SESSIONS

PRE-CONFERENCE WORKSHOPS - 20/09/2021

Workshop Registration

7:15am - 8:00am

ROOM 204AB: Part 1: Nonclinical Perspective

8:00am - 9:30am
Workshop #1: Path to a Successful IND/IMPD for Oligonucleotide Therapeutics

Workshop Leaders:

Jennifer Lockridge, Ph.D., Senior Vice President, Program Development, Dicerna Pharmaceuticals, Inc.

G. Susan Srivatsa, Ph.D. , President, ElixinPharma

Workshop will achieve the following:

This highly strategic, introductory workshop will address the regulatory requirements in pharmacology, toxicology, and CMC to support a successful IND/IMPD submission towards initiation of clinical studies for oligonucleotide therapeutics in the US, Europe, and Canada. Participants will gain a broad understanding of the content of an IND/IMPD submission and the design and execution of activities to acquire the comprehensive data package required for a successful IND/IMPD dossier.

Who should attend?

Anyone interested in preclinical/clinical development of oligonucleotide therapeutics including scientists in R&D, preclinical safety, pharmacology, pharmacokinetics, drug metabolism, manufacturing, quality control, quality assurance, project management, business development and regulatory affairs.

Topics to be covered:

- Regulatory expectations: Pharm/Tox and CMC
- Content of the Nonclinical and CMC Sections of an IND/IMPD dossier
- Considerations for design of GLP toxicology studies
- ADME characterization
- Drug Substance manufacturing, quality control and stability
- Drug Product manufacturing, quality control and stability

Participants

Jennifer Lockridge, PhD - Senior Vice President, Program Development, Dicerna Pharmaceuticals, Inc.

ROOM 205A: Workshop Introduction

8:00am - 8:15am
Workshop #2: Best Practices in Peptide Drug Development: Avoiding Pitfalls and Roadblocks through the Product Lifecycle

Participants

Gary Musso, PhD - President, Musso and Associates LLC

Bruce Morimoto, PhD - Vice President, Cerecin

ROOM 205A: Best Practices for Nonclinical Peptide Development: Bioanalysis, Pharmacokinetics, and Toxicity

8:15am - 9:00am
Workshop #2: Best Practices in Peptide Drug Development: Avoiding Pitfalls and Roadblocks through the Product Lifecycle

As therapeutics, peptides represent a unique class of molecules having properties which span both small molecules and biologics. This presentation and discussion will focus on incorporating these properties into the design and execution of bioanalytical method development and nonclinical pharmacokinetic studies as well as considerations for IND-enabling toxicity studies. Case studies will be used to illustrate these principles.

Participants

Bruce Morimoto, PhD - Vice President, Cerecin

ROOM 205A: Peptide Formulation: Initial Clinical Development, Final Dosage Forms and Life Cycle Management

9:00am - 9:45am
Workshop #2: Best Practices in Peptide Drug Development: Avoiding Pitfalls and Roadblocks through the Product Lifecycle

The talk will outline typical early clinical development strategies for peptide formulation, considerations for final immediate release dosage forms, and options for life cycle management of peptides in late stage clinical or commercial.

Participants

Christopher Rhodes, PhD - President and CEO, Drug Delivery Experts

ROOM 204AB: Q&A for Part 1

9:30am - 9:45am
Workshop #1: Path to a Successful IND/IMPD for Oligonucleotide Therapeutics

Refreshment Break

9:45am - 10:15am
Workshop #1: Path to a Successful IND/IMPD for Oligonucleotide Therapeutics

Networking Refreshment Break

9:45am - 10:15am
Workshop #2: Best Practices in Peptide Drug Development: Avoiding Pitfalls and Roadblocks through the Product Lifecycle

ROOM 204AB: Part 2: CMC Perspective

10:15am - 11:45am
Workshop #1: Path to a Successful IND/IMPD for Oligonucleotide Therapeutics

Workshop Leaders:

Jennifer Lockridge, Ph.D., Senior Vice President, Program Development, Dicerna Pharmaceuticals, Inc.

G. Susan Srivatsa, Ph.D. , President, ElixinPharma

Workshop will achieve the following:

This highly strategic, introductory workshop will address the regulatory requirements in pharmacology, toxicology, and CMC to support a successful IND/IMPD submission towards initiation of clinical studies for oligonucleotide therapeutics in the US, Europe, and Canada. Participants will gain a broad understanding of the content of an IND/IMPD submission and the design and execution of activities to acquire the comprehensive data package required for a successful IND/IMPD dossier.

Who should attend?

Anyone interested in preclinical/clinical development of oligonucleotide therapeutics including scientists in R&D, preclinical safety, pharmacology, pharmacokinetics, drug metabolism, manufacturing, quality control, quality assurance, project management, business development and regulatory affairs.

Topics to be covered:

- Regulatory expectations: Pharm/Tox and CMC
- Content of the Nonclinical and CMC Sections of an IND/IMPD dossier
- Considerations for design of GLP toxicology studies
- ADME characterization
- Drug Substance manufacturing, quality control and stability
- Drug Product manufacturing, quality control and stability

Participants

G. Susan Srivatsa, PhD - President, ElixinPharma

SESSIONS

PRE-CONFERENCE WORKSHOPS - 20/09/2021

ROOM 205A: Best Practices for CMC Regulatory Strategy for Peptides

10:15am - 11:00am
Workshop #2: Best Practices in Peptide Drug Development: Avoiding Pitfalls and Roadblocks through the Product Lifecycle

In the US, FDA reviews peptides as small molecules. In the EU, guidance allows for looser specifications for impurities for peptides. Although EMA is more generous with impurities, IMPD filings become much more demanding for Phase 2 or Phases 3 studies than US INDs. This workshop will focus on highlighting the right balance between too little or too much detail and clinical phase appropriate content.

Participants

Gary Musso, PhD - President, Musso and Associates LLC

ROOM 205A: Quality Strategies for Peptide Development

11:00am - 11:45am
Workshop #2: Best Practices in Peptide Drug Development: Avoiding Pitfalls and Roadblocks through the Product Lifecycle

Peptides are generally not classified as biologics or small molecules and there is a lack of specific guidance for this important class of therapeutics. Furthermore, peptides are often excluded from regulatory guidance documents and the resulting lack of clarity can create challenges during their clinical development. The workshop will review current guidance and provide recommendations for establishing appropriate quality standards throughout the development lifecycle.

Participants

Michael Verlander, D.Phil. - President, Proactive Quality Compliance, Inc.

ROOM 204AB: Q&A for Part 2

11:45am - 12:00pm
Workshop #1: Path to a Successful IND/IMPD for Oligonucleotide Therapeutics

ROOM 205A: Q&A and Discussion

11:45am - 12:00pm
Workshop #2: Best Practices in Peptide Drug Development: Avoiding Pitfalls and Roadblocks through the Product Lifecycle

ROOM 205C: SPOTLIGHT PRESENTATION LUNCHEON: Advanced Analytical Tools for the Quality Assessment of Oligonucleotides and Oligo-based Gene Therapeutics

12:00pm - 1:00pm

Synthetic oligonucleotides can be used as independent therapies or in combination with viral vectors in the expanding area of gene therapies. Recent interest in this topic has stemmed from nucleic acid sidechain modifications that improve target binding and drug efficacy, and provide increased resistance against nucleases. However, the quality assessment of oligonucleotides at production, formulation and quality control stages introduces challenges. Additionally, the synthesis process introduces various impurities, and the formulation of the final drug product adds to the complexity of characterization. Identifying and confirming these oligonucleotide-based product features is integral to the quality, safety, and successful delivery of the drug. Mass spectrometry (MS) and capillary electrophoresis (CE) enable seamless confirmation of transgene integrity and dependable detection of target drugs from process-related impurities. This work will demonstrate the use of high-resolution mass spectrometry to identify impurities from the target oligonucleotide. It will also compare the use of single and multicapillary electrophoresis with laser-induced fluorescence detection to determine the genome size analysis of the packaged genome in an adeno-associated virus (AAV), otherwise known as genome integrity.

Participants

Matthew Stone, PhD - Advanced Workflow Specialist, SCIEX

James Dougherty - Senior Territory Manager, SCIEX

ROOM 204AB: Workshop Introduction

1:00pm - 1:15pm
Workshop #4: Strategies and Alternative Technologies for the Manufacturing Process of Therapeutic Oligonucleotides

Participants

Marc Jacob, Ph.D. - Director of Business Development, Analytical Services, AMPAC Fine Chemical

Yogesh Sanghvi, PhD - President, Rasayan Inc.

ROOM 205A: Workshop Introduction

1:00pm - 1:15pm
Workshop #5: FDA Guidance on ANDA Submission for Peptides

Trishul Shah, Director, Business Development, North America, PolyPeptide Laboratories

Participants

Trishul Shah, MS - Director Business Development, PolyPeptide Group

ROOM 204AB: Recent Trends and Emerging Technologies for Manufacturing Therapeutic Oligonucleotides

1:15pm - 1:45pm
Workshop #4: Strategies and Alternative Technologies for the Manufacturing Process of Therapeutic Oligonucleotides

Participants

Yogesh Sanghvi, PhD - President, Rasayan Inc.

ROOM 205A: The PANDA Approach to Immunogenicity Assessment for New Drugs and Generic ANDAs

1:15pm - 2:00pm
Workshop #5: FDA Guidance on ANDA Submission for Peptides

Under the recent ANDA draft guidance for the generic peptide industry, the U.S. Food and Drug Administration (FDA) recommends that sponsors of generic peptide drugs assess the product for the presence of product-related impurities that may create the potential for differences in immunogenicity, and potentially affecting both the safety and efficacy of the generic drug product. T cell epitopes are a specific area of focus for the draft guidance. T cell epitopes may be introduced or modified as a result of changes to the sequence of the API that delete, modify, or introduce amino acids that are not present in the API sequence, resulting in changes to their immunogenicity. I will review two case studies in which we used in silico and in vitro approaches to assess the immunogenicity of impurities found in generic calcitonin and teriparatide products. Importantly, we find that certain generic peptides may be inherently immunogenic or tolerogenic, and this core property also impacts the immunogenicity risk of associated impurities, by establishing a 'set point' from which the impurities deviate based on the risk inherent to the API sequence, itself. Use of in silico tools provides a framework for evaluating the inherent immunogenic potential of the generic drug API and corresponding product-related impurities, while improving the assessment of safety and efficacy of peptide drug products intended for human use.

Goal: Update understanding of the in silico and in vitro tools that are available for assessing immunogenicity risk of peptide drugs and impurities, using case studies.

Learning objectives. Individuals who listen to this talk will be able to:

1. Understand how the sequence of a generic peptide drug and the peptide drug substance-associated impurities can be analyzed using computational tools for T cell epitope content.

2. Explain how T cell tolerance to certain T cell epitopes may occur and how such epitopes can be identified using information about the natural human HLA ligandome.

3. Know how refining input data improves the accuracy of silico predictions of T cell epitopes.

4. Describe how peptide immunogenicity and tolerance can be tested and validated using in vitro assays such as HLA binding and T cell assays (including Treg assays).

5. Understand the issue of appropriate sample sizes, such as the appropriate number of HLA alleles for in silico analyses, the minimum number of donor samples for in vitro assays.

Participants

Annie De Groot, M.D. - Founder, CEO and CSO, Epivax, Inc.

ROOM 204AB: P(III) vs. P(V): A General P(V) Reagent Platform for Phosphorous Linkages and Application to An Asymmetric Synthesis of a Cyclic Dinucleotide STING Agonist and Antisense Oligonucleotides

1:45pm - 2:30pm
Workshop #4: Strategies and Alternative Technologies for the Manufacturing Process of Therapeutic Oligonucleotides

The development of a reagent class to enable various phosphorus linkages is described. Application of these to a stereoselective synthesis of a cyclic dinucleotide STING agonist was >16-fold higher yielding than a P(III) approach and required less hazardous reagents and chromatographic purifications. Preliminary work to applying this platform to solid phase oligonucleotide synthesis is described.

Participants

Dr. Michael Schmidt, Ph.D. - Associate Scientific Director, Chemical Process De, Bristol-Myers Squibb

ROOM 205A: Considerations in Submitting Abbreviated New Drug Application of Generic Peptide Drug Products

2:00pm - 2:45pm
Workshop #5: FDA Guidance on ANDA Submission for Peptides

In 2017, FDA published a guidance on "ANDAs for certain highly purified synthetic peptide drug products that refer to listed drugs of rDNA origin", which opened the possibility for generic approval of the synthetic version of these peptide products. The recent approval of generic synthetic glucagon is the first success story from the guidance and demonstrated its usefulness and impact in developing generic peptide drugs. Yet, there are still questions about the guidance, the scope it can be applied and the ANDA process in general. In this presentation, we will discuss how to apply the Agency's peptide guidance and how to use the available tools to facilitate the ANDA submission of generic peptide drug products.

Participants

Deyi Zhang - Senior Chemist, Office of Research and Standards, US FDA

ROOM 204AB: Solution Phase Synthesis of Stereospecific Oligonucleotide Dimers and Trimers

2:30pm - 3:00pm
Workshop #4: Strategies and Alternative Technologies for the Manufacturing Process of Therapeutic Oligonucleotides

Participants

Dr. Ravi Natarajan, Ph.D. - President, Socrates Biosciences

Networking Refreshment Break

2:45pm - 3:15pm
Workshop #5: FDA Guidance on ANDA Submission for Peptides

Networking Refreshment Break

3:00pm - 3:30pm
Workshop #4: Strategies and Alternative Technologies for the Manufacturing Process of Therapeutic Oligonucleotides

ROOM 205A: Experience with the FDA Guidance ANDAs for Certain Highly Purified Synthetic Peptide Drug Products That Refer to Listed Drugs of rDNA Origin

3:15pm - 4:00pm
Workshop #5: FDA Guidance on ANDA Submission for Peptides

When the FDA Guidance was published, many public comments from industry expressed concerns regarding the feasibility to comply with the guidance expectations. In the meantime, experience has been obtained with some of the peptides covered by the guidance. This presentation will reiterate the guidance requirements together with Bachem's respective experience obtained so far.

Participants

Gerhard Haas, PhD - Vice President, Quality Assurance and Regulatory Affairs, Bachem AG

ROOM 204AB: Purification of Synthetic Oligonucleotides: A CMO Perspective

3:30pm - 4:00pm
Workshop #4: Strategies and Alternative Technologies for the Manufacturing Process of Therapeutic Oligonucleotides

Purification of synthetic oligonucleotides is a very material intensive process, consuming huge amounts of solvent per kg of drug substance. Different purification strategies and their impact on the PMI of the manufacturing process and the applications of MCSGP for process intensification and solvent reduction will be discussed.

Participants

Dr. Ralf Eisenhuth, PhD - Process Manager Technology Transfer and Chromatography, Bachem AG

ROOM 204AB: Boronic Acid-Assisted Purification of GalNAc-Conjugated ASO's

4:00pm - 4:30pm
Workshop #4: Strategies and Alternative Technologies for the Manufacturing Process of Therapeutic Oligonucleotides

Enhanced purification of GalNAc-conjugated oligonucleotides has been realized using boronic acids as mobile phase additives during reversed-phase chromatography. Several combinations of boronic acid, additive concentration, and pH were evaluated with some demonstrating substantial improvements in resolution over additive-free control purifications.

Participants

Christopher Gabriel, Ph.D. - Senior Scientist, Process Organic Chemistry, Ionis Pharmaceuticals, Inc.

ROOM 205A: Peptide Processes and Impurities in Light of the FDA Guidance on ANDAs

4:00pm - 4:45pm
Workshop #5: FDA Guidance on ANDA Submission for Peptides

Participants

Trishul Shah, MS - Director Business Development, PolyPeptide Group

ROOM 204AB: Q&A and Discussion

4:30pm - 5:00pm
Workshop #4: Strategies and Alternative Technologies for the Manufacturing Process of Therapeutic Oligonucleotides

ROOM 205A: Q&A and Discussion

4:45pm - 5:00pm
Workshop #5: FDA Guidance on ANDA Submission for Peptides

Close of Workshop

5:00pm - 5:05pm

SCHEDULE

PRE-CONFERENCE WORKSHOPS - 20/09/2021

TIDES USA: Oligonucleotide & Peptide Therapeutics

Delivered as Hybrid Event from September 20-30, 2021
Live In-Person Experience Delivered September 20-23; On-demand experience Delivered September 28-30
Boston Convention and Exhibition Center

TIME	WORKSHOP #1: PATH TO A SUCCESSFUL IND/IMPD FOR OLIGONUCLEOTIDE THERAPEUTICS	WORKSHOP #2: BEST PRACTICES IN PEPTIDE DRUG DEVELOPMENT: AVOIDING PITFALLS AND ROADBLOCKS THROUGH THE PRODUCT LIFECYCLE	WORKSHOP #4: STRATEGIES AND ALTERNATIVE TECHNOLOGIES FOR THE MANUFACTURING PROCESS OF THERAPEUTIC OLIGONUCLEOTIDES	WORKSHOP #5: FDA GUIDANCE ON ANDA SUBMISSION FOR PEPTIDES
7:00AM	**7:15am** - Workshop Registration	**7:15am** - Workshop Registration	**7:15am** - Workshop Registration	**7:15am** - Workshop Registration
8:00AM	**8:00am** - ROOM 204AB: Part 1: Nonclinical Perspective	**8:00am** - ROOM 205A: Workshop Introduction **8:15am** - ROOM 205A: Best Practices for Nonclinical Peptide Development: Bioanalysis, Pharmacokinetics, and Toxicity		
9:00AM	**9:30am** - ROOM 204AB: Q&A for Part 1 **9:45am** - Refreshment Break	**9:00am** - ROOM 205A: Peptide Formulation: Initial Clinical Development, Final Dosage Forms and Life Cycle Management **9:45am** - Networking Refreshment Break		
10:00AM	**10:15am** - ROOM 204AB: Part 2: CMC Perspective	**10:15am** - ROOM 205A: Best Practices for CMC Regulatory Strategy for Peptides		
11:00AM	**11:45am** - ROOM 204AB: Q&A for Part 2	**11:00am** - ROOM 205A: Quality Strategies for Peptide Development **11:45am** - ROOM 205A: Q&A and Discussion		
12:00PM	**12:00pm** - ROOM 205C: SPOTLIGHT PRESENTATION LUNCHEON: Advanced Analytical Tools for the Quality Assessment of Oligonucleotides and Oligo-based Gene Therapeutics	**12:00pm** - ROOM 205C: SPOTLIGHT PRESENTATION LUNCHEON: Advanced Analytical Tools for the Quality Assessment of Oligonucleotides and Oligo-based Gene Therapeutics	**12:00pm** - ROOM 205C: SPOTLIGHT PRESENTATION LUNCHEON: Advanced Analytical Tools for the Quality Assessment of Oligonucleotides and Oligo-based Gene Therapeutics	**12:00pm** - ROOM 205C: SPOTLIGHT PRESENTATION LUNCHEON: Advanced Analytical Tools for the Quality Assessment of Oligonucleotides and Oligo-based Gene Therapeutics

SCHEDULE

PRE-CONFERENCE WORKSHOPS - 20/09/2021

TIME	WORKSHOP #1: PATH TO A SUCCESSFUL IND/IMPD FOR OLIGONUCLEOTIDE THERAPEUTICS	WORKSHOP #2: BEST PRACTICES IN PEPTIDE DRUG DEVELOPMENT: AVOIDING PITFALLS AND ROADBLOCKS THROUGH THE PRODUCT LIFECYCLE	WORKSHOP #4: STRATEGIES AND ALTERNATIVE TECHNOLOGIES FOR THE MANUFACTURING PROCESS OF THERAPEUTIC OLIGONUCLEOTIDES	WORKSHOP #5: FDA GUIDANCE ON ANDA SUBMISSION FOR PEPTIDES
1:00PM			**1:00pm** - ROOM 204AB: Workshop Introduction **1:15pm** - ROOM 204AB: Recent Trends and Emerging Technologies for Manufacturing Therapeutic Oligonucleotides **1:45pm** - ROOM 204AB: P(III) vs. P(V): A General P(V) Reagent Platform for Phosphorous Linkages and Application to An Asymmetric Synthesis of a Cyclic Dinucleotide STING Agonist and Antisense Oligonucleotides	**1:00pm** - ROOM 205A: Workshop Introduction **1:15pm** - ROOM 205A: The PANDA Approach to Immunogenicity Assessment for New Drugs and Generic ANDAs
2:00PM			**2:30pm** - ROOM 204AB: Solution Phase Synthesis of Stereospecific Oligonucleotide Dimers and Trimers	**2:00pm** - ROOM 205A: Considerations in Submitting Abbreviated New Drug Application of Generic Peptide Drug Products **2:45pm** - Networking Refreshment Break
3:00PM			**3:00pm** - Networking Refreshment Break **3:30pm** - ROOM 204AB: Purification of Synthetic Oligonucleotides: A CMO Perspective	**3:15pm** - ROOM 205A: Experience with the FDA Guidance ANDAs for Certain Highly Purified Synthetic Peptide Drug Products That Refer to Listed Drugs of rDNA Origin
4:00PM			**4:00pm** - ROOM 204AB: Boronic Acid-Assisted Purification of GalNAc-Conjugated ASO's **4:30pm** - ROOM 204AB: Q&A and Discussion	**4:00pm** - ROOM 205A: Peptide Processes and Impurities in Light of the FDA Guidance on ANDAs **4:45pm** - ROOM 205A: Q&A and Discussion
5:00PM	**5:00pm** - Close of Workshop	**5:00pm** - Close of Workshop	**5:00pm** - Close of Workshop	**5:00pm** - Close of Workshop

SESSIONS

MAIN CONFERENCE - DAY 1 KEYNOTE SESSIONS - 21/09/2021

Registration and Breakfast

7:00am - 8:00am

ROOM BALLROOM WEST: Chairman's Remarks

8:00am - 8:15am

Participants

Muthiah (Mano) Manoharan, PhD - Senior Vice President of Drug Discovery, Alnylam Pharmaceuticals, Inc.

ROOM BALLROOM WEST: RNAi Therapeutics: Past, Present, and (Mostly) Future

8:15am - 9:00am

As we approach the 20-year anniversary of its first description in mammalian cells, RNAi has now emerged as a whole new class of medicines. With 4 marketed products and over a dozen programs in clinical development, Alnylam aims to continue to harness RNAi therapeutics for human health. An update will be provided on the past, present, and future efforts to bring RNAi therapeutics to patients.

Participants

John Maraganore, PhD - Chief Executive Officer, Alnylam Pharmaceuticals, Inc

ROOM BALLROOM WEST: My Long Road from the 2'-5' Oligos to the mRNA Vaccine

9:00am - 9:45am

Different types of RNA are weapons to fight against viruses. Inhibition of the viral replication can be achieved directly using 2'-5'-linked oligonucleotide that activates RNaseL, which degrades viral RNA. Long double-stranded RNA induce interferon with strong antiviral activity. Vaccination using nucleoside-modified mRNA encoding the viral antigen and formulated in lipid nanoparticles induces humoral and cellular immune responses and provides protection against viral infection.

Participants

Katalin Karikó - Senior Vice President RNA Protein Replacement Therapy, BioNTech RNA Pharmaceuticals GmbH, Germany

Networking Refreshment Break

9:45am - 10:30am

ROOM BALLROOM EAST: Development of Melanocortin-based Peptide Therapeutics: Vyleesi (FDA Approved) and Next Generation of Novel Peptides That Resolve Inflammation

10:30am - 11:15am

The melanocortin system, implicated in regulation of multiple physiological functions, is a fruitful target for development of peptide therapeutics. Topics discussed include Vyleesi® (bremelanotide injection), the first melanocortin peptide agonist approved by FDA, and development of melanocortin-targeted clinical stage anti-inflammatory peptide therapeutics for ocular and gastrointestinal indications.

Participants

Carl Spana - CEO & President, Palatin Technologies

ROOM BALLROOM EAST: Bicycle Tumor Targeted Immune Cell Agonists "Bicycle TICAs" (TM) Are Fully Synthetic, Modular and Tunable Anti-cancer Peptide Therapeutics

11:15am - 12:00pm

Participants

Kevin McDonnell, PhD - Vice President, Chemistry, Bicycle Therapeutics

ROOM 204AB: Turning Tides Together. Manufacturing of Peptides and Oligonucleotides

12:05pm - 12:50pm
Spotlight Presentation Luncheon 1

Bachem known as the leading CMO for pharmaceutical grade peptides is expanding its technology platform to offer chemical manufacturing services for nucleic acid based APIs. We would like to take the opportunity to present Bachem's capabilities as the first CMO servicing the global TIDES market.

Participants

Joseph Fraone - Business Development Manager, Oligonucleotides, Bachem

ROOM 205A: Optimizing Process Development for Biomolecule Purification

12:05pm - 12:50pm
Spotlight Presentation Luncheon 2

The optimal resin for your process is not always commercially available. Resin parameters are fixed by the manufacturer and may limit performance for many applications. Therefore, processes are developed within those parameters which can lead to loss of yield, throughput, or require more steps than desired to meet the production goals. In this workshop, ABT, a manufacturer of high-quality agarose resins, will discuss how through their decades of experience, they leverage their ability to control all aspects of the resin. Case studies will be presented on the development of custom resins, that lead to higher resolution, capacity, and simplified purification processes. The result being that optimization of agarose resins is an efficient and inexpensive means to optimize your purification process development.

Participants

Hernan Alarcon, PhD - Research & Development Scientist, Agarose Bead Technologies

Jurgen Machielse - Director, Life Sciences, AIC

ROOM 205C: Unlocking the Potential of Oligonucleotide Therapeutics for Myotonic Dystrophy through Enhanced Delivery

12:05pm - 12:50pm
Spotlight Presentation Luncheon 3

Oligonucleotide therapeutics have the potential to treat a wide range of genetic disorders in a precise and effective manner, but systemic delivery of oligonucleotides has demonstrated poor penetrance of muscle and cardiac tissue, limiting the efficacy of such therapies in neuromuscular disorders such as myotonic dystrophy type 1 (DM1). PepGen has addressed this delivery challenge through the rational development of enhanced delivery oligonucleotides (EDOs) that efficiently and effectively deliver oligonucleotides to skeletal, smooth and cardiac muscle, and to the central nervous system as demonstrated in both mice and non-human primates. PepGen's lead candidate for DM1, PGN-EDODM1, addresses the root cause of DM1 and shows impressive, sustained correction of mis-splicing events, and complete correction of myotonia in a mouse model of the disease where only skeletal muscle is affected. The broad delivery of PepGen's EDO's suggests that EDO-DM1 may successfully treat the many symptoms of this multi-systemic disease.

Participants

James McArthur, PhD - Chief Executive Officer, PepGen

SESSIONS

MAIN CONFERENCE - DAY 1 KEYNOTE SESSIONS - 21/09/2021

ROOM 208: Use of Mustang® E Membrane Chromatography as Risk Mitigation for Endotoxin and Viral Clearance Contamination During UF/DF Processing

12:05pm - 12:50pm
Spotlight Presentation Luncheon 4

An aqueous-based purification process for antisense oligonucleotides (ASO), which ends with a UF/DF step required risk mitigation to avoid endotoxin and viral contamination that could enter late in the process. This study demonstrated that Mustang E membrane chromatography can effectively remove viruses and endotoxin in late-stage processing buffers. This supports a robust approach for clearance of contaminants needed for the more stringent requirements for intrathecal (IT) delivery.

Participants

Robert Gronke, PhD - Senior Principal Scientist, Technical Development, Biogen, Inc

Sarah Blackmore - Viral Vector and Gene Therapy Technology Manager, Pall Biotech

ROOM 205B: Numaswitch - An Efficient Production Platform to Produce Peptides and Small Proteins

12:05pm - 12:50pm
Spotlight Presentation Luncheon 5

We believe in the untapped potential of peptides as feedstock for better, safer, and healthier products in life sciences and beyond. Cost of goods, development times, safety profiles and material supply should never be limiting factors. That is why we have pioneered Numaswitch, an approach to produce peptides and small proteins at highest quality, needed scales, in time, affordable and in a sustainable way. Our mission is to enable your innovations. At TIDES 2021 we are going to share selected case studies and demonstrate how Numaswitch makes a difference.

Participants

Christian Schwarz, PhD - CEO, Numaferm GmbH

Networking Refreshment Break

12:50pm - 1:25pm

ROOM BALLROOM EAST: Chairman's Remarks

1:25pm - 1:30pm

Participants

Mimoun Ayoub, Ph.D. - Vice President, Global Head Sales and Key Account Management, CordenPharma International

ROOM BALLROOM EAST: Antisense Oligonucleotides for the Treatment of Neurological Diseases

1:30pm - 2:15pm

Antisense oligonucleotides (ASOs) have emerged as a viable therapeutic modality for treating previously intractable diseases of the central nervous system. The advancements, opportunities, challenges, and lessons learned in the development of ASOs for the treatment of neurological indications will be discussed.

Participants

Holly Kordasiewicz, Ph.D. - Vice President Neurology Research, Ionis Pharmaceuticals

ROOM BALLROOM EAST: Peptide Drugs Discovery at a Pandemic Speed? Combining Human and Artificial Intelligence? A Point of View

2:15pm - 3:00pm

Participants

Philippe Karoyan, PhD - CEO, X-Pharma and Professor, Sorbonne Université

Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break

3:00pm - 4:00pm

ROOM BALLROOM EAST: How We're Developing the Next Generation of mRNA Delivery LNPs

4:00pm - 4:45pm

At Moderna, we are invested in the development of new modalities for the delivery of mRNA, with a major focus on new classes of lipid nanoparticles (LNPs). The ability of a LNP to deliver mRNA is a function of the components of the particle and the particle architecture. Over the course of our work we have identified critical design criteria for the LNP components, specifically for mRNA cargoes. These efforts have resulted in the development of multiple new classes of LNPs with demonstrated clinical translation.

Participants

Kerry Benenato, PhD - Vice President, Platform Chemistry and Formulation Discovery, Moderna Therapeutics

ROOM BALLROOM EAST: Discovery of Nucleic Acid Binding Molecules and Machine Learning Enabled Delivery of Antisense Oligonucleotides

4:45pm - 5:30pm

I will discuss a platform for discovering nucleic acid binding molecules from combinatorial biohybrid nucleobase peptide libraries and the development of machine learning approaches to deliver antisense oligonucleotides into cells.

Participants

Bradley Pentelute, PhD - Professor, Chemistry, Massachusetts Institute of Technology

Networking Reception in the Poster and Exhibit Hall

5:30pm - 7:00pm

SCHEDULE

MAIN CONFERENCE - DAY 1 KEYNOTE SESSIONS - 21/09/2021

TIDES USA: Oligonucleotide & Peptide Therapeutics

Delivered as Hybrid Event from September 20-30, 2021
Live In-Person Experience Delivered September 20-23; On-demand experience Delivered September 28-30
Boston Convention and Exhibition Center

TIME	SPOTLIGHT PRESENTATION LUNCHEON 1	SPOTLIGHT PRESENTATION LUNCHEON 2	SPOTLIGHT PRESENTATION LUNCHEON 3	SPOTLIGHT PRESENTATION LUNCHEON 4	SPOTLIGHT PRESENTATION LUNCHEON 5
7:00AM	**7:00am** - Registration and Breakfast	**7:00am** - Registration and Breakfast	**7:00am** - Registration and Breakfast	**7:00am** - Registration and Breakfast	**7:00am** - Registration and Breakfast
8:00AM	**8:00am** - ROOM BALLROOM WEST: Chairman's Remarks **8:15am** - ROOM BALLROOM WEST: RNAi Therapeutics: Past, Present, and (Mostly) Future	**8:00am** - ROOM BALLROOM WEST: Chairman's Remarks **8:15am** - ROOM BALLROOM WEST: RNAi Therapeutics: Past, Present, and (Mostly) Future	**8:00am** - ROOM BALLROOM WEST: Chairman's Remarks **8:15am** - ROOM BALLROOM WEST: RNAi Therapeutics: Past, Present, and (Mostly) Future	**8:00am** - ROOM BALLROOM WEST: Chairman's Remarks **8:15am** - ROOM BALLROOM WEST: RNAi Therapeutics: Past, Present, and (Mostly) Future	**8:00am** - ROOM BALLROOM WEST: Chairman's Remarks **8:15am** - ROOM BALLROOM WEST: RNAi Therapeutics: Past, Present, and (Mostly) Future
9:00AM	**9:00am** - ROOM BALLROOM WEST: My Long Road from the 2'-5' Oligos to the mRNA Vaccine **9:45am** - Networking Refreshment Break	**9:00am** - ROOM BALLROOM WEST: My Long Road from the 2'-5' Oligos to the mRNA Vaccine **9:45am** - Networking Refreshment Break	**9:00am** - ROOM BALLROOM WEST: My Long Road from the 2'-5' Oligos to the mRNA Vaccine **9:45am** - Networking Refreshment Break	**9:00am** - ROOM BALLROOM WEST: My Long Road from the 2'-5' Oligos to the mRNA Vaccine **9:45am** - Networking Refreshment Break	**9:00am** - ROOM BALLROOM WEST: My Long Road from the 2'-5' Oligos to the mRNA Vaccine **9:45am** - Networking Refreshment Break
10:00AM	**10:30am** - ROOM BALLROOM EAST: Development of Melanocortin-based Peptide Therapeutics: Vyleesi (FDA Approved) and Next Generation of Novel Peptides That Resolve Inflammation	**10:30am** - ROOM BALLROOM EAST: Development of Melanocortin-based Peptide Therapeutics: Vyleesi (FDA Approved) and Next Generation of Novel Peptides That Resolve Inflammation	**10:30am** - ROOM BALLROOM EAST: Development of Melanocortin-based Peptide Therapeutics: Vyleesi (FDA Approved) and Next Generation of Novel Peptides That Resolve Inflammation	**10:30am** - ROOM BALLROOM EAST: Development of Melanocortin-based Peptide Therapeutics: Vyleesi (FDA Approved) and Next Generation of Novel Peptides That Resolve Inflammation	**10:30am** - ROOM BALLROOM EAST: Development of Melanocortin-based Peptide Therapeutics: Vyleesi (FDA Approved) and Next Generation of Novel Peptides That Resolve Inflammation
11:00AM	**11:15am** - ROOM BALLROOM EAST: Bicycle Tumor Targeted Immune Cell Agonists "Bicycle TICAs" (TM) Are Fully Synthetic, Modular and Tunable Anti-cancer Peptide Therapeutics	**11:15am** - ROOM BALLROOM EAST: Bicycle Tumor Targeted Immune Cell Agonists "Bicycle TICAs" (TM) Are Fully Synthetic, Modular and Tunable Anti-cancer Peptide Therapeutics	**11:15am** - ROOM BALLROOM EAST: Bicycle Tumor Targeted Immune Cell Agonists "Bicycle TICAs" (TM) Are Fully Synthetic, Modular and Tunable Anti-cancer Peptide Therapeutics	**11:15am** - ROOM BALLROOM EAST: Bicycle Tumor Targeted Immune Cell Agonists "Bicycle TICAs" (TM) Are Fully Synthetic, Modular and Tunable Anti-cancer Peptide Therapeutics	**11:15am** - ROOM BALLROOM EAST: Bicycle Tumor Targeted Immune Cell Agonists "Bicycle TICAs" (TM) Are Fully Synthetic, Modular and Tunable Anti-cancer Peptide Therapeutics

SCHEDULE

MAIN CONFERENCE - DAY 1 KEYNOTE SESSIONS - 21/09/2021

TIDES USA: Oligonucleotide & Peptide Therapeutics

Delivered as Hybrid Event from September 20-30, 2021
Live In-Person Experience Delivered September 20-23; On-demand experience Delivered September 28-30
Boston Convention and Exhibition Center

TIME	SPOTLIGHT PRESENTATION LUNCHEON 1	SPOTLIGHT PRESENTATION LUNCHEON 2	SPOTLIGHT PRESENTATION LUNCHEON 3	SPOTLIGHT PRESENTATION LUNCHEON 4	SPOTLIGHT PRESENTATION LUNCHEON 5
12:00PM	**12:05pm** - ROOM 204AB: Turning Tides Together. Manufacturing of Peptides and Oligonucleotides **12:50pm** - Networking Refreshment Break	**12:05pm** - ROOM 205A: Optimizing Process Development for Biomolecule Purification **12:50pm** - Networking Refreshment Break	**12:05pm** - ROOM 205C: Unlocking the Potential of Oligonucleotide Therapeutics for Myotonic Dystrophy through Enhanced Delivery **12:50pm** - Networking Refreshment Break	**12:05pm** - ROOM 208: Use of Mustang® E Membrane Chromatography as Risk Mitigation for Endotoxin and Viral Clearance Contamination During UF/DF Processing **12:50pm** - Networking Refreshment Break	**12:05pm** - ROOM 205B: Numaswitch - An Efficient Production Platform to Produce Peptides and Small Proteins **12:50pm** - Networking Refreshment Break
1:00PM	**1:25pm** - ROOM BALLROOM EAST: Chairman's Remarks **1:30pm** - ROOM BALLROOM EAST: Antisense Oligonucleotides for the Treatment of Neurological Diseases	**1:25pm** - ROOM BALLROOM EAST: Chairman's Remarks **1:30pm** - ROOM BALLROOM EAST: Antisense Oligonucleotides for the Treatment of Neurological Diseases	**1:25pm** - ROOM BALLROOM EAST: Chairman's Remarks **1:30pm** - ROOM BALLROOM EAST: Antisense Oligonucleotides for the Treatment of Neurological Diseases	**1:25pm** - ROOM BALLROOM EAST: Chairman's Remarks **1:30pm** - ROOM BALLROOM EAST: Antisense Oligonucleotides for the Treatment of Neurological Diseases	**1:25pm** - ROOM BALLROOM EAST: Chairman's Remarks **1:30pm** - ROOM BALLROOM EAST: Antisense Oligonucleotides for the Treatment of Neurological Diseases
2:00PM	**2:15pm** - ROOM BALLROOM EAST: Peptide Drugs Discovery at a Pandemic Speed? Combining Human and Artificial Intelligence? A Point of View	**2:15pm** - ROOM BALLROOM EAST: Peptide Drugs Discovery at a Pandemic Speed? Combining Human and Artificial Intelligence? A Point of View	**2:15pm** - ROOM BALLROOM EAST: Peptide Drugs Discovery at a Pandemic Speed? Combining Human and Artificial Intelligence? A Point of View	**2:15pm** - ROOM BALLROOM EAST: Peptide Drugs Discovery at a Pandemic Speed? Combining Human and Artificial Intelligence? A Point of View	**2:15pm** - ROOM BALLROOM EAST: Peptide Drugs Discovery at a Pandemic Speed? Combining Human and Artificial Intelligence? A Point of View
3:00PM	**3:00pm** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**3:00pm** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**3:00pm** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**3:00pm** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**3:00pm** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break
4:00PM	**4:00pm** - ROOM BALLROOM EAST: How We're Developing the Next Generation of mRNA Delivery LNPs **4:45pm** - ROOM BALLROOM EAST: Discovery of Nucleic Acid Binding Molecules and Machine Learning Enabled Delivery of Antisense Oligonucleotides	**4:00pm** - ROOM BALLROOM EAST: How We're Developing the Next Generation of mRNA Delivery LNPs **4:45pm** - ROOM BALLROOM EAST: Discovery of Nucleic Acid Binding Molecules and Machine Learning Enabled Delivery of Antisense Oligonucleotides	**4:00pm** - ROOM BALLROOM EAST: How We're Developing the Next Generation of mRNA Delivery LNPs **4:45pm** - ROOM BALLROOM EAST: Discovery of Nucleic Acid Binding Molecules and Machine Learning Enabled Delivery of Antisense Oligonucleotides	**4:00pm** - ROOM BALLROOM EAST: How We're Developing the Next Generation of mRNA Delivery LNPs **4:45pm** - ROOM BALLROOM EAST: Discovery of Nucleic Acid Binding Molecules and Machine Learning Enabled Delivery of Antisense Oligonucleotides	**4:00pm** - ROOM BALLROOM EAST: How We're Developing the Next Generation of mRNA Delivery LNPs **4:45pm** - ROOM BALLROOM EAST: Discovery of Nucleic Acid Binding Molecules and Machine Learning Enabled Delivery of Antisense Oligonucleotides

SCHEDULE

MAIN CONFERENCE - DAY 1 KEYNOTE SESSIONS - 21/09/2021

TIME	SPOTLIGHT PRESENTATION LUNCHEON 1	SPOTLIGHT PRESENTATION LUNCHEON 2	SPOTLIGHT PRESENTATION LUNCHEON 3	SPOTLIGHT PRESENTATION LUNCHEON 4	SPOTLIGHT PRESENTATION LUNCHEON 5
5:00PM	**5:30pm** - Networking Reception in the Poster and Exhibit Hall	**5:30pm** - Networking Reception in the Poster and Exhibit Hall	**5:30pm** - Networking Reception in the Poster and Exhibit Hall	**5:30pm** - Networking Reception in the Poster and Exhibit Hall	**5:30pm** - Networking Reception in the Poster and Exhibit Hall

SESSIONS

MAIN CONFERENCE - DAY 2 - 22/09/2021

Morning Fun Run

6:00am - 7:00am

Start off your day with a fun run around Boston's Beautiful Seaport! The run will be led by our marathon running staff. We will start at the North Lobby Entrance to the BCEC at 6:00AM, run past some of Boston's magnificent landmarks and see some of the most beautiful views of the skyline. This quick 2 mile run will get you ready for day 3 of BWB! The first 50 registrants will get a free t-shirt! Space is limited, sign up today at the registration desk at East Registration – Exhibit Level outside of Hall C.

Registration

7:30am - 7:45am

ROOM 204AB: Regulatory Experience Sharing: Excipients for use in Lipid Nanoparticles

7:45am - 8:15am
Breakfast Spotlight Presentations 2

The use of excipients in lipid nanoparticles can enhance the drug absorption or delivery and is therefore of high interest in various therapeutic fields such as chemotherapy, gene therapy, or vaccinations for example. However, the unique characteristics of such excipients pose also challenges to regulatory agencies. This concerns for example the assessment of purity and safety when predicting the in vivo performance of the formulation. Authority requirements related to the control strategy for each lipid are comparable to that of active substances. Excipients for use in lipid nanoparticles exceed by far the authorityâ€™s expectations on data for regulatory filing compared to other excipients in drug products. The purity and safety of lipid excipients needs to be demonstrated, because of their crucial role in the function of the drug product, distinct physicochemical properties and complex potential interactions with other ingredients or the physicochemical environment.

Participants

Bethany Walls - Senior Regulatory Expert, MilliporeSigma

ROOM 204AB: Chairman's Remarks

8:15am - 8:20am
Oligonucleotide CMC and Targeted Delivery TRACK: Analytics of Oligonucleotides

Participants

Claus Rentel, PhD - Vice President, Analytical Development/QC, Ionis Pharmaceuticals, Inc.

ROOM 204AB: Analytical Method Development for Critical Impurities in Phosphoramidites

8:20am - 8:45am
Oligonucleotide CMC and Targeted Delivery TRACK: Analytics of Oligonucleotides

An overview of various analytical approaches for the detection, identification, and control of critical impurities in phosphoramidites will be presented with a focus on UHPLC-MS methods capable of resolving isomeric and 2'-alkyl modified impurities.

Participants

Dennis Rhodes - Assistant Director, Analytical Development and QC, Ionis Pharmaceuticals

ROOM 205A: Chairman's Remarks

8:25am - 8:30am
Oligonucleotide Discovery to Clinic and CMC TRACK: Emerging Oligonucleotide Drug Discovery

Participants

P. Peter Ghoroghchian, M.D., Ph.D. - President and CEO, Silagene, Inc.

ROOM 205B: Chairman's Remarks

8:25am - 8:30am
Peptides TRACK: Peptides for COVID-19 Therapy

Participants

Trishul Shah, MS - Director Business Development, PolyPeptide Group

ROOM 205C: Chairman's Remarks

8:25am - 8:30am
mRNA and Genome Editing TRACK: mRNA Discovery, Delivery and Translation to the Clinic

Participants

Tasuku Kitada, Ph.D. - Co-founder, Director, President, and Head of R&D, Strand Therapeutics

ROOM 205A: Targeting ANGPTL4 in the Liver with ASO – A Novel Treatment for Cardiometabolic Disease

8:30am - 9:00am
Oligonucleotide Discovery to Clinic and CMC TRACK: Emerging Oligonucleotide Drug Discovery

The lipase inhibitor ANGPTL4 is a well-studied and genetically validated target for cardiometabolic diseases. Using an antibody approach, early discovery efforts showed that global inhibition may cause severe side effects. Using an ASO-GalNAc approach we have shown that these side effects can be avoided while still displaying a potent efficacy in disease models.

Participants

Stefan Nilsson, Ph.D. - CEO, Lipigon Pharmaceuticals AB

ROOM 205B: Treatment with Vasoactive Intestinal Peptide (aviptadil) is Life-saving in Patients with Critical COVID-19

8:30am - 9:00am
Peptides TRACK: Peptides for COVID-19 Therapy

The primary lethal effect of COVID-19 is caused by binding of the SARS-CoV-2 virus to the Angiotensin Converting Enzyme 2 (ACE2) receptor on the Alevolar Type 2 (AT2) cell in the human lung, with resulting viral replication, suppression of surfactant production, cytokine formation, and cytopathy of AT2 cells. Vasoactive Intestinal Peptide (VIP) is known to protect the AT2 cell against numerous forms of experimental lung injury via its binding to the VPAC1 receptor. Aviptadil is a synthetic form of VIP manufactured by protein synthesis. In SARS-CoV-2 infected human calu3 cells (a model for AT2), VIP blocks viral replication, blocks cytokine synthesis, and prevents cytopathy. Benefits of VIP have also been reported in patients with Checkpoint-inhibitor pneumonitis and sarcoidosis. In a 196 person randomized clinical trial of patients with Critical COVID-19 and respiratory failure, ZYESAMI®(aviptadil acetate) given intravenously significantly increases the likelihood of being alive and free of respiratory failure at 60 days compared to placebo and shortens hospitalization time by a median 11 days (P<.02). Similar survival and recovery have been seen in more than 350 patients treated under expanded access protocols. The National Institutes of Health is currently conducting a 660-person global clinical trial to confirm these results.

Participants

Jonathan Javitt - Founder & Chief Executive Officer, NeuroRx Inc

ROOM 205C: Programming mRNA for Cancer Immunotherapy

8:30am - 9:00am
mRNA and Genome Editing TRACK: mRNA Discovery, Delivery and Translation to the Clinic

The next generation of mRNA therapeutics will utilize synthetic biology to enable programmable drugs capable of delivering precise, multi-functional, and curative treatments. In this presentation, I will talk about our efforts in programming mRNA to improve response rates to checkpoint inhibitor therapy for solid tumors as well as other early work that has the potential to revolutionize cancer immunotherapy.

Participants

Tasuku Kitada, Ph.D. - Co-founder, Director, President, and Head of R&D, Strand Therapeutics

ROOM 204AB: Automated Online UPLC and Data Processing for Oligonucleotide Synthesis and Purification Monitoring

8:45am - 9:10am
Oligonucleotide CMC and Targeted Delivery TRACK: Analytics of Oligonucleotides

At-line reaction monitoring is a beneficial but time-consuming analytical technique requiring expertise that may not exist on the manufacturing floor. Deploying an online solution like the Waters PATrol provides an opportunity to understand and control our synthesis/purification processes in real-time in conjunction with automation software.

Participants

Andrew Argo - Scientist I, Quality Control Analytical Technology, Biogen

ROOM 205A: U1 Adaptors Bioconjugates for Targeted Gene Silencing in Extrahepatic Tissues

9:00am - 9:30am
Oligonucleotide Discovery to Clinic and CMC TRACK: Emerging Oligonucleotide Drug Discovery

Proprietary to Silagene, Inc., U1 Adaptors are bivalent oligonucleotides that engage the U1 small nuclear ribonucleoprotein (U1 snRNP) to inhibit gene-specific polyadenylation. When compared to other silencing technologies, they exhibit several advantageous properties, including stoichiometrically favored engagement of an abundant effector (~106 U1snRNP/cell) to enable specific gene silencing that lasts weeks-to-months. The current presentation will update their detailed mechanisms of action and the company's current therapeutic development efforts

Participants

P. Peter Ghoroghchian, M.D., Ph.D. - President and CEO, Silagene, Inc.

ROOM 205B: Developing a Highly Conserved, Non-human-like, and Cross-reactive SARS-CoV-2 Peptide-based COVID-19 Vaccine

9:00am - 9:30am
Peptides TRACK: Peptides for COVID-19 Therapy

Background: The rapid induction of protective immunity following a single dose of currently approved COVID-19 vaccines clearly points to pre-existing T cell-mediated immune memory responses that are activated by vaccination. It is also well established that T cells support the SARS-CoV-2 antibody response, clear virus-infected cells, and may be required to block transmission. Furthermore, lack of information about the durability of antibody-focused vaccines, and their ability to control virus in the upper respiratory tract, and efficacy against variants, motivates development of T cell-directed vaccines capable of stimulating tissue-resident memory T cells. In this study, we set out to identify conserved peptide epitopes associated with SARS-CoV-2 T cell immunity and began the process of developing the peptides for a Phase I Trial.

Methods: Highly conserved, promiscuous SARS-CoV-2 spike, membrane and envelope sequences epitopes were selected using advanced immunoinformatics tools. Selected epitopes were assayed for antigenicity by ex vivo and cultured IFNg ELISpot assays using PBMCs donated by SARS-CoV-2 convalescents and healthy individuals. Immunogenicity of peptide epitopes co-formulated with poly-ICLC adjuvant was evaluated by prime-boost immunization of HLA-DR3 transgenic mice. Comparisons of multiple restimulation conditions in each of the evaluated groups used a two-way ANOVA. For all analyses, significance was attributed to calculated p values <0.05.

Results & Conclusions: Sixty-six percent of 32 predicted epitopes were recognized in direct ex vivo assays by persons who mounted a protective immune response to SARS-CoV-2 infection. T cell responses correlated with total spike-specific IgG and neutralizing antibody responses ($p<0.01$). Persons with no SARS-CoV-2 experience demonstrated ex vivo responses to only 9% of epitopes. However, these individuals demonstrated robust T cell responses to 97% of SARS-CoV-2 epitopes, following a period of epitope-specific T cell expansion in culture, similar to convalescents. These data suggest that pre-existing immunity, potentially to common cold coronaviruses, may contribute to natural immunity and enhance vaccine efficacy. From a starting set of 32, 19 T cell epitopes were moved into formulation studies. These peptides will be pooled andco-formulated with poly-ICLC in 4 pools for intradermal immunization. In mice, the vaccine generates an epitope-specific memory population and sharply focuses the T cell response on Th1 and Tc1 populations. Robust type 1 immunity skewing (>100-fold over type 2; $p<0.05$) alleviates concern that this T cell-targeted vaccine may enhance respiratory disease commonly associated with Th2 responses. Taken together, these epitopes may be used to improve our understanding of natural and vaccine-induced immunity and to facilitate the development of T cell-targeted vaccines that harness pre-existing SARS-CoV-2 immunity. Plans for Phase I and the eventual target population for this vaccine will be described.

Participants

Annie De Groot, M.D. - Founder, CEO and CSO, Epivax, Inc.

ROOM 205C: A Novel Vaccine Approach Using Messenger RNA-Lipid Nanoparticles: Preclinical and Clinical Perspective

9:00am - 9:30am
mRNA and Genome Editing TRACK: mRNA Discovery, Delivery and Translation to the Clinic

Acuitas is developing lipid nanoparticulate systems (LNP) that allow efficient delivery and expression of mRNA. Through internal research and multiple industry and academic collaborations, Acuitas is enabling mRNA in a broad range of therapeutic areas. The most advanced therapeutic application is as prophylactic vaccines against infectious disease. Results from collaborations demonstrating the ability of mRNA-LNP vaccines to protect against infectious diseases will be shown. Further, clinical translation of our mRNA-LNP as a prophylactic vaccine against SARS-CoV2 will be presented.

Participants

Ying Tam, Ph.D. - Chief Scientific Officer, Acuitas Therapeutics, Inc.

ROOM 204AB: Control of GalNAc siRNA Conjugates

9:10am - 9:35am
Oligonucleotide CMC and Targeted Delivery TRACK: Analytics of Oligonucleotides

Inclisiran is a GalNAc conjugated siRNA that is designed to reduce LDL cholesterol levels by inhibiting PCSK9 synthesis in the liver. The use of GalNAc conjugated siRNA's as a therapeutic class has shown promise in a number of therapeutic areas. Based on experience with Inclisiran, a strategy for control of the quality of this complex drug substance with particular emphasis on purity has emerged and will be presented here.

Participants

Adel Rafai Far, Ph.D. - Principal Fellow, Chemical and Analytical R&D, Novartis Pharmaceuticals

ROOM 205A: A Novel, RNA-based Anti-viral Approach to Limit BK Virus Propagation in Immunocompromised Patients

9:30am - 10:00am
Oligonucleotide Discovery to Clinic and CMC TRACK: Emerging Oligonucleotide Drug Discovery

BK virus (BKV) is a universal latent renal virus that reactivates in kidney transplantation patients and can lead to graft loss. No treatment exists for combatting BK virus. As such, treatment options for BKV are vital. At Hybridize, we have developed an innovative RNA-based strategy that prevents BKV-mediated disease.

Participants

Eric van der Veer, Ph.D. - Chief Scientific Officer, Hybridize Therapeutics

ROOM 205B: When the Process Doesn't Fit - 8 Weeks to Build a Purification Suite to Support A Critical Covid-19 Vaccine Adjuvant

9:30am - 10:00am
Peptides TRACK: Peptides for COVID-19 Therapy

It's late August and I am sitting at my desk. We are scheduled to produce the key adjuvant for one of the crucial COVID-19 vaccines. We have been working on this for multiple months and it has become apparent our planned fit will not work and for multiple reasons – flammable solvent, large volumes, larger column size, high temperatures, and near continuous run rate for much of the coming year. One or more of these could be overcome with scheduling, planning, reducing the run rate in one area or product mix in another area for a short period of time but the full combination of these meant the space we intended to use and the equipment we intended to use was not a good fit. Dramatic changes would be needed for the processing space, the equipment used and the physical layout. Under the best-case scenario instead of starting our engineering run in late December we would be starting in June (six months late) and the costs would exceed the planned costs by over $ 10 Million. I gave myself five minutes to cry and then it was time to get busy. A lot of hard work and even more trust, teamwork and perseverance would be needed to address these challenges.

Participants

Tim Culbreth - Site Director, Polypeptide

ROOM 205C: Preclinical Immunogenicity Characterization of ARCT-021 SARS-CoV-2 Vaccine

9:30am - 10:00am
mRNA and Genome Editing TRACK: mRNA Discovery, Delivery and Translation to the Clinic

The self-transcribing and replicating RNA (STARRTM) technology combined with Arcturus Therapeutics proprietary lipid nanoparticle (LNP) delivery technology has produced a safe and effective vaccine against SARS-CoV-2 virus. Mouse immunogenicity studies showed continuous increase in neutralizing antibody titers for up to 60 days after a single vaccination along with a strong Th1 cell mediated immune response. Lethal virus challenge studies using a human ACE2 transgenic mouse model yielded 100% protection after a single 2 Âµg RNA dose. T cell and B cell depletion studies with a sublethal virus challenge in the same transgenic mouse model showed complete protection after B cell depletion and no protection after T cell depletion. Rhesus macaque immunogenicity studies showed high neutralizing antibody titers after two prime injections 28 days apart. A further increase in neutralizing antibody titers were observed with a boost injection 120 days after the second prime injection. Non-human primate virus challenge studies showed significant reduction in bronchoalveolar virus genomes after single and double prime vaccinations. Preliminary preclinical results for second generation vaccines designed with improved anti-viral immunogenicity exhibited cross neutralization against alpha, beta, gamma and delta circulating viral variants in mice and non-human primates. The first generation vaccines are in late stage clinical trials and the second generation vaccines are planned for entry into the clinic.

Participants

Sean Sullivan, Ph.D. - Executive Director, Process Development, Arcturus Therapeutics

ROOM 204AB: Suppressing the Inherent Reactivity of Labile Moieties during Purity and Identity Characterization

9:35am - 10:00am
Oligonucleotide CMC and Targeted Delivery TRACK: Analytics of Oligonucleotides

Nucleoside phosphoramidites (NPAs) set sugar structure and nucleobase identity (sequence) in therapeutic oligonucleotides during solid phase syntheses. As such, control over their purities and impurity profiles are of paramount importance. Under reverse-phase (RP) chromatography conditions, water is ubiquitous, posing a fundamental threat to NPA stability during the analysis, and may contain adventitious oxidants. Challenges in analyzing NPAs include poor peak shape, diminished sensitivity, and rising baselines. Here, we report mitigating strategies for protection of NPAs during LCMS analyses.

Participants

Vanessa Momaney, Ph.D. - Research Scientist II, Analytical Development, Nitto Denko Avecia Inc.

Networking Refreshment Break in Poster and Exhibit Hall

10:00am - 10:45am

ROOM 204AB: Analytical Method Development of Complementary Related Organic Impurity Methods for Oligonucleotides

10:45am - 11:15am
Oligonucleotide CMC and Targeted Delivery TRACK: Analytics of Oligonucleotides

Oligonucleotides have a complex related impurity profile and require orthogonal analysis, typically by LC/UV/MS. Analytical Method Quality by Design work that led to the development of complementary LC/UV and LC/MS methods for the late-phase quantitation of related impurities will be presented, and the advantages of this approach will be discussed.

Participants

Chris Gripton, Ph.D. - Principal Scientist, GSK Medicines Research Centre

ROOM 205A: Platform Optimization to Enable Therapeutic Development of ADAR-mediated RNA editing

10:45am - 11:15am
Oligonucleotide Discovery to Clinic and CMC TRACK: Emerging Oligonucleotide Drug Discovery

RNA editing by ADARs (Adenosine Deaminases Acting on RNA) is a nearly ubiquitous cellular process by which coding and non-coding RNAs are deaminated resulting in the conversion of adenosine to inosine. Since inosine Watson-Crick base pairs as guanosine, our approach enables the functional conversion of adenosine to guanosine. We will share data demonstrating the general utility of this approach as well as our progress to unlock this new therapeutic modality. Data demonstrating endogenous ADARs can be co-opted to edit a variety of target mRNAs with high specificity and activity using synthetic oligonucleotides in vitro and in vivo will be presented.

Participants

Stuart Milstein, Ph.D. - VP Platform Biology, Korro Bio

ROOM 205B: Solid-Phase Peptide Synthesis Using a Four-Dimensional Protecting Group Scheme

10:45am - 11:15am
Peptides TRACK: Peptide Discovery and Development

Most of the peptides for both research and production are synthesized in the solid phase using a two-dimensional protection scheme. For the synthesis of some peptides such as cyclic peptides, a third kind of protecting group is incorporated, which is usually orthogonal to the first two. Herein, we introduce a fourth category of protecting groups that are stable under the conditions used to remove the first three and that are removed at the end of the synthetic process. This step can be performed when the peptide is still anchored to the resin or once the peptide is in solution. This new concept of protecting group facilitate the synthesis and manipulation of difficult peptides.

Participants

Fernando Albericio, PhD - Research Professor, School of Chemistry, University of Kwazulu-Natal

ROOM 205C: Subcutaneous Administration of mRNA LNPs to Achieve Systemic Exposures of Protein

10:45am - 11:15am
mRNA and Genome Editing TRACK: mRNA Discovery, Delivery and Translation to the Clinic

Subcutaneous administration opens the possibility of patient self-administration that could enable mRNA to be used in chronic treatments for protein replacement or regenerative therapies. In this work, we demonstrate that subcutaneous administration of mRNA formulated within LNPs results in measurable plasma exposure of a secreted protein, albeit with dose-limiting local inflammatory responses. Inclusion of steroid prodrugs in the LNPs resulted in increased protein expression and improved tolerability following subcutaneous administration.

Participants

Nigel Davies, PhD - Principal Scientist, Advanced Drug Delivery, AstraZeneca

ROOM 204AB: Identification and Quantitation of Thermal Degradation Products of Antisense Oligonucleotides (ASOs) Enables Understanding of Degradation Mechanism

11:15am - 11:45am
Oligonucleotide CMC and Targeted Delivery TRACK: Analytics of Oligonucleotides

Under thermal stress, 2'-modified anti-sense oligonucleotides (ASOs) with a deoxy-gap undergo strand cleavage, initiated by depurination. Detailed characterization of such thermal degradation products was performed on seven platform ASOs using LC-MS. Quantitative comparison of a group of shortmers resulting from abasic cleavage at various deoxypurine sites indicated sequence-related effects on the extent of degradation.

Participants

Rasika Phansalkar, Ph.D. - Scientist I, Technical Development, Biogen

ROOM 205A: Development of an Antisense Oligonucleotide, STK-001, for Dravet Syndrome

11:15am - 11:45am
Oligonucleotide Discovery to Clinic and CMC TRACK: Emerging Oligonucleotide Drug Discovery

TANGO is a novel technology which exploits antisense oligonucleotide (ASO)-mediated modulation of pre-mRNA splicing to increase protein expression. The effectiveness of this approach for treating Dravet syndrome (DS) was tested using the DS mouse model. This model has been shown to recapitulate features of DS including seizures and sudden unexpected death. DS mice were administered STK-001 via intracerebroventricular (ICV) injection. Pharmacology was assessed by measuring productive Scn1a mRNA and NaV1.1 protein in mouse brain samples and efficacy was evaluated by quantification of spontaneous seizures by electroencephalography (EEG) and survival monitoring. A single administration of STK-001 significantly decreased seizure frequency by 76% and significantly increased survival by 97% in the DS mouse model. In addition, a single administration of STK-001 in healthy mice, rat, and monkey resulted in lasting brain exposure levels, increased productive Scn1a gene expression, and NaV1.1 protein expression in the brain tissues. These results provide evidence that TANGO technology can be used to rescue both the seizure and survival phenotypes in a mouse model of DS and that STK-001 is pharmacologically active in rodents and non-human primates. STK-001 has potential to be the first gene-specific, disease-modifying treatment to restore NaV1.1 to physiological levels and provide a therapeutic benefit. STK-001 is currently being evaluated in Phase 1/2a clinical trials in patients with DS.

Participants

Meena Meena, Ph.D. - Vice President of Bioanalytical, DMPK and Biomarker Development, Stoke Therapeutics

ROOM 205B: Discovery and Optimization of Long-acting, Unimolecular Peptide Triple Agonists of the GLP-1, GIP, and Glucagon Receptors

11:15am - 11:45am
Peptides TRACK: Peptide Discovery and Development

Concurrent activation of the GLP-1, GIP, and glucagon receptors imparts combinatorial metabolic benefits through multiple pharmacological mechanisms. Long-acting peptides were designed with potent agonistic activities at all three receptors. Chronic treatment of obese rodents with such optimized triple agonists exhibited profound efficacy to normalize body weight to a pre-obese state.

Participants

Patrick Knerr, Ph.D. - Principal Scientist, Novo Nordisk Research Center Indianapoli

ROOM 205C: A Platform Approach for mRNA Vaccine Development

11:15am - 11:45am
mRNA and Genome Editing TRACK: mRNA Discovery, Delivery and Translation to the Clinic

Participants

Don Parsons, PhD - Head, Early Technical Development and LNP Process, Moderna Therapeutics

ROOM 204AB: Advancing the Pharmaceutical Science of Lipid Delivery Systems and their Translation to the Clinic

11:45am - 12:15pm
Oligonucleotide CMC and Targeted Delivery TRACK: Analytics of Oligonucleotides

Oligonucleotides (OGN) systems have emerged not only as a paradigm-shifting tool for the elucidation of gene function and for target validation, but also as promising new therapeutic modalities with the potential to address previously undruggable targets. A number of OGN therapeutics have already progressed into preclinical and clinical development. Of these, lipid-based systems have emerged as one of the most mature classes of delivery technologies. Despite tremendous advances in development, the development of lipid nanoparticle (LNP)-based therapeutics still presents unique and significant pharmaceutical and regulatory challenges. With the increase in number of OGNs in development and more prescriptive regulatory guidance, the prosecution of quality, reproducibility, and heterogeneity of the formulations is ever-present. Here, we address specific challenges inherent to the pharmaceutical development of lipid-based therapeutics, with focus on the development of a robust manufacturing process, the setting of appropriate product specifications and controls, development of strategies to assess and ensure product stability, and the evaluation of product comparability throughout development. Self-assembly of LNPs via rapid precipitation requires several consecutive steps, including electrostatic capture and nucleation of OGN with lipid at high supersaturation in a uniform mixture, subsequent precipitate growth and lipid bilayer formation, and steric stabilization of the formed particles. Optimization of LNPs as therapeutic drug products is enabled by the development of structure-activity relationships linking particle physiochemical and macromolecular properties to manufacturing control and bioperformance. Methods by which LNP properties can be rationally manipulated are thus critical enablers of this fundamental knowledge build. To speed progression into the clinic and through development, lipid-based delivery systems require manufacturing and analytical tools beyond those typically required for conventional drugs. In-depth biophysical design and characterization capabilities, as well as engineering expertise, are required to build the fundamental understanding needed for successful clinical development. Here, through cross-functional collaboration, the team has developed a suite of advanced characterization tools to gain insight into the critical properties of the LNP system and connect these observations with variation of formulation process and composition. The state-of-the-art analytical tools guide manufacturing process development, formulation composition selection, and ensure process consistency. Ultimately, understandings obtained in this work can help to facilitate the development of LNPs as a well-defined pharmaceutical product.

Participants

Angela Wagner, PhD - Associate Principal Scientist, Sterile & Specialty, Merck Sharpe & Dohme

Please Choose Another Track

11:45am - 12:15pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Emerging Oligonucleotide Drug Discovery

ROOM 205B: Oral Delivery of PTH 1-34 in the Treatment of Hypoparathyroidism and Osteoporosis

11:45am - 12:15pm
Peptides TRACK: Peptide Discovery and Development

The oral delivery of macromolecules and biologic drugs has been limited by lack of absorption and degradation of such molecules in the Gastrointestinal tract. Entera Bio has developed a proprietary drug delivery platform which it has successful utilized for the oral delivery of PTH as well as many other biological molecules. In two Phase 2 clinical trials, Entera has successfully treated patients with surgical and idiopathic forms of the orphan disease hypoparathyroidism. There was a dramatic decrease in the need for exogenous calcium, a decrease in urinary calcium, as well as a stabilization of increase in serum calcium. Data from these studies has recently been published in the Journal of Bone & Mineral Research.

Participants

Phillip Schwartz, Ph.D. - President of R&D and EVP, Entera Bio Ltd.

ROOM 205C: CMC Regulatory Strategies for mRNA Vaccines

11:45am - 12:15pm
mRNA and Genome Editing TRACK: mRNA Discovery, Delivery and Translation to the Clinic

With the development of mRNA Vaccines and the role they have played in combatting the global COVID-19 pandemic, regulators are developing guidance to assist manufacturers to navigate the emerging regulatory landscape. This session will provide an in-depth analysis of the recently released draft WHO Guidance for CMC Regulatory Strategies for mRNA Vaccines

Participants

Paul Dawidczyk - VP, Regulatory Affairs CMC, Moderna Therapeutics

Transition to Spotlight Presentation Rooms

12:15pm - 12:20pm

ROOM 204AB: An Introduction to OCELOT™ System Control

12:20pm - 12:50pm
Spotlight Presentation 1

This presentation will offer a close look at our newly developed automation platform – OCELOT™ System Control – as it would be run on an Asahi Oligosynthesizer™. The presentation will highlight details on process steps, data tracking/analysis and other functionality, in addition to a brief overview of Asahi Kasei Bioprocess and our full oligo equipment offering.

Participants

Stefan Hyde - Automation Manager, Asahi Kasei Bioprocess America

ROOM 205A: Development, Optimization and Scale up of Therapeutic Peptides: Approaches to Solid Phase Synthesis

12:20pm - 12:50pm
Spotlight Presentation 2

The application and reliance on Solid Phase Peptide Synthesis (SPPS) has increased dramatically over recent years with the rise of peptides in diagnostic and therapeutic research. With this rapid increase in development activity, the ability of automated synthesis instruments to screen multiple reaction conditions simultaneously is essential. Here we describe the automated screening of multiple reaction conditions for the synthesis of a selection of therapeutic peptides, enabling the optimum synthetic conditions to be selected for each target, followed by the scale-up of the synthesis on a pilot-scale instrument.

Participants

Colton Quick - Product Specialist, Gyros Protein Technologies

ROOM 205B: Leveraging High Resolution Mass Spectrometry for the Analysis of Process and Product Related Impurities for Synthetic Oligonucleotides

12:20pm - 12:50pm
Spotlight Presentation 3

Therapeutic oligonucleotides represent an emerging drug modality. However, the characterization of impurities associated to oligos is challenging, as oligos are polar compounds, with a number of different product and process related impurities . Here, we present an Ion Pairing Reversed Phase/High Resolution Mass Spectrometry workflow and data analysis to facilitate characterization of therapeutic oligos.

Participants

Dr. Ramesh Indrakanti, PhD - Biologics Product Specialist/Senior Account Manager, Phenomenex

ROOM 205C: Plan Now or Fail Later: Why Raw Materials Matter

12:20pm - 12:50pm
Spotlight Presentation 4

Accelerate mRNA vaccines and therapeutics from pre-clinical development to commercialization by utilizing raw materials designed to meet critical process, scale, quality, and regulatory needs. During process development, teams usually focus on process fit. But raw material fit is equally as important when considering line-of-sight to GMP manufacturing. Choosing these appropriately saves time and money. This presentation discusses two real examples of how raw material selection either accelerated or stalled regulatory approvals.

Participants

Darwin Asa PhD - Global Market Development Manager - Nucleic Acid Therapeutics, Thermo Fisher Scientific

Networking Luncheon in Poster and Exhibit Hall

12:50pm - 1:55pm

ROOM 204AB: Chairman's Remarks

1:55pm - 2:00pm
Oligonucleotide CMC and Targeted Delivery TRACK: Sustainable Manufacturing of Oligos

Firoz D. Antia, Ph.D., Director, Antisense Oligonucleotide Process Development and Manufacturing, Biogen

Participants

Firoz Antia, PhD - Head of Oligonucleotide Process Development, Biogen

ROOM 205A: Chairman's Remarks

1:55pm - 2:00pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Nucleic Acid Chemical Modifications

Participants

Punit Seth, PhD - Vice President, Medicinal Chemistry, Ionis Pharmaceuticals

ROOM 205B: Chairman's Remarks

1:55pm - 2:00pm
Peptides TRACK: Targeted Radioligand Therapies and Tumor Imaging with Peptides and Other Molecules

Participants

Jesse Dong, Ph.D. - Vice President, Peptide Chemistry, BioNTech

ROOM 205C: Chairman's Remarks

1:55pm - 2:00pm
mRNA and Genome Editing TRACK: mRNA Vaccines for Non-COVID-19 Indications

Participants

Tasuku Kitada, Ph.D. - Co-founder, Director, President, and Head of R&D, Strand Therapeutics

ROOM 204AB: Sustainability Challenges and Opportunities in Oligonucleotide Manufacturing

2:00pm - 2:30pm
Oligonucleotide CMC and Targeted Delivery TRACK: Sustainable Manufacturing of Oligos

This presentation will discuss the work performed by the ACS Green Chemistry Institute Pharma Roundtable Oligonucleotide sub-team.

Participants

Ben Andrews, Ph.D. - Scientific Investigator, GlaxoSmithKline

ROOM 205A: Old Dogs and New Puppies of Chemical Modifications for RNA Therapeutics

2:00pm - 2:30pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Nucleic Acid Chemical Modifications

Chemical modifications aimed to improve metabolic stability, potency, specificity, and delivery of RNA Therapeutics will be summarized.

Participants

Muthiah (Mano) Manoharan, PhD - Senior Vice President of Drug Discovery, Alnylam Pharmaceuticals, Inc.

ROOM 205B: Tumor-targeted Radionuclides Using De Novo Macrocyclic Peptide-mimetics

2:00pm - 2:30pm
Peptides TRACK: Targeted Radioligand Therapies and Tumor Imaging with Peptides and Other Molecules

Targeted systemic delivery of potent radionuclides such as alpha-emitter Actinium-225 or beta-emitter Lutetium-177 offers robust potential to ablate solid tumors. Efforts in this field typically focus on repurposing small molecule enzyme inhibitors or engineering endogenous peptide GPCR ligands to enable therapeutic effect on tumors. The discussion will focus on radiotherapy starting with judicious tumor target selection, discovery of de novo chemical matter to those targets, and application of rigorous drug development principles to design precision radionuclide conjugates.

Participants

Deborah Charych, Ph.D. - Advisor and Co-founder, RayzeBio, Inc.

ROOM 205C: Development of Broadly Protective Influenza Vaccines Using Nucleoside-modified mRNA

2:00pm - 2:30pm
mRNA and Genome Editing TRACK: mRNA Vaccines for Non-COVID-19 Indications

Influenza virus infection causes significant morbidity and mortality every year. Seasonal influenza virus vaccines are employed to prevent disease, but they have limited effectiveness. Development of a universal influenza virus vaccine with the potential to elicit long-lasting, broadly cross-reactive immune responses is necessary for reducing influenza virus prevalence. We have utilized the flexible and very promising lipid nanoparticle-encapsulated, nucleoside-modified mRNA vaccine platform to deliver a combination of conserved influenza virus antigens and induce strong immune responses with substantial breadth and potency in a murine model.

Participants

Norbert Pardi, PhD - Research Assistant Professor of Medicine, University of Pennsylvania

ROOM 204AB: Small Materials – Drivers (or Disasters) for Oligonucleotide Sustainability

2:30pm - 3:00pm
Oligonucleotide CMC and Targeted Delivery TRACK: Sustainable Manufacturing of Oligos

Despite the complexity of oligonucleotide manufacturing, it is easy to overlook that the environmental footprint of oligonucleotides can be dominated by its constituent building blocks. I will consider specialist amidites and a conjugating agent to show the continued importance of small molecule processing to deliver a sustainable API as volumes increase.

Participants

Louis Diorazio, Ph.D. - Principal Scientist, Chemical Development, AstraZeneca

ROOM 205A: Chemical Modification of Guide RNA in Genome Editing

2:30pm - 3:00pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Nucleic Acid Chemical Modifications

Participants

Rubina Parmar, PhD - Director, Chemistry, Intellia Therapeutics

SESSIONS

MAIN CONFERENCE - DAY 2 - 22/09/2021

ROOM 205B: Development of Targeted Alpha Therapeutics for the Treatment of Solid Tumors

2:30pm - 3:00pm
Peptides TRACK: Targeted Radioligand Therapies and Tumor Imaging with Peptides and Other Molecules

Targeted alpha therapies (TATs) offer significant potential safety and potency advantages over other forms of anticancer radiotherapies. Fusion is developing a pipeline of proprietary and partnered TATs utilizing the patented Fast-Clear™ bifunctional chelator for the treatment of solid tumors. Fusion's technology can be applied to adapt a wide variety of targeting molecules into TATs, many of which have shown compelling preclinical efficacy and safety profiles. Fusion's lead TAT, FPI-1434, is currently in a Ph1 study.

Participants

John Rhoden, Ph.D. - Director, Preclinical Development, Fusion Pharmaceuticals

ROOM 205C: Fully Protective Anti-malaria Immunity by a Self-amplifying RNA Vaccine

2:30pm - 3:00pm
mRNA and Genome Editing TRACK: mRNA Vaccines for Non-COVID-19 Indications

Immunity against malaria infection is hindered by the inability of the host to develop immunologic memory. Effective memory can be induced in mice by vaccination with a self-amplifying RNA (saRNA) encoding PMIF, the Plasmodium-encoded orthologue of macrophage migration inhibitory factor, which acts during infection to suppress memory.

Participants

Dr. Richard Bucala, M.D., Ph.D. - Professor of Medicine, Pathology, and Epidemiology, Yale University School of Medicine

ROOM 204AB: Delivering the Promise of Nucleic Acid Medicines: The Challenge of Scale Cost and Sustainability

3:00pm - 3:30pm
Oligonucleotide CMC and Targeted Delivery TRACK: Sustainable Manufacturing of Oligos

To move nucleic acid medicines from rare disease/niche indications to large patient populations requires us to ask 2 questions: Can we make enough? Can we get the cost of goods down? Embedded in these 2 questions is the need to address sustainability. Multi tonne oligonucleotide synthesis will need multiple incremental innovations and some step changes. This talk will explore the economics of large scale oligonucleotide synthesis and options to get there.

Participants

Dr. Paul McCormac, , Ph.D. - SVP Technical Operations, LEXEO Therapeutics

ROOM 205A: Oligonucleotide Backbone Engineering for Enhancing Metabolic Stability, Potency, and Selectivity of siRNAs

3:00pm - 3:30pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Nucleic Acid Chemical Modifications

Oligonucleotide phosphate backbone modification without compromising potency is one of essential strategy to have metabolically stable and efficient therapeutic oligonucleotide in vivo. We will present novel advances of siRNA backbone modification that enhances metabolic stabilization, efficacy, and specificity.

Participants

Ken Yamada, Ph.D. - Assistant Professor, University of Massachusetts Medical School

ROOM 205B: Targeted Delivery of Radionuclides

3:00pm - 3:30pm
Peptides TRACK: Targeted Radioligand Therapies and Tumor Imaging with Peptides and Other Molecules

Targeted radioligand therapy uses peptide-based ligands designed to carry radioisotope payloads specifically to receptors that are highly expressed on cancer cells. When administered systemically, the ligand rapidly seeks out cancer cells and delivers the radioisotope payload in a manner that spares healthy tissue. This talk will review recent developments in tumor targeting and radioisotope technology that are allowing the field to advance rapidly in the development of even safer and more effective radioligand therapies and enabling these therapies to move earlier in the treatment of cancer.

Participants

Justyna Kelly - VP, Medical Isotope Development and Operations, POINT Biopharma Inc.

ROOM 205C: mRNA Vaccines for Malaria

3:00pm - 3:30pm
mRNA and Genome Editing TRACK: mRNA Vaccines for Non-COVID-19 Indications

Malaria affects hundreds of millions each year and efforts to develop effective vaccines have only been partially successful. Targeting mosquito stage of the parasite to block its transmission can be an effective strategy to combat the disease. We are exploring mRNA technology for malaria transmission blocking vaccine development.

Participants

Puthupparampil Scaria, Ph.D. - Head, Conjugation Group, NIAID/NIH

Networking Refreshment Break in Poster and Exhibit Hall

3:30pm - 4:00pm
Oligonucleotide CMC and Targeted Delivery TRACK: Sustainable Manufacturing of Oligos

Networking Refreshment Break in Poster and Exhibit Hall

3:30pm - 4:00pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Nucleic Acid Chemical Modifications

Networking Refreshment Break in Poster and Exhibit Hall

3:30pm - 4:00pm
Peptides TRACK: Targeted Radioligand Therapies and Tumor Imaging with Peptides and Other Molecules

Networking Refreshment Break in Poster and Exhibit Hall

3:30pm - 4:00pm
mRNA and Genome Editing TRACK: mRNA Vaccines for Non-COVID-19 Indications

ROOM 204AB: Development of a Scalable Synthesis Process for Oligonucleotides

4:00pm - 4:30pm
Oligonucleotide CMC and Targeted Delivery TRACK: Sustainable Manufacturing of Oligos

A practical and scalable convergent liquid phase synthesis of a full-length antisense oligonucleotide has been developed. The synthesis design and some key technical breakthroughs will be discussed.

Participants

Xianglin Shi - Principal Scientist, Biogen

ROOM 205A: Redefining the Chemical Space for Antisense Therapeutics

4:00pm - 4:30pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Nucleic Acid Chemical Modifications

Advancements in the chemical design of oligonucleotide drugs have improved potency, safety and duration leading to better clinical outcomes. A number of building blocks including backbone and sugar modifications have been used to enhance the therapeutic properties of nucleic acid therapeutics. Efforts to expand the boundaries of existing chemical space to further enhance the properties of antisense therapeutics will be presented.

Participants

Punit Seth, PhD - Vice President, Medicinal Chemistry, Ionis Pharmaceuticals

SESSIONS

MAIN CONFERENCE - DAY 2 - 22/09/2021

ROOM 205B: New Peptide Receptor Radionuclide Therapy Theranostic Indications

4:00pm - 4:30pm
Peptides TRACK: Targeted Radioligand Therapies and Tumor Imaging with Peptides and Other Molecules

Novel theranostics enabling the application of molecular medicine with small molecules as well as MAbs for tumor targeted therapy may enable non-invasive diagnosis and determination of efficacy of treatment, and for some cures for oncological, neurological, and infectious diseases, including COVID-19.

Participants

Dr. Stanley Satz, Ph.D. - Chief Scientific Officer, Advanced Innovative Partners, Inc.

ROOM 205C: RNActive® - CureVac's mRNA Vaccines Against Different Pathogens

4:00pm - 4:30pm
mRNA and Genome Editing TRACK: mRNA Vaccines for Non-COVID-19 Indications

Participants

Janine Mühe, PhD - Scientist, Infectious Diseases Research, CureVac AG

ROOM 204AB: In Defense of Solid-Phase Oligonucleotide Synthesis (Costs/Strategic/Technical)

4:30pm - 5:00pm
Oligonucleotide CMC and Targeted Delivery TRACK: Sustainable Manufacturing of Oligos

As the oligonucleotide field has grown, the standard solid-phase synthesis approach to large-scale manufacturing has been subjected to increased scrutiny and criticism. It is almost certain however, that the first metric ton of oligonucleotide will be produced using solid phase synthesis. Solid-phase synthesis is ideally suited for the development of platform manufacturing processes, which allow oligonucleotide sponsor companies and contract manufacturing organizations to scale-up rapidly. The presentation will address recent criticisms of solid-phase synthesis and identify important areas for research to ensure the industry meets the inevitable metric ton demand.

Participants

Isaiah Cedillo - Director, Manufacturing & Operations, Ionis Pharmaceuticals

ROOM 205A: Sequence-specific Recognition of Double-stranded RNA by Cationic Nucleobase and Backbone-modified Peptide Nucleic Acids

4:30pm - 5:00pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Nucleic Acid Chemical Modifications

We discovered that triple-helical binding of PNA to double-stranded RNA structures inhibited translation of mRNA and maturation of pre-microRNA hairpins. This presentation will discuss our most recent results on sequence-specific recognition and functional modulation of complex RNA molecules and potential applications of this recognition in biomedical research and biotechnology.

Participants

Eriks Rozners, Ph.D. - Professor and Chair of Chemistry Department, Binghamton University

ROOM 205B: Journey Beyond the Margin: Development of Imaging System to Assist Tumor Resections

4:30pm - 5:00pm
Peptides TRACK: Targeted Radioligand Therapies and Tumor Imaging with Peptides and Other Molecules

A personal tragedy inspired hope and determination that drove the technological developments created at Lumicell. We embarked into this journey guided by experts in the fields of cancer biology, imaging and surgical oncology to understand the unmet needs in cancer surgery and how to cleverly address those needs. Lumicell decided that a complete solution was required to introduce a new paradigm: a method to label cancer and surrounding tissue so they produce a characteristic signal, a method to collect such signal directly from surgical cavity and a method to interpret that signal to provide actionable information to the surgeon in real time; and all of these completed with first and foremost, patient safety in mind. This talk summarizes the development of the LUM Imaging System – a cancer imaging peptide, a hand-held imager and detection software and how each component was designed to work with each from the ground-up. Special focus on regulatory aspects of our drug, including CMC, as well as the trajectory of the clinical trials will also be presented.

Participants

Jorge Ferrer, Ph.D. - SVP of Clinical Research and Strategy, Lumicell

ROOM 205C: Optimization of LNPs for Improved mRNA Vaccines

4:30pm - 5:00pm
mRNA and Genome Editing TRACK: mRNA Vaccines for Non-COVID-19 Indications

LNPs are the most advanced delivery system for mRNA vaccines. This talk will summarize different approaches used to optimize mRNA vaccine formulations.

Participants

Luis Brito, PhD - Senior Director, Novel Delivery Technologies, Moderna Therapeutics

ROOM 204AB: How Honeywell is Meeting the Need For Sustainable and Cost-Effective Solvent and Reagent Technology

5:00pm - 5:30pm
Oligonucleotide CMC and Targeted Delivery TRACK: Sustainable Manufacturing of Oligos

The Burdick & JacksonTM BioSynTM product line of solvents and reagents by Honeywell has evolved in close collaboration with the oligo synthesis community worldwide. With substantial technological insight emphasized by several patents in the field, Honeywell continues investing in sustainable and cost-effective solvent and reagent technology to meet the growing demands of the oligo industry from laboratory to production scale. This presentation will provide an overview of Honeywell product offerings that enable our customers to optimize synthesis efficiency and costs while reducing their environmental footprint and drive supply security. During the presentation, we will also introduce an innovative and patented process technology for solvent recovery and reuse in oligonucleotide synthesis.

Participants

Aparajita Kapoor - Director of Offering Management, Honeywell

ROOM 205A: Exploring New Oligonucleotide Backbone Chemistries and Their Deployment to Improve the Properties of Stereopure Oligonucleotides

5:00pm - 5:30pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Nucleic Acid Chemical Modifications

We have expanded our investigations beyond phosphorothioate (PS) and phosphodiester (PO) backbones to include nitrogen-containing modifications, called PN backbones. We apply PN backbone chemistry to stereopure PS-modified oligonucleotides across multiple modalities, including RNase H-mediated silencing and RNA editing with endogenous ADAR (adenosine deaminases acting on RNA) enzymes, illustrating that the inclusion of a few PN linkages generally improves the activity of stereopure oligonucleotides.

Participants

Chandra Vargeese, Ph.D. - SVP, Head of Drug Discovery, WAVE Life Sciences

Close of Track

5:00pm - 5:30pm
Peptides TRACK: Targeted Radioligand Therapies and Tumor Imaging with Peptides and Other Molecules

Close of Track

5:00pm - 5:30pm
mRNA and Genome Editing TRACK: mRNA Vaccines for Non-COVID-19 Indications

Party on Lawn D

5:30pm - 8:00pm

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
6:00AM	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run	**6:00am** - Morning Fun Run
7:00AM	**7:30am** - Registration **7:45am** - ROOM 204AB: Regulatory Experience Sharing: Excipients for use in Lipid Nanoparticles	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
8:00AM		**8:15am** - ROOM 204AB: Chairman's Remarks **8:20am** - ROOM 204AB: Analytical Method Development for Critical Impurities in Phosphoramidites **8:45am** - ROOM 204AB: Automated		**8:25am** - ROOM 205A: Chairman's Remarks **8:30am** - ROOM 205A: Targeting ANGPTL4 in the Liver with ASO – A Novel Treatment for Cardiometabolic Disease			**8:25am** - ROOM 205B: Chairman's Remarks **8:30am** - ROOM 205B: Treatment with Vasoactive Intestinal Peptide (aviptadil) is Life-saving in Patients with Critical COVID-19						**8:25am** - ROOM 205C: Chairman's Remarks **8:30am** - ROOM 205C: Programming mRNA for Cancer Immunotherapy	

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
		Online UPLC and Data Processing for Oligonucleotide Synthesis and Purification Monitoring												

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
9:00AM		**9:10am** - ROOM 204AB: Control of GalNAc siRNA Conjugates **9:35am** - ROOM 204AB: Suppressing the Inherent Reactivity of Labile Moieties during Purity and Identity Characterization		**9:00am** - ROOM 205A: U1 Adaptors Bioconjugates for Targeted Gene Silencing in Extrahepatic Tissues **9:30am** - ROOM 205A: A Novel, RNA-based Anti-viral Approach to Limit BK Virus Prop-			**9:00am** - ROOM 205B: Developing a Highly Conserved, Non-human-like, and Cross-reactive SARS-CoV-2 Peptide-based COVID-19 Vaccine **9:30am** - ROOM 205B: When the Process						**9:00am** - ROOM 205C: A Novel Vaccine Approach Using Messenger RNA-Lipid Nanoparticles: Preclinical and Clinical Perspective **9:30am** - ROOM 205C: Preclinical Immuno-	

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
				agation in Immunocompromised Patients			Doesn't Fit - 8 Weeks to Build a Purification Suite to Support A Critical Covid-19 Vaccine Adjuvant						genicity Characterization of ARCT-021 SARS-CoV-2 Vaccine	

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
10:00AM	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall **10:45am** - ROOM 204AB: Analytical Method Development of Complementary Related Organic Impurity Methods for	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall **10:45am** - ROOM 205A: Platform Optimization to Enable Therapeutic Development of ADAR-mediated RNA editing	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall **10:45am** - ROOM 205B: Solid-Phase Peptide Synthesis Using a Four-Dimensional Protecting Group Scheme	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall **10:45am** - ROOM 205C: Subcutaneous Administration of mRNA LNPs to Achieve Systemic Exposures of Protein	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
		Oligonucleotides												

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
11:00AM		**11:15am** - ROOM 204AB: Identification and Quantitation of Thermal Degradation Products of Antisense Oligonucleotides (ASOs) Enables Understanding of Degradation Mechanism		**11:15am** - ROOM 205A: Development of an Antisense Oligonucleotide, STK-001, for Dravet Syndrome **11:45am** - Please Choose Another Track		**11:15am** - ROOM 205B: Discovery and Optimization of Long-acting, Unimolecular Peptide Triple Agonists of the GLP-1, GIP, and Glucagon Receptors **11:45am** - ROOM 205B: Oral Delivery of							**11:15am** - ROOM 205C: A Platform Approach for mRNA Vaccine Development **11:45am** - ROOM 205C: CMC Regulatory Strategies for mRNA Vaccines	

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
		11:45am - ROOM 204AB: Advancing the Pharmaceutical Science of Lipid Delivery Systems and their Translation to the Clinic				PTH 1-34 in the Treatment of Hypoparathyroidism and Osteoporosis								

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIDES USA: Oligonucleotide & Peptide Therapeutics

Delivered as Hybrid Event from September 20-30, 2021

Live In-Person Experience Delivered September 20-23; On-demand experience Delivered September 28-30

Boston Convention and Exhibition Center

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
12:00PM	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:20pm** - ROOM 204AB: An Introduction to OCELOT™ System Control **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:20pm** - ROOM 205A: Development, Optimization and Scale up of Therapeutic Peptides: Approaches to Solid Phase Synthesis **12:50pm** -	**12:15pm** - Transition to Spotlight Presentation Rooms **12:20pm** - ROOM 205B: Leveraging High Resolution Mass Spectrometry for the Analysis of Process and Product Related Impurities for Synthet-	**12:15pm** - Transition to Spotlight Presentation Rooms **12:20pm** - ROOM 205C: Plan Now or Fail Later: Why Raw Materials Matter **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
										Networking Luncheon in Poster and Exhibit Hall	ic Oligonucleotides **12:50pm** - Networking Luncheon in Poster and Exhibit Hall			
1:00PM			**1:55pm** - ROOM 204AB: Chairman's Remarks		**1:55pm** - ROOM 205A: Chairman's Remarks			**1:55pm** - ROOM 205B: Chairman's Remarks						**1:55pm** - ROOM 205C: Chairman's Remarks

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
2:00PM			**2:00pm** - ROOM 204AB: Sustainability Challenges and Opportunities in Oligonucleotide Manufacturing **2:30pm** - ROOM 204AB: Small Materials – Drivers (or Disasters) for		**2:00pm** - ROOM 205A: Old Dogs and New Puppies of Chemical Modifications for RNA Therapeutics **2:30pm** - ROOM 205A: Chemical Modification of Guide RNA in Genome Editing			**2:00pm** - ROOM 205B: Tumor-targeted Radionuclides Using De Novo Macrocyclic Peptide-mimetics **2:30pm** - ROOM 205B: Development of Targeted Alpha Therapeutics for the						**2:00pm** - ROOM 205C: Development of Broadly Protective Influenza Vaccines Using Nucleoside-modified mRNA **2:30pm** - ROOM 205C: Fully Protective Anti-malaria Immunity by a Self-amplifying

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
			Oligonucleotide Sustainability					Treatment of Solid Tumors						RNA Vaccine

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
3:00PM			**3:00pm** - ROOM 204AB: Delivering the Promise of Nucleic Acid Medicines: The Challenge of Scale Cost and Sustainability **3:30pm** - Networking Refreshment Break in Poster and Exhibit Hall		**3:00pm** - ROOM 205A: Oligonucleotide Backbone Engineering for Enhancing Metabolic Stability, Potency, and Selectivity of siRNAs **3:30pm** - Networking Refreshment Break in Poster			**3:00pm** - ROOM 205B: Targeted Delivery of Radionuclides **3:30pm** - Networking Refreshment Break in Poster and Exhibit Hall						**3:00pm** - ROOM 205C: mRNA Vaccines for Malaria **3:30pm** - Networking Refreshment Break in Poster and Exhibit Hall

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
					and Exhibit Hall									

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
4:00PM			**4:00pm** - ROOM 204AB: Development of a Scalable Synthesis Process for Oligonucleotides **4:30pm** - ROOM 204AB: In Defense of Solid-Phase Oligonucleotide Synthesis (Costs/		**4:00pm** - ROOM 205A: Redefining the Chemical Space for Antisense Therapeutics **4:30pm** - ROOM 205A: Sequence-specific Recognition of Double-stranded RNA by			**4:00pm** - ROOM 205B: New Peptide Receptor Radionuclide Therapy Theranostic Indications **4:30pm** - ROOM 205B: Journey Beyond the Margin: Development of Imaging System to Assist Tu-						**4:00pm** - ROOM 205C: RNActive® - CureVac's mRNA Vaccines Against Different Pathogens **4:30pm** - ROOM 205C: Optimization of LNPs for Improved mRNA Vaccines

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
			Strategic/ Technical)		Cationic Nucleobase and Backbone-modified Peptide Nucleic Acids			mor Resections						

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
5:00PM	**5:30pm** - Party on Lawn D	**5:30pm** - Party on Lawn D	**5:00pm** - ROOM 204AB: How Honeywell is Meeting the Need For Sustainable and Cost-Effective Solvent and Reagent Technology **5:30pm** - Party on Lawn D	**5:30pm** - Party on Lawn D	**5:00pm** - ROOM 205A: Exploring New Oligonucleotide Backbone Chemistries and Their Deployment to Improve the Properties of Stereopure Oligonucleotides **5:30pm** - Party on	**5:30pm** - Party on Lawn D	**5:30pm** - Party on Lawn D	**5:00pm** - Close of Track **5:30pm** - Party on Lawn D	**5:30pm** - Party on Lawn D	**5:30pm** - Party on Lawn D	**5:30pm** - Party on Lawn D	**5:30pm** - Party on Lawn D	**5:30pm** - Party on Lawn D	**5:00pm** - Close of Track **5:30pm** - Party on Lawn D

SCHEDULE

MAIN CONFERENCE - DAY 2 - 22/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: ANALYTICS OF OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: SUSTAINABLE MANUFACTURING OF OLIGOS	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: EMERGING OLIGONUCLEOTIDE DRUG DISCOVERY	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: NUCLEIC ACID CHEMICAL MODIFICATIONS	PEPTIDES TRACK: PEPTIDE DISCOVERY AND DEVELOPMENT	PEPTIDES TRACK: PEPTIDES FOR COVID-19 THERAPY	PEPTIDES TRACK: TARGETED RADIOLIGAND THERAPIES AND TUMOR IMAGING WITH PEPTIDES AND OTHER MOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: MRNA DISCOVERY, DELIVERY AND TRANSLATION TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MRNA VACCINES FOR NON-COVID-19 INDICATIONS
					Lawn D									

SESSIONS

MAIN CONFERENCE - DAY 3 - 23/09/2021

Registration

7:30am - 7:45am

ROOM 205A: LGC's NAT Extended Services: Physicochemical Property Characterization of an Oligonucleotide

7:45am - 8:15am
Breakfast Spotlight Presentations 2

Characterization of the solid state properties of multiple batches of a 90mer Aptamer has been undertaken. LGC intends to shed light into the following physico-chemical properties of the solid oligonucleotide, such as: collapse temperature (Tc), eutectic temperature (Teu), skin formation potential, annealing effects: on “ice” structure, particle size, surface area, solute crystallization, critical temperature(s), relative rates of drying for different formulations, or for the same formulation at different temperatures or vacuum levels. These investigations do not only lead to a comprehensive analysis of the freeze dried oligonucleotide, beyond, it may serve as an rationale for the design and optimization of the lyophilization step and offering tool on hands to have full control on the lyo process, an underestimated part of the oligonucleotide production process. In addition, from a CMC perspective, it will provide a comprehensive CMC package for the IND filing.

Participants

Pierre Barratt - Director CMC, LGC

Juergen Mueller, PhD - Commercial and Strategic Development Director Oligo-therapeutics, LGC Axolabs

ROOM 205C: Long-Acting Injectable Microparticle Formulations

7:45am - 8:15am
Breakfast Spotlight Presentations 3

Long-acting injectables (LAI) have been around for decades for the delivery of small molecules and peptides to treat chronic and site-specific diseases. However, when it comes to more sensitive biological therapeutics the classical polylactide and polylactide/glycolide based systems suffer from several limitations (e.g. uncontrolled release kinetics, in situ pH drop, protein degradation) making them unsuitable. The SynBiosysÂ® biodegradable polymeric microparticle technology combines all the features required for LAI formulations for biologics. Through case studies we will showcase sustained release formulations for peptides and proteins and demonstrate their potential via extensive in vitro and in vivo characterization.

Participants

Lyndon Salins - Application Manager, Biomolecular/Liquid Formulati, MilliporeSigma

ROOM 204AB: Chairman’s Remarks

8:25am - 8:30am
Oligonucleotide CMC and Targeted Delivery TRACK: Targeted Delivery of Therapeutic Oligonucleotides

Participants

Muthiah (Mano) Manoharan, PhD - Senior Vice President of Drug Discovery, Alnylam Pharmaceuticals, Inc.

ROOM 205A: Chairman’s Remarks

8:25am - 8:30am
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotides for CNS, Skin Cancer, NAFLD and Other Indications

Participants

Yogesh Sanghvi, PhD - President, Rasayan Inc.

ROOM 205B: Chairman’s Remarks

8:25am - 8:30am
Peptides TRACK: Strategies for Peptide CMC

Participants

Lael Cheung - Business Development Manager, Bachem Americas

ROOM 205C: Chair Remarks

8:25am - 8:30am
mRNA and Genome Editing TRACK: Genome Editing Advances from Preclinical to the Clinic

Participants

Cecilia Fernández, Ph.D. - VP of Strategic Planning and Operations, Chroma Medicine

ROOM 204AB: Targeted Delivery of Base Editors to Hepatocytes in vivo

8:30am - 9:00am
Oligonucleotide CMC and Targeted Delivery TRACK: Targeted Delivery of Therapeutic Oligonucleotides

CRISPR base editing holds potential to permanently modify disease-causing genes. Delivery of base editors to tissue is vital for developing therapeutics. RNA delivery using ionizable lipid nanoparticles (iLNP) to liver is facilitated by endogenous ApoE protein and LDLR on hepatocytes. We demonstrate robust liver delivery of base editors encapsulated iLNPs to an LDLR-deficient disease model using a targeting ligand.

Participants

Kallanthottathil G. Rajeev, PhD - Vice Presideent, CMC, Verve Therapeutics

ROOM 205A: Enhancing the Pharmacologic Profiles of CNS Targeting Therapeutic Oligonucleotides

8:30am - 9:00am
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotides for CNS, Skin Cancer, NAFLD and Other Indications

Chemically modified stereopure oligonucleotides hold great promise for treating human disease. Backbone chemistry modifications called PN backbones, where a nitrogen-containing moiety replaces a non-bridging oxygen in the phosphodiester bond, significantly impact the pharmacological properties of CNS targeting stereopure oligonucleotides, enhancing potency, distribution, and durability of effect across a range of targets and model systems. These novel modifications, combined with sequence selection, 2’-modification, and chirally controlled phosphorothioate (PS) modification, have become a critical tool for optimization of preclinical pharmacology and rational drug design in targeting neurological diseases.

Participants

Elena Dale, PhD - Senior Director, Head of CNS Biology, Wave Life Sciences

ROOM 205B: Refractive Index: The Ultimate Tool for Real-Time Monitoring of Solid-Phase Peptide Synthesis. Greening the Process

8:30am - 9:00am
Peptides TRACK: Strategies for Peptide CMC

The refractive index (RI) of a liquid provides key information about its physical properties and the composition of any solution. Here we provide the first demonstration that the RI is a Process Analytical Tool (PAT) suitable for the real-time monitoring of Solid-Phase Peptide Synthesis (SPPS). This strategy comprises three steps: coupling, deprotection, and washes, that can be monitored on-line by refractometry. Given that monitoring capacity helps to the determination of the endpoint of the reactions and the optimization of all synthetic steps, it has a direct impact on the consumption of reagents, solvents, and time, thereby contributing to greener SPPS.

Participants

Beatriz De La Torre, Ph.D. - Research Professor, Laboratory of Medicine and Med, University Of KwaZulu-Natal

ROOM 205C: Clinical Progress in Genome Editing

8:30am - 9:00am
mRNA and Genome Editing TRACK: Genome Editing Advances from Preclinical to the Clinic

Participants

Laura Sepp-Lorenzino, Ph.D. - Chief Scientific Officer, Intellia Therapeutics

SESSIONS

MAIN CONFERENCE - DAY 3 - 23/09/2021

ROOM 204AB: Improved Delivery of RNA Therapeutics in the Eye, Muscle, and Beyond Using Fatty-Acid Conjugation

9:00am - 9:30am
Oligonucleotide CMC and Targeted Delivery TRACK: Targeted Delivery of Therapeutic Oligonucleotides

The functional delivery of RNA therapeutics to many tissues remains a key technological hurdle. While lipid and fatty-acid conjugation has previously been explored as a potential solution to oligonucleotide bioavailability, this strategy remains underutilized. DTx Pharma is building a platform of optimized lipid and fatty acid conjugation strategies to improve the functional distribution and uptake of RNA therapeutics. Recent efforts have led to the identification of fatty-acid motifs, in particular motifs containing clusters of two or more fatty acids, that enable efficient, durable, and functional delivery to a wide variety of tissues and cell types.

Participants

Charles Allerson, Ph.D. - Vice President of Chemistry & Drug Development, DTx Pharma

ROOM 205A: Treating Repeat Expansion Disorders by Stopping Somatic Expansion

9:00am - 9:30am
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotides for CNS, Skin Cancer, NAFLD and Other Indications

Somatic expansion of short DNA repeats drives a number of devastating CNS disorders, and requires key members of the DNA Damage Response (DDR) pathway. We will present an overview of our DDR targeting oligonucleotide approach, the development of our lead clinical candidate TTX-3360, and our clinical trial progress in Huntington's Disease.

Participants

Satya Kuchimanchi, PhD - Vice President, CMC, Triplet Therapeutics

ROOM 205B: Pegcetacoplan – Towards the Implementation of an Efficient Manufacturing Process for a PEG-ylated Peptide at an Industrial Scale, and the Associated Strategy for a Regulatory Approval

9:00am - 9:30am
Peptides TRACK: Strategies for Peptide CMC

We present new developments for the manufacturing of pegcetacoplan and illustrate how a commercial manufacturing process for a PEG-ylated peptide is achieved at the industrial scale with the required efficiencies. Solutions for eliminating the main bottleneck operations and resulting efficiency gains as well as strategy for regulatory approval are discussed.

Participants

Dr. Philipp Wenter, Ph.D. - Senior Director of Drug Substance Manufacturing, Technical Operations, Apellis Pharmaceuticals

ROOM 205C: CRISPR in the Clinic – from Hemoglobinopathy to Immunoocology

9:00am - 9:30am
mRNA and Genome Editing TRACK: Genome Editing Advances from Preclinical to the Clinic

CRISPR is a revolutionary technology that has the potential to transform medicine. CRISPR Therapeutics has leveraged this technology to bring investigational CRISPR-based products to the clinic. Early clinical data from CTX001 show transfusion independence and freedom from vaso-occlusive crises in thalassemia and sickle cell patients, respectively. In addition, initial clinical data from the CARBON study with CTX110 has shown dose-dependent efficacy and response rates that are comparable to the early autologous CAR-T trials. The path to bring these novel, investigational agents into clinical trials will be presented.

Participants

Dr. Tony Ho, M.D. - Executive Vice President, Research and Development, CRISPR Therapeutics

ROOM 204AB: Development of An antimiR-based Therapy to Treat Myotonic Dystrophy Disease

9:30am - 10:00am
Oligonucleotide CMC and Targeted Delivery TRACK: Targeted Delivery of Therapeutic Oligonucleotides

Myotonic Dystrophy type 1 (DM1) is a chronically debilitating rare genetic disease that originates from an expansion of a non-coding CTG repeat in the DMPK gene. The expansion becomes pathogenic when DMPK transcripts contain 50 or more repetitions due to the sequestration of the muscleblind-like (MBNL) family of proteins. Depletion of MBNLs causes alterations in splicing patterns in transcripts that contribute to clinical symptoms such as myotonia and muscle weakness and wasting. We previously found that miR-23b directly regulates MBNL1 in DM1 myoblasts and mice and that antisense technology ("antagomiRs") blocking this miRNA boosts MBNL1 protein levels. Here we show the therapeutic effect over time in response to administration of antagomiR-23b as a treatment in HSALR mice. Subcutaneous administration of antagomiR-23b up-regulated the expression of MBNL1 protein and rescued splicing alterations, grip strength, and myotonia, in a dose-dependent manner. The pharmacokinetic and preliminary toxicity data obtained provide further evidence that miR-23b could be a valid therapeutic target for DM1.

Participants

Beatriz Llamusi, Ph.D. - Chief Executive Officer & Co Founder, Arthex Biotech

ROOM 205A: Technology-derived Antisense Oligonucleotide Therapeutics for Diseases of Excitable Cells

9:30am - 10:00am
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotides for CNS, Skin Cancer, NAFLD and Other Indications

We have developed an integrated platform for therapeutic discovery across three core technology axes: human cell-based models of disease, all-optical electrophysiology engineering and machine learning analytics of large cellular data sets. Our platform enables high-throughput assessment of therapeutic antisense oligonucleotides (ASOs), including in vitro phenotype rescue in neuronal models of diseases as well as potential sequence-specific neurotoxicities. We are applying these tools to severe monogenic epilepsies, chronic pain, and other CNS-based disorders.

Participants

Graham T. Dempsey, PhD - Chief Scientific Officer, Q-State Biosciences

ROOM 205B: Synthesis and Formulation of Amphiphile Modified mKRAS Peptides

9:30am - 10:00am
Peptides TRACK: Strategies for Peptide CMC

Speaker TBA

Participants

Charles Chase, PhD - Vice President, Pharmaceutical Development, Elicio Therapeutics

ROOM 205C: In vivo CRISPR Base Editing of PCSK9 Durably Lowers Cholesterol in Primates

9:30am - 10:00am
mRNA and Genome Editing TRACK: Genome Editing Advances from Preclinical to the Clinic

Gene-editing technologies, including CRISPR-Cas9 nucleases and CRISPR base editors, have the potential to permanently modify disease-causing genes in human patients. CRISPR base editors delivered in vivo using lipid nanoparticles (LNP) can efficiently and precisely modify disease-related genes in living NHPs. We observed near-complete knockdown of PCSK9 in the liver following a single infusion of LNP, with a concomitant ≈90% reduction in blood PCSK9 protein and ≈60% reduction in blood low-density lipoprotein cholesterol (LDL-C), all of which remain stable for at least 10 months following a single treatment. We provide proof-of-concept for a once-and-done approach to reduce cholesterol and treat coronary heart disease, the leading cause of death worldwide.

Participants

Andrew Bellinger, M.D., Ph.D. - Chief Scientific Officer, Verve Therapeutics

Networking Refreshment Break in Poster and Exhibit Hall

10:00am - 10:45am
Oligonucleotide CMC and Targeted Delivery TRACK: Targeted Delivery of Therapeutic Oligonucleotides

Networking Refreshment Break in Poster and Exhibit Hall

10:00am - 10:45am
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotides for CNS, Skin Cancer, NAFLD and Other Indications

Networking Refreshment Break in Poster and Exhibit Hall

10:00am - 10:45am
Peptides TRACK: Strategies for Peptide CMC

Networking Refreshment Break in Poster and Exhibit Hall

10:00am - 10:45am
mRNA and Genome Editing TRACK: Genome Editing Advances from Preclinical to the Clinic

Panel Discussion: Exploring the Massachusetts Biotech Ecosystem; Fostering Innovation, Spinning out, & Maximising Growth Potential

10:30am - 10:45am
Exhibition Hall Content

- Reviewing the Incubator model and advantages of going down this path.
- Best practices for spinning out from academia
- Considering manufacturing capacity early to secure investment and maximise growth potential.
- Working with corporate partners to move towards commercialization

Participants

Shashi Murthy, PhD - Founder and CTO, Flaskworks

Brian Goodman, PhD - Principal, MPM Capital, Inc.

Vinit Nijhawan - Managing Director, MassVentures, Lecturer, Boston University, USA

Peter Strack - Scientific VP, Integrative Sciences, Bristol Myers Squibb, USA

ROOM 204AB: Muscle Targeted Delivery of Therapeutic Oligonucleotides with the FORCE™ Platform

10:45am - 11:15am
Oligonucleotide CMC and Targeted Delivery TRACK: Targeted Delivery of Therapeutic Oligonucleotides

We developed the FORCE™ platform to enable effective antibody-mediated delivery of oligonucleotides to skeletal, cardiac, and smooth muscles. FORCE is designed to deliver targeted, redosable and titratable therapies for the treatment of genetic muscle disorders. Preclinical data demonstrate potent and durable therapeutic effect in disease models in vitro and in vivo.

Participants

Oxana Beskrovnaya, Ph.D. - Chief Scientific Officer, Dyne Therapeutics

ROOM 205A: Identification and Optimization of a Minor Allele-specific siRNA to Prevent PNPLA3 I148M-driven Nonalcoholic Fatty Liver Disease

10:45am - 11:15am
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotides for CNS, Skin Cancer, NAFLD and Other Indications

Human genome wide association studies (GWAS) confirm the association of the rs738409 single nucleotide polymorphism (SNP) in the gene encoding patatin like phospholipase domain containing 3 (PNPLA3) with hepatic steatosis and its sequelae. Expression of the PNPLA3I148M mutant protein results in impaired lipid metabolism and accumulation of triglycerides on hepatic lipid droplets. Reducing expression of the mutant protein via antisense therapy served to alleviate liver inflammation and fibrosis in Pnpla3I148M knock-in mice fed a nonalcoholic steatohepatitis (NASH)-inducing diet (1). While Pnpla3-deficient mice do not display an adverse phenotype (2, 3), the safety of knocking down endogenous PNPLA3 in humans remains unknown. To expand the scope of a potential targeted nonalcoholic fatty liver disease (NAFLD) therapeutic to both homozygous and heterozygous PNPLA3 rs738409 populations, we sought to identify a minor allele-specific siRNA that could prevent human PNPLA3I148M-driven NASH phenotypes. Limiting our search to SNP-spanning triggers, a series of chemically modified siRNA were tested in vitro for activity and selectivity toward PNPLA3 rs738409 mRNA. Conjugation of the siRNA to a triantennary N-acetylgalactosamine (GalNAc) ligand enabled in vivo screening using adeno-associated virus to over-express human PNPLA3I148M versus human PNPLA3I148I in mouse livers. Structure-activity relationship optimization yielded potent and minor allele-specific compounds that achieved high levels of mRNA and protein knockdown of human PNPLA3I148M but not PNPLA3I148I. Testing of the minor allele-specific siRNA in PNPLA3I148M-expressing mice fed a NASH-inducing diet prevented PNPLA3I148M-driven disease phenotypes, thus demonstrating the potential of a precision medicine approach to treating NAFLD.

Participants

Justin Murray, PhD - Senior Principal Scientist, Selection & Modality Engineering, Amgen, Inc.

ROOM 205B: DMF Replacement in SPPS: Key Findings and Latest Results

10:45am - 11:15am
Peptides TRACK: Strategies for Peptide CMC

The main SPPS solvent for complex peptides is DMF. Even though DMF is an excellent and well-established solvent for SPPS, replacement for a less toxic solvent (or solvent mixture) is advisable in the light of recent REACH regulations. Bachem has been working on that topic for some time now. Results have been published recently together with our cooperation partners. In this presentation, we will present our key findings and latest results.

Participants

Stefan Eissler, PhD - Director, API Manufacturing, Bachem AG

ROOM 205C: Introduction to Prime Editing, The Search-and-replace Genome Editing Technology

10:45am - 11:15am
mRNA and Genome Editing TRACK: Emerging Genome Editing Technologies

Prime editing, which precisely installs mutations, insertions, or deletions at targeted genomic loci without DNA repair templates or double-strand breaks, is a promising technology for correction of pathological mutations or genetic variants to treat disease. A prime editor comprises an RNA-guided DNA-nickase and a reverse transcriptase fusion protein complexed to a prime editing gRNA (pegRNA). We will discuss its therapeutic potential.

Participants

Jennifer Gori - VP, Research, Prime Medicine

Panel Discussion: Exploring the Massachusetts Biotech Ecosystem; Fostering Innovation, Spinning out, & Maximising Growth Potential (CONTINUED)

10:45am - 11:15am
Exhibition Hall Content

- Reviewing the Incubator model and advantages of going down this path.
- Best practices for spinning out from academia
- Considering manufacturing capacity early to secure investment and maximise growth potential.
- Working with corporate partners to move towards commercialization

Participants

Shashi Murthy, PhD - Founder and CTO, Flaskworks

Brian Goodman, PhD - Principal, MPM Capital, Inc.

Vinit Nijhawan - Managing Director, MassVentures, Lecturer, Boston University, USA

Peter Strack - Scientific VP, Integrative Sciences, Bristol Myers Squibb, USA

ROOM 204AB: Engineering Antibody Oligonucleotide Conjugates (AOCs): Taking Receptor-Mediated Uptake One Step Further

11:15am - 11:45am
Oligonucleotide CMC and Targeted Delivery TRACK: Targeted Delivery of Therapeutic Oligonucleotides

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. Receptor-mediated uptake through bioconjugation oligonucleotides has changed that. 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.

Participants

Arthur Levin, PhD - Chief Scientific Officer, Avidity Biosciences, Inc.

ROOM 205A: DCR-AUD: Applying GalXC™ RNAi Technology to the Treatment of Alcohol Use Disorder (AUD)

11:15am - 11:45am
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotides for CNS, Skin Cancer, NAFLD and Other Indications

DCR-AUD, Dicerna's developmental GalXC™ RNAi candidate for alcohol use disorder, targets aldehyde dehydrogenase 2, an enzyme in the biochemical pathway that converts ethanol to acetic acid in the liver, which detoxifies acetaldehyde by converting it to acetate. Preclinical research showed DCR-AUD reduced ALDH2 expression in the livers of mice, non-human primates, and human cells in culture. In this presentation, Dr. Brown will discuss DCR-AUD's preclinical discovery and entry into nonclinical development.

Participants

Bob Brown, PhD - Chief Scientific Officer, EVP, R&D, Dicerna Pharmaceuticals

ROOM 205B: Nasal Glucagon: Overcoming Product Development Challenges

11:15am - 11:45am
Peptides TRACK: Strategies for Peptide CMC

Participants

Jeffrey Lampert - Research Scientist - API External Manufacturing TS, Eli Lilly and Company

ROOM 205C: Optimization of LNP for in vivo Base Editing in Liver

11:15am - 11:45am
mRNA and Genome Editing TRACK: Emerging Genome Editing Technologies

Base editing enables programmable single-base mutations in genomic DNA and has the potential to permanently cure serious genetic diseases. Realizing this potential requires development of safe and effective methods for delivery of base editing reagents to the intracellular compartments of target organs. LNPs are a clinically validated technology for delivery of RNA therapeutics. In this work, we have optimized LNPs for the delivery of mRNA encoding a base editor and guide RNA to hepatocytes.

Participants

Delai Chen, PhD - Associate Director Nanoparticle Formulation, Beam Therapeutics Inc.

Panel: Talent Acquisition, Development and Retention in Novel Therapeutics

11:15am - 12:00pm
Exhibition Hall Content

- Addressing key challenges in TA in novel therapeutics
- How can companies best work with personnel to develop talent into their next roles; ready now/ ready later talent.
- In a competitive area such as Boston, how should we strike a balance between working to retain talent and embracing and supporting personnel moving around.

Participants

Andrew Rigoglioso - Associate Director, Talent Acquisition, Flagship Pioneering

Juliette Hilliard - Director, TA, Novartis GT

Georgette Verdin - Chief HRO, Avrobio

Stacey Veysey - Director, Talent Acquisition, Vertex Pharmaceuticals, USA

ROOM 204AB: Extra-hepatic Delivery of Centyrin-targeted siRNA Conjugates

11:45am - 12:15pm
Oligonucleotide CMC and Targeted Delivery TRACK: Targeted Delivery of Therapeutic Oligonucleotides

Centyrins are ~10kDa proteins that combine the affinity and specificity properties of antibodies with significantly improved biophysical properties. Using large libraries of Centyrin variants, we have identified a panel of Centyrins that bind to human transferrin receptor 1 (hCD71) and have demonstrated potent functional delivery of oligonucleotide conjugates into skeletal and heart muscle while sparing knockdown in liver and kidney.

Participants

Karyn O'Neil, PhD - Chief Scientific Officer, Aro Biotherapeutics

ROOM 205A: Novel Dual Targeting siRNA Therapeutic Offers Innovative Solution for Derm-Oncology Treatment

11:45am - 12:15pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotides for CNS, Skin Cancer, NAFLD and Other Indications

Non-melanoma Skin Cancer patients currently have limited therapeutic options apart from surgical excision with associated risks and sub-optimal cosmetic appearance. A therapeutic that can achieve histological clearance and prevent scarring has the potential to have a profound impact on patient care. Sirnaomics will present clinical trial results showing STP705, used to target TGF-β1 and COX-2 siRNAs for the treatment of Nonmelanoma Skin Cancer, has demonstrated rates of histological clearance that rival surgical excision combined with improved cosmetic appearance.

Participants

Michael Molyneaux, M.D. - Chief Medical Officer, Sirnaomics

ROOM 205B: Impurity Control Strategy - Liraglutide Case Study

11:45am - 12:15pm
Peptides TRACK: Strategies for Peptide CMC

Liraglutide is a peptide utilized for the treatment of Diabetes. In this case study, analytical control strategy considerations are discussed in traversing from the innovators recombinant (bio-synthetic) Liraglutide to chemically synthesized Liraglutide (SPPS). Special emphasis is given on the control of deletion impurities through process optimization. A diastereomer control strategy was developed that utilized batch data and an occurrence risk assessment to subsequently demonstrate detectability by the analytical control strategy.

Participants

Eran Benjamin, Ph.D. - Global Director, QC AD, PolyPeptide Group

ROOM 205C: An Engineered AsCas12A Nuclease Facilitates the Rapid Generation of Therapeutic Cell Medicines

11:45am - 12:15pm
mRNA and Genome Editing TRACK: Emerging Genome Editing Technologies

This presentation will discuss these results: 1) Editing efficiencies reach nearly 100% at all sites examined in HSPCs, iPSCs, T cells, and NK cells using an engineered AsCas12a CRISPR nuclease. 2) High intrinsic specificity of Cas12a maintained in this high activity variant. 3) Achieved simultaneous targeting of three clinically relevant genes in T cells at >90% efficiency and demonstrated transgene knock-in efficiencies of up to 60% and dual transgene knock-in efficiencies approaching 40%. 4) Demonstrated site-specific knock-in of a CAR in NK cells, which affords enhanced anti-tumor NK cell recognition, potentially enabling the next generation of allogeneic cell-based therapies in oncology. 5) Leveraging this nuclease in EDIT-301 which is our clinical-stage gene editing program for sickle cell disease.

Participants

John Zuris, PhD - Associate Director, Editing Technologies, Editas Medicine

Transition to Spotlight Presentation Rooms

12:15pm - 12:20pm

ROOM 205A: Self-adjuvanting Lipid nanoparticles for Next Generation Nucleic Acid Vaccines

12:20pm - 12:50pm
Spotlight Presentation 2

In the development of nucleic acid vaccines, there is limited understanding of the mechanisms of antigen presentation and critical to long-term immunity. Lipid nanoparticles (LNP) composed of ionizable lipids are important components of such vaccines as they can convey and present the nucleic acid effectively to the immune system. NOF has built a platform of biodegradable, ionizable lipids with engineered properties to decrease systemic toxicity, improved endosomal escape and elicit potent T-cell responses. An update on the preclinical development and immunology of SS-Lipids will be presented.

Participants

Dr. Syed Reza - Scientific and Sales Consultant, NOF AMERICA CORPORATION

SESSIONS

MAIN CONFERENCE - DAY 3 - 23/09/2021

ROOM 205C: Arromer: Receptor Directed Delivery for Ultra-precise RNAi Multi-targeting

12:20pm - 12:50pm
Spotlight Presentation 3

One of the greatest barriers to successfully treating complex polygenic disease states, such as those that lead to cancer therapy resistance, has been the ability to target the right combination of genes and proteins selectively in precisely the tissue to avoid unwanted toxicity. Arromers are single agent chemically modified RNA oligonucleotides with a unique chimeric structure, which combines the precise selectivity of dual aptamer directed binding with the power and flexibility of multiple siRNA to silence the right combination of target genes in the right cell types. The Arromer technology has next generation pharmacological properties and multi-targeting features which make it a best in class therapeutic modality to address many unmet needs for complex polygenic diseases.

Participants

Spyro Mousses, PhD - CEO, Systems Oncology

ROOM 205B: Oligo Drug Delivery Systems: Evolution of the Formulation Development Landscape from Traditional Approaches to LNP-based Systems

12:20pm - 12:50pm
Spotlight Presentation 4

Oligonucleotides can be used to modulate gene expression via a range of processes including RNAi, target degradation by RNase H-mediated cleavage, splicing modulation, non-coding RNA inhibition, gene activation and programmed gene editing. As such, these molecules have potential therapeutic applications for a myriad of indications, with several oligonucleotide drugs recently gaining approval. However, despite recent technological advances, achieving efficient oligonucleotide delivery, particularly to extrahepatic tissues, remains a major translational limitation.

In fact, before oligos can become drugs, they must overcome a billion years of evolutionary defenses designed to keep invading nucleic acids on the outside of cells from getting to the inside of cells. Not surprisingly, significant effort has been placed in developing a wide array of delivery technologies. Foremost among these is the development of N-acetylgalactosamine (GalNAc), which is especially effective for delivering siRNA conjugates to the liver.

More recently, a very efficient drug delivery system has taken center-stage: Lipid NanoParticle (LNP) systems are currently the leading non-viral delivery systems for enabling the clinical potential of genetic drugs.

This presentation covers the evolution of formulation development for oligo-based drug products, starting from more traditional approaches to the latest formulation strategies, including LNPs.

Participants

Umberto Romeo - Head of R&D, Corden Pharma Caponago

Networking Luncheon in Poster and Exhibit Hall

12:50pm - 1:55pm

ROOM 204AB: Chairman's Remarks

1:55pm - 2:00pm
Oligonucleotide CMC and Targeted Delivery TRACK - Targeted Delivery of Therapeutic Oligonucleotides

Participants

Muthiah (Mano) Manoharan, PhD - Senior Vice President of Drug Discovery, Alnylam Pharmaceuticals, Inc.

ROOM 205A: Chairman's Remarks

1:55pm - 2:00pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotide CMC Strategies

Participants

Andrew Rodriguez, PhD - Assistant Director, Process Chemistry, Ionis Pharmaceuticals

ROOM 205B: Chairman's Remarks

1:55pm - 2:00pm
Peptides TRACK: Peptide Nucleic Acids

Participants

Marc Thibonnier, M.D. - Founder & President, AptamiR Therapeutics

ROOM 205C: Chairman's Remarks

1:55pm - 2:00pm
mRNA and Genome Editing TRACK: Manufacturing and Analytics for CRISPR Applications

Participants

Keith Jarvis - Senior Director, Editas Medicine

ROOM 204AB: Extra-hepatic Delivery

2:00pm - 2:30pm
Oligonucleotide CMC and Targeted Delivery TRACK - Targeted Delivery of Therapeutic Oligonucleotides

Participants

Haiyan Peng - Senior Scientist, Alnylam Pharmaceuticals

ROOM 205A: Improved Oxidizer Formulations for the Synthesis of PO/PS Mixed Backbone Oligonucleotides

2:00pm - 2:30pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotide CMC Strategies

Oxidation is conveniently accomplished during oligonucleotide synthesis with a solution of iodine in pyridine/water solvent, but problems can arise if the solution is not aged enough. In this presentation, we will discuss the problems associated with fresh oxidizer solution and new formulations that do not require aging.

Participants

Andrew Rodriguez, PhD - Assistant Director, Process Chemistry, Ionis Pharmaceuticals

ROOM 205B: 30 Years of Peptide Nucleic Acids and On the Road to Precision Antisense Antibiotics Against Multidrug Resistant Bacteria

2:00pm - 2:30pm
Peptides TRACK: Peptide Nucleic Acids

During the past 30 years peptide nucleic acids (PNA) have attracted attention and found applications in a multitude of scientific areas ranging from organic and physical chemistry over origin of life and nanotechnology to diagnostics and drug discovery. For many reasons drug discovery progress has been slow but has recently gained new momentum. This also concerns efforts towards novel precision antisense antibiotics. Multidrug-resistant Gram-negative bacteria pose an increasing threat to human health, and development of novel antibiotics would be one answer to this challenge. Most efforts to date have focused on development of broad spectrum antibiotics and unfortunately with limited success. We would argue that precision, narrow spectrum antibiotics optimized against resistant strains are more likely to succeed, both by directly addressing the challenge as well as limiting the risk of new resistance development and spreading. Thus, we are aiming at developing precision antisense antibiotics based on peptide nucleic acids (PNA) specifically targeting (essential) bacterial genes (1-7). PNA oligomers targeting the acpP gene and conjugated to bacterial penetrating peptides (BPP) (2-7), have provided antimicrobials showing (sub)micromolar antibacterial activity against Escherichia coli, Klebsiella pneumoniae, Acinetobacter baumannii and Pseudomonas aeruginosa (including multi resistant clinical isolates), as well as in vivo activity in mouse infection models. Based on recent in vitro as well as in vivo results the prospects of developing novel precision antibiotics against infections by multi resistant Gram-negative bacteria will be discussed.

Participants

Peter Nielsen, PhD - Professor, Center for Peptide-Based Antibiot, University of Copenhagen

ROOM 205C: IND-enabling Small-Scale Guide RNA Production Under GMP for CRISPR Based Cell Therapies

2:00pm - 2:30pm
mRNA and Genome Editing TRACK: Manufacturing and Analytics for CRISPR Applications

This presentation will focus on the appropriate scale, final purity release specifications and GMP compliance for internal small-scale guide RNA production necessary to support our pre-clinical programs. It will also highlight the quality management system we have created and the guide RNA production clean rooms we have implemented at our Boulder satellite location.

Participants

Keith Jarvis - Senior Director, Editas Medicine

ROOM 204AB: Redefining the chemical space for nucleic acid therapeutics

2:30pm - 3:00pm
Oligonucleotide CMC and Targeted Delivery TRACK - Targeted Delivery of Therapeutic Oligonucleotides

Phosphorothioate (PS) ASOs interact with plasma and cell-surface proteins that facilitates tissue distribution and cellular uptake. Delivery of PS ASOs to specific cell-types within tissues can be accomplished by targeting cell-surface receptors expressed in the cell-type of interest. Recent progress in delivery of ASOs to tissues beyond the liver will be presented.

Participants

Punit Seth, PhD - Vice President, Medicinal Chemistry, Ionis Pharmaceuticals

ROOM 205A: Impurity Strategy - Evaluating Consensus Impurity Lists

2:30pm - 3:00pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotide CMC Strategies

This presentation will focus on the creation and evaluation of “consensus impurity lists” for testing of oligonucleotide therapeutics. New approaches to the analysis of impurity data from orthogonal chromatographic methods will be discussed.

Participants

Matthias Kretschmer, PhD - Senior Director Analytical Sciences, Alnylam Pharmaceuticals

ROOM 205B: Molecular Bivalents for Recognition of RNA-repeated Expansion

2:30pm - 3:00pm
Peptides TRACK: Peptide Nucleic Acids

This talk highlights the latest results in our effort to develop a novel “Janus-base” (or JB) molecular platform for targeting RNA repeat expansion in a sequence-specific and selective manner, one that could be tailor-designed to bind to any repeat sequence. The newly designed “ligands” are relatively small in size (3 units in length). They bear a closer resemblance to small molecules than they do to oligonucleotides. However, unlike small molecules, they engage their targets in a sequence-specific and selective manner through bifacial H-bonding interactions with the adjoining nucleobases in both strands of RNA double helix. The work provides a proof-of-concept that such relatively small nucleic acid “ligands” could be developed for recognition of CUGexp-RNA transcripts.

Participants

Danith Ly, Ph.D. - Professor of Chemistry, Carnegie Mellon University

ROOM 205C: Gram-Scale Good Manufacturing Practice (GMP) Single Guide RNA for CRISPR-based Gene Editing

2:30pm - 3:00pm
mRNA and Genome Editing TRACK: Manufacturing and Analytics for CRISPR Applications

Over the past several years, CRISPR has revolutionized the field of gene editing. A major benefit of this technology is the ability to use ‘programmable’ single guide RNAs (sgRNAs) to precisely target areas of the genome for subsequent editing. However, this approach requires both high-quality and high purity sgRNAs to achieve optimal editing efficiency and obtaining these in sufficient quantities can be challenging. In this presentation, we demonstrate our current manufacturing capabilities and how they allow us to deliver up to multigram-quantity custom sgRNAs with the option to manufacture under either non-GMP or GMP conditions under stringent quality control. Additionally, we will highlight our SureGuide and ClinGuide sgRNAs and solutions that are currently being used in both the research lab and in active clinical studies.

Participants

Amanda Haas - Business Development Manager, Gene Editing Therapeutics, Agilent Technologies

ROOM 204AB: Delivery of Genetic Medicines with Non-Viral Polymer Nanoparticles

3:00pm - 3:30pm
Oligonucleotide CMC and Targeted Delivery TRACK - Targeted Delivery of Therapeutic Oligonucleotides

Safe and efficient non-viral delivery technology will broaden the clinical utility of gene therapy for many genetic diseases. GenEdit’s NanoGalaxy platform enables the generation of a large library of diverse polymer nanoparticles that can deliver a variety of gene therapy cargos to different target tissues. Initial proof-of-concept studies showed PNP delivery of CRISPR to the central nervous system in diseased mouse models resulted in behavior changes.

Participants

Kunwoo Lee, Ph.D. - Chief Executive Officer, GenEdit

SESSIONS

MAIN CONFERENCE - DAY 3 - 23/09/2021

ROOM 205A: Investigations into Disruptive Delivery Approaches for LNA ASOs - Evaluation of Bovine Milk EVs and LNPs as Oral DDS

3:00pm - 3:30pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotide CMC Strategies

The evaluation of bovine milk derived extracellular vesicles (EV) as a drug delivery system for Locked Nucleic Acid Antisense Oligonucleotides (LNA ASO) is presented. The talk covers the optimization of downstream processing and purification methodology, proteomics and lipidomics analysis to better understand the composition of highly purified milk, EV biophysical characterization, stability in biorelevant fluids. Furthermore, the in vitro cell culture evaluation using hPSC-derived neurons and primary human cells, PK/PD evaluation in vivo using malat1 LNA ASO loaded EVs are presented.

Participants

Michael Keller, Ph.D. - Senior Principal Scientist, pRED, pCMC, Roche

ROOM 205B: On/Off/Edit: Drugging the Genome with HIgh Specificity

3:00pm - 3:30pm
Peptides TRACK: Peptide Nucleic Acids

NeuBase will discuss pharmaceuticalization of peptide-nucleic acids (PNAs) so as to drug the genome to address multiple causal mechanisms underlying diseases, with single nucleotide selectivity and high tolerability.

Participants

Dietrich Stephan, Ph.D. - Chairman and CEO, NeuBase Therapeutics, Inc.

ROOM 205C: Analytical Characterization of Guide RNAs and Ribonucleoproteins for CRISPR Applications

3:00pm - 3:30pm
mRNA and Genome Editing TRACK: Manufacturing and Analytics for CRISPR Applications

Characterization of the longer guide RNAs (gRNAs, 40-100+ nucleotides) and the ribonucleoprotein (RNP) complexes utilized in CRISPR applications represent an analytical challenge. Key parameters include purity considerations and complex/dynamic secondary structure of the oligonucleotides, as well the noncovalent nature and potential multiple stoichiometries of the RNP.

Participants

Steven Wolk, Ph.D. - Vice President, Analytical Chemistry & Site Head, Boulder, Editas Medicine

ROOM 204AB: Delivery of RNA Therapeutics: The Great Endosomal Escape!

3:30pm - 4:00pm
Oligonucleotide CMC and Targeted Delivery TRACK - Targeted Delivery of Therapeutic Oligonucleotides

RNA therapeutics have great potential to selectively treat human disease, especially cancer. However, due to their 8-14 kiloDalton size and 20-40 negative phosphate charges, RNAs have limited (<1%) to no ability to overcome a billion years of evolutionary defenses that prevent RNAs from escaping across the endosomal lipid bilayer membrane into the cytoplasm of cells. Consequently, endosomal escape remains The Technological Problem to solve for development of RNA therapeutics.

Participants

Steven Dowdy, PhD - Professor, UCSD School of Medicine

ROOM 205A: Characterization of mRNA Vaccines

3:30pm - 4:00pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotide CMC Strategies

Participants

Huijuan Li, PhD - Vice President, Analytical Development, Moderna Therapeutics

ROOM 205B: Oligonucleotide Therapeutics with a Peptide Nucleic Acid Backbone to Treat Metabolic Pandemics

3:30pm - 4:00pm
Peptides TRACK: Peptide Nucleic Acids

AptamiR Therapeutics is developing its second generation of miRNA antagomirs with a Peptide Nucleic Acid backbone which are conjugated to a fatty acid or a short peptide for targeted and preferential cytoplasmic delivery to adipocytes through the trans-membrane transporter Fatty Acid Translocase (FAT). The effective dose of these drug candidates should be much lower with a greatly improved safety and PK/PD profile, especially the mean residence time inside the targeted cells.

Participants

Marc Thibonnier, M.D. - Founder & President, AptamiR Therapeutics

Close of TIDES Conference

3:30pm - 3:35pm
mRNA and Genome Editing TRACK: Manufacturing and Analytics for CRISPR Applications

Close of TIDES Conference

4:00pm - 4:05pm
Oligonucleotide CMC and Targeted Delivery TRACK - Targeted Delivery of Therapeutic Oligonucleotides

Close of TIDES Conference

4:00pm - 4:05pm
Oligonucleotide Discovery to Clinic and CMC TRACK: Oligonucleotide CMC Strategies

Close of TIDES Conference

4:00pm - 4:05pm
Peptides TRACK: Peptide Nucleic Acids

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
7:00AM	**7:30am** - Registration **7:45am** - ROOM 205A: LGC's NAT Extended Services: Physicochemical Property Characterization of an Oligonu-	**7:30am** - Registration **7:45am** - ROOM 205C: Long-Acting Injectable Microparticle Formulations	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration	**7:30am** - Registration

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAK-FAST SPOT-LIGHT PRESEN-TATIONS 2	BREAK-FAST SPOT-LIGHT PRESEN-TATIONS 3	EXHIBI-TION HALL CONTENT	OLIGONU-CLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERA-PEUTIC OLIGONU-CLEOTIDE S	OLIGONU-CLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERA-PEUTIC OLIGONU-CLEOTIDE S	OLIGONU-CLEOTIDE DISCOV-ERY TO CLINIC AND CMC TRACK: OLIGONU-CLEOTIDE CMC STRATE-GIES	OLIGONU-CLEOTIDE DISCOV-ERY TO CLINIC AND CMC TRACK: OLIGONU-CLEOTIDE S FOR CNS, SKIN CANCER, NAFLD AND OTH-ER INDI-CATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATE-GIES FOR PEPTIDE CMC	SPOT-LIGHT PRESEN-TATION 2	SPOT-LIGHT PRESEN-TATION 3	SPOT-LIGHT PRESEN-TATION 4	MRNA AND GENOME EDITING TRACK: EMERG-ING GENOME EDITING TECH-NOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING AD-VANCES FROM PRECLINI-CAL TO THE CLIN-IC	MRNA AND GENOME EDITING TRACK: MANU-FACTUR-ING AND ANALYT-ICS FOR CRISPR APPLICA-TIONS
	cleotide														

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
8:00AM					**8:25am** - ROOM 204AB: Chairman's Remarks **8:30am** - ROOM 204AB: Targeted Delivery of Base Editors to Hepatocytes in vivo		**8:25am** - ROOM 205A: Chairman's Remarks **8:30am** - ROOM 205A: Enhancing the Pharmacologic Profiles of CNS Targeting Therapeu-		**8:25am** - ROOM 205B: Chairman's Remarks **8:30am** - ROOM 205B: Refractive Index: The Ultimate Tool for Real-Time Monitoring of Sol-					**8:25am** - ROOM 205C: Chair Remarks **8:30am** - ROOM 205C: Clinical Progress in Genome Editing	

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAK-FAST SPOT-LIGHT PRESEN-TATIONS 2	BREAK-FAST SPOT-LIGHT PRESEN-TATIONS 3	EXHIBI-TION HALL CONTENT	OLIGONU-CLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERA-PEUTIC OLIGONU-CLEOTIDE S	OLIGONU-CLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERA-PEUTIC OLIGONU-CLEOTIDE S	OLIGONU-CLEOTIDE DISCOV-ERY TO CLINIC AND CMC TRACK: OLIGONU-CLEOTIDE CMC STRATE-GIES	OLIGONU-CLEOTIDE DISCOV-ERY TO CLINIC AND CMC TRACK: OLIGONU-CLEOTIDE S FOR CNS, SKIN CANCER, NAFLD AND OTH-ER INDI-CATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATE-GIES FOR PEPTIDE CMC	SPOT-LIGHT PRESEN-TATION 2	SPOT-LIGHT PRESEN-TATION 3	SPOT-LIGHT PRESEN-TATION 4	MRNA AND GENOME EDITING TRACK: EMERG-ING GENOME EDITING TECH-NOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING AD-VANCES FROM PRECLINI-CAL TO THE CLIN-IC	MRNA AND GENOME EDITING TRACK: MANU-FACTUR-ING AND ANALYT-ICS FOR CRISPR APPLICA-TIONS
							tic Oligonu-cleotides		id-Phase Peptide Synthesis. Greening the Process						

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
9:00AM					**9:00am** - ROOM 204AB: Improved Delivery of RNA Therapeutics in the Eye, Muscle, and Beyond Using Fatty-Acid Conjugation **9:30am** - ROOM		**9:00am** - ROOM 205A: Treating Repeat Expansion Disorders by Stopping Somatic Expansion **9:30am** - ROOM 205A: Technology-derived		**9:00am** - ROOM 205B: Pegcetacoplan – Towards the Implementation of an Efficient Manufacturing Process for a PEGylated Peptide at an Industrial Scale,					**9:00am** - ROOM 205C: CRISPR in the Clinic – from Hemoglobinopathy to Immunooncology **9:30am** - ROOM 205C: In vivo CRISPR	

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
					204AB: Development of An antimiR-based Therapy to Treat Myotonic Dystrophy Disease		Antisense Oligonucleotide Therapeutics for Diseases of Excitable Cells		and the Associated Strategy for a Regulatory Approval **9:30am** - ROOM 205B: Synthesis and Formulation of Amphiphile Modified					Base Editing of PCSK9 Durably Lowers Cholesterol in Primates	

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
									mKRAS Peptides						

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
10:00AM			**10:30am** - Panel Discussion: Exploring the Massachusetts Biotech Ecosystem; Fostering Innovation, Spinning out, & Maximising Growth Potential		**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall **10:45am** - ROOM 204AB: Muscle Targeted Delivery of Therapeutic		**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall **10:45am** - ROOM 205A: Identification and Optimization of a Minor Al-		**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall **10:45am** - ROOM 205B: DMF Replacement in SPPS: Key Findings and				**10:45am** - ROOM 205C: Introduction to Prime Editing, The Search-and-replace Genome Editing Technology	**10:00am** - Networking Refreshment Break in Poster and Exhibit Hall	

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
			10:45am - Panel Discussion: Exploring the Massachusetts Biotech Ecosystem; Fostering Innovation, Spinning out, & Maximising Growth		Oligonucleotides with the FORCE™ Platform		lele-specific siRNA to Prevent PNPLA3 I148M-driven Non-alcoholic Fatty Liver Disease		Latest Results						

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAK-FAST SPOT-LIGHT PRESEN-TATIONS 2	BREAK-FAST SPOT-LIGHT PRESEN-TATIONS 3	EXHIBI-TION HALL CONTENT	OLIGONU-CLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERA-PEUTIC OLIGONU-CLEOTIDE S	OLIGONU-CLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERA-PEUTIC OLIGONU-CLEOTIDE S	OLIGONU-CLEOTIDE DISCOV-ERY TO CLINIC AND CMC TRACK: OLIGONU-CLEOTIDE CMC STRATE-GIES	OLIGONU-CLEOTIDE DISCOV-ERY TO CLINIC AND CMC TRACK: OLIGONU-CLEOTIDE S FOR CNS, SKIN CANCER, NAFLD AND OTH-ER INDI-CATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATE-GIES FOR PEPTIDE CMC	SPOT-LIGHT PRESEN-TATION 2	SPOT-LIGHT PRESEN-TATION 3	SPOT-LIGHT PRESEN-TATION 4	MRNA AND GENOME EDITING TRACK: EMERG-ING GENOME EDITING TECH-NOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING AD-VANCES FROM PRECLINI-CAL TO THE CLIN-IC	MRNA AND GENOME EDITING TRACK: MANU-FACTUR-ING AND ANALYT-ICS FOR CRISPR APPLICA-TIONS
			Potential (CONTIN-UED)												

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
11:00AM			**11:15am** - Panel: Talent Acquisition, Development and Retention in Novel Therapeutics		**11:15am** - ROOM 204AB: Engineering Antibody Oligonucleotide Conjugates (AOCs): Taking Receptor-Mediated Uptake One Step Further		**11:15am** - ROOM 205A: DCR-AUD: Applying GalXC™ RNAi Technology to the Treatment of Alcohol Use Disorder (AUD) **11:45am** - ROOM 205A:		**11:15am** - ROOM 205B: Nasal Glucagon: Overcoming Product Development Challenges **11:45am** - ROOM 205B: Impurity Control				**11:15am** - ROOM 205C: Optimization of LNP for in vivo Base Editing in Liver **11:45am** - ROOM 205C: An Engineered AsCas12A Nuclease		

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
					11:45am - ROOM 204AB: Extra-hepatic Delivery of Centyrin-targeted siRNA Conjugates		Novel Dual Targeting siRNA Therapeutic Offers Innovative Solution for Derm-Oncology Treatment		Strategy - Liraglutide Case Study				Facilitates the Rapid Generation of Therapeutic Cell Medicines		

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
12:00PM	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:20pm** - ROOM 205A: Self-adjuvanting Lipid nanoparticles for Next Generation	**12:15pm** - Transition to Spotlight Presentation Rooms **12:20pm** - ROOM 205C: Arromer: Receptor Directed Delivery for Ultra-precise RNAi Multi-tar-	**12:15pm** - Transition to Spotlight Presentation Rooms **12:20pm** - ROOM 205B: Oligo Drug Delivery Systems: Evolution of the Formulation Develop-	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	**12:15pm** - Transition to Spotlight Presentation Rooms **12:50pm** - Networking Luncheon in Poster and Exhibit Hall

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
										Nucleic Acid Vaccines **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	geting **12:50pm** - Networking Luncheon in Poster and Exhibit Hall	ment Landscape from Traditional Approaches to LNP-based Systems **12:50pm** - Networking Luncheon in Poster and Exhibit Hall			

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
1:00PM				**1:55pm** - ROOM 204AB: Chairman's Remarks		**1:55pm** - ROOM 205A: Chairman's Remarks		**1:55pm** - ROOM 205B: Chairman's Remarks							**1:55pm** - ROOM 205C: Chairman's Remarks

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
2:00PM				**2:00pm** - ROOM 204AB: Extra-hepatic Delivery **2:30pm** - ROOM 204AB: Redefining the chemical space for nucleic acid therapeutics		**2:00pm** - ROOM 205A: Improved Oxidizer Formulations for the Synthesis of PO/PS Mixed Backbone Oligonucleotides **2:30pm** - ROOM		**2:00pm** - ROOM 205B: 30 Years of Peptide Nucleic Acids and On the Road to Precision Antisense Antibiotics Against Multidrug Resistant Bacteria							**2:00pm** - ROOM 205C: IND-enabling Small-Scale Guide RNA Production Under GMP for CRISPR Based Cell Therapies **2:30pm** -

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
						205A: Impurity Strategy - Evaluating Consensus Impurity Lists		**2:30pm** - ROOM 205B: Molecular Bivalents for Recognition of RNA-repeated Expansion							ROOM 205C: Gram-Scale Good Manufacturing Practice (GMP) Single Guide RNA for CRISPR-based Gene Editing

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
3:00PM				**3:00pm** - ROOM 204AB: Delivery of Genetic Medicines with Non-Viral Polymer Nanoparticles **3:30pm** - ROOM 204AB: Delivery of RNA Ther-		**3:00pm** - ROOM 205A: Investigations into Disruptive Delivery Approaches for LNA ASOs - Evaluation of Bovine Milk EVs and LNPs as Oral DDS		**3:00pm** - ROOM 205B: On/Off/Edit: Drugging the Genome with HIgh Specificity **3:30pm** - ROOM 205B: Oligonucleotide Therapeutics with a							**3:00pm** - ROOM 205C: Analytical Characterization of Guide RNAs and Ribonucleoproteins for CRISPR Applications **3:30pm** - Close of

SCHEDULE

MAIN CONFERENCE - DAY 3 - 23/09/2021

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	EXHIBITION HALL CONTENT	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK - TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE CMC AND TARGETED DELIVERY TRACK: TARGETED DELIVERY OF THERAPEUTIC OLIGONUCLEOTIDES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDE CMC STRATEGIES	OLIGONUCLEOTIDE DISCOVERY TO CLINIC AND CMC TRACK: OLIGONUCLEOTIDES FOR CNS, SKIN CANCER, NAFLD AND OTHER INDICATIONS	PEPTIDES TRACK: PEPTIDE NUCLEIC ACIDS	PEPTIDES TRACK: STRATEGIES FOR PEPTIDE CMC	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	MRNA AND GENOME EDITING TRACK: EMERGING GENOME EDITING TECHNOLOGIES	MRNA AND GENOME EDITING TRACK: GENOME EDITING ADVANCES FROM PRECLINICAL TO THE CLINIC	MRNA AND GENOME EDITING TRACK: MANUFACTURING AND ANALYTICS FOR CRISPR APPLICATIONS
				apeutics: The Great Endosomal Escape!		**3:30pm** - ROOM 205A: Characterization of mRNA Vaccines		Peptide Nucleic Acid Backbone to Treat Metabolic Pandemics							TIDES Conference
4:00PM				**4:00pm** - Close of TIDES Conference		**4:00pm** - Close of TIDES Conference		**4:00pm** - Close of TIDES Conference							

The TIDES 2021 Digital Agenda (Available for viewing from September 28, 2021) will consist of 100+ On-Demand Speaker Presentation Videos Recorded from the In-Person TIDES Boston conference PLUS A Scheduled Agenda of the Following Presentations taking place September 28-30.

On demand

Sophisticated Manufacturing Method for High Quality gRNA under GMP

9:15am - 9:45am

Chemical synthesis of high-quality short oligonucleotides has been possible for more than a decade. However, chemical synthesis of high quality long RNAs such as guide RNAs for CRISPR has been a long-standing challenge. In this presentation, we report on the development and successful results of a high-quality long RNA production under GMP.

Participants

Ikuya Oshiro, PhD - Research Scientist R&D, SUMITOMO CHEMICAL Co., Ltd.

RNA Mass-Production by Microbial Fermentation

10:00am - 10:30am

- RNA production by Corynebacteirum glutamicum
- Large-scale RNA production technology newly developed

Participants

Shuhei Hashiro - Research Scientist, Ajinomoto Co., Inc.

Plan Now or Fail Later: Why Raw Materials Matter

10:45am - 11:15am

Accelerate mRNA vaccines and therapeutics from pre-clinical development to commercialization by utilizing raw materials designed to meet critical process, scale, quality, and regulatory needs. During process development, teams usually focus on process fit. But raw material fit is equally as important when considering line-of-sight to GMP manufacturing. Choosing these appropriately saves time and money. This presentation discusses two real examples of how raw material selection either accelerated or stalled regulatory approvals.

Participants

Darwin Asa PhD - Global Market Development Manager - Nucleic Acid Therapeutics, Thermo Fisher Scientific

FEATURED LIVE PRESENTATION WITH LIVE Q&A: Combinatorial Optimization of mRNA Structure, Stability, and Translation for RNA-based Therapeutics

11:30am - 12:00pm

Therapeutic mRNAs and vaccines are being developed for a broad range of human diseases, including COVID-19. However, their optimization is hindered by mRNA instability and inefficient protein expression. Here, we describe design principles that overcome these barriers. We develop a new RNA sequencing-based platform called PERSIST-seq to systematically delineate in-cell mRNA stability, ribosome load, as well as in-solution stability of a library of diverse mRNAs. We find that, surprisingly, in-cell stability is a greater driver of protein output than high ribosome load. We further introduce a method called In-line-seq, applied to thousands of diverse RNAs, that reveals sequence and structure-based rules for mitigating hydrolytic degradation. Our findings show that “superfolder” mRNAs can be designed to improve both stability and expression that are further enhanced through pseudouridine nucleoside modification. Together, our study demonstrates simultaneous improvement of mRNA stability and protein expression and provides a computational-experimental platform for the enhancement of mRNA medicines.

Participants

Kathrin Leppek, PhD - Postdoctoral Scholar, Maria Barna Lab, Dept. of Developmental Biology and Genetics, Stanford University

Networking Break

12:00pm - 12:45pm

Gram-Scale Good Manufacturing Practice (GMP) Single Guide RNA for CRISPR-based Gene Editing

12:45pm - 1:15pm

Over the past several years, CRISPR has revolutionized the field of gene editing. A major benefit of this technology is the ability to use ‘programmable’ single guide RNAs (sgRNAs) to precisely target areas of the genome for subsequent editing. However, this approach requires both high-quality and high purity sgRNAs to achieve optimal editing efficiency and obtaining these in sufficient quantities can be challenging. In this presentation, we demonstrate our current manufacturing capabilities and how they allow us to deliver up to multigram-quantity custom sgRNAs with the option to manufacture under either non-GMP or GMP conditions under stringent quality control. Additionally, we will highlight our SureGuide and ClinGuide sgRNAs and solutions that are currently being used in both the research lab and in active clinical studies.

Participants

Joe Guiles, PhD - Head of Development - Nucleic Acid Solution Divis, Agilent Technologies, Inc.

Preclinical Immunogenicity Characterization of ARCT-021 SARS-CoV-2 Vaccine

1:30pm - 2:00pm

The self-transcribing and replicating RNA (STARRTM) technology combined with Arcturus Therapeutics proprietary lipid nanoparticle (LNP) delivery technology has produced a safe and effective vaccine against SARS-CoV-2 virus. Mouse immunogenicity studies showed continuous increase in neutralizing antibody titers for up to 60 days after a single vaccination along with a strong Th1 cell mediated immune response. Lethal virus challenge studies using a human ACE2 transgenic mouse model yielded 100% protection after a single 2 Âµg RNA dose. T cell and B cell depletion studies with a sublethal virus challenge in the same transgenic mouse model showed complete protection after B cell depletion and no protection after T cell depletion. Rhesus macaque immunogenicity studies showed high neutralizing antibody titers after two prime injections 28 days apart. A further increase in neutralizing antibody titers were observed with a boost injection 120 days after the second prime injection. Non-human primate virus challenge studies showed significant reduction in bronchoalveolar virus genomes after single and double prime vaccinations. Preliminary preclinical results for second generation vaccines designed with improved anti-viral immunogenicity exhibited cross neutralization against alpha, beta, gamma and delta circulating viral variants in mice and non-human primates. The first generation vaccines are in late stage clinical trials and the second generation vaccines are planned for entry into the clinic.

Participants

Sean Sullivan, Ph.D. - Executive Director, Process Development, Arcturus Therapeutics

Advancements in mRNA Capping and Manufacturing

2:15pm - 2:45pm

mRNA has revolutionized the field of vaccines and TriLink has been at the forefront. In this presentation, we demonstrate the advantages of CleanCap® co-transcriptional capping and advancements in manufacturing to enable cost-effective vaccine production. With the capability of delivering up to multigram-quantity custom mRNAs under either non-GMP or GMP conditions, TriLink is the experienced development partner for research and clinical studies.

Participants

Jessica Madigan - Director, Business Development GMP, TriLink BioTechnologies

SCHEDULE

DIGITAL - SEP. 28 - 28/09/2021

TIDES USA: Oligonucleotide & Peptide Therapeutics

Delivered as Hybrid Event from September 20-30, 2021
Live In-Person Experience Delivered September 20-23; On-demand experience Delivered September 28-30
Boston Convention and Exhibition Center

TIME	
9:00AM	**On demand** - The TIDES 2021 Digital Agenda (Available for viewing from September 28, 2021) will consist of 100+ On-Demand Speaker Presentation Videos Recorded from the In-Person TIDES Boston conference PLUS A Scheduled Agenda of the Following Presentations taking place September 28-30. **9:15am** - Sophisticated Manufacturing Method for High Quality gRNA under GMP
10:00AM	**10:00am** - RNA Mass-Production by Microbial Fermentation **10:45am** - Plan Now or Fail Later: Why Raw Materials Matter
11:00AM	**11:30am** - FEATURED LIVE PRESENTATION WITH LIVE Q&A: Combinatorial Optimization of mRNA Structure, Stability, and Translation for RNA-based Therapeutics
12:00PM	**12:00pm** - Networking Break **12:45pm** - Gram-Scale Good Manufacturing Practice (GMP) Single Guide RNA for CRISPR-based Gene Editing
1:00PM	**1:30pm** - Preclinical Immunogenicity Characterization of ARCT-021 SARS-CoV-2 Vaccine
2:00PM	**2:15pm** - Advancements in mRNA Capping and Manufacturing

The TIDES 2021 Digital Agenda (Available for viewing from September 28, 2021) will consist of 100+ On-Demand Speaker Presentation Videos Recorded from the In-Person TIDES Boston conference PLUS A Scheduled Agenda of the Following Presentations taking place September 28-30.

On demand

Liquid-phase Synthesis (LPS) of Oligonucleotide by Using Blockmer™

9:15am - 9:45am

Liquid-phase synthesis (LPS) of oligonucleotides is an attractive method for oligonucleotide API manufacture, enabling large scale production with high cost performances. However, LPS remains significant challenges due to its complex operations and low quality of target oligonucleotides. Therefor LPS has not yet become a major method for the API manufacture. We have developed Blockmer™ as building blocks and “fluorous unit” to overcome these problems and our novel LPS system suitable for oligonucleotide APIs will be presented.

Participants

Ichiro Mori, PhD - Director of R&D, NATiAS Inc.

Turning Tides Together. Manufacturing of Peptides and Oligonucleotides

10:00am - 10:30am

Bachem known as the leading CMO for pharmaceutical grade peptides is expanding its technology platform to offer chemical manufacturing services for nucleic acid based APIs. We would like to take the opportunity to present Bachem’s capabilities as the first CMO servicing the global TIDES market.

Participants

Joseph Fraone - Business Development Manager, Oligonucleotides, Bachem

Development, Optimization and Scale up of Therapeutic Peptides: Approaches to Solid Phase Synthesis

10:45am - 11:15am

The application and reliance on Solid Phase Peptide Synthesis (SPPS) has increased dramatically over recent years with the rise of peptides in diagnostic and therapeutic research. With this rapid increase in development activity, the ability of automated synthesis instruments to screen multiple reaction conditions simultaneously is essential. Here we describe the automated screening of multiple reaction conditions for the synthesis of a selection of therapeutic peptides, enabling the optimum synthetic conditions to be selected for each target, followed by the scale-up of the synthesis on a pilot-scale instrument.

Participants

Colton Quick - Product Specialist, Gyros Protein Technologies

FEATURED LIVE PRESENTATION WITH LIVE Q&A: Clinical Development of Peptide Nucleic Acids: A First-in-Class Modality

11:30am - 12:00pm

Participants

Dietrich Stephan, Ph.D. - Chairman and CEO, NeuBase Therapeutics, Inc.

Short Break

12:00pm - 12:10pm

Numaswitch - An Efficient Production Platform to Produce Peptides and Small Proteins

12:10pm - 12:40pm

We believe in the untapped potential of peptides as feedstock for better, safer, and healthier products in life sciences and beyond. Cost of goods, development times, safety profiles and material supply should never be limiting factors. That is why we have pioneered Numaswitch, an approach to produce peptides and small proteins at highest quality, needed scales, in time, affordable and in a sustainable way. Our mission is to enable your innovations. At TIDES 2021 we are going to share selected case studies and demonstrate how Numaswitch makes a difference.

Participants

Christian Schwarz, PhD - CEO, Numaferm GmbH

Short Break

12:40pm - 12:45pm

Optimizing Process Development for Biomolecule Purification

12:45pm - 1:15pm

The optimal resin for your process is not always commercially available. Resin parameters are fixed by the manufacturer and may limit performance for many applications. Therefore, processes are developed within those parameters which can lead to loss of yield, throughput, or require more steps than desired to meet the production goals. In this workshop, ABT, a manufacturer of high-quality agarose resins, will discuss how through their decades of experience, they leverage their ability to control all aspects of the resin. Case studies will be presented on the development of custom resins, that lead to higher resolution, capacity, and simplified purification processes. The result being that optimization of agarose resins is an efficient and inexpensive means to optimize your purification process development.

Participants

Hernan Alarcon, PhD - Research & Development Scientist, Agarose Bead Technologies

Jurgen Machielse - Director, Life Sciences, AIC

Impurity Control Strategy - Liraglutide Case Study

1:30pm - 2:00pm

Liraglutide is a peptide utilized for the treatment of Diabetes. In this case study, analytical control strategy considerations are discussed in traversing from the innovators recombinant (bio-synthetic) Liraglutide to chemically synthesized Liraglutide (SPPS). Special emphasis is given on the control of deletion impurities through process optimization. A diastereomer control strategy was developed that utilized batch data and an occurrence risk assessment to subsequently demonstrate detectability by the analytical control strategy.

Participants

Eran Benjamin, Ph.D. - Global Director, QC AD, PolyPeptide Group

Use of Mustang® E Membrane Chromatography as Risk Mitigation for Endotoxin and Viral Clearance Contamination During UF/DF Processing

2:15pm - 2:45pm

An aqueous-based purification process for antisense oligonucleotides (ASO), which ends with a UF/DF step required risk mitigation to avoid endotoxin and viral contamination that could enter late in the process. This study demonstrated that Mustang E membrane chromatography can effectively remove viruses and endotoxin in late-stage processing buffers. This supports a robust approach for clearance of contaminants needed for the more stringent requirements for intrathecal (IT) delivery.

Participants

Robert Gronke, PhD - Senior Principal Scientist, Technical Development, Biogen, Inc

Sarah Blackmore - Viral Vector and Gene Therapy Technology Manager, Pall Biotech

SCHEDULE

DIGITAL - SEP. 29 - 29/09/2021

TIDES USA: Oligonucleotide & Peptide Therapeutics

Delivered as Hybrid Event from September 20-30, 2021
Live In-Person Experience Delivered September 20-23; On-demand experience Delivered September 28-30
Boston Convention and Exhibition Center

TIME	
9:00AM	**On demand** - The TIDES 2021 Digital Agenda (Available for viewing from September 28, 2021) will consist of 100+ On-Demand Speaker Presentation Videos Recorded from the In-Person TIDES Boston conference PLUS A Scheduled Agenda of the Following Presentations taking place September 28-30. **9:15am** - Liquid-phase Synthesis (LPS) of Oligonucleotide by Using Blockmer™
10:00AM	**10:00am** - Turning Tides Together. Manufacturing of Peptides and Oligonucleotides **10:45am** - Development, Optimization and Scale up of Therapeutic Peptides: Approaches to Solid Phase Synthesis
11:00AM	**11:30am** - FEATURED LIVE PRESENTATION WITH LIVE Q&A: Clinical Development of Peptide Nucleic Acids: A First-in-Class Modality
12:00PM	**12:00pm** - Short Break **12:10pm** - Numaswitch - An Efficient Production Platform to Produce Peptides and Small Proteins **12:40pm** - Short Break **12:45pm** - Optimizing Process Development for Biomolecule Purification
1:00PM	**1:30pm** - Impurity Control Strategy - Liraglutide Case Study
2:00PM	**2:15pm** - Use of Mustang® E Membrane Chromatography as Risk Mitigation for Endotoxin and Viral Clearance Contamination During UF/DF Processing

The TIDES 2021 Digital Agenda (Available for viewing from September 28, 2021) will consist of 100+ On-Demand Speaker Presentation Videos Recorded from the In-Person TIDES Boston conference PLUS A Scheduled Agenda of the Following Presentations taking place September 28-30.

On demand

Fujifilm's Capabilities for Manufacturing Lipid Nanoparticles

9:15am - 9:45am

FUJIFILM Corporation is going to transform into a healthcare company through leveraging original technologies cultivated in photo-films' manufacturing, such as nanotechnologies, fine-chemistry and analysis technologies. Based on these assets, we have started a CDMO business to manufacture Lipid nanoparticles. We can provide proprietary ionizable lipids and a one-stop service from formulation research to GMP manufacturing for lipid nanoparticles.

Participants

Naoki Yamada - Director, Pharmaceutical Drug Development, FUJIFILM Pharmaceuticals U.S.A., Inc.

Oligo Drug Delivery Systems: Evolution of the Formulation Development Landscape from Traditional Approaches to LNP-based Systems

10:00am - 10:30am

Oligonucleotides can be used to modulate gene expression via a range of processes including RNAi, target degradation by RNase H-mediated cleavage, splicing modulation, non-coding RNA inhibition, gene activation and programmed gene editing. As such, these molecules have potential therapeutic applications for a myriad of indications, with several oligonucleotide drugs recently gaining approval. However, despite recent technological advances, achieving efficient oligonucleotide delivery, particularly to extrahepatic tissues, remains a major translational limitation. In fact, before oligos can become drugs, they must overcome a billion years of evolutionary defenses designed to keep invading nucleic acids on the outside of cells from getting to the inside of cells. Not surprisingly, significant effort has been placed in developing a wide array of delivery technologies. Foremost among these is the development of N-acetylgalactosamine (GalNAc), which is especially effective for delivering siRNA conjugates to the liver. More recently, a very efficient drug delivery system has taken center-stage: Lipid NanoParticle (LNP) systems are currently the leading non-viral delivery systems for enabling the clinical potential of genetic drugs.This presentation covers the evolution of formulation development for oligo-based drug products, starting from more traditional approaches to the latest formulation strategies, including LNPs.

Participants

Umberto Romeo - Head of R&D, Corden Pharma Caponago

An Introduction to OCELOT™ System Control

10:45am - 11:15am

This presentation will offer a close look at our newly developed automation platform – OCELOT™ System Control – as it would be run on an Asahi Oligosynthesizer™. The presentation will highlight details on process steps, data tracking/analysis and other functionality, in addition to a brief overview of Asahi Kasei Bioprocess and our full oligo equipment offering.

Participants

Stefan Hyde - Automation Manager, Asahi Kasei Bioprocess America

FEATURED MINI-SYMPOSIUM AND LIVE PANEL DISCUSSION WITH LIVE Q&A: Targeted Delivery of Oligonucleotides: Tissue-Specific Challenges and Novel Conjugation Strategies

11:30am - 1:00pm

Participants

Muthiah (Mano) Manoharan, PhD - Senior Vice President of Drug Discovery, Alnylam Pharmaceuticals, Inc.

Punit Seth, PhD - Vice President, Medicinal Chemistry, Ionis Pharmaceuticals

Anastasia Khvorova, PhD - Professor, RNA Therapeutics Institute and Program in Molecular Medicine, University of Massachusetts Medical School

Arthur Levin, PhD - Chief Scientific Officer, Avidity Biosciences, Inc.

Steven Dowdy, PhD - Professor, UCSD School of Medicine

Maja Janas, Ph.D. - Senior Scientist, Investigative Toxicology, Alnylam Pharmaceuticals

Michael Skynner, Ph.D. - Chief Operating Officer, Bicycle Therapeutics

Networking Break

1:00pm - 1:45pm

Suppressing the Inherent Reactivity of Labile Moieties during Purity and Identity Characterization

1:45pm - 2:15pm

Nucleoside phosphoramidites (NPAs) set sugar structure and nucleobase identity (sequence) in therapeutic oligonucleotides during solid phase syntheses. As such, control over their purities and impurity profiles are of paramount importance. Under reverse-phase (RP) chromatography conditions, water is ubiquitous, posing a fundamental threat to NPA stability during the analysis, and may contain adventitious oxidants. Challenges in analyzing NPAs include poor peak shape, diminished sensitivity, and rising baselines. Here, we report mitigating strategies for protection of NPAs during LCMS analyses.

Participants

Vanessa Momaney, Ph.D. - Research Scientist II, Analytical Development, Nitto Denko Avecia Inc.

Leveraging High Resolution Mass Spectrometry for the Analysis of Process and Product Related Impurities for Synthetic Oligonucleotides

2:30pm - 3:00pm

Therapeutic oligonucleotides represent an emerging drug modality. However, the characterization of impurities associated to oligos is challenging, as oligos are polar compounds, with a number of different product and process related impurities . Here, we present an Ion Pairing Reversed Phase/High Resolution Mass Spectrometry workflow and data analysis to facilitate characterization of therapeutic oligos.

Participants

Dr. Ramesh Indrakanti, PhD - Biologics Product Specialist/Senior Account Manager, Phenomenex

SCHEDULE

DIGITAL - SEP. 30 - 30/09/2021

TIDES USA: Oligonucleotide & Peptide Therapeutics

Delivered as Hybrid Event from September 20-30, 2021
Live In-Person Experience Delivered September 20-23; On-demand experience Delivered September 28-30
Boston Convention and Exhibition Center

TIME	
9:00AM	**On demand** - The TIDES 2021 Digital Agenda (Available for viewing from September 28, 2021) will consist of 100+ On-Demand Speaker Presentation Videos Recorded from the In-Person TIDES Boston conference PLUS A Scheduled Agenda of the Following Presentations taking place September 28-30. **9:15am** - Fujifilm's Capabilities for Manufacturing Lipid Nanoparticles
10:00AM	**10:00am** - Oligo Drug Delivery Systems: Evolution of the Formulation Development Landscape from Traditional Approaches to LNP-based Systems **10:45am** - An Introduction to OCELOT™ System Control
11:00AM	**11:30am** - FEATURED MINI-SYMPOSIUM AND LIVE PANEL DISCUSSION WITH LIVE Q&A: Targeted Delivery of Oligonucleotides: Tissue-Specific Challenges and Novel Conjugation Strategies
12:00PM	
1:00PM	**1:00pm** - Networking Break **1:45pm** - Suppressing the Inherent Reactivity of Labile Moieties during Purity and Identity Characterization
2:00PM	**2:30pm** - Leveraging High Resolution Mass Spectrometry for the Analysis of Process and Product Related Impurities for Synthetic Oligonucleotides