The Story of Drisapersen Exon Skipping Development for Duchenne Muscular Dystrophy

Annemieke Aartsma-Rus, PhD, Professor of Translational Genetics, Leiden University Medical Center

Antisense oligonucleotides (ASOs) will hide a target exon from the machinery, preventing its inclusion into mRNA. This restores the reading frame, allowing the production of a partially functional dystrophin. The presentation will outline the development of this approach through proof-of-concept studies in cell and animal models, preclinical optimization studies and clinical trials, but also discuss required multilateral education of stakeholders to develop tools to measure clinical efficacy of the approach.

2:00 siRNA Therapeutics for Ocular Indications

Elena Feinstein, MD, PhD, CSO, Quark Pharmaceuticals

Advances in platform technology as well as proof of concept data from non-clinical and clinical studies in ocular indications will be discussed.

2:20 Refresh Break - View Our Virtual Exhibit Hall

2:50 Next-Generation Oligonucleotide Therapy Candidates with Tunable Backbones

David Tabatadze, PhD, President, ZATA Pharmaceuticals, Inc.

The ZON platform is a novel class of oligonucleotides synthesized via phosphoramidite chemistry that permits facile attachment to the internucleoside phosphate charge-neutralizing groups (CNG), bearing positive charges at their termini. ZONs have length-optimized CNG branches, allowing these positive charges to reach their neighboring phosphate groups where they can neutralize negative charges. ZON technology can be equally applied to DNA and RNA derivatives and be applied in all oligotherapy approaches.

3:10 Divalent siRNA Scaffold for Robust Gene Modulation in the Central Nervous System

Chantal Ferguson, Senior PhD Student, RNA Therapeutic Institute, University of Massachusetts Medical School

We developed a divalent (Di)-siRNA scaffold that supports potent and sustained gene silencing in the CNS upon intra-cerebroventricular (ICV) injection. In mice, di-siRNA silences target mRNA in mouse CNS for at least 6 months without detectable toxicity. In cynomologus macaques, a bolus injection of di-siRNA showed substantial distribution and robust silencing throughout CNS without detectable toxicity and minimal off-target effect.

3:30 PANEL DISCUSSION : Live Q&A with Session Speakers

Moderator: Mano Manoharan, PhD, Distinguished Scientist & Sr VP Innovation Chem, Innovation Chemistry, Alnylam Pharmaceuticals

Panelists:

Annemieke Aartsma-Rus, PhD, Professor of Translational Genetics, Leiden University Medical Center

Elena Feinstein, MD, PhD, CSO, Quark Pharmaceuticals
David Tabatadze, PhD, President, ZATA Pharmaceuticals, Inc.
Chantal Ferguson, Senior PhD Student, RNA Therapeutic Institute, University of Massachusetts Medical School

3:30 **Recommended Short Course***

SC1: Examining the Safety and Toxicity of Nucleic Acid Therapeutics
**Separate registration required. See short course page for details.*

3:50 **Happy Hour - View Our Virtual Exhibit Hall**

4:20 **Close of Day**

WEDNESDAY, OCTOBER 21

RECENT ADVANCES WITH RNAs



10:20 am **KEYNOTE PRESENTATION: MiRNA Therapeutics: From Bench to Bedside**
Ekkehard Leberer, PhD, Senior Director, R&D Alliance Management, Sanofi; Scientific Managing Director, COMPACT Consortium
The presentation will summarize the opportunities and challenges of developing microRNA-based drugs, discuss challenges and solutions for delivery to target tissues, and will illustrate the successful generation of an anti-fibrotic microRNA-based therapeutic approach by targeting microRNA-21 with an antisense oligonucleotide (anti-miR-21).

10:40 **Thought Leader Presentation: Method Development for the Characterization of Synthetic Oligonucleotides by LC-MS**
, Phenomenex

11:00 **Session Break**

11:20 **Coffee Break - View Our Virtual Exhibit Hall**

11:50 **Asymmetric siRNAs Targeting Age-Related Macular Degeneration**
Dong-Ki Lee, PhD, CEO, OliX Pharmaceuticals
OliX pharmaceuticals is developing novel RNAi therapeutics against a variety of diseases with unmet medical needs, based on its proprietary asymmetric siRNA (asiRNA) platform. In this presentation, we will introduce preclinical data from OLX301A and 301D, ocular asiRNA therapeutic programs targeting age-related macular degeneration (AMD).

12:10 pm **How Do miRNAs and RNAi Function Inside Cells?**
David Corey, PhD, Professor, Department of Pharmacology, UT Southwestern
RNA interference can be a potent regulatory mechanism in human cells. Synthetic RNAs can control gene expression and have been developed to be successful drugs while endogenous miRNAs can control natural physiologic processes and disease. Despite two decades of study, however, the full scope of natural RNAi in cells has remained obscure. Our results suggest that the action of miRNAs in cells is complex and must be justified with care.

12:30 **Breakout Roundtable Discussions**

BREAKOUT: Nucleic Acid-Based Therapies for Prevention and Treatment of COVID-19: Pros and Cons
David Tabatadze, PhD, President, ZATA Pharmaceuticals, Inc.

BREAKOUT: Recent Advances in the Following Areas: The CNS, the Liver, Inhalation Drugs, Immuno-Oncology and Ocular Diseases
Patrick Lu, PhD, President & CEO, Sirnaomics

BREAKOUT: Examining the Safety and Toxicity of Nucleic Acid Therapeutics
Steven Kates, PhD, Vice President, Regulatory Affairs, Dicerna Pharmaceuticals

BREAKOUT: Building a Robust, Targeted-Centered Development Program
David Corey, PhD, Professor, Department of Pharmacology, UT Southwestern

1:15 **Lunch Break - View Our Virtual Exhibit Hall**

2:00 **Novel siRNA Therapeutics for Enhancing Antitumor Activity of Immune Checkpoint Inhibitor**
Patrick Lu, PhD, President & CEO, Sirnaomics
Using a proprietary and optimized polypeptide nanoparticle (PNP) -based delivery technology, we have developed the novel anti-fibrosis and anti-cancer therapeutics with siRNAs targeting both TGFβ1 and Cox-2 simultaneously (STP705), resulting in human fibroblasts apoptosis. STP705 was initially used for local treatments for skin hypertrophic scar and non-melanoma skin cancer. STP707 (a systemic formulation of STP705) was further advanced for treatment of liver fibrosis and cholangiocarcinoma.

2:20 **LIVE PANEL DISCUSSION: Opportunities and Challenges with RNAs**
Moderator: Ekkehard Leberer, PhD, Senior Director, R&D Alliance Management, Sanofi; Scientific Managing Director, COMPACT Consortium
Panelists:
Patrick Lu, PhD, President & CEO, Sirnaomics
David Corey, PhD, Professor, Department of Pharmacology, UT Southwestern
Dong-Ki Lee, PhD, CEO, OliX Pharmaceuticals

2:40 **Refresh Break - View Our Virtual Exhibit Hall**

CLOSING PLENARY



3:10 **Biological Activity of Thiomorpholino Oligonucleotides**
Marvin Caruthers, PhD, Distinguished Professor, University of Colorado
Thiomorpholino oligonucleotides are analogues containing morpholino- and 2'deoxyribonucleosides joined through thiophosphor internucleotide linkages. These analogues stimulate biological activity in a dual luciferase assay, in exon skipping with Marfan Syndrome and Duschenne Muscular Dystrophy, and in regulating TUG 1 RNA. Current research includes regulating microRNA maturation, editing transcription termination, exon skipping of additional genetic diseases, and antisense experiments with RNase H.



3:30 **We Love Conjugates: Targeting RNA using the GalNAc-Oligonucleotide Conjugates - Approval of the First Conjugate GIVLAARI™**
Mano Manoharan, PhD, Distinguished Scientist & Sr VP Innovation Chem, Innovation Chemistry, Alnylam Pharmaceuticals

3:50 **PANEL DISCUSSION : Live Q&A with Session Speakers**
Moderator: Dmitry Samarsky, PhD, CTO, Sirnaomics
Panelists:
Marvin Caruthers, PhD, Distinguished Professor, University of Colorado
Mano Manoharan, PhD, Distinguished Scientist & Sr VP Innovation Chem, Innovation Chemistry, Alnylam Pharmaceuticals

4:10 **Close of Conference**

Oligonucleotide CMC and Regulatory Strategies

Accelerating Product Development and Commercial Success

TUESDAY, OCTOBER 20

INNOVATIVE CMC AND MANUFACTURING STRATEGIES



10:05 am KEYNOTE PRESENTATION: Reinventing Oligonucleotide Synthesis

Phil S. Baran, PhD, Chair & Professor, Chemistry, Scripps Research Institute

The advent of phosphoramidite-based coupling chemistry and solid-phase organic synthesis democratized oligonucleotide synthesis for the biochemistry community thus paving the way for stunning developments in the field. This talk will present a fundamentally different approach to constructing oligonucleotides containing P(S)2, chiral P(S)O and P(Me)O, and canonical P(O)2 linkages to create chimeric sequences that would be outside the realm of what can be obtained with current methods.

10:25 Learnings from Process Validation of a Full-Scale Oligonucleotide Manufacturing Process

Jesse Faber, Senior Process Engineer III, ASO Process Engineering and Manufacturing, Biogen

In 2016, Biogen began construction of their first ASO manufacturing facility. After the successful manufacture of several clinical oligonucleotides, Biogen has just completed its first process validation of a commercial oligonucleotide in 2019. This presentation will focus on key learnings from the process validation effort including process characterization, risk assessment, and platform strategies.

10:45 Session Break

11:05 PANEL DISCUSSION : Live Q&A with Session Speakers

Moderator: Marc Lemaitre, PhD, Consultant, ML_Consult LLC

Panelists:

Phil S. Baran, PhD, Chair & Professor, Chemistry, Scripps Research Institute

Jesse Faber, Senior Process Engineer III, ASO Process Engineering and Manufacturing, Biogen

11:25 Coffee Break - View Our Virtual Exhibit Hall

PLENARY



11:55 Recent Advances in Antisense Technology at Ionis Pharmaceuticals

Brett P. Monia, PhD, CEO, Ionis Pharmaceuticals, Inc.

The development of antisense technology to treat a broad range of rare and common diseases is now validated. Antisense medicines offer tremendous hope for patients afflicted with serious diseases that cannot be addressed with traditional drug discovery platforms. With numerous antisense medicines recently receiving market authorization, along with a large and diverse pipeline of medicines on the horizon, antisense is poised to revolutionize the practice of medicine for generations.



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Moderator: Dmitry Samarsky, PhD, CTO, Sirnaomics

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- Opportunities of neurological diseases in drug discovery, development and delivery
- Challenges that lie ahead

Panelists:

Brett P. Monia, PhD, CEO, Ionis Pharmaceuticals, Inc.

Edward M. Kaye, MD, CEO, Stoke Therapeutics

1:00 Lunch Break - View Our Virtual Exhibit Hall

INNOVATIVE CMC AND MANUFACTURING STRATEGIES

1:40 Monte Carlo Simulations of Amidite Starting Material Impurity Incorporations for Drug Substance Specification Justifications

Francis Ring, Assistant Director, Manufacturing and Operations, Ionis Pharmaceuticals

For starting material-related product impurities, Ionis supplemented the product impurity data with Monte Carlo simulations of the corresponding starting material impurity. By building the Monte Carlo simulations from the entire amidite production history, significantly more historical variability was captured than any individual Ionis product experienced. The methodology for building the starting material impurity and product incorporation models will be discussed along with the Monte Carlo simulation results.

2:00 Oligonucleotide DP Development and Manufacturing for Orphan Ophthalmic Diseases

Vera Brinks, PhD, Director Pharmaceutics, ProQR Therapeutics

Oligonucleotide drug product (DP) development for (ultra) orphan ophthalmic diseases can be challenging from a formulation, primary packaging and manufacturing point of view. This presentation will elaborate on some of the challenges related to intravitreal (IVT) administered products, such as endotoxin and sub-visible particle specifications of DP, but also dosing accuracy. Also, terminal sterilization as part of DP manufacturing will be discussed.

2:20 Refresh Break - View Our Virtual Exhibit Hall

OPTIMIZING ANALYTICAL APPROACHES



2:50 FEATURED PRESENTATION: Accelerated Stability Assessment of Oligonucleotides

Mark Madsen, PhD, Associate Director, Analytical Development/ Quality Control, Ionis Pharmaceuticals

Development of an accelerated stability assessment program to determine the activation energy required for degradation of antisense oligonucleotides will be presented. The results from these studies enable calculation of drug product stability at the long-term storage conditions and the rates of formation for individual degradation products. Study design, example data, and potential applications will be discussed.

3:10 Modern Statistical Methods for the Data Challenges of C&GT

Tara Scherder, Partner, SynoloStats

Determination of specifications and performance prediction of C&G therapy products can be challenging due to small sample size and complex relationships. Bayesian statistical methods, which can leverage all relevant information, plus allow more flexibility to model complex data relationships, are uniquely suited for these challenges. These methods are used with great success in adaptive clinical trials and can be leveraged in challenging CMC applications to develop more accurate models.

3:30 PANEL DISCUSSION
: Live Q&A with Session Speakers
Moderator: Marc Lemaitre, PhD, Consultant, ML_Consult LLC
Panelists:
Francis Ring, Assistant Director, Manufacturing and Operations, Ionis Pharmaceuticals
Vera Brinks, PhD, Director Pharmaceutics, ProQR Therapeutics
Mark Madsen, PhD, Associate Director, Analytical Development/Quality Control, Ionis Pharmaceuticals
Tara Scherder, Partner, SynoloStats

3:30 Recommended Short Course*

SC1: Examining the Safety and Toxicity of Nucleic Acid Therapeutics
**Separate registration required. See short course page for details.*

3:50 Happy Hour - View Our Virtual Exhibit Hall

4:20 Close of Day

WEDNESDAY, OCTOBER 21

OPTIMIZING ANALYTICAL APPROACHES

10:00 am Industry Case Study from Quark Pharmaceuticals
Anne Oommen, Sr Scientist, Quark Pharmaceuticals Inc
In this presentation, I will be speaking on analytical development considerations from IND to NDA specifically using an siRNA as case study, the challenges during development, optimizations of analytical approaches, associated IND and NDA enabling activities and the evolution of overall quality control strategies. I will also be speaking on the expectations for establishing specifications at various stages, from starting materials through drug substance.

10:20 Phase Appropriate Analytical Control Strategy for Antisense Oligonucleotides
Hong Jiang, Senior Scientist, Analytical Development, Biogen
It is vitally important to develop a phase appropriate analytical control strategy to ensure the safety and efficacy of the product while keeping the drug accessible to patients. Challenges and considerations in developing such a phase appropriate strategy will be discussed. Examples of development and implementation of new technology and methods will be provided.

10:40 Thought Leader Presentation: Method Development for the Characterization of Synthetic Oligonucleotides by LC-MS
, Phenomenex

11:00 PANEL DISCUSSION: Live Q&A with Session Speakers
Moderator: Paul Manley, President & Principal Consultant, Orvieto Consulting, LLC
Panelists:
Anne Oommen, Sr Scientist, Quark Pharmaceuticals Inc
Hong Jiang, Senior Scientist, Analytical Development, Biogen
, Phenomenex

11:20 Coffee Break - View Our Virtual Exhibit Hall

CRITICAL FEEDBACK ON REGULATORY SUBMISSIONS

11:50 Practical, Quality and Regulatory CMC Considerations to Manufacture Clinical Trial Materials for Early Phase. What Do You Really Need to Do?
Kevin Fettes, PhD, Consultant and Founder, FTS Pharma Consulting
The complexity of oligonucleotide drug candidates being selected for clinical development has increased in recent years. These oligonucleotides often have significant chemical modifications requiring novel starting materials as well as technical innovations in process development, analytical chemistry, manufacturing and controls. This places extraordinary demands on both sponsor companies and contract manufacturing organizations to meet regulatory expectations under aggressive timelines.

12:10 pm Submitting Your First Investigational New Drug (IND) Application: A Roadmap of Key Activities
Paul Manley, President & Principal Consultant, Orvieto Consulting, LLC
An IND application can be daunting for a small company making such a submission for the first time. This presentation will discuss key activities to consider as you plan for this important FDA interaction, including: Pre-IND dialogue, project plans, use of internal and external resources, creation of your electronic submission and the IND review process.

12:30 Breakout Roundtable Discussions

BREAKOUT: Nucleic Acid-Based Therapies for Prevention and Treatment of COVID-19: Pros and Cons
David Tabatadze, PhD, President, ZATA Pharmaceuticals, Inc.

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Steven Kates, PhD, Vice President, Regulatory Affairs, Dicerna Pharmaceuticals

BREAKOUT: Building a Robust, Targeted-Centered Development Program
David Corey, PhD, Professor, Department of Pharmacology, UT Southwestern

1:15 Lunch Break - View Our Virtual Exhibit Hall

2:00 CMC for Oligonucleotides and the Regulatory Landscape
Marc Lemaitre, PhD, Consultant, ML_Consult LLC
Development of oligonucleotide therapeutics is generally well understood with more than 10 approved molecules. However, it's not yet a routine. In this presentation, we will address the specificities that need to be understood to avoid issues and delay when receiving questions from authorities. We will also try to explain how to manage tight timelines.

Oligonucleotide CMC and Regulatory Strategies

Accelerating Product Development and Commercial Success

2:20 LIVE PANEL DISCUSSION: How to Successfully Prepare for a Regulatory Submission and Overcome Common Hurdles

Moderator: Paul Manley, President & Principal Consultant, Orvieto Consulting, LLC

- Challenges: Manufacturing development history for regulatory filings
- Global dossiers: FDA, EMA, Japan, Brazil and Canada
- Stability data: What was permitted and what was not?

Panelists:
Steven Kates, PhD, Vice President, Regulatory Affairs, Dicerna Pharmaceuticals
Kevin Fettes, PhD, Consultant and Founder, FTS Pharma Consulting
Marc Lemaitre, PhD, Consultant, ML_Consult LLC

2:40 Refresh Break - View Our Virtual Exhibit Hall

CLOSING PLENARY



3:10 Biological Activity of Thiomorpholino Oligonucleotides

Marvin Caruthers, PhD, Distinguished Professor, University of Colorado

Thiomorpholino oligonucleotides are analogues containing morpholino- and 2'deoxyribonucleosides joined through thiophosphor internucleotide linkages. These analogues stimulate biological activity in a dual luciferase assay, in exon skipping with Marfan Syndrome and Duschenne Muscular Dystrophy, and in regulating TUG 1 RNA. Current research includes regulating microRNA maturation, editing transcription termination, exon skipping of additional genetic diseases, and antisense experiments with RNase H.



3:30 We Love Conjugates: Targeting RNA using the GalNAc-Oligonucleotide Conjugates - Approval of the First Conjugate GIVLAARI™

Mano Manoharan, PhD, Distinguished Scientist & Sr VP Innovation Chem, Innovation Chemistry, Alnylam Pharmaceuticals

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Panelists:
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Mano Manoharan, PhD, Distinguished Scientist & Sr VP Innovation Chem, Innovation Chemistry, Alnylam Pharmaceuticals

4:10 Close of Conference

> "Well balanced content, perfectly executed logistics."
>
> ***Dmitry Samarsky, PhD, CTO, Sirnaomics***

> "The scientific quality and value of the presentations was very high. The opportunities for networking have been excellent. I very much enjoyed attending the conference."
>
> ***Ekkehard Leberer, PhD, Senior Director, R&D Alliance Management, Sanofi***

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October 20, 2020
SC1: Examining the Safety and Toxicity of Nucleic Acid Therapeutics

CONFERENCE PRICING

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Standard Rate after October 2, 2020	$1195	$645

Premium POST-EVENT ON-DEMAND Conference Pricing (Includes access to ALL recorded conference and short course presentations only for one year. Does not include Q&A sessions or networking)

Standard Rate after October 2, 2020	$1025	$555

Standard REAL-TIME VIRTUAL Conference Pricing (Includes Real-Time access to both OPT Congress virtual conference streams, event features and On-Demand access for one year. You are allowed to move between virtual sessions to attend presentations taking place at the same time. This Package excludes the Short Course.)

Standard Rate after October 2, 2020	$1099	$545

Standard POST-EVENT ON-DEMAND Conference Pricing (Includes Post-Event access to both conference presentation recordings only for one year. Does not include short courses, Q&A sessions or networking.)

Standard Rate after October 2, 2020	$875	$495

October 20, 2020	October 21, 2020
C1: Oligonucleotide Discovery and Delivery	C1: Oligonucleotide Discovery and Delivery
C2: Oligonucleotide CMC and Regulatory Strategies	C2: Oligonucleotide CMC and Regulatory Strategies

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