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MARCH 13 - 14, 2023 REVERE HOTEL BOSTON COMMON | BOSTON, MA & VIRTUAL

OPT Congress

8th Annual

Oligonucleotide & Precision Therapeutics

IN-PERSON ■ VIRTUAL
FLEXIBLE REGISTRATION

DISCOVERY ■ DEVELOPMENT ■ DELIVERY

PLENARY SPEAKERS



Marvin Caruthers, PhD, Distinguished Professor, University of Colorado



Rubina Parmar, PhD, Vice President, Chemistry & Delivery Sciences, Intellia Therapeutics, Inc.



Arthur Levin, PhD, CSO, Avidity Biosciences



Daniel Anderson, PhD, Professor, Chemical Engineering, Massachusetts Institute of Technology



Mano Manoharan, PhD, Distinguished Scientist & Senior Vice President, Innovation Chemistry, Alnylam Pharmaceuticals



Steve Pascolo, PhD, Senior Scientist, University Hospital of Zurich; Founder & CEO, Miescher Pharma GmbH

2023 Conference Programs

Oligonucleotide Discovery and Delivery

Oligonucleotide CMC and Regulatory Strategies

Emerging mRNA Therapeutics NEW

SHORT COURSE
Safety and Toxicity of Nucleic Acids

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Welcome to OPT Congress

OPT Congress is the premier conference for professionals involved in discovering and developing oligonucleotide therapeutics. Now in its 8th year, this unique event brings together leading discovery scientists, developers, CMC experts, regulatory specialists, and technology providers to discuss advances in next-generation oligonucleotide and precision therapeutics throughout the drug development process. New this year, we are delighted to announce a third conference program dedicated to Emerging mRNA Therapeutics to complement our established Oligo Discovery & Delivery and CMC & Regulatory programs. In addition to 2 days of inspiring keynotes, interactive discussions and more than 55 scientific presentations, we deliver an in-depth course focusing on the Safety & Toxicity of Nucleic Acids. Join your peers and industry experts as together we optimize drug design, delivery and performance, accelerate time to market and achieve commercial success.

EVENT-AT-A-GLANCE

DAY 1 MONDAY MARCH 13TH

Short Course 1

Oligonucleotide Discovery & Delivery	Oligonucleotide CMC & Regulatory Strategies	Emerging mRNA Therapeutics

Plenary Session

DAY 2 TUESDAY MARCH 14TH

Oligonucleotide Discovery & Delivery	Oligonucleotide CMC & Regulatory Strategies	Emerging mRNA Therapeutics

Plenary Session

WITH THANKS TO OUR EXECUTIVE ADVISORY BOARD



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



Arthur Levin, PhD
Executive Vice President, Research and Development, Avidity Biosciences



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



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



Dmitry Samarsky, PhD
CTO, Sirnaomics

Plenary and Featured Presentations



Biological Studies with Thiomorpholino Oligonucleotides
Marvin Caruthers, PhD, Distinguished Professor, University of Colorado



Advances in CRISPR Genome Editing for Therapeutics Application
Rubina Parmar, PhD, Vice President, Chemistry & Delivery Sciences, Intellia Therapeutics, Inc.



Recent Developments in Oligo Conjugates
Arthur Levin, PhD, CSO, Avidity Biosciences



Nucleic Acid Delivery Systems for RNA Therapy and Genome Editing
Daniel Anderson, PhD, Professor, Chemical Engineering, Massachusetts Institute of Technology



Advances in Chemistry Made RNAi Therapeutics Possible
Mano Manoharan, PhD, Distinguished Scientist & Senior Vice President, Innovation Chemistry, Alnylam Pharmaceuticals



Development of mRNA-Based Vaccines
Steve Pascolo, PhD, Senior Scientist, University Hospital of Zurich; Founder & CEO, Miescher Pharma GmbH



GalAhead: A Novel Therapeutic GalNAc-RNAi Platform to Downregulate Single and Multiple Genes
Dmitry Samarsky, PhD, CTO, Sirnaomics



Finding the Needle in the miRNA Haystack: Rethinking the miRNA Discovery Platform
David Corey, PhD, Professor, Department of Pharmacology, UT Southwestern



Robust Gene Silencing in the Brain and Lung
Jonathan Watts, PhD, Associate Professor, RNA Therapeutics Institute, University of Massachusetts Chan Medical School



Developing Manufacturing and Quality Control Strategy for siRNA Therapeutics
Lubomir Nechev, PhD, Senior Vice President, CMC Development, Alnylam Pharmaceuticals



Strategies for Optimizing Performance of the Ionis Platform Method for Determination of Assay, Purity, and Impurity Profile of Oligonucleotide Therapeutics Using IP-HPLC-UV-MS
Kimmy Kolkey, Associate Director, Analytical Development and Quality Control, Ionis Pharmaceuticals



Novel RNAs for Treating Cancer
Mark Kay, MD, PhD, Dennis Farrey Family Professor of Pediatrics and Professor of Genetics, Department of Pediatrics and Genetics, Stanford University



Innovations in mRNA Therapeutics
Drew Weissman, MD, PhD, Roberts Family Professor in Vaccine Research, Department of Infectious Diseases; Director of the Penn Institute for RNA Innovation and Professor, Department of Medicine, University of Pennsylvania

Your Safety Is Our Top Priority

To ensure maximum safety, CHI has instituted mandatory health and safety protocols for all attendees, exhibitors, speakers and staff who attend in person. Attendees who cannot participate because of this policy, or due to travel restrictions, are encouraged to participate using our highly praised virtual event platform. Our virtual events are designed to provide you with an in-person experience at your convenience, anywhere, anytime. We are actively following news and recommendations around COVID-19 and the Omicron variant. These protocols are subject to change as we continue to learn more.

ALL IN-PERSON ATTENDEES *MUST*:

Have a negative COVID-19 test result from an FDA-authorized, a WHO-authorized, or European authorized over-the-counter antigen test within 24 hours prior to arriving at the event. You will be asked your results at registration.

CHI *RECOMMENDS* ALL ATTENDEES:

Have an updated COVID-19 vaccination and wear a mask in public spaces at the event.

Oligonucleotide Discovery and Delivery

Optimizing Design, Delivery and Performance

MONDAY, MARCH 13

9:15 am Main Conference Registration (Gallery Foyer)

10:40 Welcome Remarks by Conference Organizer

ROOM LOCATION: Singlo
GalNAc-RNAi THERAPEUTIC PLATFORM

10:50 Chairperson's Opening Remarks

Chandra Vargeese, PhD, CTO & Head, Platform Discovery Sciences, Wave Life Sciences



10:55 FEATURED PRESENTATION: GalAhead: A Novel Therapeutic GalNAc-RNAi Platform to Downregulate Single and Multiple Genes

Dmitry Samarsky, PhD, CTO, Sirnaomics

Sirnaomics has developed a proprietary GalNAc-RNAi therapeutic platform, GalAhead, comprising two key technological components – mxRNA (miniaturized RNAi triggers) and muRNA (multi-unit RNAi triggers). We will present data validating these two technologies both *in vivo* and *in vitro*, as well as a progress report on quickly expending GalAhead therapeutic pipeline.

TRANSLATION TO THE CLINIC

11:25 Impact of Phosphoryl Guanidine on Oligonucleotide Pharmacology: Translation to the Clinic

Chandra Vargeese, PhD, CTO & Head, Platform Discovery Sciences, Wave Life Sciences

We have applied phosphoryl guanidine backbone chemistry (PN chemistry) to oligonucleotides. Similar to the phosphorothioate (PS) backbone, PN is a chiral modification, so we apply it as part of our stereopure platform. The application of PN chemistry has improved the pharmacological properties of our stereopure molecules in preclinical studies across oligonucleotide modalities. Early data from our ongoing clinical trials suggests these improved pharmacological properties are translating into the clinic.

11:55 FORCE Platform for Delivery of Oligonucleotides for the Treatment of DM1 and DMD: Moving from Bench to Clinic with DYNE-101 and DYNE-251

Oxana Beskrovnaya, PhD, CSO, Dyne Therapeutics

Data from Duchenne muscular dystrophy (DMD) and myotonic dystrophy type 1 (DM1) preclinical models demonstrate that administration of DYNE-251 and DYNE-101, respectively, results in delivery of oligonucleotides directly to cardiac and skeletal muscle. DYNE-251 and DYNE-101 are expected to begin patient dosing in Phase I/II clinical trials in mid-2022.

12:25 pm Control and Mitigation of Endotoxin Contamination in Single Stranded Oligonucleotides

Chad Ratterman, Process Development, Process Development, Nitto Avecia

As the list of oligonucleotide therapeutics targeting CNS diseases grows, control of bacterial endotoxincontamination within the API has become critical.Endotoxin can be found on almost every surface andinmanyraw materialswithin the laboratory environment and cannot be eliminated via standard sterilizationmethods. Therefore, control of endotoxin contamination within the API is largely a matter of avoidingintroduction,which requires precautions begin early and continue throughout the manufacturing process.However, unforeseen complications can allow for contamination to occur despite the most rigid ofprecautions. Nitto Avecia has developed a processforresolvingendotoxin contamination withinlyophilized API allowing for rescue of non-conforming batches. This talk will focus on effective controlstrategiesfor endotoxin contamination andoutline a simpleand robust chromatographicpost-hocmitigationprocedure.

12:55 Grand Opening Dessert Break in the Exhibit Hall with Poster Viewing (Bohea)

RECENT ADVANCES WITH RNAs

1:35 Chairperson's Remarks

Dmitry Samarsky, PhD, CTO, Sirnaomics



1:40 FEATURED PRESENTATION: Finding the Needle in the miRNA Haystack: Rethinking the miRNA Discovery Platform

David Corey, PhD, Professor, Department of Pharmacology, UT Southwestern

Human miRNAs have the potential to be versatile agents for regulating gene expression. Connecting a miRNA first to recognition of mRNA sequences and then to control of specific cellular functions is more difficult than has been widely assumed, slowing therapeutic applications. Here I describe approaches to find out when and where miRNAs are most likely to play important roles in disease.

2:10 Expanding Oligonucleotide Target Space through RNA Editing

Paloma H. Giangrande, PhD, Vice President, Platform and Discovery Sciences Biology, Wave Life Sciences

Our first RNA editing program was launched to correct a disease-causing, protein-coding mutation in SERPINA1 mRNA. RNA editing is a versatile technology, with enormous opportunity to address diseases with unmet need. We will provide an update on our preclinical efforts to expand the target space for this new technology, including modulating protein-protein interactions and upregulating gene expression.

2:40 Choosing the Right GMP Partner for Your CRISPR-Enabled Cell and Gene Therapy

Kevin Holden, PhD, Head of Science, Synthego

Synthego is a genome engineering company that supports the development of CRISPR-based cell and gene therapies, from discovery to first in human clinical trials. We present our approach to supporting scientists in both industry and academia with high quality CRISPR reagents for their preclinical research work, through IND-enabling studies, and into the clinic. In addition, we will showcase our new 18,000 sq. ft. GMP facility that opens in mid-2023.

2:55 Bridging Oligo and Two Ligands: A Facile Dual-Conjugation Strategy Enabling Rapid Sequence/Ligand Screening

Jun Zhou, PhD, Director, Oligonucleotide Discovery Chemistry, WuXi STA

Ligand conjugation opens up opportunities to deliver oligonucleotide to a specific cell or tissue and to fine-tune the 'drug-like' properties. However, the complexity of conjugation chemistry poses significant challenges to screen ligands for ASO/siRNA, in particular when linking to two different ligands. We've investigated numerous orthogonal linker chemistries and identified a facile dual-conjugation strategy that accommodates rapid sequence/ligand screening in plate format.

3:10 Refreshment Break in the Exhibit Hall with Poster Viewing (Bohea)

ROOM LOCATION: Singlo
PLENARY SESSION

3:50 Plenary Chairperson's Remarks

Dmitry Samarsky, PhD, CTO, Sirnaomics



3:55 PLENARY PRESENTATION: Nucleic Acid Delivery Systems for RNA Therapy and Genome Editing

Daniel Anderson, PhD, Professor, Chemical Engineering, Massachusetts Institute of Technology

Here we describe our work developing nanoformulations for RNA therapy and genome editing. Libraries of degradable polymers and lipid-like materials have been synthesized, formulated, and screened for

their ability to deliver RNA payloads inside cells. These nanoformulations facilitate *in vivo* delivery to a range of tissues and can enable targeted gene suppression with siRNA, gene expression with mRNA, or even permanent genetic editing using the CRISPR/Cas9 system.



4:25 PLENARY PRESENTATION: Advances in CRISPR Genome Editing for Therapeutics Application

Rubina Parmar, PhD, Vice President, Chemistry & Delivery Sciences, Intellia Therapeutics, Inc.

At Intellia, we are building a full-spectrum genome editing company. We are deploying the industry's broadest and deepest toolbox, including novel editing and delivery solutions, to harness the immense power of CRISPR-based technologies for *in vivo* and *ex vivo* therapeutic applications, each with the potential to revolutionize the future of medicine. In this presentation, we will share the advances in the therapeutic application of CRISPR/Cas9 for genome editing.



4:55 PLENARY PRESENTATION: Recent Developments in Oligo Conjugates

Arthur Levin, PhD, CSO, Avidity Biosciences

The promise of oligonucleotide therapeutics is to use Watson-Crick-Franklin base-pairing rules to design drugs directly and rationally based on genomic information. Until recently, that promise has remained elusive because of cell barriers to oligonucleotide uptake. Avidity's AOC technology uses monoclonal antibodies to cell surface proteins that are internalized in order to facilitate the functional delivery of oligonucleotide therapeutics into a broad range of cell and tissue types.

5:25 Welcome Reception in the Exhibit Hall with Poster Viewing (Bohea)

6:30 Close of Day

TUESDAY, MARCH 14

7:45 am Registration and Morning Coffee (Gallery Foyer)

ROOM LOCATION: Singlo
RECENT ADVANCES WITH RNAs

8:30 Chairperson's Remarks

Radhakrishnan Iyer, PhD, CSO, RIGImmunce, Inc.

8:35 Asymmetric siRNA Therapeutics Targeting Skin, Eye, and Liver Diseases

Dong-Ki Lee, PhD, CEO, OliX Pharmaceuticals

RNAi therapeutics have the potential to target hepatic as well as extra-hepatic diseases with the use of optimal delivery methods. We have established lipid-conjugated asymmetric siRNA (asiRNA) targeting skin and ocular tissues, to develop therapeutic programs against hypertrophic scar, androgenic alopecia, and age-related macular degeneration. We also present GalNAcTang, a GalNAc-based liver targeting strategy to tackle diseases such as HBV and NASH. Preclinical and clinical updates will be presented.

9:05 Development of Stem-Loop RNA Oligonucleotide Therapeutics

Radhakrishnan Iyer, PhD, CSO, RIGImmunce, Inc.

RIGImmune is developing a new class of RNA oligonucleotides that activate the innate immune sensor RIG-I. The lead compound is being developed as a pan-viral agent and for immuno-oncology.

9:35 Therapeutic Potential of an RNA Editing Platform Using Editing Oligonucleotides (EONs)

Gerard Platenburg, CSO and Co-Founder, ProQR Therapeutics

Axiomer, an RNA editing technology, uses Editing Oligonucleotides (EONs) to target specific RNA for A-to-I editing by recruiting endogenous Adenosine Deaminase Acting on RNA (ADAR). This presentation will highlight the therapeutic possibilities of this platform that are not limited to disease causing mutations and can potentially address high unmet medical needs by editing wild-type RNA to engineer proteins or modify their function as well as creating *de novo* mutations.

ROOM LOCATION: Gallery Foyer and Bigelow

10:05 Breakout Discussions

Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations, or commiserate about persistent challenges. Please visit https://www.optcongress.com/interactive-discussions for a complete listing of topics and descriptions.

IN-PERSON BREAKOUT DISCUSSION: Integrating Innovative Technologies into Oligo Therapeutics Discovery and Delivery

June Park, PhD, CEO, siRNAgen Therapeutics

Gerard Platenburg, CSO and Co-Founder, ProQR Therapeutics

IN-PERSON BREAKOUT DISCUSSION: IN-PERSON BREAKOUT DISCUSSION: From Early Discovery and the Clinic to Collaborations Between Big Pharma and Small Biotechs

Marvin Caruthers, PhD, Distinguished Professor, University of Colorado

10:50 Coffee Break in the Exhibit Hall with Poster Viewing (Bohea)

11:30 GalNac-RNA for the Lung – Targeted Oligonucleotide Therapy for Respiratory Diseases

Thomas Frischmuth, PhD, CEO, rnatics

rnatics develops first-in-class, carbohydrate-coupled oligonucleotide therapeutics to inhibit disease-causing RNAs in macrophages. This mode of delivery enables access to organs previously not accessible to RNA therapy offering tremendous therapeutic opportunities. The most advanced drug candidate, RCS-21, is in development for the treatment of severe cases of COVID-19. RCS-21 is delivered by inhalation and addresses the macrophage-mediated lung damage following a SARS-CoV-2 infection. We aim to start clinical trials in 2023.

OPTIMIZING DESIGN, DELIVERY, AND PERFORMANCE



12:00 pm FEATURED PRESENTATION: Oligonucleotide Therapeutics to Treat Neurological Disease by Correcting RNA Processing

Jonathan Watts, PhD, Professor, RNA Therapeutics Institute, University of Massachusetts Chan Medical School

In this talk, we will describe work in two diseases of unmet need in the brain, with the theme of using ASOs to correct disease-induced defects in RNA processing. First, we describe an ASO to treat sporadic ALS by correcting mis-splicing of Stathmin2 that results from TDP-43-pathology. We also describe ASO-induced redirection of RNA processing as a potential therapeutic for a common cause of inherited intellectual disability, Fragile X Syndrome.

12:30 Luncheon in the Grand Master Ballroom

1:15 Dessert Break in the Exhibit Hall with Poster Viewing (Bohea)

1:40 Chairperson's Remarks

Sweta Girgenrath, PhD, Head of Cardiovascular and Neuromuscular Therapeutic Areas, Entrada Therapeutics

1:45 Endosomal Escape Vehicle-Oligonucleotide Conjugates for the Targeted Upregulation and Downregulation of Gene Expression

Sweta Girgenrath, PhD, Head of Cardiovascular and Neuromuscular Therapeutic Areas, Entrada Therapeutics

We have developed a novel oligonucleotide delivery platform by conjugation with Entrada's proprietary Endosomal Escape Vehicle (EEV) technology, a class of cyclic peptides designed to facilitate intracellular delivery and endosomal escape. Preclinical studies in several disease models have confirmed the significant therapeutic potential of this approach and demonstrated that the EEV platform can serve as a general and highly efficient delivery technology for the targeted delivery of therapeutic oligonucleotides.

DEEP LEARNING AND ADVANCED COMPUTATIONAL APPROACHES

2:15 Using Computation to Enhance Target ID in RNAi Drug Discovery

James Longden, PhD, Head, Discovery Biology, e therapeutics plc

e-therapeutics combines data, computation and biology to discover and develop RNAi medicines at speed. We generate mechanistic network biology models, then analyze these networks to interrogate disease process complexity, test therapeutic hypotheses, identify novel targets, and then design nucleotide sequences. ETX has built a comprehensive knowledgebase of proprietary and public hepatocyte-relevant data that it mines using deep learning and then employs proprietary GalNAc-siRNA technology to target hepatocytes in the liver.

2:45 Refreshment Break in the Exhibit Hall. Last Chance for Poster Viewing (Bohea)

AsahiKASEI BIOPROCESS

ROOM LOCATION: Singlo
PLENARY SESSION

3:15 Plenary Chairperson's Remarks

Jonathan Watts, PhD, Professor, RNA Therapeutics Institute, University of Massachusetts Chan Medical School



3:20 PLENARY PRESENTATION: Biological Studies with Thiomorpholino Oligonucleotides

Marvin Caruthers, PhD, Distinguished Professor, University of Colorado

Currently underway are more than 20 biological studies focused on the use of thiomorpholino oligonucleotides (TMOs) as a therapeutic drug for the treatment of various genetic diseases. Without exception, TMOs are more active than any other analogue tested and, in one case, a TMO is active under conditions where the 2'-MOE analogue is toxic to mice in the same treatment group.



3:50 PLENARY PRESENTATION: Advances in Chemistry Made RNAi Therapeutics Possible

Mano Manoharan, PhD, Distinguished Scientist & Senior Vice President, Innovation Chemistry, Alnylam Pharmaceuticals

We have evaluated numerous chemical modifications beyond the standard 2'-O-methyl, 2'-fluoro, and phosphorothioate linkages. These include backbone chiral phosphorothioates, glycol nucleic acids, altriol nucleic acids, gem 2'-deoxy-2'-α-F-2'-β-C-methyl, 5'-morpholino, and amino-oxy click chemistry (AOCC) mediated conjugates. Furthermore, novel spatial architectures like circular siRNAs have also been evaluated. This presentation will summarize how chemistry has made possible the currently exciting world of RNAi therapeutics.



4:20 PLENARY PRESENTATION: Development of mRNA-Based Vaccines

Steve Pascolo, PhD, Senior Scientist, University Hospital of Zurich; Founder & CEO, Miescher Pharma GmbH

Less than one year after the publication of the sequence of SARS-CoV-2, mRNA vaccines against COVID-19 were approved. More mRNA vaccines (against infectious diseases and cancer) are in clinical developments and are expected to be approved in the coming years. New mRNA formats (circular, replicating) and formulations (lipoplexes and polyplexes) are also being tested to further improve mRNA vaccines. I will present the past, present and future of mRNA vaccines.

4:50 Close of Conference

Oligonucleotide CMC and Regulatory Strategies

Accelerating Product Development and Commercial Success

MONDAY, MARCH 13

9:15 am Main Conference Registration (Gallery Foyer)

10:40 Welcome Remarks by Conference Organizer

ROOM LOCATION: Salada
INNOVATIVE MANUFACTURING STRATEGIES

10:50 Chairperson's Opening Remarks

Lubomir Nechev, PhD, Senior Vice President, CMC Development, Alnylam Pharmaceuticals



10:55 FEATURED PRESENTATION: Developing Manufacturing and Quality Control Strategy for siRNA Therapeutics

Lubomir Nechev, PhD, Senior Vice President, CMC Development, Alnylam Pharmaceuticals

The last few years showed a significant increase in the number of approved oligonucleotide products, not only in the liver-targeting rare disease area but also in CNS and prevalent diseases. This increased the interest in the process of systematic development of CMC strategies in the field. The talk will focus on successful manufacturing and quality control strategies for synthetic siRNA therapeutics, utilizing different delivery solutions.

11:25 CMC Considerations for siRNA Drug Substance Manufacturing – Improving Processes and Learnings from Outsourcing

Jeske Smink, Head of Drug Substance, Manufacturing, Silence Therapeutics

The quality attributes of siRNA drug substances are affected by used raw materials including customized starting materials such as linkers and conjugates, as well as by the manufacturing process, process parameters, and potential degradation products of the compounds. It is important to increase the understanding of processes and products to allow for improvements of processes and product quality. One of the prerequisites for this is to have good analytics in place.

11:55 Adoption and Implementation of Innovative Technologies – Reducing OPEX and Improving Efficiency in Manufacturing

Robert Dream, PhD, Managing Director, HDR Co. LLC

Driving manufacturing process improvement is one of the most effective ways to increase quality, operational efficiency, and ROI (return on investment). Improving the processes that contribute to the final product is an easy way to create scalable and sustainable change. The right improvements can reduce defects, decrease production time, and boost client satisfaction.

12:25 pm Control and Mitigation of Endotoxin Contamination in Single Stranded Oligonucleotides

Nitto Avecia

Chad Ratterman, Process Development, Process Development, Nitto Avecia

As the list of oligonucleotide therapeutics targeting CNS diseases grows, control of bacterial endotoxincontamination within the API has become critical.Endotoxin can be found on almost every surface andinmanyraw materialswithin the laboratory environment and cannot be eliminated via standard sterilizationmethods. Therefore, control of endotoxin contamination within the API is largely a matter of avoidingintroduction,which requires precautions begin early and continue throughout the manufacturing process.However, unforeseen complications can allow for contamination to occur despite the most rigid ofprecautions. Nitto Avecia has developed a processforresolvingendotoxin contamination withinlyophilized API allowing for rescue of non-conforming batches. This talk will focus on effective controlstrategiesfor endotoxin contamination andoutline a simpleand robust chromatographicpost-hocmitigationprocedure.

12:55 Grand Opening Dessert Break in the Exhibit Hall with Poster Viewing (Bohea)

CPC scientific Peptide Partners Concept to Commercial

REGULATORY INTELLIGENCE

1:35 Chairperson's Remarks

Lubomir Nechev, PhD, Senior Vice President, CMC Development, Alnylam Pharmaceuticals

1:40 Regulatory CMC Interactions and Intelligence for Development and Late-Phase ASO Programs

Tracey Burr, PhD, Director, CMC Regulatory Affairs, Ionis Pharmaceuticals

Current regulatory intelligence for early development and late-phase filings will be discussed, including common health authority requests and recent regulatory guidance, and approaches for their phase-appropriate implementation. The impact of recent regulations on development activities will be discussed and examples provided.

2:10 Phosphoramidite Standards to Support Quality of Oligonucleotide Biotherapeutics

Xiaolei Zhuang, PhD, Scientific Liaison, Global Biologics, USP

This talk will focus on the development of DNA amidites RS. The reference standard materials were analyzed for identification and purity. Water content and residual solvent level were also determined for quality control. All the DNA amidites RS have shown NLT 98% purity within the specification limits. The results collected during collaborative study support the suitability of use as reference standards for DNA amidites, the raw materials of oligonucleotide-related products.

2:40 Novel Approaches to Oligonucleotide Synthesis Using Enzymes

ALMAC

Darren Gray, Biology Technical Leader, Sciences, Almac Group

Biocatalysis has transformed chemical synthesis of small molecule APIs in recent years by shortening routes, lowering waste and reducing costs. Enzymes are now seeing applications beyond small molecule and are being exploited in oligonucleotide synthesis. This presentation will showcase how enzymes can be used in both single and double stranded oligonucleotide synthesis.

3:10 Refreshment Break in the Exhibit Hall with Poster Viewing (Bohea)

SynOligo BIOTECHNOLOGIES

ROOM LOCATION: Singlo
PLENARY SESSION

3:50 Plenary Chairperson's Remarks

Dmitry Samarsky, PhD, CTO, Sirnaomics



3:55 PLENARY PRESENTATION: Nucleic Acid Delivery Systems for RNA Therapy and Genome Editing

Daniel Anderson, PhD, Professor, Chemical Engineering, Massachusetts Institute of Technology

Here we describe our work developing nanoformulations for RNA therapy and genome editing. Libraries of degradable polymers and lipid-like materials have been synthesized, formulated, and screened for their ability to deliver RNA payloads inside cells. These nanoformulations facilitate *in vivo* delivery to a range of tissues and can enable targeted gene suppression with siRNA, gene expression with mRNA, or even permanent genetic editing using the CRISPR/Cas9 system.



4:25 PLENARY PRESENTATION: Advances in CRISPR Genome Editing for Therapeutics Application

Rubina Parmar, PhD, Vice President, Chemistry & Delivery Sciences, Intellia Therapeutics, Inc.

At Intellia, we are building a full-spectrum genome editing company. We are deploying the industry's broadest and deepest toolbox, including novel editing and delivery solutions, to harness the immense power of CRISPR-based technologies for *in vivo* and *ex vivo* therapeutic

applications, each with the potential to revolutionize the future of medicine. In this presentation, we will share the advances in the therapeutic application of CRISPR/Cas9 for genome editing.



4:55 PLENARY PRESENTATION: Recent Developments in Oligo Conjugates

Arthur Levin, PhD, CSO, Avidity Biosciences

The promise of oligonucleotide therapeutics is to use Watson-Crick-Franklin base-pairing rules to design drugs directly and rationally based on genomic information. Until recently, that promise has remained elusive because of cell barriers to oligonucleotide uptake. Avidity's AOC technology uses monoclonal antibodies to cell surface proteins that are internalized in order to facilitate the functional delivery of oligonucleotide therapeutics into a broad range of cell and tissue types.

5:25 Welcome Reception in the Exhibit Hall with Poster Viewing (Bohea)

6:30 Close of Day

TUESDAY, MARCH 14

7:45 am Registration and Morning Coffee (Gallery Foyer)

ROOM LOCATION: Salada

OPTIMIZING CMC & ANALYTICAL CHARACTERIZATION

8:30 Chairperson's Remarks

Rachel Johns, Senior Director, Formulations, Avidity Biosciences LLC



8:35 FEATURED PRESENTATION: Strategies for Optimizing Performance of the Ionis Platform Method for Determination of Assay, Purity, and Impurity Profile of Oligonucleotide Therapeutics Using IP-HPLC-UV-MS

Kimmy Kolkey, Associate Director, Analytical Development and Quality Control, Ionis Pharmaceuticals

Ionis Pharmaceuticals developed an analytical method for the determination of assay, purity, and impurities of ASOs using IP-HPLC-UV-MS. The method permits identification and quantitation of oligonucleotide impurities and has excellent analytical selectivity. Leveraging over 15 years of experience gained through application of the method for compounds in all phases of development at global testing sites, recommended best practices to ensure successful analysis, and strategies to minimize downtime will be presented.

9:05 Identifying the Best Analytical Methods for Characterizing Impurities

Nadim Akhtar, PhD, Senior Principal Scientist, New Modalities, AstraZeneca

Sharing novel methods developed to characterise challenging impurities and method simplification for QC methods.

9:35 Therapeutic Potential of an RNA Editing Platform Using Editing Oligonucleotides (EONs)

Gerard Platenburg, CSO and Co-Founder, ProQR Therapeutics

Axiomer, an RNA editing technology, uses Editing Oligonucleotides (EONs) to target specific RNA for A-to-I editing by recruiting endogenous Adenosine Deaminase Acting on RNA (ADAR). This presentation will highlight the therapeutic possibilities of this platform that are not limited to disease causing mutations and can potentially address high unmet medical needs by editing wild-type RNA to engineer proteins or modify their function as well as creating *de novo* mutations.

ROOM LOCATION: Gallery Foyer and Bigelow

10:05 Breakout Discussions

Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations, or commiserate about persistent challenges. Please visit https://www.optcongress.com/interactive-discussions for a complete listing of topics and descriptions.

IN-PERSON BREAKOUT DISCUSSION: Evaluating CMC and Analytical Strategies

Mike Webb, PhD, Founder and CEO, Mike Webb Pharma; Former Vice President, API Chemistry & Analysis, GSK

10:50 Coffee Break in the Exhibit Hall with Poster Viewing (Bohea)

11:30 Focusing on the Key Control Challenges for Therapeutic Oligonucleotide Drug Substances

Mike Webb, PhD, Founder and CEO, Mike Webb Pharma; Former Vice President, API Chemistry & Analysis, GSK

There are a number of challenges in developing a control strategy for therapeutic oligonucleotides. In this presentation, we will discuss these challenges. Ensuring identity is confirmed with the correct sequence, that reproducible quantitation of impurities is maintained across batches, providing reliable batch purity for drug product compounding, and demonstrating consistent diastereomeric ratios across batches of phosphorothiolated oligonucleotides will be discussed as some of the current hot topics.

12:00 pm Advanced CMC Strategies for Novel Modalities

Rachel Johns, Senior Director, Formulations, Avidity Biosciences LLC

Avidity Biosciences is a clinical phase company developing a new type of modality, Antibody Oligonucleotide Conjugates (AOCs). This therapeutic consists of a monoclonal antibody, as well as an oligonucleotide and, is therefore a hybrid of a biologic and a small molecule. In this talk, we will discuss the challenges and pathways associated with the Chemistry, Manufacture, and Control of these novel hybrid therapeutics.

12:30 Luncheon in the Grand Master Ballroom

1:15 Dessert Break in the Exhibit Hall with Poster Viewing (Bohea)

1:40 Chairperson's Remarks

Kimmy Kolkey, Associate Director, Analytical Development and Quality Control, Ionis Pharmaceuticals

1:45 Oligonucleotide CMC: Control Strategy

Marc Lemaitre, PhD, Oligonucleotide Therapeutics CMC/Strategy Consultant, ML_Consult LLC

CMC (Chemistry, Manufacturing, and Control) for oligonucleotides synthesized by solid phase synthesis and purified by chromatography is an unusual regulatory situation compared to small molecules or synthetic peptides due to that situation and the various types of oligonucleotides. The presentation will describe solutions for a smooth development and control strategy.

FEEDBACK ON REGULATORY SUBMISSIONS

2:15 Key Elements of a Well Composed Nonclinical Section of an IND Application

Emily Noonan Place, PhD, Senior Consultant, Biologics Consulting

2:45 Refreshment Break in the Exhibit Hall. Last Chance for Poster Viewing (Bohea)

ROOM LOCATION: Singlo
PLENARY SESSION

3:15 Plenary Chairperson's Remarks
Jonathan Watts, PhD, Professor, RNA Therapeutics Institute, University of Massachusetts Chan Medical School



3:20 PLENARY PRESENTATION: Biological Studies with Thiomorpholino Oligonucleotides
Marvin Caruthers, PhD, Distinguished Professor, University of Colorado
Currently underway are more than 20 biological studies focused on the use of thiomorpholino oligonucleotides (TMOs) as a therapeutic drug for the treatment of various genetic diseases. Without exception, TMOs are more active than any other analogue tested and, in one case, a TMO is active under conditions where the 2'-MOE analogue is toxic to mice in the same treatment group.



3:50 PLENARY PRESENTATION: Advances in Chemistry Made RNAi Therapeutics Possible
Mano Manoharan, PhD, Distinguished Scientist & Senior Vice President, Innovation Chemistry, Alnylam Pharmaceuticals
We have evaluated numerous chemical modifications beyond the standard 2'-O-methyl, 2'-fluoro, and phosphorothioate linkages. These include backbone chiral phosphorothioates, glycol nucleic acids, altriol nucleic acids, gem 2'-deoxy-2'-α-F-2'-β-C-methyl, 5'-morpholino, and amino-oxy click chemistry (AOCC) mediated conjugates. Furthermore, novel spatial architectures like circular siRNAs have also been evaluated. This presentation will summarize how chemistry has made possible the currently exciting world of RNAi therapeutics.



4:20 PLENARY PRESENTATION: Development of mRNA-Based Vaccines
Steve Pascolo, PhD, Senior Scientist, University Hospital of Zurich; Founder & CEO, Miescher Pharma GmbH
Less than one year after the publication of the sequence of SARS-CoV-2, mRNA vaccines against COVID-19 were approved. More mRNA vaccines (against infectious diseases and cancer) are in clinical developments and are expected to be approved in the coming years. New mRNA formats (circular, replicating) and formulations (lipoplexes and polyplexes) are also being tested to further improve mRNA vaccines. I will present the past, present and future of mRNA vaccines.

4:50 Close of Conference

Emerging mRNA Therapeutics

Design, Delivery, Optimization, and Indications

MONDAY, MARCH 13

9:15 am Main Conference Registration (Gallery Foyer)

10:40 Welcome Remarks by Conference Organizer

ROOM LOCATION: Bigelow

EMERGING RNA MODALITIES

10:50 Chairperson's Opening Remarks

Paloma H. Giangrande, PhD, Vice President, Platform and Discovery Sciences Biology, Wave Life Sciences

10:55 The Therapeutic Potential of oRNA

Nelson Chau, PhD, Senior Vice President, Platform, Orna Therapeutics

Orna has developed a highly efficient circularization process for generating large quantities of purified circular RNA (oRNA). We demonstrate, in the absence of modified nucleotides, that oRNA is immunoquiescent via *in vitro* and *in vivo* assessment. We present case studies using novel ionizable lipids for the generation of lipid nanoparticles to systemically deliver oRNA therapies for oncology (*in situ* CAR) and protein replacement.

11:25 Programmable Epigenomic Medicines: A New Class of mRNA Therapeutics

Thomas McCauley, PhD, CSO, Omega Therapeutics, Inc.

Gene regulation, in health and disease, is mediated by the epigenetic modification to tune gene expression in a cell state-specific manner. Omega Therapeutics is developing programmable epigenetic mRNA therapeutics through an innovative platform capable of precisely and durably modifying chromatin state to treat disease. Based on a computationally guided, modular, and engineered approach, our epigenetic medicines can tune-expression one or many targets with high likelihood of translational success.

11:55 RNA Activation in Acquired and Genetic Diseases

Nagy Habib, ChM, FRCS, Head of R&D and CMO, MiNA Therapeutics Ltd.

Small activating RNAs (saRNA) are double-stranded 21 nucleotide RNA that either target promoters or enhance genes leading to mRNA upregulation. MTL-CEBPA is an investigative drug that resulted from the conjugation of saRNA CEBPA with NOV 340 liposomes that target tumor-associated macrophages in order to alter favorably the tumor microenvironment. MTL-CEBPA improved clinical outcomes in a subset of patients when co-administered with TKI or checkpoint inhibitors.

12:25 pm Control and Mitigation of Endotoxin Contamination in Single Stranded Oligonucleotides

Chad Ratterman, Process Development, Process Development, Nitto Avecia

As the list of oligonucleotide therapeutics targeting CNS diseases grows, control of bacterial endotoxincontamination within the API has become critical.Endotoxin can be found on almost every surface andinmanyraw materialswithin the laboratory environment and cannot be eliminated via standard sterilizationmethods. Therefore, control of endotoxin contamination within the API is largely a matter of avoidingintroduction,which requires precautions begin early and continue throughout the manufacturing process.However, unforeseen complications can allow for contamination to occur despite the most rigid ofprecautions. Nitto Avecia has developed a processforresolvingendotoxin contamination withinlyophilized API allowing for rescue of non-conforming batches. This talk will focus on effective controlstrategiesfor endotoxin contamination andoutline a simpleand robust chromatographicpost-hocmitigationprocedure.

12:55 Grand Opening Dessert Break in the Exhibit Hall with Poster Viewing (Bohea)

UNDERSTANDING RNA-DRIVEN CANCER BIOLOGY

1:35 Chairperson's Opening Remarks

Mark Kay, MD, PhD, Dennis Farrey Family Professor of Pediatrics and Genetics, Department of Pediatrics and Genetics, Stanford University

1:40 miRNA-Based Logic Circuits Encoded on Self-Amplifying RNA for Highly Specific Cancer Cell Classification

Ron Weiss, PhD, Professor, Biological Engineering, Massachusetts Institute of Technology

We developed self-amplifying RNA and modified RNA platforms into vectors capable of carrying synthetic circuitry payloads that can provide a variety of desirable dynamics. We also encoded miRNA target sites on our RNA vectors to provide for highly specific cell type classification. We are using this technology to create next-generation cancer immunotherapy RNA vectors capable of activating therapeutic payloads discriminately in cancer cells.



2:10 FEATURED PRESENTATION: Novel RNAs for Treating Cancer

Mark Kay, MD, PhD, Dennis Farrey Family Professor of Pediatrics and Genetics, Department of Pediatrics and Genetics, Stanford University

I will discuss a miRNA that has long been known to have anti-oncogenic properties and is derived from a long primary transcript, which we recently discovered has a separate tumor suppressor function. The role and anti-oncogenic mechanism by which this long non-coding RNA functions will be discussed. In addition, the therapeutic implications for therapeutic intervention will also be examined.

2:40 Making Innovation Reality: Strategies for Future Manufacturing Requirements

Katarina Stenklo, Enterprise Solutions Commercial Activation Leader, Bioprocess, Cytiva

mRNA technology has changed the way therapies are developed. The overall potential of mRNA is clear, with mRNA therapeutics being developed in many research areas. Much of the current equipment used in manufacturing is repurposed from the biotech industry and is designed for much larger scales than needed for mRNA.What are the challenges associated with smaller scales and how can manufacturers prepare for rapid response?

3:10 Refreshment Break in the Exhibit Hall with Poster Viewing (Bohea)

ROOM LOCATION: Singlo
PLENARY SESSION

3:50 Plenary Chairperson's Remarks

Dmitry Samarsky, PhD, CTO, Sirnaomics



3:55 PLENARY PRESENTATION: Nucleic Acid Delivery Systems for RNA Therapy and Genome Editing

Daniel Anderson, PhD, Professor, Chemical Engineering, Massachusetts Institute of Technology

Here we describe our work developing nanoformulations for RNA therapy and genome editing. Libraries of degradable polymers and lipid-like materials have been synthesized, formulated, and screened for their ability to deliver RNA payloads inside cells. These nanoformulations facilitate *in vivo* delivery to a range of tissues and can enable targeted gene suppression with siRNA, gene expression with mRNA, or even permanent genetic editing using the CRISPR/Cas9 system.



4:25 PLENARY PRESENTATION: Advances in CRISPR Genome Editing for Therapeutics Application

Rubina Parmar, PhD, Vice President, Chemistry & Delivery Sciences, Intellia Therapeutics, Inc.

At Intellia, we are building a full-spectrum genome editing company. We are deploying the industry's broadest and deepest toolbox, including novel editing and delivery solutions, to harness the immense power of CRISPR-based technologies for *in vivo* and *ex vivo* therapeutic applications, each with the potential to revolutionize the future of medicine. In this presentation, we will share the advances in the therapeutic application of CRISPR/Cas9 for genome editing.



4:55 PLENARY PRESENTATION: Recent Developments in Oligo Conjugates

Arthur Levin, PhD, CSO, Avidity Biosciences

The promise of oligonucleotide therapeutics is to use Watson-Crick-Franklin base-pairing rules to design drugs directly and rationally based on genomic information. Until recently, that promise has remained elusive because of cell barriers to oligonucleotide uptake. Avidity's AOC technology uses monoclonal antibodies to cell surface proteins that are internalized in order to facilitate the functional delivery of oligonucleotide therapeutics into a broad range of cell and tissue types.

5:25 Welcome Reception in the Exhibit Hall with Poster Viewing (Bohea)

6:30 Close of Day

TUESDAY, MARCH 14

7:45 am Registration and Morning Coffee (Gallery Foyer)

ROOM LOCATION: Bigelow

INNOVATIONS IN mRNA MODIFICATIONS

8:30 Chairperson's Opening Remarks

Iris Grossman, PhD, Chief Therapeutics Officer, R&D, Eleven Therapeutics US Inc.

8:35 Chemically Modified mocRNAs for Highly Efficient Protein Expression in Mammalian Cells

Xiao Wang, PhD, Assistant Professor, Department of Chemistry, Broad Institute of MIT and Harvard

I will introduce messenger-oligonucleotide conjugated RNAs (mocRNAs) to enable site-specific, robust, and modularized encoding of chemical modifications for highly-efficient and stable protein expression. By directly linking enzymatic and organic synthesis of mRNA, we envision that the mocRNA design will open new avenues to expand the chemical space and translational capacity of RNA-based vectors in basic research and therapeutic applications.



9:05 FEATURED PRESENTATION: Innovations in mRNA Therapeutics

Drew Weissman, MD, PhD, Roberts Family Professor in Vaccine Research, Department of Infectious Diseases; Director of the Penn Institute for RNA Innovation and Professor, Department of Medicine, University of Pennsylvania

Vaccines prevent 4-5 million deaths a year, making them the principal tool of medical intervention worldwide. Nucleoside-modified mRNA (modmRNA) is responsible for the first 2 FDA-approved vaccines. These vaccines show greater than 90% efficacy and outstanding safety. Vaccines against many pathogens are in development. ModmRNA is also being developed for therapeutic protein delivery and gene therapy. modmRNA will have an enormous potential in the development of new medical therapies.

10:05 Breakout Discussions

Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations, or commiserate about persistent challenges. Please visit https://www.optcongress.com/interactive-discussions for a complete listing of topics and descriptions.

IN-PERSON BREAKOUT DISCUSSION: Developing mRNA Therapeutics

Paloma H. Giangrande, PhD, Vice President, Platform and Discovery Sciences Biology, Wave Life Sciences

Mark Kay, MD, PhD, Dennis Farrey Family Professor of Pediatrics and Genetics, Department of Pediatrics and Genetics, Stanford University

Robert Mabry, PhD, CSO, Orna Therapeutics

Drew Weissman, MD, PhD, Roberts Family Professor in Vaccine Research, Department of Infectious Diseases; Director of the Penn Institute for RNA Innovation and Professor, Department of Medicine, University of Pennsylvania

10:50 Coffee Break in the Exhibit Hall with Poster Viewing (Bohea)

ASYMCHEM

NEW FORMULATIONS FOR TARGETED DELIVERY

11:30 Lipid Nanoparticles for Overcoming Biological Barriers to mRNA Delivery

Michael Mitchell, PhD, Skirkanich Assistant Professor of Innovation, Department of Bioengineering, University of Pennsylvania

In this talk, I will discuss our efforts towards the development of lipid and polymer-based nanoparticles that enable the delivery of nucleic acid therapeutics to target cells and tissues *in vivo*. Furthermore, I will describe new therapeutic strategies utilizing these nanoparticles (i) to reprogram immune cells for cancer immunotherapy applications, (ii) for in utero mRNA delivery for treating disease before birth.

12:00 pm Therapeutic RNA Delivery Using Protein-Based Nanocapsules

Ekkehard Leberer, PhD, Senior Life Sciences Consultant, ELBIOCON; Advisor, Neuway Pharma

The presentation will introduce NEUWAY's protein-based nanocapsules as a scalable and versatile delivery technology for a broad range of therapeutic RNA applications. The use of these nanocapsules to deliver therapeutic mRNAs across the blood-brain barrier will be described. The therapeutic potential of this delivery technology will be illustrated for the mRNA treatment of monogenetic CNS disorders such as metachromatic leukodytrophy (MLD).

12:30 Luncheon in the Grand Master Ballroom

1:15 Dessert Break in the Exhibit Hall with Poster Viewing (Bohea)

OPTIMIZING mRNA THERAPEUTICS

1:40 Chairperson's Remarks

Lior Zangi, PhD, Associate Professor, Department of Medicine, Cardiology and Genetics and Genomic Sciences, Icahn School of Medicine at Mount Sinai

1:45 The SMRTs (Specific Modified mRNA Translational System) Way to Fix the Heart

Lior Zangi, PhD, Associate Professor, Department of Medicine, Cardiology and Genetics and Genomic Sciences, Icahn School of Medicine at Mount Sinai

Ischemic heart failure is a leading cause of mortality. This urgent unresolved medical need led scientists to evaluate whether induction of cardiac protection, post cardiac ischemic injury, can attenuate cardiomyocyte (CM) loss. In my talk, I will describe a minimal invasive method, using tail vain I.V injection to deliver therapeutic and protective modRNA directly to CM to attenuate CM cell death and improve the outcome post cardiac ischemic injury.

2:15 mRNA-Based Cancer Immunotherapies: Encoding Vaccines to Immunomodulatory Proteins

Sushma Gurumurthy, PhD, Director, Oncology Research, Moderna, Inc.

Modified mRNA technology has been successfully applied in the development of vaccines, most recently against SARS-CoV-2 virus in the fight against COVID-19. I will present our application of mRNA therapies to develop novel immune-mediate therapies that activate the ability of the immune system to develop durable anti-tumor immunity with approaches ranging from encoding cancer vaccines against shared and personalized tumor antigens to different immunomodulatory proteins.

2:45 Refreshment Break in the Exhibit Hall. Last Chance for Poster Viewing (Bohea)

AsahiKASEI BIOPROCESS

ROOM LOCATION: Singlo
PLENARY SESSION

3:15 Plenary Chairperson's Remarks

Jonathan Watts, PhD, Professor, RNA Therapeutics Institute, University of Massachusetts Chan Medical School



3:20 PLENARY PRESENTATION: Biological Studies with Thiomorpholino Oligonucleotides

Marvin Caruthers, PhD, Distinguished Professor, University of Colorado

Currently underway are more than 20 biological studies focused on the use of thiomorpholino oligonucleotides (TMOs) as a therapeutic drug for the treatment of various genetic diseases. Without exception, TMOs are more active than any other analogue tested and, in one case, a TMO is active under conditions where the 2'-MOE analogue is toxic to mice in the same treatment group.



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Mano Manoharan, PhD, Distinguished Scientist & Senior Vice President, Innovation Chemistry, Alnylam Pharmaceuticals

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4:50 Close of Conference

SHORT COURSE

Safety and Toxicity of Nucleic Acids





SC1: Safety and Toxicity of Nucleic Acids

Instructors:

Sarah Lamore, PhD, Director, Toxicology, Pre-Clinical Development Sciences, Wave Life Sciences

Xiao Shelley Hu, PhD, Vice President, 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



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Conference Venue and Hotel:
Revere Hotel Boston Common
200 Stuart Street| Boston, MA 02116

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Discounted Room Rate Cut-off Date: February 13, 2023

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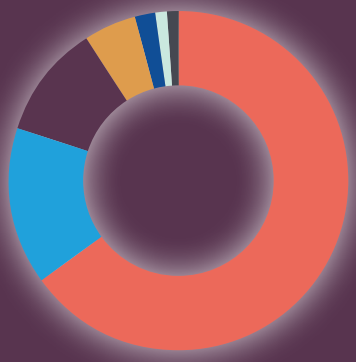
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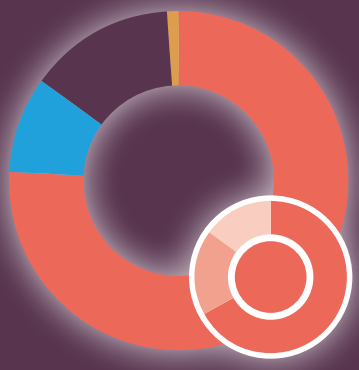
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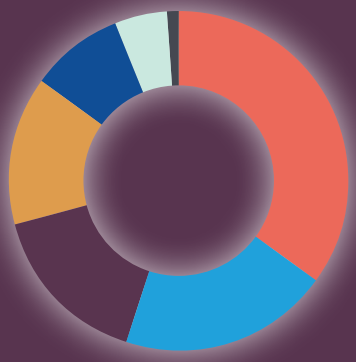
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2022 Attendee Demographics







COMPANY TYPE

Biotech	65%
Pharma	15%
Academic	11%
Services	5%
Government	2%
Financial	1%
Healthcare	1%

GEOGRAPHIC LOCATION

USA	76%
Asia	9%
Europe	14%
Rest of World	1%

US BREAKDOWN

East Coast	67%
West Coast	18%
Midwest	15%

DELEGATE TITLE

Scientist/Technologist	35%
Director	20%
Executive	16%
Sales & Marketing	14%
Manager	9%
Professor	5%
Assistant	1%

Registration & Pricing Information

SHORT COURSE PRICING

	Commercial	Academic, Government, Hospital-affiliated
One short course	$599	$349

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Early Bird Registration until November 18	$1699	$849
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Advance Rate Registration until January 27	$2099	$999
Standard Registration and on-site	$2299	$1099

STANDARD IN-PERSON AND VIRTUAL PACKAGE (Includes access to 3 conferences and On-Demand for 1 year. Excludes short course)

Early Bird Registration until November 18	$1399	$579
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Standard Registration and on-site	$1999	$849

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