SESSIONS

PRE-CONFERENCE DAY - 14/05/2024

Strategies for Oligonucleotide Purification Using Reverse Phase Resins

08:30 - 09:00
Breakfast Spotlight Presentations 1

Oligonucleotides are short synthetic DNA sequences that are used for diagnostic and therapeutic purposes. They are manufactured through multi-step chemical synthesis processes which are prone to generate impurities. Multiple options for oligonucleotide purification are available....but which route is best? In this presentation we explore oligonucleotide purification challenges in process development and production through different purification examples. We will discuss: 1) Comparison of reverse phase and ion exchange purification strategies; 2) Reverse phase chromatography options for DMT-On and DMT-Off purification and 3) Purification of short and long oligonucleotides.

Participants

Martin Deetz, PhD - Senior Technical Laureate, DuPont

Plant-based Squalene for Parenteral Applications

08:30 - 09:00
Breakfast Spotlight Presentations 2

This plant-based alternative to animal-derived squalene has many benefits. PhytoSquene® reduces the need to source pharma grade squalene from sharks, therefore helping to preserve biodiversity and ecosystems. It has a high and consistent quality and no harmful (carcinogenic, mutagenic or toxic) processes are involved in manufacturing.

Participants

Lars Geiger, PhD - Global Director of Project Management Drug Substance, Evonik Health Care

Recent Technological Innovation in Peptide and Oligonucleotide Manufacturing

08:30 - 09:00
Breakfast Spotlight Presentations 3

Recent Technological Innovation in Peptide and Oligonucleotide Manufacturing

To accelerate the development of peptides and oligonucleotides as pharmaceuticals, it is necessary to develop technologies that overturn traditional approaches and overcome cost and quality challenges in all manufacturing processes, including synthesis, purification, and freeze-drying. We will introduce specific examples of new technologies that can solve these issues using our model compounds.

Participants

Yoshitaka Nemoto - Vice President R&D, PeptiStar

Workshop AM1: Introduction to Therapeutic Oligonucleotides - Design, Function and Delivery

09:00 - 12:30
Workshop AM1: Introduction to Therapeutic Oligonucleotides - Design, Function and Delivery

The purpose of this workshop is to introduce scientists to therapeutic oligonucleotides. This workshop will discuss the different types of therapeutic oligonucleotides and how they work in the body to treat disease. Difference modalities such as SiRNA, ASO, MiRNA and Aptamer will be covered. The key factors for the design of the molecules will be described. The workshop will also discuss the challenges of delivery to the appropriate tissue and into the appropriate cell, and the strategies currently employed to address these. The toxicology, metabolism and clearance in the body will be covered. Finally, the workshop will discuss formulation options and how the drug substances and drug products are manufactured and controlled.

Participants

Mike Webb, Ph.D. - Founder and CEO, Mike Webb Pharma

Workshop Leaders' Introduction and Overview: Stereochemistry of Synthetic Oligonucleotides - Challenges and Opportunities

09:00 - 09:20
Workshop AM2: Stereochemistry of Synthetic Oligonucleotides: Strategies for Analysis and Control

Participants

G. Susan Srivatsa, PhD - President, ElixinPharma

Fran Wincott, PhD - President, Wincott & Associates, LLC

Workshop AM3: Introduction to Genome Editing

09:00 - 12:30
Workshop AM3: Introduction to Genome Editing

Scientists will be introduced to the technology of gene editing in this workshop. Participants in the workshop will learn about the different gene editing technologies available and how they work for therapeutic purposes. In this workshop, we will cover the delivery technologies available for delivering these gene editing technologies to appropriate tissues. Some of the other questions to be considered in this workshop include: What are the recent improvements and advances in genome editing technologies? What are some strategies for handling off-target detection and mitigation? What is the current status, as well as the challenges and risks of in vivo gene editing beyond the liver? How are companies handling precision targeted integration of large genetic cargo? What are the latest tools for DMPK and (bio)analytics of gRNAs? What are the current regulatory perspectives and guidances in the current landscape of gene edited products?

Participants

Rubina Parmar, PhD - Vice President, Chemistry and Delivery Sciences, Intellia Therapeutics

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

Workshop Leader's Welcome and Opening Remarks

09:00 - 09:15
Workshop AM4: Analytical Strategies for Therapeutic Peptides

Participants

Matteo Villain, PhD - Vice President and Global Peptides Technical Lead, Piramal Pharma Solutions

The Regulatory Perspective: Analytical Expectations for Peptide Therapeutics

09:15 - 09:45
Workshop AM4: Analytical Strategies for Therapeutic Peptides

Regulatory authorities anticipate greater emphasis on analytical characterization at earlier stages of peptide therapeutics development. Let's delve into recent insights from regulatory authorities in the United States and European Union concerning peptide characterization and the analytical pre-requisite across different developmental phases. Discover the applicability of ICH Q3A/B to peptides, the instances necessitating specific numerical values in specification, and the characterization methodology expected by regulators.

Participants

Jamie Brugnano, Ph.D. - Director of Regulatory Affairs, Bachem Americas

Nonstereoselective Synthesis: Process, Control and Regulatory Considerations

09:20 - 10:00
Workshop AM2: Stereochemistry of Synthetic Oligonucleotides: Strategies for Analysis and Control

Participants

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

Practical Considerations for Characterization of Peptide Drug Products

09:45 - 10:15
Workshop AM4: Analytical Strategies for Therapeutic Peptides

Dosage form characterization, impurity profiling and accurate stability assessments are all critical parts of any drug development program. Peptide drug products have specific characterization challenges not encountered with typical small molecule drug products. This talk is intended to highlight attributes that are unique to peptide drug products and provide practical analytical approaches for the drug development chemist.

Participants

Lisa Caralli - Sr. Director of Scientific Advisory, Pharmaceutics, Catalent Pharma Solutions

Stereoselective Synthesis: Process, Control and Regulatory Considerations

10:00 - 10:40
Workshop AM2: Stereochemistry of Synthetic Oligonucleotides: Strategies for Analysis and Control

Participants

Keith Bowman - Vice President of Process Development, Wave Life Sciences

High-end Analytical Tools to Meet the Latest Regulatory Requirements for Generic Peptides – A Case Study

10:15 - 10:45
Workshop AM4: Analytical Strategies for Therapeutic Peptides

While regulatory requirements for generic peptides applications are becoming more and more demanding, the existing analytical tools and techniques are pushed to their limits to deliver the appropriate performance (resolution, sensitivity, precision). This presentation will go through a recent case study for a synthetic generic peptide of recombinant origin, where several high-end analytical techniques have been used to overcome the challenges of the most recent FDA guideline in this area.

Participants

Jean-Marc Poudrel, PhD - Head of Regulatory Affairs, PolyPeptide Group

Networking Refreshment Break

10:40 - 11:10
Workshop AM2: Stereochemistry of Synthetic Oligonucleotides: Strategies for Analysis and Control

Networking Refreshment Break

10:45 - 11:15
Workshop AM4: Analytical Strategies for Therapeutic Peptides

Characterization and Stereochemical Control Strategy of siRNA

11:10 - 11:40
Workshop AM2: Stereochemistry of Synthetic Oligonucleotides: Strategies for Analysis and Control

Participants

Lubomir Nechev, PhD - Senior Vice President CMC Development, Alnylam Pharmaceuticals, Inc.

Making Waves in Peptide Analysis: Solving Isomer Challenges with Ion Mobility

11:15 - 11:45
Workshop AM4: Analytical Strategies for Therapeutic Peptides

Ion mobility (IM) interest and implementation has grown in pharmaceutical industry over the last 10 years. IM technology can be hyphenated with traditional LC-MS providing an orthogonal separation to aid in the identification of isomeric modified peptides. A review of commercially available IM with a case study to demonstrate how structures for lossless ion manipulation (SLIM) high resolution ion mobility (HRIM) can be leveraged to study peptide therapeutics.

Participants

Ashli Simone - Technical Product Specialist, MOBILion Systems

Characterization and Control Strategy of PMO Stereochemistry

11:40 - 12:10
Workshop AM2: Stereochemistry of Synthetic Oligonucleotides: Strategies for Analysis and Control

PMO drug substance, comprised of morpholino subunits and phosphorodiamidate linkages, is manufactured by linear chain elongation using activated morpholino subunits (building blocks) via solid-phase oligomer synthesis. Each subunit contains three chiral centers, two at the 1' and 4' carbons of the morpholino ring and one at the phosphorus of the inter-subunit linkage. The stereochemistry of the chiral centers in the morpholine rings are derived from the corresponding starting ribonucleoside and maintain the same absolute configuration. The phosphorus atoms of the inter-subunit linkages are introduced as an approximately 1:1 mixture of the two epimers at each position and thus PMO drug substance is a mixture of 2n diastereomers, where n is the length of the oligo chain. It is impossible to analyze and characterize each individual isomer for the final drug substance due to the large number of isomers present, and therefore an alternative strategy is employed to analyze the stereochemistry of drug substance inter-linkage, i.e., each activated morpholino subunit is analyzed, and the stereochemistry of drug substance is correlated with that of activated morpholino subunits by the outcome of the coupling reaction. Using the combination of dimer models and 31P NMR in both solution and solid phase, it is found that the stereochemistry of the coupling reaction is stereospecific, therefore the overall drug substance backbone stereochemistry is controlled by that of each building block.

Participants

Bao Cai, PhD - Executive Director, Process Development, Sarepta Therapeutics

Analytical Procedure Development for Novel Peptides: From Column Screening to GMP Releases

11:45 - 12:15
Workshop AM4: Analytical Strategies for Therapeutic Peptides

In the current pharmaceutical landscape, therapeutic peptides are on the rise. To ensure the large-scale production of peptides in the highest quality, Bachem implements a holistic analytical control strategy. Beginning with product-specific understanding of e.g. related impurities and aggregation behavior, state-of-the-art chromatographic detection methods are developed and validated. This approach alongside the process development enables that stringent product specifications in line with current regulatory demands are continuously met.

Participants

Priska Frei, Ph.D. - Scientist QC, Bachem AG

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Panel Discussion with Workshop Speakers

12:10 - 12:30
Workshop AM2: Stereochemistry of Synthetic Oligonucleotides: Strategies for Analysis and Control

Closing Remarks and Discussion

12:15 - 12:30
Workshop AM4: Analytical Strategies for Therapeutic Peptides

Enzymatic Oligonucleotide Synthesis Process Flow and Substance Impurity Profile

12:30 - 13:30
Spotlight Presentation Luncheon 1

This workshop will provide an overview of a template-independent, enzymatic oligonucleotide synthesis process to produce small interfering RNA therapeutics and discuss how this novel technology will fit into, supplement, or replace existing oligonucleotide manufacturing infrastructure. Topics will also include comparisons to solid-phase synthesis in terms of performance, capital investment, raw material requirements, waste management, as well as downstream processing and impurity profiling.

Participants

Derek Gauntlett - Director, Process Chemistry, Codexis

A Scalable Versatile System for mRNA-LNP Formulation Based on Impingement Jets Mixing Technology - From R&D to Commercial Drug Substance Production

12:30 - 13:30
Spotlight Presentation Luncheon 2

The formulation step in the Lipid Nanoparticle (LNP) production is critical and needs to be scalable without losing quality. The KNAUER´s Impingement Jets Mixing (IJM) systems provide vertical solution for the scaling the formulation methods from R&D to Production. The results of the technology transfer show no changes in the particle characterization parameters, such as particle size (<100 nm, depends on payload), polydispersity (<0.1) and encapsulation efficiency (>98%).

Participants

Lilit Avagyan - Team Leader Customized Solutions, Knauer

Sciex Luncheon Spotlight Presentation

12:30 - 13:30
Spotlight Presentation Luncheon 3

Workshop Leader's Welcome and Opening Remarks

13:30 - 13:40
Workshop PM1: CMC and Nonclinical Strategies for an Oligonucleotide Phase 1 IND

Who should attend?

Executives leading a company working towards and IND, anyone interested in CMC and nonclinical oligonucleotide activities, anyone involved in early-stage drug development especially those who may be new to oligonucleotides. Quality assurance personnel, QC/analytical development chemists, toxicologists.

Participants

Kathryn Ackley, PhD - CMC Consultant Specializing in Oligonucleotides, Independent Consultant

Workshop PM2: Introduction to Analytical Control Strategies for Therapeutic Oligonucleotides

13:30 - 17:00
Workshop PM2: Introduction to Analytical Control Strategies for Therapeutic Oligonucleotides

The workshop will focus on the specific requirements for control that are common to all therapeutic oligonucleotides. For example, common impurities from solid-state synthesis especially those which come from the starting materials, the synthetic process and degradation products. The issues of determining water in hygroscopic products. Issues with assays for both single and double stranded oligonucleotides. In addition, we will discuss how establishing an ongoing control strategy is important to determine and monitor critical quality attributes that affect the drug product and the key aspects of specification setting across the phases of development. The workshop will also touch on risk assessment in late phase quality by design approaches and the role of analysis in determining critical process parameters and their relationship to critical quality attributes.

Participants

Mike Webb, Ph.D. - Founder and CEO, Mike Webb Pharma

Workshop Leader's Welcome and Opening Remarks

13:30 - 13:40
Workshop PM3: sgRNA as Intermediate Drug Substance for CRISPR Therapeutics: Analytics, Manufacturing, Regulatory and Beyond

Participants

Marc Jacob, PhD - Executive Director of Business Development, SK pharmteco

Workshop Leader's Welcome and Opening Remarks

13:30 - 13:35
Workshop PM4: CMC Regulatory Strategies for Peptides

Participants

Gary Musso, PhD - President, Musso and Associates LLC

CMC Regulatory Challenges During Peptide Development

13:35 - 14:15
Workshop PM4: CMC Regulatory Strategies for Peptides

The presentation will explore the current state of peptide therapeutics, ongoing progress, and future directions. Additionally, it will discuss the importance of effective CMC approaches for discovering, optimizing, assessing, and delivering combination peptide therapeutics for the treatment of various diseases.

Participants

Samrat Sisodia, Ph.D. - Vice President RA and QA, Palatin Technologies

Nonclinical Aspects of an IND

13:40 - 14:25
Workshop PM1: CMC and Nonclinical Strategies for an Oligonucleotide Phase 1 IND

Participants

Peter Korytko, Ph.D. - President, Preclinical GPS

Oligonucleotide Analytics Overview with a Focus on Guide RNAs for CRISPR Applications

13:40 - 14:10
Workshop PM3: sgRNA as Intermediate Drug Substance for CRISPR Therapeutics: Analytics, Manufacturing, Regulatory and Beyond

Dr. Gilar will discuss the general methods for oligonucleotides analysis (ASO, PMO, DNA, siRNA, sgRNA etc.), tips and tricks from 25 years of experience and will also cover the guidelines for oligonucleotides LC purification as well as mas spec methods for characterization of long oligonucleotides such as sgRNA used for CRISPR.

Participants

Martin Gilar, PhD - Scientific Fellow, Separations R&D, Waters Corporation

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Overview of Guide RNAs for CRISPR Applications

14:10 - 14:40
Workshop PM3: sgRNA as Intermediate Drug Substance for CRISPR Therapeutics: Analytics, Manufacturing, Regulatory and Beyond

The guide RNAs used in CRISPR applications are typically long oligonucleotides (40-100+ nucleotides). Their impurity level and complexity generally increase as a function of length due to the iterative nature of the synthesis process. In addition, different Cas nucleases require different gRNAs, with specific sequence characteristics. The impact of guide RNA purity and sequence fidelity on the safety attributes as well as the efficiency and specificity of different CRISPR Cas systems will be discussed.

Participants

Jean-Noel Lemercier, PhD - Associate Director, Chemistry, Editas Medicine

Regulatory CMC Strategies for Early Peptide Development

14:15 - 15:00
Workshop PM4: CMC Regulatory Strategies for Peptides

Limited experience in peptide manufacture (typically one preclinical lot and one clinical lot) present challenges with setting specifications and supporting data. This presentation will discuss issues and challenges in early peptide development with a focus on strategies and priorities of a small companies including case studies with FDA feedback.

Participants

Aileen Ryan - Senior Regulatory Advisor, Prometrika, LLC

Manufacturing Aspects of an IND

14:25 - 15:00
Workshop PM1: CMC and Nonclinical Strategies for an Oligonucleotide Phase 1 IND

Participants

Kathryn Ackley, PhD - CMC Consultant Specializing in Oligonucleotides, Independent Consultant

Important Aspects to Successful Manufacturing Outcomes Through Agilent's Six Years of Making GMP sgRNA

14:40 - 15:20
Workshop PM3: sgRNA as Intermediate Drug Substance for CRISPR Therapeutics: Analytics, Manufacturing, Regulatory and Beyond

Important evolutionary elements of the GMP production process for continuously improving purity outcome. How these elements address a diversity in length and modifications of the sgRNA. Additionally, a key quality attribute of sgRNA is sequence fidelity. The failure mode scenarios and controls for sequence fidelity will be presented.

Participants

Kaizhang He, Ph.D. - Director of Process Chemistry, Agilent Technologies

Joe Guiles, PhD - Head of Chemical Development, Agilent Technologies

Networking Refreshment Break

15:00 - 15:30
Workshop PM1: CMC and Nonclinical Strategies for an Oligonucleotide Phase 1 IND

Networking Refreshment Break

15:00 - 15:30
Workshop PM4: CMC Regulatory Strategies for Peptides

Networking Refreshment Break

15:20 - 15:45
Workshop PM3: sgRNA as Intermediate Drug Substance for CRISPR Therapeutics: Analytics, Manufacturing, Regulatory and Beyond

Quality and Regulatory Aspects of an IND

15:30 - 16:15
Workshop PM1: CMC and Nonclinical Strategies for an Oligonucleotide Phase 1 IND

Participants

Judy Carmody, Ph.D. - Founder and Principal Consultant, Carmody Quality Solutions, LLC

CMC Regulatory Areas of Increased Interest for Peptides

15:30 - 16:15
Workshop PM4: CMC Regulatory Strategies for Peptides

For many years, CMC regulatory reviews focused on the process and quality. Process related areas included CPPs, CQAs and design space concepts to support the commercial process. Quality focused on related substance impurities with FDA moving away from Ph Eur Limits for impurities to a much tighter ICH Q3 limitations. Regulatory review of other quality features have more recently been identified in dossier review. With the recent update of Annex 1, CMC information for sterile medicinal products have become much more defined and generally has resulted in significant upgrades required. An overview of some of these newer areas of interest and constructive approaches to address these will be discussed.

Participants

Gary Musso, PhD - President, Musso and Associates LLC

Characterization and QC of Therapeutic gRNAs for Non-Viral CRISPR Editing

15:45 - 16:15
Workshop PM3: sgRNA as Intermediate Drug Substance for CRISPR Therapeutics: Analytics, Manufacturing, Regulatory and Beyond

Identifying and verifying genomic alterations resulting from off-target editing, gRNA synthesis errors, cross-contamination, or other unintended gRNA activity is critical to addressing unexpected genotoxic effects for gene and cell therapies. However, assembling the necessary components and expertise for genotoxicity characterization studies is expensive and labor intensive. To better enable the GCT community, we demonstrate a series of tools, workflows, and services that can be leveraged to perform characterization of CRISPR reagents.

Participants

Garrett Rettig, Ph.D. - Senior Director of Product Development, Integrated DNA Technologies

Panel Discussion and Ask the Consultants

16:15 - 17:00
Workshop PM1: CMC and Nonclinical Strategies for an Oligonucleotide Phase 1 IND

Gene Editing Regulatory and Delivery Challenges, and Other Perspectives

16:15 - 16:45
Workshop PM3: sgRNA as Intermediate Drug Substance for CRISPR Therapeutics: Analytics, Manufacturing, Regulatory and Beyond

The presentation will delve into cutting-edge advancements in Gene Editing platforms, and the current challenges linked to the extrahepatic delivery of genomic medicines, and share regulatory challenges and perspectives in this dynamic field. In addition, we will discuss opportunities for integrating CMC and modular manufacturing technology platforms early in development to expedite the development and translation of genome editing into transformative medicines that can change patients' lives.

Participants

Luis Santos, Ph.D. - Director, Non-viral Delivery, mRNA and LNP Product, Prime Medicine

Nitrosamine Risk Assessment

16:15 - 17:00
Workshop PM4: CMC Regulatory Strategies for Peptides

Participants

Laurin Melzig, PhD - Director Process Development, Bachem AG

Closing Remarks and Discussion

16:45 - 17:00
Workshop PM3: sgRNA as Intermediate Drug Substance for CRISPR Therapeutics: Analytics, Manufacturing, Regulatory and Beyond

Close of Workshop

17:00 - 17:05
Workshop PM1: CMC and Nonclinical Strategies for an Oligonucleotide Phase 1 IND

Close of Workshop

17:00 - 17:05
Workshop PM2: Introduction to Analytical Control Strategies for Therapeutic Oligonucleotides

Close of Workshop

17:00 - 17:05
Workshop PM3: sgRNA as Intermediate Drug Substance for CRISPR Therapeutics: Analytics, Manufacturing, Regulatory and Beyond

Close of Workshop

17:00 - 17:05
Workshop PM4: CMC Regulatory Strategies for Peptides

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08:00	**08:30** - Strategies for Oligonucleotide Purification Using Reverse Phase Resins	**08:30** - Plant-based Squalene for Parenteral Applications	**08:30** - Recent Technological Innovation in Peptide and Oligonucleotide Manufacturing											

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09:00				**09:00** - Workshop AM1: Introduction to Therapeutic Oligonucleotides - Design, Function and Delivery	**09:00** - Workshop Leaders' Introduction and Overview: Stereochemistry of Synthetic Oligonucleotides - Challenges and Opportunities	**09:00** - Workshop AM3: Introduction to Genome Editing	**09:00** - Workshop Leader's Welcome and Opening Remarks **09:15** - The Regulatory Perspective: Analytical Expectations for Peptide							

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					09:20 - Nonstereoselective Synthesis: Process, Control and Regulatory Considerations		Therapeutics **09:45** - Practical Considerations for Characterization of Peptide Drug Products							

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10:00					**10:00** - Stereoselective Synthesis: Process, Control and Regulatory Considerations **10:40** - Networking Refreshment Break		**10:15** - High-end Analytical Tools to Meet the Latest Regulatory Requirements for Generic Peptides – A Case Study **10:45** - Network-							

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11:00					**11:10** - Characterization and Stereochemical Control Strategy of siRNA **11:40** - Characterization and Control Strategy of PMO Stere-		**11:15** - Making Waves in Peptide Analysis: Solving Isomer Challenges with Ion Mobility **11:45** - Analytical Procedure Develop-							

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SCHEDULE

PRE-CONFERENCE DAY - 14/05/2024

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 1	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	WORKSHOP AM1: INTRODUCTION TO THERAPEUTIC OLIGONUCLEOTIDES - DESIGN, FUNCTION AND DELIVERY	WORKSHOP AM2: STEREOCHEMISTRY OF SYNTHETIC OLIGONUCLEOTIDES: STRATEGIES FOR ANALYSIS AND CONTROL	WORKSHOP AM3: INTRODUCTION TO GENOME EDITING	WORKSHOP AM4: ANALYTICAL STRATEGIES FOR THERAPEUTIC PEPTIDES	SPOTLIGHT PRESENTATION LUNCHEON 1	SPOTLIGHT PRESENTATION LUNCHEON 2	SPOTLIGHT PRESENTATION LUNCHEON 3	WORKSHOP PM1: CMC AND NONCLINICAL STRATEGIES FOR AN OLIGONUCLEOTIDE PHASE 1 IND	WORKSHOP PM2: INTRODUCTION TO ANALYTICAL CONTROL STRATEGIES FOR THERAPEUTIC OLIGONUCLEOTIDES	WORKSHOP PM3: SGRNA AS INTERMEDIATE DRUG SUBSTANCE FOR CRISPR THERAPEUTICS: ANALYTICS, MANUFACTURING, REGULATORY AND BEYOND	WORKSHOP PM4: CMC REGULATORY STRATEGIES FOR PEPTIDES
12:00					**12:10** - Panel Discussion with Workshop Speakers		**12:15** - Closing Remarks and Discussion	**12:30** - Enzymatic Oligonucleotide Synthesis Process Flow and Substance Impurity Profile	**12:30** - A Scalable Versatile System for mRNA-LNP Formulation Based on Impingement Jets Mixing Technology - From R&D to Com-	**12:30** - Scilex Luncheon Spotlight Presentation				

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									mercial Drug Substance Production					

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13:00											**13:30** - Workshop Leader's Welcome and Opening Remarks **13:40** - Nonclinical Aspects of an IND	**13:30** - Workshop PM2: Introduction to Analytical Control Strategies for Therapeutic Oligonucleotides	**13:30** - Workshop Leader's Welcome and Opening Remarks **13:40** - Oligonucleotide Analytics Overview with a Focus on	**13:30** - Workshop Leader's Welcome and Opening Remarks **13:35** - CMC Regulatory Challenges During Peptide Development

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PRE-CONFERENCE DAY - 14/05/2024

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													Guide RNAs for CRISPR Applications	

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PRE-CONFERENCE DAY - 14/05/2024

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14:00											**14:25** - Manufacturing Aspects of an IND		**14:10** - Overview of Guide RNAs for CRISPR Applications **14:40** - Important Aspects to Successful Manufacturing Outcomes	**14:15** - Regulatory CMC Strategies for Early Peptide Development

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PRE-CONFERENCE DAY - 14/05/2024

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													Through Agilent's Six Years of Making GMP sgRNA	

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15:00											**15:00** - Networking Refreshment Break **15:30** - Quality and Regulatory Aspects of an IND		**15:20** - Networking Refreshment Break **15:45** - Characterization and QC of Therapeutic gRNAs for Non-Viral CRISPR Editing	**15:00** - Networking Refreshment Break **15:30** - CMC Regulatory Areas of Increased Interest for Peptides

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16:00											**16:15** - Panel Discussion and Ask the Consultants		**16:15** - Gene Editing Regulatory and Delivery Challenges, and Other Perspectives **16:45** - Closing Remarks and Discussion	**16:15** - Nitrosamine Risk Assessment

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17:00											**17:00** - Close of Workshop	**17:00** - Close of Workshop	**17:00** - Close of Workshop	**17:00** - Close of Workshop

SESSIONS

MAIN CONFERENCE - DAY 1 KEYNOTE SESSIONS (MAY 15) - 15/05/2024

Registration and Breakfast

07:00 - 07:30

Pioneering a Versatile LNP Production Process for mRNA Vaccines, Therapeutics, and Gene Editing - Unveiling the Proof of Concept

07:30 - 08:00
Breakfast Spotlight Presentations 1

RNA-based therapeutics exhibit significant potential in treating various diseases, including Covid-19. These therapeutics function by either suppressing pathological genes through siRNA delivery or expressing therapeutic proteins via the introduction of exogenous mRNA into cells. Despite their promise, mRNA molecules pose challenges due to their size, fragility, and susceptibility to degradation. Crossing plasma membranes to access target cells is not facile, necessitating the development of an effective delivery solution. Amongst all delivery solutions enabling the therapeutic capabilities of siRNA, mRNA, or CRISPR for systemic applications, lipid nanoparticles (LNPs) have emerged as pivotal delivery systems. LNPs, presently at the forefront of RNA delivery platforms, have progressed into human clinical trials as well as approved market product. Their safety profile have been thoroughly assessed in both human and non-human primates. While lipid nanoparticle delivery platforms have undergone extensive research and optimization for formulating oligonucleotide drug products, they now offer a solid foundation for mRNA-based systems. However, it is crucial to note that LNPs containing mRNA require distinct treatment compared to those containing oligonucleotides, as the particle structure significantly influences stability under processing conditions.

Participants

Thomas Vrucina - Deputy Head Liposome Technology, Polymun Scientific

Millipore Breakfast Spotlight Presentation

07:30 - 08:00
Breakfast Spotlight Presentations 2

Our Progress in Taking xRNA Manufacturing Digital

07:30 - 08:00
Breakfast Spotlight Presentations 3

ReciBioPharm and MIT are in the midst of developing fully integrated and digitally controlled production lines for xRNA. Through this project we will develop an innovative production stream from DNA to Drug Product—IVT to fill-finish (1); compatible with a wide range of xRNA modalities and nanoparticle formulations (2); integrated process analytical technologies (3); include digital twins to accelerate development time (4); and include machine-learning for predictive control (5).

Participants

Aaron Cowley, PhD - Chief Scientific Officer, ReciBioPharm

Chairperson's Remarks

08:10 - 08:15
Keynote and Plenary Session

Antisense Based Therapy for Neurological Diseases

08:15 - 09:00
Keynote and Plenary Session

Currently there are multiple genetic based medicines being pursued for rare neurological diseases including antisense technology, gene therapy and gene editing technologies. Antisense oligonucleotides. Of these platforms, ASOs are one of the more advanced technologies. ASOs are synthetic, chemical modified nucleic acid analogs designed to bind to RNA by Watson-Crick base paring. Upon binding to the RNA, ASOs modulate the function of the targeted RNA through a variety of mechanisms. Both protein coding, as well as non-coding RNAs, can be targets of ASO based drugs, significantly broadening therapeutic targets for drug discovery compared to small molecules and protein based therapeutics. The approvals of nusinersen (Spinraza) as a treatment for spinal muscular atrophy (SMA) and tofersen (Qalsody) for ALS patients with SOD1 mutations validates the utility of antisense drugs for the treatment of motor neuron diseases. The application of antisense technology as potential therapy for other neurodegenerative diseases and neurodevelopmental disorders will be discussed.

Participants

Frank Bennett, PhD - Executive Vice President and Chief Scientific Officer, Ionis Pharmaceuticals

Machine Learning + Multiplex Libraries

09:00 - 09:45
Keynote and Plenary Session

Pioneering barcoded multiplexing since 1984 has enabled libraries of up to trillions of 'tides for selection &/or quantitation. Combined with AI (e.g. LLM) enables radical changes & denovo 'tides with novel properties & lower off-targeting -- including specificity for tissues, genomic location & active sites(MOA).

Participants

George Church, PhD - Professor of Genetics, Harvard Medical School

Networking Refreshment Break

09:45 - 10:15

Personalized Medicine for Agriculture – How Natural and Designed Macromolecules Are Reshaping Crop Protection and How We Grow Food

10:15 - 11:00
Keynote and Plenary Session

Agriculture is now seeing a surge in targeted and sustainable macromolecular solutions to crop protection. The US EPA has approved the first peptide bioinsecticide (Vestaron) and is currently evaluating the first RNAi bioinsecticide (Greenlight Biosciences). Challenges with the development, stabilization, and delivery of these novel crop protection classes is discussed with emphasis on cysteine-rich natural peptides.

Participants

Kyle Schneider, PhD - R&D Director, Vestaron

Targeting Transferrin Receptor to Enable Uniform Biodistribution of Antisense Oligonucleotides Using a Systemic Dose Route

11:00 - 11:45
Keynote and Plenary Session

ASOs are promising therapies, though do not cross the BBB. We use a human TfR binding molecule to transport ASO across the BBB after systemic delivery, termed oligonucleotide transport vehicle (OTV). OTV drives widespread ASO biodistribution and target knockdown across the CNS, supporting OTV's potential therapeutic use in neurological disorders.

Participants

Sarah DeVos, PhD - Director and Principal Scientist, Denali Therapeutics

SESSIONS

MAIN CONFERENCE - DAY 1 KEYNOTE SESSIONS (MAY 15) - 15/05/2024

Transition to Spotlight Presentation/Panel Luncheons

11:45 - 12:05

Expanding the mRNA Ecosystem

12:05 - 12:35
Spotlight Presentation Luncheon 1

Aldevron's significantly expanded mRNA capabilities includes enhancements to drug product and drug substance manufacturing, as well as advanced analytical testing capabilities. At this luncheon, learn more about those advancements, and how Aldevron continues to expand its sequence-to-vial mRNA ecosystem.

Participants

Todd Howren, PhD - Vice President RNA Client Services, Aldevron

Going Large-scale with Manufacturing of Oligonucleotides

12:05 - 12:35
Spotlight Presentation Luncheon 2

The growing number of oligonucleotide-based APIs is accompanied by an increasing need for efficient routes for their large-scale manufacturing. It is therefore essential to develop more efficient, more sustainable, and highly scalable manufacturing techniques. The speaker will give an overview of Bachem's existing oligonucleotide capacity based on traditional packed bed synthesizers from small-, mid-, pilot- to large-scale and according chromatography. Besides scalability considerations and equipment comparisons, the talk will also outline currently ongoing capacity expansion, where a new, additional large-scale line for metric ton oligonucleotide output is commissioned.

Participants

Daniel Samson, PhD - Vice President, Head Oligonucleotides, Bachem AG

Synthesis of Nucleic Acid Therapeutics: Next Generation Processes and Solutions

12:05 - 12:35
Spotlight Presentation Luncheon 3

The development of processes for streamlined and rapid production of mRNA is critical to enable the availability of treatments. This is required not only for pandemic situations but also for several other diseases that currently lack treatment or need an improved form. In this talk we will share data for enzymatic synthesis of DNA, personalized scale synthesis of mRNA, and other such solutions that will address needs and challenges of development of therapeutics.

Participants

Sirat Sikka - Senior Scientist, Applications & Innovation, Thermo Fisher Scientific

Investigating Critical Quality Attributes of Cap2 mRNA Using Cap Analog Libraries

12:05 - 12:35
Spotlight Presentation Luncheon 4

mRNA caps are involved in modulating translation initiation, mRNA stability, and self/non-self-recognition of mRNAs by the immune system. Cap 0 is not abundant in higher eukaryotes and can trigger innate immune responses. Cap 1 is found in all eukaryotes and promotes mRNA translation. Cap2 is present in 50% of eukaryrotic mRNA in nature and is believed to be a dynamic marker for mRNA aging. Also, cap2 functions to reduce activation of the innate immune response. In this presentation, we will discuss the results of screening cap 2 analogs for their impacts on protein expression and immunogenicity in mice.

Participants

Chunping Xu, Ph.D - Director of Chemistry R&D, TriLink BioTechnologies

LGC Axolabs Spotlight Presentation

12:05 - 12:35
Spotlight Workshop Luncheon 5

Nitto Avecia Spotlight Presentation

12:05 - 12:35
Spotlight Presentation Luncheon 6

Short Networking Break

12:35 - 12:55

Chairperson's Remarks

12:55 - 13:00
Keynote and Plenary Session

Tackling ASCVD at Scale Via a Suite of Long-acting Oligonucleotide Therapies

13:00 - 13:45
Keynote and Plenary Session

Atherosclerotic cardiovascular disease (ASCVD) is the number one killer in the world. Although effective oral medicines are available for key ASCVD drivers, including high cholesterol and hypertension, poor adherence is a major barrier to real-world efficacy. Building on Novartis' foundational, cholesterol-lowering siRNA Leqvio, our goal is to improve and extend people's lives by tacking multiple ASCVD risk factors with a suite of long-acting, oligonucleotide therapies.

Participants

Meg Brousseau, Ph.D. - Executive Director, Cardiovascular & Metabolic Diseases, Novartis

Development and Approval of RIVFLOZA® (Nedosiran)

13:45 - 14:30
Keynote and Plenary Session

This presentation will discuss the development program and strategies for RIVFLOZA® including thoughts on the current and future label in US and RoW. I will also share thoughts on biotech development compared to "big pharma" as well as thoughts on supply chain.

Participants

Jacob Hyllested-Winge, M.D. - Project Vice President, Boston Global Development, Novo Nordisk

Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break

14:30 - 15:30

GLP-1 Clinical Progress, Pipeline and Innovation

15:30 - 16:15
Keynote and Plenary Session

Participants

Kieren Mather, MD - Associate VP-Medical-Incretins and Diabetes Breakthroughs, Early Clinical Research, Eli Lilly and Company

Late Breaking Keynote Presentation

16:15 - 17:00
Keynote and Plenary Session

Networking Reception in the Poster and Exhibit Hall

17:00 - 18:30

Join fellow attendees, speakers and exhibitors in the exhibit hall for an evening of fun food, drink, poster/ exhibit viewing and networking. This evening reception is a great opportunity to make new contacts, re-connect with old colleagues and browse the exciting technologies, products and services in the exhibition and poster area.

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MAIN CONFERENCE - DAY 1 KEYNOTE SESSIONS (MAY 15) - 15/05/2024

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07:00	**07:30** - Pioneering a Versatile LNP Production Process for mRNA Vaccines, Therapeutics, and Gene Editing - Unveiling the Proof of Concept **07:00** - Registration and Breakfast	**07:30** - Millipore Breakfast Spotlight Presentation **07:00** - Registration and Breakfast	**07:30** - Our Progress in Taking xRNA Manufacturing Digital **07:00** - Registration and Breakfast	**07:00** - Registration and Breakfast	**07:00** - Registration and Breakfast	**07:00** - Registration and Breakfast	**07:00** - Registration and Breakfast	**07:00** - Registration and Breakfast	**07:00** - Registration and Breakfast	**07:00** - Registration and Breakfast
08:00				**08:10** - Chairperson's Remarks **08:15** - Antisense Based Therapy for Neurological Diseases						
09:00	**09:45** - Networking Refreshment Break	**09:45** - Networking Refreshment Break	**09:45** - Networking Refreshment Break	**09:00** - Machine Learning + Multiplex Libraries **09:45** - Networking Refreshment Break	**09:45** - Networking Refreshment Break	**09:45** - Networking Refreshment Break	**09:45** - Networking Refreshment Break	**09:45** - Networking Refreshment Break	**09:45** - Networking Refreshment Break	**09:45** - Networking Refreshment Break

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10:00				**10:15** - Personalized Medicine for Agriculture – How Natural and Designed Macromolecules Are Reshaping Crop Protection and How We Grow Food						
11:00	**11:45** - Transition to Spotlight Presentation/ Panel Luncheons	**11:45** - Transition to Spotlight Presentation/ Panel Luncheons	**11:45** - Transition to Spotlight Presentation/ Panel Luncheons	**11:00** - Targeting Transferrin Receptor to Enable Uniform Biodistribution of Antisense Oligonucleotides Using a Systemic Dose Route **11:45** - Transition to Spotlight Presentation/ Panel Luncheons	**11:45** - Transition to Spotlight Presentation/ Panel Luncheons	**11:45** - Transition to Spotlight Presentation/ Panel Luncheons	**11:45** - Transition to Spotlight Presentation/ Panel Luncheons	**11:45** - Transition to Spotlight Presentation/ Panel Luncheons	**11:45** - Transition to Spotlight Presentation/ Panel Luncheons	**11:45** - Transition to Spotlight Presentation/ Panel Luncheons

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MAIN CONFERENCE - DAY 1 KEYNOTE SESSIONS (MAY 15) - 15/05/2024

TIDES USA: Oligonucleotide & Peptide Therapeutics

May 14-17, 2024 | In-Person + Digital
Boston, MA, USA
Hynes Convention Center

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12:00	**12:35** - Short Networking Break	**12:35** - Short Networking Break	**12:35** - Short Networking Break	**12:55** - Chairperson's Remarks **12:35** - Short Networking Break	**12:05** - Expanding the mRNA Ecosystem **12:35** - Short Networking Break	**12:05** - Going Large-scale with Manufacturing of Oligonucleotides **12:35** - Short Networking Break	**12:05** - Synthesis of Nucleic Acid Therapeutics: Next Generation Processes and Solutions **12:35** - Short Networking Break	**12:05** - Investigating Critical Quality Attributes of Cap2 mRNA Using Cap Analog Libraries **12:35** - Short Networking Break	**12:05** - LGC Axolabs Spotlight Presentation **12:35** - Short Networking Break	**12:05** - Nitto Avecia Spotlight Presentation **12:35** - Short Networking Break
13:00				**13:00** - Tackling ASCVD at Scale Via a Suite of Long-acting Oligonucleotide Therapies **13:45** - Development and Approval of RIVFLOZA® (Nedosiran)						
14:00	**14:30** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**14:30** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**14:30** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**14:30** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**14:30** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**14:30** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**14:30** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**14:30** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**14:30** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break	**14:30** - Grand Opening of Poster and Exhibit Hall and Networking Refreshment Break

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15:00				**15:30** - GLP-1 Clinical Progress, Pipeline and Innovation						
16:00				**16:15** - Late Breaking Keynote Presentation						
17:00	**17:00** - Networking Reception in the Poster and Exhibit Hall	**17:00** - Networking Reception in the Poster and Exhibit Hall	**17:00** - Networking Reception in the Poster and Exhibit Hall	**17:00** - Networking Reception in the Poster and Exhibit Hall	**17:00** - Networking Reception in the Poster and Exhibit Hall	**17:00** - Networking Reception in the Poster and Exhibit Hall	**17:00** - Networking Reception in the Poster and Exhibit Hall	**17:00** - Networking Reception in the Poster and Exhibit Hall	**17:00** - Networking Reception in the Poster and Exhibit Hall	**17:00** - Networking Reception in the Poster and Exhibit Hall

SESSIONS

MAIN CONFERENCE - DAY 2 (MAY 16) - 16/05/2024

TIDES Early Morning Healthy Activity TBA

06:30 - 07:30

Registration

07:30 - 07:45

Single-use Considerations for Research and Manufacturing in Oligonucleotide Therapeutics

07:45 - 08:15
Breakfast Spotlight Presentations 1

It's time for oligonucleotide therapeutics to step into the light. With an increase in commercialized therapies, therapeutic developers need to scale up quickly to meet demand while using the same manufacturing suite for a diversity of drugs. Keeping the end in mind while considering manufacturability, scale-up, and the use of single-use technologies will help deliver process efficiency. In this presentation, we'll discuss strategies to future proof your manufacturing by considering decisions early on in your therapeutics development.

Participants

Justin Townsend - Oligonucleotide Solid Phase Synthesis Specialist, Cytiva

New Ligation Approach: Technology for High Quality Manufacturing of Over 150 mer RNA

07:45 - 08:15
Breakfast Spotlight Presentations 2

Participants

Masato Sanosaka, PhD - Group Leader of Research & Process Development, Ajinomoto Biopharma Services

GenScript Breakfast Spotlight Presentations

07:45 - 08:15
Breakfast Spotlight Presentations 3

Chairman's Remarks: Macrocyclic Peptides and Peptide Discovery

08:15 - 08:20
Peptide Discovery to CMC

Participants

Trishul Shah, M.S. - Director, Business Development, North America, PolyPeptide Laboratories Inc.

Discovery of Zilucoplan: A Potent Macrocyclic Peptide Complement Component 5 (C5) Inhibitor in Acetylcholine Receptor Antibody-positive Generalized Myasthenia Gravis

08:20 - 08:45
Peptide Discovery to CMC

Cyclic peptides are diverse molecules that are now a focus in drug discovery efforts. Their molecular size, between small molecules and biologics, provides attractive scaffolds to screen against some challenging targets, including protein-protein interactions and those considered to be "undruggable" proteins. With messenger ribonucleic acid (mRNA) display screening technology now able to produce trillions of peptide molecules for screening and quickly identify tight binders against targeting proteins, an exciting time of cyclic peptide drug discovery has come. We have been working on cyclic peptide drug discovery since 2010 and have successfully identified two compounds derived from mRNA display that have entered clinical trials. One of them is a complement C5 inhibitor, zilucoplan. Here we present the discovery of zilucoplan, starting from hits identification via mRNA display screening against C5, followed by medicinal chemistry modifications to improve the potency, plasma stability and PK properties, leading to the clinical candidate.

Participants

Lihu Yang, PhD - Head of Discovery Chemistry US, UCB

Chairman's Remarks: Oligonucleotide Chemistry, Mechanisms and Preclinical

08:25 - 08:30
Oligonucleotide Discovery, Preclinical and Clinical

Participants

Troels Koch, PhD - Senior Vice President, Chemistry, MiNa Therapeutics

Chairman's Remarks: Emerging Trends in Oligonucleotide Synthesis

08:25 - 08:30
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

Yogesh Sanghvi, PhD - President, Rasayan Inc.

Chairman's Remarks: Optimization of mRNA Sequence and Structure

08:25 - 08:30
mRNA Technology and Applications

Participants

Andreas Kuhn, PhD - Senior Vice President RNA Biochemistry & CMC Management, BioNTech SE

Co-Chairs' Remarks: Genome Editing Delivery

08:25 - 08:30
Delivery of Macromolecules

Participants

Rubina Parmar, PhD - Vice President, Chemistry and Delivery Sciences, Intellia Therapeutics

Luis Brito, PhD - Vice President, Delivery Platform, Beam Therapeutics

siRNA Phosphate Backbone Engineering to Enhance Potency and Extrahepatic Tissue Accumulation

08:30 - 09:00
Oligonucleotide Discovery, Preclinical and Clinical

Modulating backbone structures of siRNAs has a profound impact on pharmacokinetics and pharmacodynamics profiles. Our recent chemistry advances such as extended nucleic acids (exNA) that enables significantly improved plasma pharmacokinetics and 4 to 20-fold increased extrahepatic tissue accumulation will be presented.

Participants

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

Biocatalytic Approaches to Nucleic Acid Therapeutics Manufacturing

08:30 - 09:00
Oligonucleotide Chemistry, Manufacturing and Controls

The rapidly growing number of therapies approved and in advanced clinical trials is placing unprecedented demands on our capacity to manufacture oligonucleotides at scale. Existing methods of chemical synthesis rely on iterative coupling, capping, oxidation and deprotection to achieve stepwise extension of sequences immobilized on solid supports and are limited by their scalability and sustainability. This talk will describe transformative biocatalytic approaches to efficiently produce oligonucleotides in a single operation, where polymerases and endonucleases work in synergy to amplify complementary sequences embedded within catalytic self-priming templates. This approach uses unprotected building blocks, aqueous conditions and can be used to produce diverse oligonucleotide sequences containing a range of pharmaceutically relevant modifications.

Participants

Sarah Lovelock, Ph.D. - Reader in Biological Chemistry, University of Manchester

SESSIONS

MAIN CONFERENCE - DAY 2 (MAY 16) - 16/05/2024

Enhancing mRNA Translation Efficiency through Trinucleotide Cap Modifications

08:30 - 09:00
mRNA Technology and Applications

Novel chemically modified trinucleotide caps enable mRNAs to be synthesized in a "one-pot" procedure with high capping rate and yield, and mRNAs capped with the modified analogs demonstrate enhanced protein expression. In addition, Series of fluorescent labelled mRNAs can be directly transcribed with fluorescent caps for ready tracing.

Participants

Jiancun Larry Zhang, Ph.D. - President and CEO, Guangzhou Henovcom Biosciences

In vivo Delivery of LNP-encapsulated RNA to Immune Cells

08:30 - 09:00
Delivery of Macromolecules

Recently, great progress has been made delivering RNA medicines—including genetic editing technologies—e*x vivo*, however, the future now focuses on their *in vivo* delivery. Using a large proprietary lipid nanoparticle library, we have made progress towards unlocking RNA delivery to immune cells, enabling us to build impactful therapeutic programs.

Participants

Pete Smith, Ph.D. - Chief Scientific Officer, ReNAgade Therapeutics

Bicycles as Modular and Precision Guided Anti-tumor Immune Cell Agonists

08:45 - 09:10
Peptide Discovery to CMC

Bicycles are low molecular weight bicyclic peptides constrained via a chemical scaffold. The pharmacologic and pharmacodynamic properties of Bicycles are highly suited to the delivery of potent payloads such as toxins, radionuclides and immune agonists in oncology. This presentation will focus on the application of Bicycle tumor targeted immune cell agonists (*Bicycle* TICA^{TM}) that simultaneously bind to overexpressed cell-surface targets on tumor cells and activating receptors on immune cells to drive highly specific anti-tumor activity.

Participants

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

Cyclic Structured Oligonucleotides for RNA Therapeutics

09:00 - 09:30
Oligonucleotide Discovery, Preclinical and Clinical

Chemistry has been crucial in providing drug-like properties to an oligonucleotide sequence. Our early work led to the design of Gapmer antisense for RNase H-mediated knockdown of targeted RNA and the design of phosphorothioate 2-substituted RNA for modulation of splicing. These chemistries have facilitated the development of antisense candidates and the approval of drugs. Recently, our focus has shifted to the chemical engineering of oligonucleotides, which led to the design of cyclic-structured oligonucleotides. Details will be provided on the cyclic structured oligonucleotides' design, potency, specificity, and applicability to various mechanisms.

Participants

Sudhir Agrawal - President and Founder, Arnay Sciences

A Platform for Controlled Template-Independent Enzymatic Synthesis of RNA Oligonucleotides and Therapeutics

09:00 - 09:30
Oligonucleotide Chemistry, Manufacturing and Controls

Therapeutic RNA oligonucleotides have shown tremendous potential to manage and treat disease, yet current manufacturing methods may not be able to deliver on this promise. Here, we report the development and optimization of a novel, aqueous-based, template-independent enzymatic RNA oligonucleotide synthesis platform as an alternative to traditional chemical methodologies. Our platform is made possible by reversible terminator nucleoside triphosphates and an enzyme capable of their incorporation. We show that many common therapeutic RNA modifications are compatible with our process and demonstrate the enzymatic synthesis of natural and modified oligonucleotides in both liquid and solid phases. Our platform offers many unique advantages over chemical synthesis, including the realization of a more sustainable process to produce therapeutic RNA oligonucleotides.

Participants

Daniel Wiegand - CEO and Co-Founder, EnPlusOne Biosciences

AvantCap – An Inspiration from Posttranscriptional Modification of mRNA 5'end

09:00 - 09:30
mRNA Technology and Applications

Eukaryotic mRNAs undergo co-transcriptional 5'-end modification with a 7-methylguanosine cap. In higher eukaryotes, the cap carries additional methylations, such as $^{m6}A_m$ – a common epitranscriptomic mark unique to the mRNA 5'-end. This modification is regulated by the Pcif1 methyltransferase and the FTO demethylase, but its biological function is still unknown. We designed and synthesized a trinucleotide FTO-resistant *N*6-benzyl analog of the $^{m6}A_m$-cap – $m^7Gppp^{Bn6}A_mpG$ (termed *AvantCap*) and incorporated it into mRNA using T7 polymerase. $m^7Gppp^{Bn6}A_mpG$-capped mRNAs encoding reporter proteins administered intravenously to mice provided up to 6-fold higher protein outputs than reference mRNAs, while mRNAs encoding tumor antigens showed superior activity in therapeutic setting as anti-cancer vaccines.

Participants

Jacek Jemielity, Ph.D. - Head of Laboratory, University of Warsaw and CEO, Explorna Therapeutics

Delivery of RNA Gene Writing Systems to Liver and Beyond

09:00 - 09:30
Delivery of Macromolecules

Tessera Therapeutics is pioneering a suite of RNA Gene Writer systems that can introduce a broad range of edits to the genome. To enable the use of this suite of gene editing technologies for in vivo editing, we have developed a nonviral lipid nanoparticle delivery platform capable of in vivo RNA delivery to multiple tissues, including T cells and hematopoietic stem cells.

Participants

Jane Wang, Ph.D. - Executive Director of Delivery Technologies, Tessera Therapeutics

MK-0616 Showcases the Potential of Macrocycles as Oral Drugs for Extracellular Targets for Atherosclerotic CVD

09:10 - 09:35
Peptide Discovery to CMC

Participants

Marc Becker, PhD - Senior Scientist, Merck

Xeno Nucleic Acid (XNA) Modifications for Improving RNAi Therapeutics

09:30 - 10:00
Oligonucleotide Discovery, Preclinical and Clinical

We have shown that acyclic (*S*)-glycol nucleic acid (*S*-GNA) and L-α-threofuranosyl nucleic acid (TNA, which has a tetrose sugar) modifications of siRNAs improve the safety of RNAi therapeutics while maintaining potency. We have also evaluated LNA and related analogs for RNAi therapeutics and our structure-activity relationship findings will be presented. (RNA. 2023 402–414; doi: 10.1261/rna.079526.122)

Participants

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

Enzymatic Synthesis of RNA with Chemical Modifications

09:30 - 10:00
Oligonucleotide Chemistry, Manufacturing and Controls

Chemically modified synthetic RNA used for therapeutic applications such as antisense oligonucleotides and RNA based genome engineering are difficult and expensive to synthesize using current phosphoramidite chemical synthesis methods. Thus, new RNA synthesis technologies designed to significantly increase yields and efficiency while greatly reducing costs are needed. We demonstrate rapid and efficient one step enzymatic synthesis of chemically modified RNA oligonucleotides over 150 nt in length with >90-95% purity. A newly discovered RNA polymerase efficiently synthesizes RNA with 100% 2'-fluoro, 2'-O-methyl or alpha-phosphorothioate modified ribonucleotides. We anticipate that scaling up of this versatile enzymatic technology will allow for significant reductions in the cost of synthesizing chemically modified RNA oligonucleotides and mRNA based therapeutics while greatly increasing yields for large scale production.

Participants

Richard Pomerantz, Ph.D. - Associate Professor, Biochemistry and Molecular Bi, Thomas Jefferson University

Discovering New Cap Analogs and Their Performances in Difference mRNA Constructs

09:30 - 10:00
mRNA Technology and Applications

With mRNA gaining acceptance as a therapeutic modality, more cap analogs may be needed to tailor for different applications, for example, burst of protein expression for gene editing vs. persistent expression for protein replacement. We employ an AI-assisted discovery platform, from in-silico design to molecular docking to performance analysis, with a goal to delineate structure-activity relationship. The top cap analog candidates were tested in-vitro and in-vivo using model mRNA constructs such as firefly luciferase. Furthermore, the top cap analogs were used to make various mRNA vaccines (e.g. RV, VZV, HPV) and tested in mice. We discovered that different cap analogs performed differently depending on mRNA sequence. More work needs to be done to identify the cause for performance variability. Since the novel cap analogs are new to the market, various testing was conducted in mice and NHP to demonstrate their safety.

Participants

May Guo - Chief Commercial Officer, Areterna

Lipid Nanoparticles for Overcoming Biological Barriers to mRNA Delivery

09:30 - 10:00
Delivery of Macromolecules

Recent years have witnessed tremendous developments and breakthroughs in the field of RNA-based therapeutics and vaccines. The distinct mechanisms of exogenous RNAs and analogs, including messenger RNAs, small interfering RNAs, microRNAs, and antisense oligonucleotides, have brought them unprecedented potential to treat a variety of pathological conditions. However, the widespread application of RNA therapeutics and vaccines is hampered by their intrinsic features (e.g., instability, large size, and dense negative charge) and formidable host barriers. Development of safe and efficient vectors is key for successful delivery and translation of RNA therapeutics and vaccines. In this talk, I will discuss our efforts towards the development of lipid nanoparticle (LNP) platforms that enable the delivery of RNA therapeutics and vaccines to a range of target cells and tissues in the body. Furthermore, I will describe new therapeutic strategies utilizing these LNPs including (i) in vivo reprogramming of immune cells for cancer immunotherapy and vaccination, (ii) in utero gene editing for treating disease before birth, and (iii) mRNA prenatal therapeutics for treating pregnancy disorders such as pre-eclampsia.

Participants

Michael Mitchell, PhD - Associate Professor of Bioengineering, University of Pennsylvania

Anti-tumor Activities of HeliconTM Peptide Inhibitors of β-catenin/TCF Interaction in Cancer Patient-derived Xenograft Models

09:35 - 10:00
Peptide Discovery to CMC

Blocking the β-catenin-TCF/LEF interaction offers an attractive therapeutic strategy to treat a large population of patients with WNT pathway mutations. We have successfully discovered and developed conformationally hyperstabilized α-helical peptides (Helicons) that bind directly to β-catenin with picomolar affinity. In vivo, the Helicons display favorable pharmacokinetic properties, broad tissue distribution and potent anti-tumor effects. Inhibiting β-catenin-TCF interaction with Helicons represents a first-in-class therapeutic approach for the treatment of cancers resulting from aberrant transcriptional signaling via β-catenin.

Participants

Yaguang Si - Senior Director Biology, FogPharma

Networking Refreshment Break in Poster and Exhibit Hall

10:00 - 10:45

Bivalent Recognition of RNA-Repeated Expansions

10:45 - 11:15
Oligonucleotide Discovery, Preclinical and Clinical

This talk highlights the latest results in an effort to develop a bifacial molecular platform, referred to as 'Janus-base' (JB), designed for targeting RNA repeat expansions in a sequence-specific and selective manner. This platform can be tailor-designed to bind to any repeat sequence. The newly designed 'ligands' are relatively small in size (3 units in length) and bear a closer resemblance to small molecules than 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 the RNA double helix. The work provides proof-of-concept that such relatively small nucleic acid 'ligands' could be developed for the recognition of CUGexp-RNA transcripts.

Participants

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

SESSIONS

MAIN CONFERENCE - DAY 2 (MAY 16) - 16/05/2024

A Platform Approach to Manufacturing Single Stranded Oligonucleotides by Enzymatic Assembly

10:45 - 11:15
Oligonucleotide Chemistry, Manufacturing and Controls

GSK has developed a templated oligonucleotide assembly platform that takes advantage of engineered DNA ligases to make single stranded oligonucleotides. The process eliminates the need for chromatography yet produces oligonucleotides with purity that exceeds that typically seen for solid supported synthesis. Data on application of this platform to different oligonucleotide types and progress on scale up will be presented.

Participants

David Tew - Senior Scientific Director, Enzyme Engineering and, Glaxosmithkline Medical Reseach Centre

Design of Highly Functional Libraries with Hyperstable Peptide and Venom Scaffolds Assisted with Machine Learning

10:45 - 11:15
Peptide Discovery to CMC

While peptides offer potential for therapeutics, their instability hampers their effectiveness. We aimed to enhance the stability of peptide scaffolds with multiple disulfides found in natural venom. Using a large dataset from yeast surface display of de novo designed hyperstable peptides, we trained a machine learning model to predict peptide folding with high accuracy. Leveraging these insights, we designed new peptide scaffold libraries optimized for folding efficiency. Successive trials yielded favorable results in folding and stability. Our innovative methodology, combining yeast experiments and machine learning, enhances therapeutic peptide design - promising greater efficiency for future peptide therapies.

Participants

Yingnan Zhang - Senior Principal Scientific Manager, Genentech

Modeling and Design of RNA, Including mRNA

10:45 - 11:15
mRNA Technology and Applications

The discovery and design of biologically important RNA molecules and medicines has lagged behind proteins, in part due to the general difficulty of RNA structural modeling. What are the prospects for an AlphaFold for RNA? I'll describe some recent progress in modeling RNA structure, including super folder mRNAs, from current and upcoming internet-scale competitions hosted on the Eterna, Kaggle, and CASP platforms.

Participants

Rhiju Das, PhD - Professor of Biochemistry, Stanford University

RNA-Based Approach to Delivering Prime Editing

10:45 - 11:15
Delivery of Macromolecules

Prime Editing (PE) is a next-generation gene editing technology that can precisely correct more than 90% of all pathogenic human mutations without the need for double-strand breaks (DSBs), with minimal byproducts at the edit site, minimal off-target activity and minimal risk of large chromosomal alterations or genotoxicity sometimes observed with CRISPR-Cas9. We have developed a lipid nanoparticle (LNP) delivery system to deliver PE drug component RNAs by intravenous infusion and have recently made several advances in engineering our Prime Editor mRNAs and Prime Editor guide RNAs (pegRNA) to provide instructions to the cell effectively for a precision gene correction event. Herein, we will overview our recent advances in our RNA platform to enable Prime Editing therapies for patients.

Participants

Seth Alexander, Ph.D. - Director of RNA Technologies, Prime Medicine

Novel Chemistries in Gene Silencing and Prime Editing

11:15 - 11:45
Oligonucleotide Discovery, Preclinical and Clinical

Participants

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

Pushing the Boundaries of Nucleic Acid Synthesis

11:15 - 11:45
Oligonucleotide Chemistry, Manufacturing and Controls

This presentation provides the background on enzymatic nucleic acid synthesis based on polymerase-nucleotide conjugates and describes how the technology can be used to generate oligos of unprecedented length and quality.

Participants

Sebastian Palluk, Ph.D. - Chief Technology Officer, Ansa Biotechnologies

First De-novo Designed Cyclic Peptides for SORT1

11:15 - 11:45
Peptide Discovery to CMC

ProteinQure has designed the first known cyclic binders to SORT1 using our proprietary computational platform. Sortilin (SORT1) is a member of the vacuolar protein sorting 10 protein (Vps10p). As a cell surface receptor, SORT1 is able to mediate efficient endocytosis of extracellular ligands to the lysosomal compartment. Numerous reports have identified enriched SORT1 expression in a variety of tumor types, including triple-negative breast cancer (TNBC), a subtype of breast cancer associated with aggressive clinical behavior and poor disease outcomes. We sought to exploit SORT1-dependent internalization of peptides as a platform for rapid and specific chemotherapy delivery into TNBC cells. Using PQStudio (our proprietary computation-enabled design capabilities), we generated high affinity SORT1 targeting peptides that exhibit efficient receptor dependent internalization and lysosomal localization. Alternative computational approaches such as AlphaFold2 and large language models failed to recapitulate the peptide design. Peptide drug conjugates (PDCs) were generated via a linkage strategy that combines our designed peptides to the antimitotic agent monomethyl auristatin E (MMAE). Our PDC molecules exhibit potent tumor regression in a MDA-MB-231 TNBC cell derived xenograft model, thereby highlighting the potential of SORT1-engaging PDCs as an efficacious targeted chemotherapeutic delivery strategy.

Participants

Lucas Siow - CEO and Co-Founder, ProteinQure

SESSIONS

MAIN CONFERENCE - DAY 2 (MAY 16) - 16/05/2024

AI-Optimized mRNA Design Improves Stability and Immunogenicity

11:15 - 11:45
mRNA Technology and Applications

Messenger RNA (mRNA) vaccines have been successful in COVID-19, but still exhibit the critical limitation of mRNA instability. Therefore, we want to optimize both structural stability and codon usage to enhance protein expression. However, due to synonymous codons, the mRNA design space is prohibitively large—for example, there are 10^632 candidate mRNA sequences for the SARS-CoV-2 spike protein, which poses insurmountable computational challenges. Here we provide a simple and unexpected solution inspired by AI (natural language processing). Our algorithm LinearDesign calculates an optimal mRNA design for the spike protein in just 11 minutes and can concurrently optimize stability and codon usage. LinearDesign substantially improves mRNA half-life and protein expression, and profoundly increases antibody titre by up to 128 times in mice compared to the codon-optimization benchmark on mRNA vaccines for COVID-19 and varicella-zoster virus. Our technology can be used for both vaccines and therapeutics and has been licensed (non-exclusively) to Sanofi.

Participants

Liang Huang, PhD - Co-Founder, Coderna.ai, Inc. and Professor of Computer Science and Biochemistry, Oregon State University

Late Breaking Presentation

11:15 - 11:45
Delivery of Macromolecules

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Late Breaking Presentation

11:45 - 12:15
Oligonucleotide Discovery, Preclinical and Clinical

The Next-generation of Oligonucleotide Chemistry Using the P(V) Platform

11:45 - 12:15
Oligonucleotide Chemistry, Manufacturing and Controls

This talk will focus on a platform of novel P(V) reagents for the synthesis of nucleic acids and other phosphorus containing molecules. The historical development of these reagents in collaboration with BMS will be described along with their application to the synthesis of therapeutically relevant molecules with an emphasis of novel backbone chemistries and how Elsie deploys these reagents in our pursuit of oligonucleotide therapeutics.

Participants

Kyle Knouse, Ph.D. - Co-Founder and Director of Chemistry, Elsie Biotechnologies

Harnessing the Power of Dual Incretin Agonists to Target Cardiometabolic Diseases

11:45 - 12:15
Peptide Discovery to CMC

Beyond GLP-1R mono-agonism, recent efforts by the biopharmaceutical industry have focused on targeting multiple incretin receptors. Pemvidutide is a novel, balanced (1:1), GLP-1R/Glucagon receptor (GCGR) agonist under development for the treatment of obesity and MASH (formerly known as NASH). GCCR agonism enhances hepatic lipid metabolism and may promote a negative energy balance. Hence, GLP-1R/GCCR co-agonism has the potential to produce additional favorable cardiometabolic benefits in people with obesity and MASH".

Participants

Shaheen Tomah, M.D. - Associate Director, Clinical Development, Altimmune

Deep Learning Guided Optimization of Translation Efficiency for mRNA Vaccine Development

11:45 - 12:15
mRNA Technology and Applications

Delivered mRNA vaccines benefit from a high protein yield to stimulate an effective immune response. We trained RiboNN, a deep learning model, to predict translation efficiency—a major determinant of protein yield—among numerous cell types. RiboNN can be used to guide the design of translation-optimized mRNA therapeutics.

Participants

Vikram Agarwal, Ph.D. - Head of mRNA Platform Design Data Science, Sanofi Pasteur

Late Breaking Presentation

11:45 - 12:15
Delivery of Macromolecules

Transition to Spotlight Presentation Rooms

12:15 - 12:20
Oligonucleotide Discovery, Preclinical and Clinical

Transition to Spotlight Presentation Rooms

12:15 - 12:20
Oligonucleotide Chemistry, Manufacturing and Controls

Transition to Spotlight Presentation Rooms

12:15 - 12:20
Peptide Discovery to CMC

Transition to Spotlight Presentation Rooms

12:15 - 12:20
mRNA Technology and Applications

Transition to Spotlight Presentation Rooms

12:15 - 12:20
Delivery of Macromolecules

Green SPPS – An Effort to Minimize the Environmental Impact Related to the SPPS Process

12:20 - 12:50
Spotlight Presentation 1

In recent years, one of CordenPharma's green initiatives has been to reduce our carbon footprint. This presentation will cover efforts to develop a SPPS protocol that minimizes or eliminates the usage of DMF and NMP, as well as reduces the total solvent consumption for the SPPS process. Preliminary results from the exploration on solvent usage reduction, including key parameters such as *in-situ* amino acid activation and coupling/deprotection/wash solvents, will be presented.

Participants

Lin Chen, PhD - Manager, Process Development, Corden Pharma Colorado

SESSIONS

MAIN CONFERENCE - DAY 2 (MAY 16) - 16/05/2024

Process Development for sgRNA in CRISPR/ Casx Therapeutics

12:20 - 12:50
Spotlight Presentation 2

The CRISPR/Casx system is widely recognized as a breakthrough technology for precisely editing DNA sequences, allowing for the removal, addition, or alteration of genetic material. Comprising two crucial elements, the system features the enzymatic scissor, Casx, and the guide RNA, known as single guide RNA (sgRNA), tasked with precision in genome targeting. sgRNA can be generated through cell transcription, in vitro transcription (IVT), or solid-supported synthesis. The growing demand for solid-supported synthesis, particularly for longmers (>100mer), to meet therapeutic needs presents unique challenges compared to ASO or siRNA synthesis. This presentation will delve into the outcomes of process optimization for longmer preparation, shedding light on the impact of key parameters.

Participants

Sungwon Kim, Ph.D. - Head of Oligonucleotide R&D, ST Pharm

Unveiling Impurities of Chemically Synthesized gRNAs

12:20 - 12:50
Spotlight Presentation 3

Cell & Gene Therapy is gaining momentum, necessitating the use of chemically synthesized gRNAs with high purity. However, conventional analytical methods can be deceptive, as gRNAs with poor quality may appear to be 80% pure or more. We will showcase hidden impurities in these gRNAs and also demonstrate that gRNA manufacturing with genuine purity of over 80% has been achieved for 100mer and more by utilizing PMM amidites and high-resolution analysis.

Participants

Akihiro Sakata - Senior Research Scientist, Sumitomo Chemical Co., Ltd.

Charting New Horizons in Guide RNA Manufacturing

12:20 - 12:50
Spotlight Presentation 4

Pioneering advancements in guide RNA manufacturing since 2016, BioSpring is a global supplier of commercial, clinical, and preclinical guide RNA. In this talk, our leading manufacturing expert will guide you through what it takes to scale GMP guide RNA manufacturing for clinical and commercial use, the extensive development involved, and the engineering and innovation that goes into evolving reliable high resolution analytical methods and achieving high purity, even in long and complex guide RNA constructs.

Participants

Raoul Hennig, Ph.D. - Head of Manufacturing Site II, BioSpring

Oligo Manufacturing Innovations – From Synthesis through Concentration

12:20 - 12:50
Spotlight Presentation 5

This presentation will explore the oligo manufacturing process – from synthesis through concentration – and the applicable equipment considerations for effective technical implementation. It will also introduce the latest innovations from Asahi Kasei Bioprocess that allow for a nearly complete manufacturing line offering.

Participants

Tom Krebstakies, PhD - Sales Manager - Europe & Asia, Asahi Kasei Bioprocess

Networking Luncheon in Poster and Exhibit Hall

12:50 - 13:55

Chairman's Remarks: Oligonucleotide Discovery and Development

13:55 - 14:00
Oligonucleotide Discovery, Preclinical and Clinical

Chairman's Remarks and Memorial Tribute to Paul McCormac

13:55 - 14:05
Oligonucleotide Chemistry, Manufacturing and Controls

Session: Oligonucleotide CMC Strategies and Case Studies

Participants

Kevin Fettes, PhD - Principal and Founder, FTS Pharma Consulting, LLC

Chairman's Remarks: Best Practices and Case Studies in Peptide Manufacturing and CMC

13:55 - 14:00
Peptide Discovery to CMC

Participants

Mimoun Ayoub, PhD - Senior VP, Global Head of Sales and Key Account Management, CordenPharma International

Chairman's Remarks: mRNA Preclinical and Clinical Progress Outside of COVID/ID Vaccines: New mRNA Therapeutic Frontiers & Novel Disease Indications

13:55 - 14:00
mRNA Technology and Applications

Participants

Frank DeRosa, PhD - CTO & Global Head of Research, mRNA Center of Exce, Sanofi

Chairman's Remarks: Next-Generation Delivery Platforms

13:55 - 14:00
Delivery of Macromolecules

Participants

Stephen Spagnol, PhD - Director, Enabling Technologies, Merck

SESSIONS

MAIN CONFERENCE - DAY 2 (MAY 16) - 16/05/2024

Ligand Mediated Delivery of Oligonucleotides Across the Blood Brain Barrier

14:00 - 14:30
Oligonucleotide Discovery, Preclinical and Clinical

Therapeutic oligonucleotides benefit patients suffering from neurological disease, but do not cross the blood brain barrier (BBB) and require intrathecal administration to reach the central nervous system (CNS). To reduce patient burden and improve distribution to deep brain regions, we developed a ligand conjugated antisense (LICA) approach that delivers ASOs and siRNAs across the BBB in mice, resulting in target mRNA reduction throughout the CNS.

Participants

Ian Huggins, Ph.D. - Research Fellow, Medicinal Chemistry, Ionis Pharmaceuticals

Perspective on Current Industry Control Strategies for Synthetic Peptides

14:00 - 14:30
Peptide Discovery to CMC

There is currently a lack of guidelines and harmonization from Health Agencies on the control strategies that should be implemented for synthetic peptide active pharmaceutical ingredients. In the US, further ambiguity is perceived since synthetic peptides > 40 amino acids are registered as BLA and synthetic peptides ≤ 40 amino acids are registered as NDA. This can result in an increased regulatory risk at the time of filing and globally divergent, non-efficient approaches. The Peptides Working Group (WG), as a part of the International Consortium for Innovation & Quality (IQ) in Pharmaceutical Development, has conducted a survey of participating IQ Consortium companies on the control strategies applied for synthetic peptides based on their phase of development and number of amino acids for both DS and DP. In this presentation, a comprehensive analysis of the survey results will be presented along with recommendations on phase- and size-appropriate specification setting strategies. The compiled survey results from ten pharmaceutical companies revealed that while most respondents follow similar control strategies for ID testing, purity measurements, and assay testing, none of the survey questions received a unanimous response. Interestingly, the number of, and type of, analytical techniques utilized for each test differed when comparing the phase of development, the number of amino acids in the peptide, and whether it was for the DS or DP. The survey questions that surprisingly had the greatest variance were what limits companies set for their reporting, identification, and qualification thresholds throughout development as well as the rationale used to justify these limits. It is evident from the results of this survey that there is a lack of alignment amongst the pharmaceutical industry on what specifications and controls should be implemented for synthetic peptides. Ultimately, the knowledge acquired from the survey results in combination with previously published literature and unique company experiences has enabled the Peptide WG to put forth appropriate recommendations to achieve harmonization on control strategies for peptides.

Participants

Jeremy Manheim, Ph.D. - Associate Principal Scientist, Merck & Co.

An Update on BioNTech's mRNA Oncology Clinical Pipeline

14:00 - 14:30
mRNA Technology and Applications

Two discrete type of therapeutic oncology mRNA-based vaccines are in clinical development at BioNTech. The individualized, patient-specific approach is studied in several randomized Phase 2 studies, based on promising early data. Similarly, disease specific TAAs were selected to create off-the-shelf mRNA vaccines also studied in randomized studies. Furthermore, early studies are ongoing for mRNA concepts encoding for antibodies and cytokines.

Participants

Michael Wenger, M.D. - Vice President Clinical Development, BioNTech SE

Machine Learning-Driven Design of Bespoke Polymer Nanoparticles for In Vivo Gene Therapies

14:00 - 14:30
Delivery of Macromolecules

This presentation will describe how Nanite's SAYER machine learning platform's predictive capabilities in designing bespoke polymer delivery vehicles for transient nucleic acid payloads. Examples will demonstrate how nanoparticle attributes, including tropism, can be tuned via machine learning based on payload type, target tissue/cell type, and delivery output metrics.

Participants

Shashi Murthy, PhD - CTO and Co-Founder, Nanite Inc.

Analytical Challenges in the Characterization of CRISPR Therapeutics

14:05 - 14:30
Oligonucleotide Chemistry, Manufacturing and Controls

The analytics needed to support the characterization and release of CRISPR-based therapeutics represent a broad landscape of techniques and methods and have unique scientific challenges. The analytical needs will change significantly across ex vivo vs. in vivo approaches, delivery modalities, and several of potency assays may be needed, and will typically be unique for each indication. Clinical phase associated validation requirements and current regulatory guidance documents must also be considered.

Participants

Steven Wolk, PhD - Vice President of Chemistry & Boulder Site Head, Editas Medicine

SESSIONS

MAIN CONFERENCE - DAY 2 (MAY 16) - 16/05/2024

Exploring the GalXC-Plus Platform for Extrahepatic Delivery of siRNA

14:30 - 15:00
Oligonucleotide Discovery, Preclinical and Clinical

We'll discuss extrahepatic oligonucleotide delivery, with an emphasis on the innovative GalXC-Plus platform and, 2nd Generation GalXC-Plus. By introducing chemical modifications to the GalXC-Plus delivery system, we can impart preferential delivery and activity in the extrahepatic space. In vivo outcomes involving mice and non-human primates (NHP) will be presented.

Participants

Robert Kolakowski, Ph.D. - Senior Director, Medicinal Chemistry, Novo Nordisk

Lessons Learned for Applying a Holistic Microbial Control Process for Oligonucleotides in Process Control Excursions

14:30 - 15:00
Oligonucleotide Chemistry, Manufacturing and Controls

Not all oligonucleotides drug substance manufacturing processes are equal. Within the industry there are ambiguities regarding microbial control for Oligos, which have characteristics of upstream synthesis similar to Small Molecules and downstream purification similar to Large Molecules. Therefore, there is no one size fits all when it comes to the application of a microbial control concept. What happens when in-process bioburden samples from a processing step exceed the control limits or preliminary target while the final API release results are well within specification? This presentation will cover lessons learned where a risk based holistic microbial approach was utilized to determine impact on patient safety and material quality of a clinical phase GMP manufactured oligonucleotide DS.

Participants

Joann Lau - Microbiology Engineer, Genentech

Tailoring Control Strategies to Meet Specific Peptide Drug Substance Complexity, Customer Needs and Regulatory Requirements

14:30 - 15:00
Peptide Discovery to CMC

Process analytical control strategies are built from the incoming starting material supply, process and purge capabilities, analytical control strategies, risk assessment tools and state of the art analytical methods. Different customers and regulatory agencies are requiring different levels of risk management and control. This presentation will showcase several peptide drug substance examples of managing customer and regulatory expectations, while assuring supply chain capabilities.

Participants

Eran Benjamin, PhD - Global Director, Analytical Development and Quality, PolyPeptide Group

mRNA-4157 Individualized Neoantigen Therapy: mRNA Therapeutics Coming of Age in Cancer

14:30 - 15:00
mRNA Technology and Applications

This presentation will provide the background story and a development update of mRNA-4157 an individualized neoantigen therapy for cancer treatment.

Participants

Robert Meehan, M.D. - Senior Director of Clinical Development, Moderna Therapeutics

Ushering in a New Era of Genetic Medicines with the Fusogenix™ Proteo-Lipid Vehicle™ Drug Delivery Platform

14:30 - 15:00
Delivery of Macromolecules

Entos Pharmaceuticals' proprietary Fusogenix Proteo-Lipid Vehicle™ (PLV™) platform, enables precision non-viral and re-dosable delivery of all nucleic acids payloads (DNA, RNA, or combinations), ushering in the new era of genetic medicine. The Fusogenix platform combines the best attributes of current viral and nonviral delivery technology that allows for delivery of all nucleic acid modalities including gene editing tools.

Participants

John D. Lewis, Ph.D. - Chief Executive Officer, Entos Pharmaceuticals

Preclinical Profile of ARO-SOD1, An siRNA therapy for SOD1-ALS

15:00 - 15:30
Oligonucleotide Discovery, Preclinical and Clinical

ARO-SOD1 is an siRNA conjugate in development for the treatment of amyotrophic lateral sclerosis (ALS) caused by SOD1 mutations. Preclinical data in non-human primates and rodent disease models demonstrate its potential as best-in-class therapy for SOD1-ALS, and highlight the broad potential of Arrowhead's CNS-targeting TRiM™ platform to treat neurodegenerative diseases.

Participants

Christine Esau, Ph.D. - Vice President, Biology, Arrowhead Pharmaceuticals

Nitto Avecia Speaker TBA

15:00 - 15:30
Oligonucleotide Chemistry, Manufacturing and Controls

Analytical Tools to Support Impurity Control Strategies for Synthetic Peptides Drug Substances

15:00 - 15:30
Peptide Discovery to CMC

European medicines agency (EMA) published a draft guidance on the development and manufacture of synthetic peptides which will address multiple quality aspects including control for peptide purity and related impurities. The EMA guidance discusses the use of orthogonal methods to minimize the risk of undetected impurities. This presentation examines the analytical toolbox available to support peptide synthesis process development and provides a case study - building an impurities control strategy for an AstraZeneca phase 3 chemically synthesized peptide drug candidate. The formation, identification, quantitation, fate and purge of certain impurity classes are discussed in the context of developing the manufacturing process and control strategy for the synthetic peptide drug substance.

Participants

Osama Chahrour, PhD - Principal Scientist, Chemical Development, AstraZeneca

SESSIONS

MAIN CONFERENCE - DAY 2 (MAY 16) - 16/05/2024

Messenger RNA Therapeutics for Primary Ciliary Dyskinesia

15:00 - 15:30
mRNA Technology and Applications

mRNA transcript therapy is envisaged to enable novel therapeutic approaches for numerous disease targets. Ethris is specialized in pulmonary delivery of mRNA. Ethris platform technology for pulmonary delivery of mRNA including details of an extraordinarily stable lipidoid formulation of mRNA will be presented as well as preclinical proof of concept for structural and functional correction of defect cilia in patient cells.

Participants

Christian Plank, PhD - Chief Technology Officer, Ethris GmbH

Clinical Translation of the FORCE™ Platform for Targeted Oligonucleotide Delivery

15:00 - 15:30
Delivery of Macromolecules

The FORCE™ Platform was developed to enable TfR1-mediated delivery of oligonucleotides to muscle for the treatment of serious genetic muscle diseases. Preclinical data showed robust muscle delivery and target engagement in DM1 and DMD disease models. Initial data from the ACHIEVE trial in DM1 and DELIVER trial in DMD demonstrated clinical proof of concept.

Participants

Timothy Weeden - Vice President- Head of Platform Discovery, Dyne Therapeutics

Networking Refreshment Break in Poster and Exhibit Hall

15:30 - 16:15

Novel Findings of Suitable Gapmer Modification for Neurological Application and Our Preclinical Progress of Sub-acute Spinal Cord Injury Treatment Drug Development

16:15 - 16:45
Oligonucleotide Discovery, Preclinical and Clinical

Luxna Biotech is a preclinical antisense drug development company based on modified nucleic acids. Currently, better modifications are being discovered that are suitable for applications in neurological diseases. Here we present new discoveries that minimize neurotoxicity while maintaining knockdown efficacy *in vivo* neurological Gapmer. As an application to the field of neurological diseases, we demonstrate that a single injection of gapmer, which contributes to significant recovering motor action in mice contusion model and suggestable skillful movements recovery of monkey hemi-section model, is a promising drug treatment for sub-acute phase spinal cord injury.

Participants

Hideaki Sato - President and CEO, Luxna Biotech

Agilent Speaker TBA

16:15 - 16:45
Oligonucleotide Chemistry, Manufacturing and Controls

Fragment-based Approaches for Acylated Peptide Synthesis; An Analysis of Cost and Capacity

16:15 - 16:45
Peptide Discovery to CMC

Acylated peptide are required in multi-tonne amounts for treating type-2 diabetes and obesity. This contribution will focus on cost and capacity models for fragment-based approaches and compare this with linear SPPS.

Participants

Leendert van ven Bos - Chief Executive Officer, EnzyTag BV

SORT LNP-formulated mRNA for the treatment of Primary Ciliary Dyskinesia

16:15 - 16:45
mRNA Technology and Applications

Primary ciliary dyskinesia (PCD) is a severe, chronic respiratory disease caused by dysfunction of the cilia. RCT1100 components, including the mRNA and Selective Organ Targeting (SORT) lipid nanoparticle (LNP) formulation, have been optimized to enhance effective DNAI1 mRNA translation in target cells of the respiratory epithelium, including ciliated cells.

Participants

David Lockhart, Ph.D. - President & Chief Scientific Officer, ReCode Therapeutics

Recent Progress with Antibody Oligonucleotide Conjugates (AOCs)

16:15 - 16:45
Delivery of Macromolecules

Using the transferrin receptor as a mechanism to target and deliver siRNAs to muscle has now been demonstrated in multiple species and in clinical trials. Exciting new data from recent preclinical studies and clinical trials demonstrate the power of receptor-mediated uptake of therapeutics to broaden the scope of cell and tissue types that can be targeted with oligonucleotide therapeutics.

Participants

Hanhua Huang, PhD - Vice President, Biology, Avidity Biosciences

Conditionally Activated siRNAs – A Biomarker-gated Approach to Genetic Medicine

16:45 - 17:15
Oligonucleotide Discovery, Preclinical and Clinical

Switch Therapeutics is developing their Conditionally Activated siRNA (CASi) platform for treatment of Central Nervous System (CNS) diseases. Our platform combines advantageous properties of traditional single and double-stranded RNA modalities into a single molecule enabling us to achieve widespread biodistribution and durable knockdown within the CNS.

Participants

Dee Datta - Co-founder & CEO, Switch Therapeutics

Optimization of the Solid-phase Oligonucleotide Detritylation Reaction Using In-line IR PAT

16:45 - 17:15
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

Steven Stanton - Senior Scientist in Oligonucleotide Process Chemistry, AstraZeneca

SESSIONS

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Study for the Novel Energy Efficient Approach for Reclaiming Acetonitrile in Peptide Manufacturing

16:45 - 17:15
Peptide Discovery to CMC

In the past, acetonitrile has primarily been recovered by multistage distillations. While these can be effective, they also come with some processing limitations and challenges. An alternative pilot scale system has developed which demonstrates a new approach to recovering this critical solvent for peptide manufacturing and greening processes.

Participants

Brad Grossman - Head of Production Torrance, PolyPeptide Group

LUNAR®-CF: An Inhaled mRNA-LNP Approach to Cystic Fibrosis Lung Disease

16:45 - 17:15
mRNA Technology and Applications

Participants

David Geller, M.D. - Vice President, Pulmonary and Rare Diseases, Arcturus Therapeutics

RAPTOR: A High Throughput Platform for Screening LNPs in Primates

16:45 - 17:15
Delivery of Macromolecules

Liberate Bio has demonstrated the potential for efficient screening of lipid nanoparticle bioaccumulation in NHPs using RNA barcoding. Empirical determination of bioaccumulation in the most relevant biological model provides the ideal training data for both deep learning and generative AI algorithms to design novel delivery vehicles. Combining these technologies sets the stage to reduce evaluation costs 100-fold and development cycles by half to achieve the extraordinary—delivering genetic medicines that transcend liver-based limitations.

Participants

Walter Strapps, PhD - Chief Scientific Officer, Intellia Therapeutics

Medicinal Chemistry Approaches to Identify Long Acting ApoC3 siRNA Candidates

17:15 - 17:45
Oligonucleotide Discovery, Preclinical and Clinical

Apolipoprotein C-III (ApoC3) is a key regulator of plasma triglyceride levels and increased levels are associated with hypertriglyceridemia and increased risk of cardiovascular disease. As a part of our effort to develop a siRNA medicine to inhibit ApoC3 mRNA we conducted an extensive medicinal chemistry structure-activity relationship evaluation of ApoC3 siRNAs and have identified candidates predicted to have an excellent safety and tolerability profile with an extended duration of action.

Participants

Thazha P. Prakash, Ph.D. - Executive Research Fellow, Ionis Pharmaceuticals

Big Molecules and Small Particles

17:15 - 17:45
Oligonucleotide Chemistry, Manufacturing and Controls

Modified messenger RNA constitutes an interesting new approach for transient protein expression in different therapies, including the recently approved SARS-Cov-2 vaccines. However, the details of the intracellular delivery of such macromolecules using so-called lipid nanoparticles remains unknown. In this work we have prepared lipid nanoparticles (LNPs) of two different ionizable lipids (DLin-MC3-DMA and DLin-DMA), cholesterol, distearylphophatidyl choline (DSPC) and a PEG lipid. We then dosed these two LNPs intravenously in mice measuring LNP uptake, mRNA delivery and the concurrent protein expression in liver cells, i.e. hepatocytes, liver sinusoidal endothelial cells (LSEC) and Kupffer cells (KC). The in vivo data clearly showed that although uptake of lipid and delivered mRNA is very similar for both types of LNPs, the protein expression in hepatocytes is order of magnitude different. In order to rationalize these in vivo observations, mRNA LNPs were characterized by several techniques e.g. 13C-NMR and small-angle x-ray scattering. Previously, we have shown that LNPs have a core-shell structure and here we focused our efforts into studying the core of LNPs, as bulk phases. By careful analysis of the inverse hexagonal phase structure of both ionizable lipids, we put forward a hypothesis on why DLin-MC3-DMA LNPs outperforms DLin-DMA LNPs in vivo.

Participants

Lennart Lindfors, Ph.D. - Senior Principal Scientist, Pharmaceutical Science, AstraZeneca

Development of DMF-free SPPS Processes – A Practical Perspective

17:15 - 17:45
Peptide Discovery to CMC

Changing from the well-established DMF-based SPPS platform to non-toxic binary solvent mixtures causes both chemical and practical challenges, but also provides new tools and opportunities for process optimisation.

Participants

Trine Puggaard Petersen, PhD - Senior Development Scientist, Novo Nordisk

Advancing mRNA-Based Epigenic Controllers in Human Disease

17:15 - 17:45
mRNA Technology and Applications

Genetic dysregulation underpins many human diseases. Omega Therapeutics has designed mRNA-based epigenetic controllers to pre-transcriptionally resolve genetic dysregulation, with potential for broad therapeutic application. Highlighting Omega's development of novel medicines in clinic for diseases with high unmet medical needs, this presentation outlines genetic specificity, modulation, and engagement of disease targets.

Participants

Russell Johnson, Ph.D. - VP, Drug Delivery & Formulations, Omega Therapeutics

Receptor-specific Targeting through Engineered VLPs

17:15 - 17:45
Delivery of Macromolecules

Cell-specific delivery remains a significant challenge for *in vivo* cell and gene therapies. Orbital has developed a modular cell specific delivery system based on virus-like particles (VLPs) using recognition of specific cellular receptors. We demonstrate the ability of this platform to target various cellular receptors when incorporating different targeting moieties and demonstrate targeted delivery to immune cells *in vivo*.

Participants

Joseph Timpona, PhD - Senior Scientist, Orbital Therapeutics

Networking Reception in Poster and Exhibit Hall

17:45 - 18:45

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06:00	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA	**06:30** - TIDES Early Morning Healthy Activity TBA
07:00	**07:45** - Single-use Considerations for Research and Manufacturing in Oligonucleotide Therapeutics **07:30** - Registration	**07:45** - New Ligation Approach: Technology for High Quality Manufacturing of Over 150 mer RNA **07:30** - Registration	**07:45** - GenScript Breakfast Spotlight Presentations **07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration

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TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 1	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY TO CMC	MRNA TECHNOLOGY AND APPLICATIONS	DELIVERY OF MACROMOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	SPOTLIGHT PRESENTATION 5
08:00				**08:25** - Chairman's Remarks: Oligonucleotide Chemistry, Mechanisms and Preclinical **08:30** - siRNA Phosphate Backbone Engineering to Enhance Potency and Extrahepatic Tissue Accumulation	**08:25** - Chairman's Remarks: Emerging Trends in Oligonucleotide Synthesis **08:30** - Biocatalytic Approaches to Nucleic Acid Therapeutics Manufacturing	**08:15** - Chairman's Remarks: Macrocyclic Peptides and Peptide Discovery **08:20** - Discovery of Zilucoplan: A Potent Macrocyclic Peptide Complement Component 5 (C5) Inhibitor in Acetylcholine Receptor Antibody-positive Generalized Myasthenia Gravis **08:45** - Bicycles as	**08:25** - Chairman's Remarks: Optimization of mRNA Sequence and Structure **08:30** - Enhancing mRNA Translation Efficiency through Trinucleotide Cap Modifications	**08:25** - Co-Chairs' Remarks: Genome Editing Delivery **08:30** - In vivo Delivery of LNP-encapsulated RNA to Immune Cells					

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TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 1	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY TO CMC	MRNA TECHNOLOGY AND APPLICATIONS	DELIVERY OF MACROMOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	SPOTLIGHT PRESENTATION 5
						Modular and Precision Guided Anti-tumor Immune Cell Agonists							

SCHEDULE

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09:00				**09:00** - Cyclic Structured Oligonucleotides for RNA Therapeutics **09:30** - Xeno Nucleic Acid (XNA) Modifications for Improving RNAi Therapeutics	**09:00** - A Platform for Controlled Template-Independent Enzymatic Synthesis of RNA Oligonucleotides and Therapeutics **09:30** - Enzymatic Synthesis of RNA with Chemical Modifications	**09:10** - MK-0616 Showcases the Potential of Macrocycles as Oral Drugs for Extracellular Targets for Atherosclerotic CVD **09:35** - Antitumor Activities of HeliconTM Peptide Inhibitors of β-catenin/TCF Interaction in Cancer Patient-derived Xenograft Models	**09:00** - AvantCap – An Inspiration from Posttranscriptional Modification of mRNA 5'end **09:30** - Discovering New Cap Analogs and Their Performances in Difference mRNA Constructs	**09:00** - Delivery of RNA Gene Writing Systems to Liver and Beyond **09:30** - Lipid Nanoparticles for Overcoming Biological Barriers to mRNA Delivery					

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10:00	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:45** - Bivalent Recognition of RNA-Repeated Expansions **10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:45** - A Platform Approach to Manufacturing Single Stranded Oligonucleotides by Enzymatic Assembly **10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:45** - Design of Highly Functional Libraries with Hyperstable Peptide and Venom Scaffolds Assisted with Machine Learning **10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:45** - Modeling and Design of RNA, Including mRNA **10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:45** - RNA-Based Approach to Delivering Prime Editing **10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall

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TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 1	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY TO CMC	MRNA TECHNOLOGY AND APPLICATIONS	DELIVERY OF MACROMOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	SPOTLIGHT PRESENTATION 5
11:00				**11:15** - Novel Chemistries in Gene Silencing and Prime Editing **11:45** - Late Breaking Presentation	**11:15** - Pushing the Boundaries of Nucleic Acid Synthesis **11:45** - The Next-generation of Oligonucleotide Chemistry Using the P(V) Platform	**11:15** - First De-novo Designed Cyclic Peptides for SORT1 **11:45** - Harnessing the Power of Dual Incretin Agonists to Target Cardiometabolic Diseases	**11:15** - AI-Optimized mRNA Design Improves Stability and Immunogenicity **11:45** - Deep Learning Guided Optimization of Translation Efficiency for mRNA Vaccine Development	**11:15** - Late Breaking Presentation **11:45** - Late Breaking Presentation					

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TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 1	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY TO CMC	MRNA TECHNOLOGY AND APPLICATIONS	DELIVERY OF MACROMOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	SPOTLIGHT PRESENTATION 5
12:00	**12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:15** - Transition to Spotlight Presentation Rooms **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:15** - Transition to Spotlight Presentation Rooms **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:15** - Transition to Spotlight Presentation Rooms **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:15** - Transition to Spotlight Presentation Rooms **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:15** - Transition to Spotlight Presentation Rooms **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:20** - Green SPPS – An Effort to Minimize the Environmental Impact Related to the SPPS Process **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:20** - Process Development for sgRNA in CRISPR/Casx Therapeutics **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:20** - Unveiling Impurities of Chemically Synthesized gRNAs **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:20** - Charting New Horizons in Guide RNA Manufacturing **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:20** - Oligo Manufacturing Innovations – From Synthesis through Concentration **12:50** - Networking Luncheon in Poster and Exhibit Hall

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13:00				**13:55** - Chairman's Remarks: Oligonucleotide Discovery and Development	**13:55** - Chairman's Remarks and Memorial Tribute to Paul McCormac	**13:55** - Chairman's Remarks: Best Practices and Case Studies in Peptide Manufacturing and CMC	**13:55** - Chairman's Remarks: mRNA Preclinical and Clinical Progress Outside of COVID/ID Vaccines: New mRNA Therapeutic Frontiers & Novel Disease Indications	**13:55** - Chairman's Remarks: Next-Generation Delivery Platforms					

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14:00				**14:00** - Ligand Mediated Delivery of Oligonucleotides Across the Blood Brain Barrier **14:30** - Exploring the GalXC-Plus Platform for Extrahepatic Delivery of siRNA	**14:05** - Analytical Challenges in the Characterization of CRISPR Therapeutics **14:30** - Lessons Learned for Applying a Holistic Microbial Control Process for Oligonucleotides in Process Control Excursions	**14:00** - Perspective on Current Industry Control Strategies for Synthetic Peptides **14:30** - Tailoring Control Strategies to Meet Specific Peptide Drug Substance Complexity, Customer Needs and Regulatory Requirements	**14:00** - An Update on BioNTech's mRNA Oncology Clinical Pipeline **14:30** - mRNA-4157 Individualized Neoantigen Therapy: mRNA Therapeutics Coming of Age in Cancer	**14:00** - Machine Learning-Driven Design of Bespoke Polymer Nanoparticles for In Vivo Gene Therapies **14:30** - Ushering in a New Era of Genetic Medicines with the Fusogenix™ Proteo-Lipid Vehicle™ Drug Delivery Platform					

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15:00	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:00** - Preclinical Profile of ARO-SOD1, An siRNA therapy for SOD1-ALS **15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:00** - Nitto Avecia Speaker TBA **15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:00** - Analytical Tools to Support Impurity Control Strategies for Synthetic Peptides Drug Substances **15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:00** - Messenger RNA Therapeutics for Primary Ciliary Dyskinesia **15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:00** - Clinical Translation of the FORCE™ Platform for Targeted Oligonucleotide Delivery **15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall

SCHEDULE

MAIN CONFERENCE - DAY 2 (MAY 16) - 16/05/2024

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 1	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY TO CMC	MRNA TECHNOLOGY AND APPLICATIONS	DELIVERY OF MACROMOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	SPOTLIGHT PRESENTATION 5
16:00				**16:15** - Novel Findings of Suitable Gapmer Modification for Neurological Application and Our Preclinical Progress of Sub-acute Spinal Cord Injury Treatment Drug Development **16:45** - Conditionally Activated siRNAs – A Biomarker-gated Approach to Genetic Medicine	**16:15** - Agilent Speaker TBA **16:45** - Optimization of the Solid-phase Oligonucleotide Detritylation Reaction Using In-line IR PAT	**16:15** - Fragment-based Approaches for Acylated Peptide Synthesis; An Analysis of Cost and Capacity **16:45** - Study for the Novel Energy Efficient Approach for Reclaiming Acetonitrile in Peptide Manufacturing	**16:15** - SORT LNP-formulated mRNA for the treatment of Primary Ciliary Dyskinesia **16:45** - LUNAR®-CF: An Inhaled mRNA-LNP Approach to Cystic Fibrosis Lung Disease	**16:15** - Recent Progress with Antibody Oligonucleotide Conjugates (AOCs) **16:45** - RAPTOR: A High Throughput Platform for Screening LNPs in Primates					

SCHEDULE

MAIN CONFERENCE - DAY 2 (MAY 16) - 16/05/2024

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 1	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY TO CMC	MRNA TECHNOLOGY AND APPLICATIONS	DELIVERY OF MACROMOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	SPOTLIGHT PRESENTATION 5
17:00	**17:45** - Networking Reception in Poster and Exhibit Hall	**17:45** - Networking Reception in Poster and Exhibit Hall	**17:45** - Networking Reception in Poster and Exhibit Hall	**17:15** - Medicinal Chemistry Approaches to Identify Long Acting ApoC3 siRNA Candidates **17:45** - Networking Reception in Poster and Exhibit Hall	**17:15** - Big Molecules and Small Particles **17:45** - Networking Reception in Poster and Exhibit Hall	**17:15** - Development of DMF-free SPPS Processes – A Practical Perspective **17:45** - Networking Reception in Poster and Exhibit Hall	**17:15** - Advancing mRNA-Based Epigenic Controllers in Human Disease **17:45** - Networking Reception in Poster and Exhibit Hall	**17:15** - Receptor-specific Targeting through Engineered VLPs **17:45** - Networking Reception in Poster and Exhibit Hall	**17:45** - Networking Reception in Poster and Exhibit Hall	**17:45** - Networking Reception in Poster and Exhibit Hall	**17:45** - Networking Reception in Poster and Exhibit Hall	**17:45** - Networking Reception in Poster and Exhibit Hall	**17:45** - Networking Reception in Poster and Exhibit Hall

SESSIONS

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

Sunrise Yoga: Wellness Event

06:45 - 07:15

Please join us for an early morning yoga session to prepare your mind and body for another full day of TIDES conference sessions, as well as to meet other attendees.

Registration

07:30 - 07:45

Case Study on the GMP Development and Manufacturing Process for DOTAGA-Labeled Urea-Based Peptide PSMA Inhibitor, DOTAGA-(I-y)fk(Sub-KuE)

07:45 - 08:15
Breakfast Spotlight Presentations 1

Peptides are advantageous as therapeutic vectors in PRRT due to their small size, favorable pharmacokinetics, high binding affinity, low immunogenicity and toxicity, and minimal off-target binding. This case study presents a manufacturing process for DOTAGA-Labeled urea-based peptide PSMA inhibitor API, DOTAGA-(I-y)fk(Sub-KuE). Multigram-scale process development and GMP manufacturing was completed in only ten months. Through optimized synthesis, cleavage, and purification an overall yield of >65% (>98% purity) was achieved.

Participants

John Phipps - Vice President of Clinical Pipeline Development, CPC Scientific

Overcoming Oligonucleotide Manufacturing Challenges

07:45 - 08:15
Breakfast Spotlight Presentations 2

Participants

Trishul Shah, M.S. - Director, Business Development, North America, PolyPeptide Laboratories Inc.

Amidite RSM Impurity Control and Impact on Oligonucleotide API Quality

07:45 - 08:15
Breakfast Spotlight Presentations 3

Effective amidite impurities control is critical in solid-phase oligonucleotide synthesis, directly impacting the quality of oligonucleotide API. This presentation will explore the sources and control strategies for various impurities in oligonucleotide API, emphasizing the critical role of amidite impurity control in maintaining API quality. Additionally, the talk will touch upon strategies to establish a resilient supply chain for oligonucleotides, including robust supplies of high-quality amidite.

Participants

William Fang - Vice President of Oligonucleotide and Peptide Development, WuXi TIDES

Chairman's Remarks: Oligonucleotide Preclinical and Clinical & Progress in the Development of Phosphorodiamidate Morpholino Oligonucleotides PMOs

08:25 - 08:30
Oligonucleotide Discovery, Preclinical and Clinical

Participants

Trishul Shah, M.S. - Director, Business Development, North America, PolyPeptide Laboratories Inc.

Chairman's Remarks: Analytics for Oligonucleotide Modalities

08:25 - 08:30
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

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

Chairman's Remarks: Targeted Radiopharmaceuticals: Progress to the Clinic

08:25 - 08:30
Peptide Discovery to CMC

Participants

Christopher McGee, Ph.D. - VP & Head, Global Business Development, Bachem

Ved Srivastava, PhD - CTO, Perpetual Medicines

Chairman's Remarks: Genome Editing Clinical and Preclinical

08:25 - 08:30
Genome Editing Technology and Applications

Participants

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

Chairman's Remarks: Targeted Delivery and Novel Delivery Approaches

08:25 - 08:30
Delivery of Macromolecules

Participants

Luis Brito, PhD - Vice President, Delivery Platform, Beam Therapeutics

SESSIONS

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

Silencing Gain-of-function KCNT1 Genetic Epilepsy with Divalent siRNA, A Novel Small Interfering RNA Technology, Durably Eliminates Spontaneous Seizures in a Mouse Epilepsy Model

08:30 - 09:00
Oligonucleotide Discovery, Preclinical and Clinical

We previously have described a new variant of siRNA, divalent siRNA, made up of two linked siRNAs, that has distribution and tolerability developed for durable transcript silencing in the central nervous system. A well-tolerated dose of di-siRNA delivered directly to the cerebrospinal fluid drives selective transcript silencing throughout the CNS of mice and of nonhuman primates, with durability of at least six months. Here, we report a di-siRNA, ATL-201, that silences transcripts of the KCNT1 gene, which encodes a potassium-selective ion channel that drives neuronal excitability. Gain-of-function variants in KCNT1 drive severe infant- or childhood-onset epilepsy that is refractory to existing anti-seizure medications and represents a substantial and unmet medical need. siRNAs targeting KCNT1 were screened in vitro for knockdown of transcript and protein as measured with RT-PCR, Western blot, and patch-clamp electrophysiology. ATL-201 siRNA sequence in vitro gave reduction in KCNT1 transcript and near-complete reduction of KCNT1 protein and of KCNT1-driven potassium ion channel currents, reflecting knockdown of functional KCNT1 protein at the plasma membrane. In mice, Kcnt1 protein was reduced by approximately three quarters in cortex as early as three days post-administration of a well-tolerated dose of ATL-201, with the protein knockdown persisting for at least four months in an ongoing study. ATL-201 was then tested for efficacy at preventing spontaneous seizures in 6- to 8-week old mice homozygous for Kcnt1-Y777H, an ortholog to the KCNT1-Y796H human disease-associated variant. Seizures were measured over a 24-hour period at multiple time points with continuous EEG recordings paired with behavioral scoring via continuous video monitoring. Kcnt1-Y777H mice dosed ICV with ATL-201 in an ongoing study had few to no seizures at three days, two weeks, and two months post-administration compared to PBS-dosed control mice. This phenotype was specific to Kcnt1 silencing, as equimolar dosing of a non-targeting di-siRNA had no discernable effect on seizures. Silencing the KCNT1 gene in the CNS using the di-siRNA platform with its broad distribution, long durability, and tolerability may be an effective treatment for KCNT1-driven epilepsies, a most severe epileptic encephalopathy with few if any effective treatments.

Participants

Stefan McDonough, Ph.D. - Senior Vice President, Head of Neuroscience, Atalanta Therapeutics

Methods to Establish Diasteromeric Content Comparability in Oligonucleotide Products

08:30 - 09:00
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

George Bou-Assaf, Ph.D. - Associate Director, Analytical and Biophysical Dev, Biogen

Next Wave of Radionuclide Theranostics

08:30 - 09:00
Peptide Discovery to CMC

Radionuclide Theranostics - thanks to the recent approvals of Lutathera and Pluvicto – have gained significant momentum. A number of new theranostic approaches are currently translated into the clinic including new targets, new binders, new radionuclides, synergistic combination treatment and radiosensitizers. Overall the field of radionuclide theranostics involves a number of moving targets. This presentation aims to provide a snapshot of the currently most promising radionuclide theranostics on the brink of being clinically translated.

Participants

Ken Herrmann, M.D. - Chair, Department of Nuclear Medicine, Universitätsklinikum Essen

Prime Editing for the Treatment of Chronic Granulomatous Disease

08:30 - 09:00
Genome Editing Technology and Applications

Chronic Granulomatous Disease (CGD) is an immunodeficiency caused by mutations in genes encoding proteins of the nicotinamide adenine dinucleotide phosphate (NADPH) oxidase enzyme. When myeloid cells detect pathogens, NADPH oxidase produces oxidative bursts that kill pathogens to control infection. CGD patients lack NADPH oxidase, causing recurrent infections and inflammatory complications. P47phox CGD is typically caused by a two-nucleotide GT deletion (delGT) in the *NCF1* gene which encodes p47 protein of NADPH oxidase. Prime Medicine is developing a Prime Edited (PE) autologous CD34+ cell drug product for the treatment of CGD. An update on the development of this drug product will be presented.

Participants

Jennifer Gori, PhD - Vice President, Research, Prime Medicine

Extrahepatic Delivery of LNPs

08:30 - 09:00
Delivery of Macromolecules

Typical lipid nanoparticles (LNPs) preferentially locate to and are metabolized through the liver. Generation Bio has developed a novel class of cell-targeted LNPs (ctLNPs) that can be redirected to extrahepatic cell types. We will discuss recent advancements in ctLNP development and optimization, and their potential applications in extrahepatic spaces.

Participants

Di Bush, PhD - Vice President, Head of Delivery, Generation Bio

Late Breaking Presentation

09:00 - 09:30
Oligonucleotide Discovery, Preclinical and Clinical

Strategies for the Characterization of Stereopure Chimeric PO/PS/PN Oligonucleotides

09:00 - 09:30
Oligonucleotide Chemistry, Manufacturing and Controls

Chemically modified oligonucleotides that modulate RNA hold great promise for the treatment of human disease. Wave Life Sciences is advancing new chemistries to generate stereopure, chimeric backbone-containing oligonucleotides—those in which the chirality of each backbone linkage has been precisely controlled during chemical synthesis—with the aim of improving their drug-like properties. We will provide an overview of the methods we have developed to synthesize, manufacture, and quality control stereopure chimeric oligonucleotides containing PN (phosphoryl guanidine) backbone linkages in combination with more traditional phosphodiester (PO) and phosphorothioate (PS) backbone linkages. We will describe how stereochemical stability and stereochemical identity are established and interrogated, using multiple analytical techniques, to support the manufacture of stereopure oligonucleotides for clinical use.

Participants

Pachamuthu Kandasamy, PhD - Vice President, Medicinal Chemistry, Wave Life Sciences

SESSIONS

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

RGD Peptide as a Theranostic Radiotracer Targeting ?vβ3 Integrin

09:00 - 09:30
Peptide Discovery to CMC

Integrin $?_v\beta_3$ plays an essential role in regulating angiogenesis, a key process in tumor growth. Restricted over expression of integrin $?_v\beta_3$ on the activated endothelial cells of neo-vasculature of tumor makes $?_v\beta_3$ an invaluable molecular target that may help in early diagnosis and management of various solid tumors. The study aims to describe bench to bedside development of radiolabeled RGD peptide targeting $?_v\beta_3$ integrin.

Participants

Rakhee Vatsa, Ph.D. - Scientific Officer D, Radiopharmacist, Tata Memorial Centre

Advances in In Vivo CRISPR Gene Editing for Therapeutic Application

09:00 - 09:30
Genome Editing Technology and Applications

Intellia is a leading clinical-stage gene editing company focused on the development of CRISPR-based therapies. Interim clinical data with NTLA-2001, an investigational *in vivo* CRISPR-based therapy with the potential to be the first single-dose treatment for ATTR amyloidosis, will be presented.

Participants

Liron Walsh, M.D. - Vice President, Head of Development, Intellia Therapeutics

Novel TRiM™ Platform for Delivery of RNAi Therapeutics to Adipose Tissue

09:00 - 09:30
Delivery of Macromolecules

Adipose tissue is a critical endocrine organ for energy and hormonal homeostasis, and disruption of these highly regulated processes can lead to the induction of metabolic disease such as obesity and type 2 diabetes. There remain significant challenges, however, in the development of novel therapeutics capable of correcting these processes in adipose tissue. To address adipose-related disease, Arrowhead has developed a TRiM™ platform for the delivery of RNAi therapeutic candidates to white adipose tissue. This platform provides a novel and highly efficient mode of delivery of siRNA, allowing for low and infrequent dosing regimens via subcutaneous administration. The TRiM™ adipose platform has achieved notable gene knockdown (≥ 90%) and long duration of effect in both rodent and non-human primates. The TRiM™ adipose delivery platform may help address the unmet need for novel therapeutics capable of treating adipose-related diseases and disorders.

Participants

Tao Pei, Ph.D. - Group Vice President, Chemistry, Arrowhead Pharmaceuticals

Late Breaking Presentation

09:30 - 10:00
Oligonucleotide Discovery, Preclinical and Clinical

Considerations for Method Development of siRNA Duplexes

09:30 - 10:00
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

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

Bicyclic Peptide Technology to Develop Bicycle Radio-conjugates

09:30 - 10:00
Peptide Discovery to CMC

This presentation will discuss 1) challenges of synthetic manufacturing of a complex macromolecule such as Concarlo's peptide; 2) Optimization of peptide solubility; 3) Our learnings in scaling up peptide manufacturing process and 4) Incorporating approaches from mRNA-LNP manufacturing such as microfluidic mixing into peptide/conventional liposome manufacturing. Loading efficiency, particle size, Cryo-TEM data to support the claims.

Participants

Johanna Lahdenranta, Ph.D. - Director In Vivo Pharmacology, Bicycle Therapeutics

Proof-of-concept for in vivo Base Editing to Inactivate the PCSK9 Gene and Lower LDL-Cholesterol in Humans

09:30 - 10:00
Genome Editing Technology and Applications

VERVE-101 is an investigational *in vivo* base editing medicine designed to inactivate the hepatic PCSK9 gene and reduce LDL-cholesterol levels. Interim data from the first-in-human heart-1 study of VERVE-101 in participants with heterozygous familial hypercholesterolemia show dose-dependent LDL-cholesterol reductions and provide the first proof-of-concept for base editing medicines.

Participants

Andrew Bellinger, MD, PhD - Chief Scientific Officer, Verve Therapeutics

In Vivo Engineering of Cells Using Targeted Lipid Nanoparticles

09:30 - 10:00
Delivery of Macromolecules

Capstan Therapeutics is developing a novel targeted LNP (tLNP) platform purpose-built for preferential delivery of mRNAs to specific cells. Due to the versatility of this non-viral, redosable platform, treatments for various diseases can be envisioned using different targeting binders to deliver a broad set of payloads to diverse cell populations.

Participants

Priya Karmali, Ph.D. - Chief Technology Officer, Capstan Therapeutics

Networking Refreshment Break in Poster and Exhibit Hall

10:00 - 10:45

Recent Progress with Antibody PMO Conjugates

10:45 - 11:15
Oligonucleotide Discovery, Preclinical and Clinical

Utilizing TfR1 receptor-mediated delivery of oligonucleotides to muscles presents a promising treatment strategy for muscular diseases such as DM1, DMD, and FSHD. Avidity's innovative Antibody Oligonucleotide Technology (AOC) holds significant potential for delivering phosphorodiamidate morpholino oligonucleotides (PMO) to muscle tissue, showcasing its applicability in the treatment of Duchenne Muscular Dystrophy (DMD). The presentation includes preclinical and clinical data on AOC 1044, an exon 44 skipping PMO conjugate.

Participants

Ramana Doppalapudi, Dr. - Vice President, Chemistry, Avidity Biosciences

SESSIONS

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

Development of a 2D-LC/MS Workflow and Its Application for Impurity Profiling of Phosphorodiamidate Morpholino Oligomers

10:45 - 11:15
Oligonucleotide Chemistry, Manufacturing and Controls

Phosphorodiamidate morpholino oligomers (PMOs) are short single-stranded oligonucleotides comprising a backbone of morpholine rings connected by phosphorodiamidate linkages. The manufacture of PMO drug substances involves solid-phase oligomer synthesis and subsequent cleavage/deprotection followed by purification and lyophilization. During the manufacturing process, a variety of impurities are generated from various sources. These process-related impurities are often structurally related to their parent PMO. Determination of impurity profile of drugs to confirm quality and thereby ensure safety and efficacy is essential. Herein, we present the development of a robust 2D-LC/MS workflow and its application for impurity profiling of PMOs. PMOs and the impurities were separated by two-dimensional LC with orthogonal modes of separation and detected by Quadrupole Time-of-Flight mass spectrometry. The developed 2D-LC/MS workflow was successfully applied to the impurity profiling of various PMOs with different sequences and lengths.

Participants

Tao Wei, Dr. - Associate Director, RNA Process Development, Sarepta Therapeutics

Development of Radiopharmaceutical Therapy agents for treatment of GPC3-Expressing tumors

10:45 - 11:15
Peptide Discovery to CMC

The recent approvals of Lutathera and Pluvicto have highlighted the potential of Radiopharmaceutical Therapy (RPT) as a secure and efficient targeted modality for treating various solid tumors. The successful development of RPT necessitates methodical optimization and a thorough evaluation of the targeting moiety, linker, chelator, and the selection of radioisotopes. RayzeBio is at the forefront of innovation in this domain, employing a data-driven drug discovery approach to systematically identify optimal RPT agents against clinically validated oncology targets that have yet to be addressed using RPT. In this presentation, we will share the application of this approach to develop and optimization of potential RPT agents for the treatment of GPC3-expressing tumors.

Participants

Alain Noncovich, Ph.D. - Associate Director of Chemistry, RayzeBio

AsCas12a Gene Editing of HBG1/2 Promoters with EDIT-301 (reni-cel) Results in Rapid and Sustained Normalization of Hemoglobin and Increased Fetal Hemoglobin in Patients with Severe Sickle Cell Disease and Transfusion-dependent Beta-thalassemia

10:45 - 11:15
Genome Editing Technology and Applications

Editas Medicine will present clinical data for the RUBY and EdiTHAL trials. In both trials, observed pharmacodynamic responses and preliminary efficacy data confirm proof of concept for reni-cel (EDIT-301) mechanism of action. Reni-cel was well-tolerated and demonstrated a safety profile consistent with myeloablative conditioning with busulfan and autologous hematopoietic stem cell transplantation in all treated patients in the two trials (n=17).

Participants

Olubunmi Afonja - Senior Director, Clinical Development, Editas Medicine

Versatile Transformable Peptidic Nanoplatform for Cancer Therapy and Detection

10:45 - 11:15
Delivery of Macromolecules

We have recently developed a tumor-targeting transformable nanoplatform capable of receptor-mediated transformation at the tumor sites from 20nm nanoparticles into nanofibrillar network. Therapeutic payloads such as cytotoxic agents, photosensitizing agents, immunomodulatory agents, and immune cell capturing ligands can be delivered efficiently to the tumor microenvironment, resulting in excellent anti-tumor response.

Participants

Kit Lam, M.D., Ph.D. - Distinguished Professor, Biochemistry and Molecular, University of California Davis

Enhanced Delivery Oligonucleotides: An Update on Preclinical and Clinical Progress

11:15 - 11:45
Oligonucleotide Discovery, Preclinical and Clinical

PepGen Inc. is a clinical-stage biotechnology company advancing the next-generation of oligonucleotide therapies with the goal of transforming the treatment of severe neuromuscular and neurological diseases. PepGen's Enhanced Delivery Oligonucleotide, or EDO platform is founded on over a decade of research and development and leverages cell-penetrating peptides to improve the uptake and activity of conjugated oligonucleotide therapeutics. Using these EDO peptides, we are generating a pipeline of oligonucleotide therapeutic candidates that are designed to target the root cause of serious diseases.

Participants

Niels Svenstrup, Ph.D. - SVP Chemistry, Manufacturing and Controls, PepGen

LC MS Methods for Characterization of Long Oligonucleotides

11:15 - 11:45
Oligonucleotide Chemistry, Manufacturing and Controls

Resolution performance of LC and MS techniques is challenged by development of long therapeutic oligonucleotides such sgRNA. We will discuss the application of modern method of ultra-performance LC for separation and MS characterization of long oligonucleotides and other classes of nucleic acids.

Participants

Martin Gilar, PhD - Scientific Fellow, Separations R&D, Waters Corporation

Pioneering Aktis Oncology's Miniprotein Radioconjugates

11:15 - 11:45
Peptide Discovery to CMC

Aktis Oncology is developing radiopharmaceuticals based on miniprotein binders. Miniproteins have ideal properties for radioconjugates being highly selective and potent with excellent tumor penetration properties while also rapidly clearing from the periphery via kidney filtration sparing healthy tissue. Aktis has generated multiple first-in-class programs using the miniprotein platform demonstrating its broad utility.

Participants

Brian Goodman, PhD - Co-Founder and Chief Operating Officer, Aktis Oncology

SESSIONS

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Late Breaking Presentation

11:15 - 11:45
Genome Editing Technology and Applications

Late Breaking Presentation

11:15 - 11:45
Delivery of Macromolecules

Strategies for Oligonucleotides Purification Applicable for Clinical Products Manufacture

11:45 - 12:15
Oligonucleotide Discovery, Preclinical and Clinical

Purification is the most time-consuming and critical step in the Oligo/modified Oligo manufacturing process. The presentation covers purification method selection criteria and strategies in minimizing the risks in the purification step which are specifically applicable for clinical products manufacture.

Participants

Mahender Gurram, Ph.D. - Senior Director, DS Development, Mfg, & CMC, Entrada Therapeutics

Analytical Development for Prime Editing Guide (peg)RNAs

11:45 - 12:15
Oligonucleotide Chemistry, Manufacturing and Controls

Prime editing is a “search-and-replace” gene editing technology that can correct disease-causing genetic mutations at their precise location in the genome, without requiring double-strand DNA breakage. Prime editing offers a potential therapeutic platform for a broad range of challenging diseases. Comprehensive analytical methods are being developed to assess the quality of gene editing critical raw materials and drug substances (e.g., prime editing mRNA or protein, nicking guide (NG)RNA, prime editing guide (PEG)RNA), because of the potential effects quality can have on, for example, gene editing accuracy and efficiency. PEGRNAs can be particularly challenging to analyze because of their length, secondary structures, and complex activities. A series of HPLC- and mass spectrometry-based analytical methods were developed to assess PEGRNA purity, stability, mass, impurity ID/quantitation, and biochemical activity.

Participants

Xiangkun Yang, Ph.D. - Senior Scientist II, Prime Medicine

Ac-FL-020, A Novel PSMA-targeting Radioligand Therapy Candidate in Development

11:45 - 12:15
Peptide Discovery to CMC

Radioligand therapy (RLT) has recently demonstrated attractive clinical benefits. Such early success has promoted the race for next wave RLTs where exciting opportunities and unique challenges have been both presented. Here we will disclose the discovery of ^{225}Ac-FL-020, a novel PSMA-targeting RLT candidate identified by our proprietary Clear-X technology platform.

Participants

Fa Liu, PhD - Chief Scientific Officer, Full-Life Technologies

Late Breaking Presentation

11:45 - 12:15
Genome Editing Technology and Applications

Late Breaking Presentation

11:45 - 12:15
Delivery of Macromolecules

Transition to Spotlight Presentation Rooms

12:15 - 12:20
Oligonucleotide Discovery, Preclinical and Clinical

Transition to Spotlight Presentation Rooms

12:15 - 12:20
Oligonucleotide Chemistry, Manufacturing and Controls

Transition to Spotlight Presentation Rooms

12:15 - 12:20
Peptide Discovery to CMC

Transition to Spotlight Presentation Rooms

12:15 - 12:20
Genome Editing Technology and Applications

Transition to Spotlight Presentation Rooms

12:15 - 12:20
Delivery of Macromolecules

Novel Ionizable Lipids and Their LNPs to Accelerate Development of RNA based Therapeutics

12:20 - 12:50
Spotlight Presentation 1

FUJIFILM has launched end-to-end CDMO services for LNPs based on our platform technologies, proprietary ionizable lipids and manufacturing process technologies. Our ionizable lipid, which consists of a head with diamino group, biodegradable linkers, and branched tails, enables the design of suitable LNPs for RNA-based therapeutics. We have identified several lead lipids and formulations through in vivo screening and these LNPs show high activity for RNA delivery and low toxicity.

Participants

Shigetomo Tsujihata - Senior Scientist, Bio Science & Engineering Laboratory, FUJIFILM Corporation

Non-viral RNA Delivery with Biodegradable Lipids

12:20 - 12:50
Spotlight Presentation 2

Understanding the role of key cellular mediators is key to developing the next generation of safe, well tolerated non-viral delivery systems. We will discuss advances in tuning the degradation rate, immune activation, and tissue targeting of biodegradable COATSOME ® SS Series for development of distinct LNPs for cell therapy, gene editing, and vaccines.

Participants

Syed Reza, MD, PhD - Scientific and Sales Consultant, NOF Corporation

Novel Cap Analogs and Modified NTPs to Enable Therapeutic mRNA Development

12:20 - 12:50
Spotlight Presentation 3

High purity DNA oligomers are important for therapeutic and research uses. Obtaining these high purity oligos requires purification steps to remove impurities generated during the synthesis. Reverse phase chromatography (RP-HPLC) is an excellent method for oligo purification, but it can be difficult to know where to start when it comes to resin selection. This talk will guide the audience through the design of an oligonucleotide purification process using DuPont™ AmberChrom™ polymeric chromatography resins.

Participants

May Guo - Chief Commercial Officer, Areterna

SESSIONS

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

Phenomenex Spotlight Presentations

12:20 - 12:50
Spotlight Presentation 4

Unlocking Guide RNA Quality: The Power of NGS Analysis

12:20 - 12:50
Spotlight Presentation 5

With an increasing number of guide RNA programs emerging in the commercial market, including for in-vivo applications, proper impurity characterization and control of guide RNA impurities are essential to ensure the safety and reliability of a therapy. Enter Next Generation Sequencing (NGS), which not only allows scientists to determine sequence-specific impurities, but also capture the fingerprint of the guide RNA impurity profile.

Participants

Barbara Pfaff, PhD - QC Manager Molecular Sequencing, BioSpring GmbH

Networking Luncheon in Poster and Exhibit Hall

12:50 - 13:55

Chairman's Remarks: Novel RNA-based Therapeutic and Vaccine Platforms

13:55 - 14:00
Oligonucleotide Discovery, Preclinical and Clinical

Chairman's Remarks: Innovations in Oligonucleotide Process Development and Manufacturing

13:55 - 14:00
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

Firoz Antia, PhD - Head of Oligonucleotide Development, Biogen

Chairman's Remarks: Delivery of Peptides and Peptides as Delivery Agents

13:55 - 14:00
Peptide Discovery to CMC

Participants

Yvonne M. Angell, PhD - Executive Director, Discovery Oligo/Peptide Project Management, STA Pharmaceutical

Chairman's Remarks: Next-Generation Genome Editing Technologies

13:55 - 14:00
Genome Editing Technology and Applications

Editing the Genome with Cas9 mRNA

14:00 - 14:30
Oligonucleotide Discovery, Preclinical and Clinical

mRNA medicine has reshaped the biopharmaceutical landscape. At Intellia, our lead clinical programs, NTLA-2001 and NTLA-2002, have generated clinical evidence that LNP delivery of Cas9 mRNA results in targeted hepatic delivery of functional Cas9 protein. As we begin the MAGNITUDE Phase 3 trial of NTLA-2001 for ATTR amyloidosis with cardiomyopathy, we are advancing the prospect of functional enzyme delivery via therapeutic mRNA into pivotal studies.

Participants

Danny Crawford, PhD - Director, Nucleic Acid Process Sciences, Intellia Therapeutics

Adoption of Innovative Technologies in Oligonucleotide Manufacturing: Improving Efficiency of siRNA Manufacturing Processes

14:00 - 14:30
Oligonucleotide Chemistry, Manufacturing and Controls

The solid phase oligonucleotide synthesis based on sequential coupling of phosphoramidite monomers is a well-established industrial manufacturing process, currently performed routinely on kilo scale mainly due to limitations of synthesis and purification processes. Novel approaches towards more efficient, scalable, and sustainable large-scale manufacture will be discussed supporting future commercialization of the expanding range of high-volume siRNA therapeutics.

Participants

Roumen Radinov, Ph.D. - Vice President, Process Sciences, Alnylam Pharmaceuticals

Peptide Drug Delivery - Roadmap to Selecting a Development Candidate and Transforming it to a Product

14:00 - 14:30
Peptide Discovery to CMC

Peptides are potent and selective modulators of endogenous process and have been the target of drug development for decades, yet comparatively not many have reached the marketplace. This is primarily due to the physicochemical and biological properties of peptides, they are large, changed, and subject to extensive degradation by peptidases. This presentation will cover a developability roadmap from a drug delivery and formulation perspective for how to select candidates with a higher chance of success and ways to transform them into a product. The focus will be on injectable peptides but conclude with a perspective on considerations for alternative route delivery.

Participants

Annette Bak, Ph.D. - Head of Advanced Drug Delivery, AstraZeneca

Enhancing Precision and Efficiency of In Vivo Gene Editing with Engineered PsCas9

14:00 - 14:30
Genome Editing Technology and Applications

CRISPR-Cas technologies offer precise genome manipulation for gene therapy. *Parasutterella secunda* Cas9 is a high-fidelity enzyme capable of gene editing in mice. We report the Cryo-EM structure of PsCas9 and engineered it and its sgRNA. The engineered variant, ePsCas9, maintains high-fidelity and shows superior gene editing in mouse liver, outperforming SpCas9 with no safety concerns. ePsCas9 is a highly efficient and precise tool for in vivo gene editing.

Participants

Grzegorz Sienski, Ph.D. - Director and Project Manager, AstraZeneca

mRNA-encoded Antibodies to Combat Infectious Diseases

14:30 - 15:00
Oligonucleotide Discovery, Preclinical and Clinical

Monoclonal antibodies have shown remarkable efficacy in the treatment and prevention of multiple viral diseases. However, traditional CHO-based manufacturing constraints have limited the pace of antibody development and the types of molecules that can be developed. In this presentation, I'll discuss the use of mRNA technology to rapidly express engineered antibodies with high potency, breadth, and resilience to escape.

Participants

Laura Walker, PhD - Head of Infectious Disease Biotherapeutics Engineering and Discovery, Moderna

SESSIONS

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

Use of Ultrafiltration/Diafiltration for the Processing of Antisense Oligonucleotides

14:30 - 15:00
Oligonucleotide Chemistry, Manufacturing and Controls

The use of ultrafiltration/diafiltration to process antisense oligonucleotides will be examined including its capabilities and limitations, membrane properties, sieving coefficients, along with concentrations, flux, and yields achieved. Additionally, effects of buffer types, permeability, viscosities, and impact on clearance will be presented.

Participants

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

BIONDD – Enabling Oral Administration of Biologics Achieving Drug Exposures Comparable to Injections

14:30 - 15:00
Peptide Discovery to CMC

Patients greatly prefer the oral route of administration for pharmaceuticals. Limited oral absorption of biologics (peptides, proteins, RNAs, and antibodies) is a major challenge. The BIONDD^{TM} capsule delivers biologic drugs with a bioavailability like SC injection creating a broad platform for oral delivery of drugs that would be injected today.

Participants

Nikolaj Skak - Chief Technology Officer, Biograil

Programmable Molecular Technologies for Genome Editing and Cell Control

14:30 - 15:00
Genome Editing Technology and Applications

Our lab has developed new molecular technologies for genome editing and cell engineering, including PASTE for large DNA integration, RNA guided CRISPR proteases, and a novel technology for programmable mRNA therapies. These advances enable precise genome editing and cell state manipulation, with significant implications for therapeutics and diagnostics.

Participants

Omar Abudayyeh, PhD - Director of Gene Editing, MGB Gene and Cell Therapy Institute

Jonathan Gootenberg, PhD - Principal Investigator, Center for Vaccines and Virology Research, Beth Israel Deaconess Medical Center

Introducing Circular RNA Vaccine Platform as Novel Alternative to RNA Vaccine

15:00 - 15:30
Oligonucleotide Discovery, Preclinical and Clinical

Since the discovery of circular RNA, a new class of single-stranded RNA, their biogenesis, regulation and function have been rapidly characterized, allowing for better understanding and their adoption as new tools for therapeutic applications. With the development of biotechnology and molecular medicine, circRNAs have been engineered as a novel class of RNA therapeutics. In the field of vaccines, compared to linear mRNA vaccine, mRNA vaccine offers an improved approach to RNA-based vaccination with increased stability, simplicity of manufacture and scalability.

Participants

Gilles Besin, Ph.D. - Chief Scientific Officer, Orbital Therapeutics

Characterization and Mitigation of Impurities in Oligonucleotides Containing Methansulfonylphosphoramidate Linkages

15:00 - 15:30
Oligonucleotide Chemistry, Manufacturing and Controls

Oligonucleotide impurities associated with the installation of mesyl phosphoramidite internucleotide linkages during solid-phase synthesis have been identified and characterized. The impurities result from modification of guanosine residues. In this presentation, we will discuss the impurities' structures and mechanisms of formation as well as effective mitigation strategies to limit their formation.

Participants

Christopher Gabriel, Ph.D. - Assistant Director, Process Organic Chemistry, Ionis Pharmaceuticals, Inc.

Potency-enhanced Peptidomimetic VHL Ligands with Improved Oral Bioavailability

15:00 - 15:30
Peptide Discovery to CMC

We present a comprehensive peptidomimetic SAR approach, combined with cellular target engagement assays to improve the current VHL ligand. We identified the 1,2,3-triazole group as an optimal substitute for the amide bond, and incorporated conformationally constrained alterations on the right-hand side, led to picomolar binding affinity and improved oral bioavailability.

Participants

Hao Wu, Ph.D. - Scientist 4, Peptide Therapeutics, Genentech

Targeted Genome Editing with a DNA-dependent DNA Polymerase and Exogenous DNA-containing Templates

15:00 - 15:30
Genome Editing Technology and Applications

Reverse transcriptases, used in prime editing systems, exhibit lower fidelity, processivity and dNTP affinity than many DNA-dependent DNA polymerases. I will present a DNA-dependent DNA polymerase (phi29), untethered from Cas9, enables efficient editing from a synthetic, end-stabilized DNA-containing template in human cells. Compared to prime editing, DNA polymerase editing avoids autoinhibitory intramolecular base pairing of the template, facilitates template synthesis and supports larger insertions.

Participants

Bin Liu - Postdoctoral Fellow, UMass Chan Medical School

Networking Refreshment Break

15:30 - 16:00

Surmounting Conventional Cell Therapy Limitations via In situ CAR Therapy Using oRNA™ Lipid Nanoparticles

16:00 - 16:30
Oligonucleotide Discovery, Preclinical and Clinical

We have been developing a novel, synthetic, circular coding RNA platform (oRNA technology) which exhibits significant improvements in production, expression and formulation compared to mRNAs. We have combined oRNA technology with novel immunotropic LNPs to address the limitations of ex vivo CAR-T therapies by creating off-the-shelf, yet "autologous", in situ CAR (isCAR™) therapies. oRNA-enabled isCAR therapies promise a transient, re-dosable and scalable immune cell therapy without requiring immunodepletion for the treatment of cancer.

Participants

Robert Mabry, PhD - Chief Scientific Officer, Orna Therapeutics

SESSIONS

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

Synthetic Challenges and Mechanisms in 2'-NMA Chemistry for Antisense Oligonucleotides

16:00 - 16:30
Oligonucleotide Chemistry, Manufacturing and Controls

The 2'-NMA chemistry employed in the synthesis of antisense oligonucleotides (ASOs) introduces distinctive synthetic challenges characterized by high branchmer levels and a low purity profile. The intricacies of branchmer formation were studied through targeted syntheses and high-resolution mass spectrometry (HRMS) analysis. It was observed that the branchmer formation can be effectively suppressed by adjusting the process parameters.

Participants

Li Xiao, PhD - Senior Scientist, ASO Development and Manufacturing, Biogen

Developing an Integrated Approach Toward Orally Bioavailable Peptide Therapeutics

16:00 - 16:30
Peptide Discovery to CMC

Participants

Stephen Buckley, Ph.D. - Vice President, Novo Nordisk AS

Cas12a Prime Editor Technology

16:00 - 16:30
Genome Editing Technology and Applications

Participants

John Murphy, Ph.D. - CSO, Arbor Biotechnologies, USA

In Vivo Engineering of the Immune System

16:30 - 17:00
Oligonucleotide Discovery, Preclinical and Clinical

To date, ex vivo engineered T cells showed great performance or promise in several categories of disease indications such as B cell malignancies and autoimmunity. Nevertheless, significant hurdles persist, limiting access, scalability, clinical performance and broad applicability. In vivo engineering of the immune system utilizing an off the shelf, scalable, tunable platform devoid of viral vectors and components, carries the potential of overcoming such challenges and greatly expanding the applicability of this concept.

Participants

Adrian Bot, MD, PhD - Chief Scientific Officer and EVP, R&D, Capstan Therapeutics

Regulatory Considerations for Solution API as a Drug Substance

16:30 - 17:00
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

Rohit Tiwari, Ph.D. - Director Global Regulatory Affairs CMC, Eli Lilly and Company

Developing a Twice-yearly, Miniaturized Subdermal GLP-1 Delivery Implant

16:30 - 17:00
Peptide Discovery to CMC

Poor real-world medication adherence prevents patients from receiving the full potential benefits of their treatment, contributing to 125,000 annually avoidable deaths and over $100B in avoidable healthcare costs in the US alone. To address this challenge, Vivani Medical is developing a miniaturized long-term subdermal implant to guarantee medication adherence over many months. The first application is a twice-yearly exenatide (GLP-1) implant under development for the treatment of Type 2 Diabetes and Obesity.

Participants

Adam Mendelsohn, Ph.D. - Chief Executive Officer, Vivani Medical

Integrase Mediated Programmable Genomic Integration (I-PGI)

16:30 - 17:00
Genome Editing Technology and Applications

I-PGI combines the specificity of CRISPR/Cas9 with proprietary integrases that allow for the insertion of any DNA sequence of any size into a specific programmed location. We will share our progress developing this technology for both in vivo (integrative gene therapy) and ex vivo (cell therapy) applications.

Participants

John Finn, Ph.D. - Chief Scientific Officer, Tome Biosciences

Interim Phase 1 Clinical Data from a 2nd Generation Self-replicating RNA Vaccine for Infectious Disease: Immune Responses and Efficacy at All Dose Levels (0.1, 1.0 and 10 mcg) with a Clean Safety Profile

17:00 - 17:30
Oligonucleotide Discovery, Preclinical and Clinical

Replicate Bioscience has developed RBI-4000, which encodes the rabies glycoprotein in a novel srRNA vector encapsulated in an LNP and was evaluated in healthy volunteers (NCT06048770). Unprecedented immune protection was achieved at the lowest dose tested (0.1 mcg) with clean safety through the highest dose tested (10 mcg). The immunogenicity and safety improvement represents a new standard for the RNA field enabling broader utilization across complex ID, oncology and protein replacement.

Participants

Andrew Geall, PhD - Co-founder and Chief Development Officer, Replicate Bioscience

Manufacturing Strategies for Chemically Modified tRNAs

17:00 - 17:05
Oligonucleotide Chemistry, Manufacturing and Controls

This talk will explore how to tackle the challenges to manufacture this new therapeutic modality, with highlights including approaches to chemical synthesis, impurity identification and control, and physicochemical characterization of a novel drug substance. We will examine novel technologies and discuss initial proof-of-concept experiments to unlock the enormous potential in tRNA biology to scale genetic medicines and create a universal precision medicine to treat thousands of diseases with shared genetic mutations.

Participants

William Kiesman, Ph.D. - Chief Technology Officer, Alltrna

Optimization of Endosomal Escape Vehicle (EEV™) Cell-Penetrating Peptides for Enhanced Delivery of Oligonucleotides to Skeletal and Cardiac Muscle

17:00 - 17:30
Peptide Discovery to CMC

To overcome current limitations of oligonucleotide therapeutic delivery, we designed a family of cyclic cell-penetrating peptides (CPPs) that form the core of our Endosomal Escape Vehicle (EEV™) technology. Through a series of medicinal chemistry modifications, EEV peptides were optimized to efficiently deliver covalently conjugated oligonucleotides to skeletal and cardiac muscle in preclinical models of Duchenne muscular dystrophy (DMD).

Participants

Leo Qian, PhD - Co-Founder and Vice President, Discovery Research, Entrada Therapeutics

Discovery of a Unified RNA-guided Mechanism for Programmable Genome Manipulation

17:00 - 17:30
Genome Editing Technology and Applications

Genomic rearrangements such as insertions, deletions, or inversions, are essential for genetic diversity. These rearrangements are typically orchestrated by enzymes involved in fundamental DNA repair processes such as homologous recombination or in the transposition of foreign genetic material by viruses and mobile genetic elements (MGEs). Here, we show that some MGEs express a non-coding RNA that binds specifically to their encoded recombinase. Reprogramming of this RNA enables multi-kilobase DNA insertion into genomic target sites as well as programmable DNA excision and inversion. The bridge mechanism expands the diversity of nucleic acid-guided systems beyond CRISPR and RNA interference, enabling a general method for genome design using the three fundamental DNA rearrangements.

Participants

Patrick Hsu, Ph.D. - Co-Founder, Arc Institute and Associate Professor, UC Berkeley

Close of TIDES 2024

17:30 - 17:35

SCHEDULE

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 1	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY TO CMC	GENOME EDITING TECHNOLOGY AND APPLICATIONS	DELIVERY OF MACROMOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	SPOTLIGHT PRESENTATION 5
06:00	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event	**06:45** - Sunrise Yoga: Wellness Event
07:00	**07:45** - Case Study on the GMP Development and Manufacturing Process for DOTAGA-Labeled Urea-Based Peptide PSMA Inhibitor, DOTAGA-(l-y)fk(SubKuE) **07:30** - Registration	**07:45** - Overcoming Oligonucleotide Manufacturing Challenges **07:30** - Registration	**07:45** - Amidite RSM Impurity Control and Impact on Oligonucleotide API Quality **07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration	**07:30** - Registration

SCHEDULE

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

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08:00				**08:25** - Chairman's Remarks: Oligonucleotide Preclinical and Clinical & Progress in the Development of Phosphorodiamidate Morpholino Oligonucleotides PMOs **08:30** - Silencing Gain-of-function KCNT1 Genetic Epilepsy with Divalent siRNA, A Novel Small Interfering RNA Technology, Durably	**08:25** - Chairman's Remarks: Analytics for Oligonucleotide Modalities **08:30** - Methods to Establish Diasteromeric Content Comparability in Oligonucleotide Products	**08:25** - Chairman's Remarks: Targeted Radiopharmaceuticals: Progress to the Clinic **08:30** - Next Wave of Radionuclide Theranostics	**08:25** - Chairman's Remarks: Genome Editing Clinical and Preclinical **08:30** - Prime Editing for the Treatment of Chronic Granulomatous Disease	**08:25** - Chairman's Remarks: Targeted Delivery and Novel Delivery Approaches **08:30** - Extrahepatic Delivery of LNPs					

SCHEDULE

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 1	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY TO CMC	GENOME EDITING TECHNOLOGY AND APPLICATIONS	DELIVERY OF MACROMOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	SPOTLIGHT PRESENTATION 5
				Eliminates Spontaneous Seizures in a Mouse Epilepsy Model									
09:00				**09:00** - Late Breaking Presentation **09:30** - Late Breaking Presentation	**09:00** - Strategies for the Characterization of Stereopure Chimeric PO/PS/PN Oligonucleotides **09:30** - Considerations for Method Development of siRNA Duplexes	**09:00** - RGD Peptide as a Theranostic Radiotracer Targeting ?vβ3 Integrin **09:30** - Bicyclic Peptide Technology to Develop Bicycle Radioconjugates	**09:00** - Advances in In Vivo CRISPR Gene Editing for Therapeutic Application **09:30** - Proof-of-concept for in vivo Base Editing to Inactivate the PCSK9 Gene and Lower LDL-Cholesterol in Humans	**09:00** - Novel TRiM™ Platform for Delivery of RNAi Therapeutics to Adipose Tissue **09:30** - In Vivo Engineering of Cells Using Targeted Lipid Nanoparticles					

SCHEDULE

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

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10:00	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:45** - Recent Progress with Antibody PMO Conjugates **10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:45** - Development of a 2D-LC/MS Workflow and Its Application for Impurity Profiling of Phosphorodiamidate Morpholino Oligomers **10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:45** - Development of Radiopharmaceutical Therapy agents for treatment of GPC3-Expressing tumors **10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:45** - As-Cas12a Gene Editing of HBG1/2 Promoters with EDIT-301 (reni-cel) Results in Rapid and Sustained Normalization of Hemoglobin and Increased Fetal Hemoglobin in Patients with Severe Sickle Cell Disease and Transfusion-dependent Beta-thalassemia **10:00** - Networking Refreshment Break in	**10:45** - Versatile Transformable Peptidic Nanoplatform for Cancer Therapy and Detection **10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall	**10:00** - Networking Refreshment Break in Poster and Exhibit Hall

SCHEDULE

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

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							Poster and Exhibit Hall						
11:00				**11:15** - Enhanced Delivery Oligonucleotides: An Update on Preclinical and Clinical Progress **11:45** - Strategies for Oligonucleotides Purification Applicable for Clinical Products Manufacture	**11:15** - LC MS Methods for Characterization of Long Oligonucleotides **11:45** - Analytical Development for Prime Editing Guide (peg)RNAs	**11:15** - Pioneering Aktis Oncology's Miniprotein Radioconjugates **11:45** - Ac-FL-020, A Novel PSMA-targeting Radioligand Therapy Candidate in Development	**11:15** - Late Breaking Presentation **11:45** - Late Breaking Presentation	**11:15** - Late Breaking Presentation **11:45** - Late Breaking Presentation					

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12:00	**12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:15** - Transition to Spotlight Presentation Rooms **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:15** - Transition to Spotlight Presentation Rooms **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:15** - Transition to Spotlight Presentation Rooms **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:15** - Transition to Spotlight Presentation Rooms **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:15** - Transition to Spotlight Presentation Rooms **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:20** - Novel Ionizable Lipids and Their LNPs to Accelerate Development of RNA based Therapeutics **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:20** - Non-viral RNA Delivery with Biodegradable Lipids **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:20** - Novel Cap Analogs and Modified NTPs to Enable Therapeutic mRNA Development **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:20** - Phenomenex Spotlight Presentations **12:50** - Networking Luncheon in Poster and Exhibit Hall	**12:20** - Unlocking Guide RNA Quality: The Power of NGS Analysis **12:50** - Networking Luncheon in Poster and Exhibit Hall
13:00				**13:55** - Chairman's Remarks: Novel RNA-based Therapeutic and Vaccine Platforms	**13:55** - Chairman's Remarks: Innovations in Oligonucleotide Process Development and Manufacturing	**13:55** - Chairman's Remarks: Delivery of Peptides and Peptides as Delivery Agents	**13:55** - Chairman's Remarks: Next-Generation Genome Editing Technologies						

SCHEDULE

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 1	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY TO CMC	GENOME EDITING TECHNOLOGY AND APPLICATIONS	DELIVERY OF MACROMOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	SPOTLIGHT PRESENTATION 5
14:00				**14:00** - Editing the Genome with Cas9 mRNA **14:30** - mRNA-encoded Antibodies to Combat Infectious Diseases	**14:00** - Adoption of Innovative Technologies in Oligonucleotide Manufacturing: Improving Efficiency of siRNA Manufacturing Processes **14:30** - Use of Ultrafiltration/Diafiltration for the Processing of Antisense Oligonucleotides	**14:00** - Peptide Drug Delivery - Roadmap to Selecting a Development Candidate and Transforming it to a Product **14:30** - BIONDD – Enabling Oral Administration of Biologics Achieving Drug Exposures Comparable to Injections	**14:00** - Enhancing Precision and Efficiency of In Vivo Gene Editing with Engineered PsCas9 **14:30** - Programmable Molecular Technologies for Genome Editing and Cell Control						

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MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

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15:00	**15:30** - Networking Refreshment Break	**15:30** - Networking Refreshment Break	**15:30** - Networking Refreshment Break	**15:00** - Introducing Circular RNA Vaccine Platform as Novel Alternative to RNA Vaccine **15:30** - Networking Refreshment Break	**15:00** - Characterization and Mitigation of Impurities in Oligonucleotides Containing Methansulfonylphosphoramidate Linkages **15:30** - Networking Refreshment Break	**15:00** - Potency-enhanced Peptidomimetic VHL Ligands with Improved Oral Bioavailability **15:30** - Networking Refreshment Break	**15:00** - Targeted Genome Editing with a DNA-dependent DNA Polymerase and Exogenous DNA-containing Templates **15:30** - Networking Refreshment Break	**15:30** - Networking Refreshment Break	**15:30** - Networking Refreshment Break	**15:30** - Networking Refreshment Break	**15:30** - Networking Refreshment Break	**15:30** - Networking Refreshment Break	**15:30** - Networking Refreshment Break

SCHEDULE

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

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16:00				**16:00** - Surmounting Conventional Cell Therapy Limitations via In situ CAR Therapy Using oRNA™ Lipid Nanoparticles **16:30** - In Vivo Engineering of the Immune System	**16:00** - Synthetic Challenges and Mechanisms in 2'-NMA Chemistry for Antisense Oligonucleotides **16:30** - Regulatory Considerations for Solution API as a Drug Substance	**16:00** - Developing an Integrated Approach Toward Orally Bioavailable Peptide Therapeutics **16:30** - Developing a Twice-yearly, Miniaturized Subdermal GLP-1 Delivery Implant	**16:00** - Cas12a Prime Editor Technology **16:30** - Integrase Mediated Programmable Genomic Integration (I-PGI)						

SCHEDULE

MAIN CONFERENCE - DAY 3 (MAY 17) - 17/05/2024

TIME	BREAKFAST SPOTLIGHT PRESENTATIONS 1	BREAKFAST SPOTLIGHT PRESENTATIONS 2	BREAKFAST SPOTLIGHT PRESENTATIONS 3	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY TO CMC	GENOME EDITING TECHNOLOGY AND APPLICATIONS	DELIVERY OF MACROMOLECULES	SPOTLIGHT PRESENTATION 1	SPOTLIGHT PRESENTATION 2	SPOTLIGHT PRESENTATION 3	SPOTLIGHT PRESENTATION 4	SPOTLIGHT PRESENTATION 5
17:00	**17:30** - Close of TIDES 2024	**17:30** - Close of TIDES 2024	**17:30** - Close of TIDES 2024	**17:00** - Interim Phase 1 Clinical Data from a 2nd Generation Self-replicating RNA Vaccine for Infectious Disease: Immune Responses and Efficacy at All Dose Levels (0.1, 1.0 and 10 mcg) with a Clean Safety Profile **17:30** - Close of TIDES 2024	**17:00** - Manufacturing Strategies for Chemically Modified tRNAs **17:30** - Close of TIDES 2024	**17:00** - Optimization of Endosomal Escape Vehicle (EEV™) Cell-Penetrating Peptides for Enhanced Delivery of Oligonucleotides to Skeletal and Cardiac Muscle **17:30** - Close of TIDES 2024	**17:00** - Discovery of a Unified RNA-guided Mechanism for Programmable Genome Manipulation **17:30** - Close of TIDES 2024	**17:30** - Close of TIDES 2024	**17:30** - Close of TIDES 2024	**17:30** - Close of TIDES 2024	**17:30** - Close of TIDES 2024	**17:30** - Close of TIDES 2024	**17:30** - Close of TIDES 2024