Final Weeks to Register!

6th ANNUAL

OPT Congress

MARCH 30 - 31, 2021 | ALL TIMES EDT

Oligonucleotide & Precision Therapeutics

VIRTUAL

DISCOVERY ■ DEVELOPMENT ■ DELIVERY

CONFERENCE PROGRAMS:

DAY 1 MARCH 30

Oligonucleotide Discovery and Delivery

Short Course: Examining the Safety and Toxicity of Nucleic Acid Therapeutics

DAY 2 MARCH 31

Oligonucleotide CMC and Regulatory Strategies

CHOOSE FROM A 1- OR 2-DAY PASS

KEYNOTE SPEAKERS:



Melissa J. Moore, PhD
CSO, Moderna



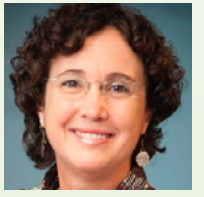
Muthiah (Mano) Manoharan, PhD
Senior Vice President, Drug Discovery, Alnylam Pharmaceuticals



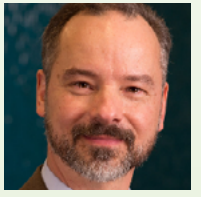
Richard S. Geary, PhD
Executive Vice President (EVP), Drug Development, Ionis Pharmaceuticals



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



Laura Sepp-Lorenzino, PhD
CSO, Intellia Therapeutics



Bob Brown, PhD
CSO and Executive Vice President R&D, Dicerna Pharmaceuticals

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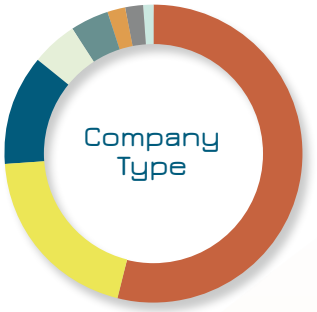
Event Testimonials

The virtual nature actually helped Q&A sessions to be more inclusive with varied content.
- Paul Manley, Orvieto Consulting

This virtual event makes me feel connected to the community. It was good to catch up with previous colleagues. People's resilience in difficult times inspired me. Thank you!
-Xiao Hu, Wave Life Sciences

It was a very well-managed virtual conference.
-Tara Scherder, SynoloStats

2020 Attendee Demographics



Biotech	54%
Pharma	20%
Academic	12%
Services	5%
Healthcare	4%
Government	2%
Financial	2%
Societies	1%



USA	79%
Asia	10%
Europe	7%
Rest of World	4%

US Breakdown

East Coast	68%
West Coast	21%
Midwest	11%



Executive +Director	40%
Scientist/Technologist	34%
Sales & Marketing	12%
Manager	6%
Professor	6%
Assistant	2%

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Dmitry Samarsky, PhD
CTO, Sirnaomics



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Senior Vice President, Drug Discovery, Alnylam Pharmaceuticals



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Lubo Nechev, PhD
Vice President, Process & Analytical Sciences, Alnylam Pharmaceuticals



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

Oligonucleotide Discovery and Delivery

TUESDAY, MARCH 30

KEYNOTE SESSION: GALNAC TECHNOLOGY, ANTISENSE AND GENOME EDITING



9:30 am Living in the World of RNA Therapeutics Using the GalNAc Conjugates: What Is New?
Muthiah (Mano) Manoharan, PhD, Senior Vice President, Drug Discovery, Alnylam Pharmaceuticals
GIVLAARI® (2019), OXLUMO® (2020) and LEQVIO®* (2020) are recently approved GalNAc conjugated RNAi therapeutics. This presentation will point out the molecular basis of their success, and will further illustrate other evolving therapies like Vutrisiran, ALN-AGT and others along with our improvements based on a rational design of siRNAs using chemical modifications.



9:50 RNA Targeting with Antisense Oligonucleotides: Present and Future
Richard S. Geary, PhD, Executive Vice President, Drug Development, Ionis Pharmaceuticals
The number and chemical breadth of antisense oligonucleotide (ASO) medicines have rapidly grown in both clinical development and in market approvals. The ASOs currently in development are well balanced between rare and broad diseases and represent significant innovations. Some of the unique aspects associated with single strand ASO chemistry, distribution, cell uptake, clinical pharmacology and safety will be discussed as applied to clinical development examples and overall research progress.

10:10 Synthesis of transcriptional activation CRISPR-Cas9 reagents for gene function and network screening
horizon INSPIRED CELL SOLUTIONS
Amanda Haas, Custom Synthesis Product Manager, Horizon Discovery
Oligonucleotide-directed targeting to regulate gene function has enabled genome-wide studies and extended to the hope of targeting any gene. We will discuss the optimization of guide RNA synthesis for CRISPR activation (CRISPRa) which requires substantially longer oligonucleotides. CRISPRa is the most promising new technology for overexpression, synthesis of CRISPRa guides has allowed rapid evaluation of large gene families.



10:30 Transforming Medicine: The Power and Promise of Genome Editing for Rare and Genetic Diseases
Laura Sepp-Lorenzino, PhD, CSO, Intellia Therapeutics
Our systemic lipid nanoparticle or LNP-based delivery system has the potential to unlock treatment of genetic diseases by selectively knocking out disease-causing genes, introducing targeted insertion of a functional gene or both. The presentation will focus on Intellia's *in vivo* platform and pipeline, highlighting the paths to gRNA selection, preclinical pharmacology and the clinic.



10:50 Recent Advances with GalNAc Technology
Bob Brown, PhD, CSO and Executive Vice President R&D, Dicerna Pharmaceuticals

11:25 KEYNOTE LIVE PANEL DISCUSSION: Q&A with Session Speakers
Moderator: Laura Sepp-Lorenzino, PhD, CSO, Intellia Therapeutics
Panelists:
Muthiah (Mano) Manoharan, PhD, Senior Vice President, Drug Discovery, Alnylam Pharmaceuticals
Richard S. Geary, PhD, Executive Vice President, Drug Development, Ionis Pharmaceuticals
Amanda Haas, Custom Synthesis Product Manager, Horizon Discovery
Bob Brown, PhD, CSO and Executive Vice President R&D, Dicerna Pharmaceuticals

11:55 Session Break - View Our Virtual Exhibit Hall

12:25 Breakout Session
Join your colleagues and fellow delegates for a focused, informal discussion moderated by a member of our speaking faculty. A small group format allows participants to meet potential collaborators, share examples from their own work and discuss ideas with peers.

BREAKOUT SESSION: Implementation of AI and Machine Learning in Oligonucleotide Design
Anastasia Khvorova, PhD, Professor, RNA Therapeutic Institute, University of Massachusetts Medical School

OPTIMIZING DESIGN, DELIVERY AND PERFORMANCE

1:20 siRNA Chemical Engineering for Extrahepatic Delivery
Anastasia Khvorova, PhD, Professor, RNA Therapeutic Institute, University of Massachusetts Medical School
RNAi enables simple and specific modulation of gene expression when the chemical architecture supporting efficient *in vivo* delivery is defined. Using huntingtin – the causative gene in Huntington disease – as a model, we demonstrate that chemically engineered siRNAs induce potent protein silencing (> 99%) in all brain regions tested one month post injection. Further chemical optimization enables broad distribution and silencing in large-brain animal models.

1:40 MicroRNA Therapeutics: Targeting the Pathways of Human Disease
Ekkehard Leberer, PhD, Senior Director, R&D Alliance Management, Sanofi; Scientific Managing Director, COMPACT Consortium
MicroRNAs are short non-coding RNAs that regulate biochemical pathways by RNA interference (RNAi). Dysregulation of microRNAs is associated with many diseases. MicroRNA-21 has been implicated with fibrotic diseases and cancer. The presentation shows the development of an anti-fibrotic antisense oligonucleotide (anti-miR-21) for the treatment of a genetic fibrotic kidney disease called Alport Syndrome, and summarizes the potential of anti-miR-21 as a drug to treat hepatocellular carcinoma.

Oligonucleotide Discovery and Delivery

2:00 Sample Preparation Strategies for Oligonucleotide Bioanalytical Workflows

Ivan Lebedev, Application Scientist, Product Management and Strategy, Phenomenex

We will give an overview of sample preparation strategies for synthetic oligonucleotides, including how to adjust experimental design depending on the oligo and sample matrix. We will we go in depth into the parameters to adjust to ensure an effective sample prep and cleanup is implemented and approaches for other challenging oligos which are unique in that they do not have the negatively charged backbone associated with other nucleic acid chemistries.

2:20 A Novel, RNA-Based Anti-Viral Approach to Limit BK Virus Propagation in Immunocompromised Patients

Bianca Matthee, Chief Operating Officer, Hybridize Therapeutics

There are currently no treatments for combating BK virus. As such, novel treatment strategies to limit BKV reactivation are vital. Hybridize has developed an innovative RNA-based strategy to combat BKV-mediated disease and in this presentation we will show our approach in abrogating viral DNA replication as well as capsid protein formation.

2:40 Treating Diseases with Antibody Oligonucleotide Conjugates: Combining the Selectivity of Antibodies with the Specificity of Oligonucleotide Drugs

Arthur A. Levin, PhD, CSO, Avidity Biosciences

Antibody oligonucleotide conjugates (AOCs™) deliver oligonucleotide therapeutics to multiple cell and tissue types, expanding the therapeutic utility of oligos by combining selective binding of antibodies and specificity of oligonucleotides. Design and engineering AOCs require optimization of the antibody, the oligonucleotide, and the linkers. Taking each of the optimized parts and then optimizing them has enabled us to deliver AOCs to multiple cells and tissues using multiple cell surface-mAb pairs.

3:00 Novel Vaccine Delivery Platforms

Ana Jaklenec, PhD, Research Scientist, Langer Lab, MIT

Innovative vaccine delivery technologies will be presented including single-injection self-boosting vaccines, 3D printed on-demand micro-needle mRNA vaccines and on patient medical records using invisible dyes.

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

Moderator: Dmitry Samarsky, PhD, CTO, Sirnaomics

Panelists:

Anastasia Khvorova, PhD, Professor, RNA Therapeutic Institute, University of Massachusetts Medical School

Ekkehard Leberer, PhD, Senior Director, R&D Alliance Management, Sanofi; Scientific Managing Director, COMPACT Consortium

Ivan Lebedev, Application Scientist, Product Management and Strategy, Phenomenex

Bianca Matthee, Chief Operating Officer, Hybridize Therapeutics

Arthur A. Levin, PhD, CSO, Avidity Biosciences

Ana Jaklenec, PhD, Research Scientist, Langer Lab, MIT

4:00 OPT Congress Connects

4:30 SC1: Examining the Safety and Toxicity of Nucleic Acid Therapeutics

Premium REAL TIME VIRTUAL Pricing or separate registration required. See short course page for details.

6:00 Close of Oligonucleotide Discovery and Delivery

> For anyone who is responsible for discovering and developing oligonucleotide therapeutics, OPT Congress provides unmatched content related to R&D, CMC, Regulatory Affairs, & Business Strategy. Despite the virtual format, the technical sessions were of top-notch quality & facilitated a lively exchange of ideas. I look forward to attending OPT in person, which I expect to be as enlightening as the virtual event, if not more so.
>
> **- Lael Cheung, Bachem Americas Inc.**

Oligonucleotide CMC and Regulatory Strategies

WEDNESDAY, MARCH 31

KEYNOTE SESSION: MODERNA'S mRNA COVID-19 VACCINE AND INNOVATIVE OLIGONUCLEOTIDE SYNTHESIS



9:30 am The Backstory of Moderna's COVID-19 Vaccine
Melissa J. Moore, PhD, CSO, Moderna



9:50 Rethinking the Synthesis of Oligonucleotides
Phil S. Baran, PhD, Chair & Professor, Chemistry, Scripps Research Institute
A new platform based on P(V), amenable to automated oligonucleotide synthesis, will be unveiled for the preparation of long oligo sequences with any desired connectivity (phosphodiester, diastereomerically pure phosphorothiolate, phosphorodithiolate) and nearly any type of nucleoside (DNA, LNA, MOE) that shows compelling advantages over the current state of the art P(III)-based technology.

10:10 Biocatalytic strategies towards Oligonucleotide synthesis
Jill Caswell, PhD, Biology Team Leader, Almac Group
Biocatalysis has transformed small molecule API chemical synthesis by shortening routes, lowering waste & reducing costs. Enzyme applications are now being exploited in oligo synthesis. This presentation showcases enzymes used in single & double-stranded oligo synthesis. This technology base can be applied to develop enzymatic methods for oligo synthesis resulting in improved yields & purity profiles compared to traditional methods.

OPTIMIZING ANALYTICAL CHARACTERISATION

10:30 Identifying the Best Analytical Methods for Characterizing Impurities
Daniel Capaldi, PhD, Vice President, Analytical and Process Development, Ionis Pharmaceuticals
The identification of product-related impurities in drug substances and products is an important aspect of development that impacts analytical methodology, stability testing, toxicology, specifications and manufacturing process optimization. Using real examples, the presentation will highlight analytical methods such as liquid chromatography-electrospray mass spectrometry and 1D and 2D NMR spectroscopy for the detection, quantification and characterization of product-related impurities in oligonucleotide therapeutics.

ACCELERATING TIME TO MARKET

10:50 Speed to Market with Computational Advances in Designed Experiments
Tara Scherder, Partner, SynoloStats
Computational advancements have led to new classes of designs with desirable properties such as reduction in the number of experiments, flexibility to assign varying focus to factors and adequate precision, even in the presence of second order terms. In this talk, examples of the advantages of these designs over classical designs will be shown, along with common mistakes made for all classes of designed experiments.

11:20 LIVE PANEL DISCUSSION: Q&A with Session Speakers
Moderator: Tara Scherder, Partner, SynoloStats
Panelists:
Melissa J. Moore, PhD, CSO, Moderna
Phil S. Baran, PhD, Chair & Professor, Chemistry, Scripps Research Institute
Jill Caswell, PhD, Biology Team Leader, Almac Group
Daniel Capaldi, PhD, Vice President, Analytical and Process Development, Ionis Pharmaceuticals

11:50 Session Break - View Our Virtual Exhibit Hall

12:20 Breakout Session
Join your colleagues and fellow delegates for a focused, informal discussion moderated by a member of our speaking faculty. A small group format allows participants to meet potential collaborators, share examples from their own work and discuss ideas with peers.

BREAKOUT SESSION: Reducing Costs and Improving the Efficiency of Manufacturing Processes
Phil S. Baran, PhD, Chair & Professor, Chemistry, Scripps Research Institute
- Biggest hurdles to manufacturing oligonucleotide therapeutics (solvent quantites, reagent costs, machinery)
- Novel technologies on the horizon, for example: 1. Solution phase/solid phase hybrid approaches, 2. Pure solid phase approaches with new linkers, 3. Solution phase approaches with soluble resins, 4. Flow chemistry
- Managing impurities, future FDA regulations on stereopure vs. stereorandom ASOs
- The prospect of P(III)-free approaches

BREAKOUT SESSION: Feedback on Successful IND and NDA Submissions
Kim Tyndall, President, CMC Tyndall Consultant

Oligonucleotide CMC and Regulatory Strategies

CRITICAL FEEDBACK ON REGULATORY SUBMISSIONS

1:15 Considerations for Oligonucleotide Submissions using M4Q
James V. McArdle, PhD, President, McArdle & Associates, LLC
This presentation will provide suggestions on organizing the effort to prepare a clinical trial application and maintaining that dossier through to the marketing application. The presentation will also provide insight into certain sections of M4Q to aid in understanding the content expected in those sections.

1:35 Current CMC Regulatory Experiences and Expectations for Oligonucleotides
René Thürmer, PhD, Deputy Head, Pharmaceutical Biotechnology, BfArM
This presentation will provide an overview about the regulatory landscape for oligonucleotides. Issues related to the pharmaceutical quality of oligonucleotides are highlighted and experiences from recent regulatory submissions will be discussed.

1:55 Sponsored Presentation *(Opportunity Available)*

2:15 Preparing an IND: CMC Part – Phase-Appropriate Development
Marc Lemaitre, PhD, Consultant, ML_Consult LLC
Leveraging on real-world cases, this presentation will provide possible strategies for the management of CMC operations towards successful global development/registration of oligonucleotide therapeutics and for addressing the regulatory expectations for assuring quality and performance in a phase appropriate way.

2:45 LIVE PANEL DISCUSSION: Q&A with Session Speakers
Moderator: Marc Lemaitre, PhD, Consultant, ML_Consult LLC
Panelists:
René Thürmer, PhD, Deputy Head, Pharmaceutical Biotechnology, BfArM
James V. McArdle, PhD, President, McArdle & Associates, LLC
Kim Tyndall, President, CMC Tyndall Consultant

3:15 OPT Congress Connects

3:45 Close of Oligonucleotide CMC and Regulatory Strategies

> OPT Congress was a great conference. I can tell a lot of hard work was involved. The on-demand presentations are great!
>
> **-Bob Wu, Gilead Sciences**

EVENT-AT-A-GLANCE

TUESDAY, MARCH 30, 2021

Oligonucleotide Discovery and Delivery

Short Course: Examining the Safety and Toxicity of Nucleic Acid Therapeutics

WEDNESDAY, MARCH 31, 2021

Oligonucleotide CMC and Regulatory Strategies

SHORT COURSE

SC1: Examining the Safety and Toxicity of Nucleic Acid Therapeutics

Instructors:

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

John Davis II, PhD, Vice President, Head of Preclinical Development, Dyne Therapeutics

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

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.

Benefits of OPT Congress — Oligonucleotide & Precision Therapeutics — VIRTUAL

- **VIRTUAL CONFERENCE** accessible from the comfort of your home or office
- **INSPIRING KEYNOTE PRESENTATIONS** from world-renowned experts and visionaries
- **INTERACTIVE NETWORKING** with live Q&A sessions, breakout discussions, poster viewing, live chat with exhibitors, sponsors, and fellow delegates
- **RESEARCH POSTERS** engage with researchers presenting the latest in oligonucleotide discovery, development and delivery
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SHORT COURSE ONLY PRICING

	Commercial	Academic, Government, Hospital-affiliated
One short course	$349	$149

March 30, 2021
SC1: Examining the Safety and Toxicity of Nucleic Acid Therapeutics

CONFERENCE PRICING

Premium REAL-TIME VIRTUAL Conference Pricing *BEST VALUE* (Includes real-time access to both OPT conference days & event features, and On-Demand access for one year. PLUS! Includes Access to Short Course.)

Advance Rate until March 12	$1099	$599
Standard Registration after March 12	$1199	$699

Standard REAL-TIME VIRTUAL Conference Pricing (Includes access to one conference day, virtual event features, and On-Demand access. Please select the date and conference you plan to attend. Package excludes short courses.)

Advance Rate until March 12	$899	$449
Standard Registration after March 12	$999	$549

March 30, 2021	March 31, 2021
C1: Oligonucleotide Discovery and Delivery	C2: Oligonucleotide CMC and Regulatory Strategies

CONFERENCE DISCOUNTS

POSTER SUBMISSION - DISCOUNT ($50 OFF): Poster materials are due by March 5, 2021. Once your registration has been fully processed, we will send an email containing a unique link and instructions for submitting your abstract and other materials. If you do not receive your link within 5 business days, please contact jring@healthtech.com. **This discount does not apply to product or service providers"*

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