SESSIONS

DAY 1 - 19/03/2024

Registration and Coffee

08:00 - 09:00

Workshop Moderator's Opening Remarks

09:00 - 09:15
Workshop #1: Overcoming Manufacturing Challenges and Defining CMC Control Strategies of Synthetic RNA Therapeutics

Participants

Thomas Rupp - Owner & Principal, Thomas Rupp Consulting, Germany

Workshop Moderator's Opening Remarks

09:00 - 09:15
Workshop #2: Analytical Characterization and Bioanalysis of Peptides

Workshop Description:

Peptides fall between small molecules and biologics in terms of molecular weight and size. Additionally, the complexity of peptide therapeutics is increasing with unnatural amino acid substitutions and various covalent modifications (lipidation, PEGylation). This results in analytical challenges! Learn from the experts on strategies and best practices. Hear case studies from companies working in this area.

Who should attend?

Anyone interested in the analysis and bioanalysis of peptide therapeutics. This includes Research scientists, Manufacturing Personnel, Quality Assurance, Project Management, Business Development and Scientific Management

Participants

Bruce Morimoto, PhD - Vice President, Drug Development, Alto Neuroscience

Workshop Agenda

09:15 - 12:30
Workshop #1: Overcoming Manufacturing Challenges and Defining CMC Control Strategies of Synthetic RNA Therapeutics

Workshop Description:

This workshop will address different strategies for overcoming manufacturing challenges of synthetic RNA therapeutics. It will also discuss methods for defining CMC control strategies along the development pathway from early clinical development through later stages of development. A variety of different examples and case studies will be presented to give attendees a good understanding of the current bottlenecks, potential solutions and future directions in oligonucleotide CMC and manufacturing.

Who should attend?

Anyone interested in development of oligonucleotide therapeutics; Anyone interested in outsourcing the manufacturing of oligonucleotide therapeutics to a CMO / CRO. This includes R&D Researchers, Manufacturing Personnel, Quality Assurance, Project Management, Business Development and Scientific Management.

Participants

Thomas Rupp - Owner & Principal, Thomas Rupp Consulting, Germany

Synthetic Peptide Purity Method Development Challenges – Illustrated Case Studies

09:15 - 10:00
Workshop #2: Analytical Characterization and Bioanalysis of Peptides

The purity and impurities analysis of peptides is a complex task requiring reliable methods when going into a clinical program, with each project bringing its share of unknowns. For traditional APIs, a robust purity method development strategy is required, whereas in the high-throughput individualised medicines field, speed is of the essence to reach patients as soon as possible. This presentation will address those challenges through recent case studies.

Participants

Alaric Desmarchelier, PhD - Business Development Manager - Peptides, Almac Group

Updates on Peptide and Protein Characterization Methodologies

10:00 - 10:45
Workshop #2: Analytical Characterization and Bioanalysis of Peptides

LC/MS is a powerful method for the characterization of peptides, proteins and drug conjugates; however other tools like MALS add complementary information that provide a better understanding of therapeutic molecules. In our lab we have been using these tools to get a more complete picture on the structural characteristics of peptide, protein, and gene therapy drug candidates and will discuss some of the unique challenges faced with each.

Participants

Michael McGinley - Director, Global Applications, Phenomenex

Networking Refreshment Break

10:45 - 11:15
Workshop #2: Analytical Characterization and Bioanalysis of Peptides

Solubility and Physical Stability Challenges in Peptide Formulation Development

11:15 - 12:00
Workshop #2: Analytical Characterization and Bioanalysis of Peptides

Solubility and physical stability challenges may be encountered during the development of injectable peptide and protein drugs. Most peptide and protein injections as liquid formulations or reconstituted solids need to comply with particulate matter specifications set by USP guidelines and be devoid of insoluble aggregates to reduce or eliminate cardiovascular risk and undesired immune responses. Case studies will be presented on how to overcome latent physical instability of small and linear cyclic peptides in liquid formulations during long-term storage.

Participants

Juerg Tschopp, PhD - Principal Scientist, Stratum Medical Corporation

Bioanalytical Challenges, Best Practices and Case Studies

12:00 - 12:30
Workshop #2: Analytical Characterization and Bioanalysis of Peptides

Speaker TBA

SESSIONS
DAY 1 - 19/03/2024

Close of Workshop and Luncheon for Morning Workshop Attendees

12:30 - 13:40

Chairperson's Remarks

13:40 - 13:45
Main Conference Plenary Keynote Session

Participants

Jennifer Lockridge, PhD - VP, Dicerna TRU Early Development, Novo Nordisk

Developing Next Generation Oligonucleotide Therapeutics

13:45 - 14:15
Main Conference Plenary Keynote Session

Advances in drug-like properties of oligonucleotides and targeted delivery approaches in the last decades have enabled the development of this class of molecules to modulate intracellular targets precisely. This has led to the expansion of druggable target space and development of oligonucleotide therapeutics for previously untreated diseases. This talk will give an overview of the opportunities and challenges that need to be addressed to develop oligonucleotides into meaningful therapies beyond rare diseases.

Participants

Shalini Andersson, PhD - Vice President of Oligonucleotide Discovery, AstraZeneca

Chemical Engineering of Oligonucleotides: Applications for RNA Therapeutic

14:15 - 14:45
Main Conference Plenary Keynote Session

RNA therapeutics has become an established drug discovery and development platform with approved drugs that employ various mechanisms of action. Chemistry has played a critical role in providing drug-like properties to oligonucleotides. Recently, we have employed chemical engineering, an approach of using established chemical modifications at strategic positions in an oligonucleotide, modifying the accessibility of both 3'- and 5'-ends, and creating transient shapes. These changes have improved the delivery, specificity, and potency of antisense. These designs are broadly applicable to various mechanisms of action of RNA therapeutics.

Participants

Sudhir Agrawal - President and Founder, Arnay Sciences

Peptide and Protein Therapeutics Conjugated with Human N-Linked Glycans

14:45 - 15:15
Main Conference Plenary Keynote Session

N-glycans are useful modifier molecules to improve the *in vivo* half-lives of various bioactive peptides and proteins. Our chemical glycosylation procedure, which exploits the beneficial characteristics of human N-glycans, enables to more quickly develop glycopeptide/protein therapeutics that are superior to the original molecules in terms of pharmacokinetic and physicochemical properties.

Participants

Yuji Nishiuchi, Ph.D. - Director of Research and Development, GlyTech, Inc.

Development of Orally Bioavailable Peptides Targeting an Intra-cellular Protein: From a Hit to a Clinical KRAS Inhibitor

15:15 - 15:45
Main Conference Plenary Keynote Session

Cyclic peptides as a therapeutic modality have received notable attention due to their potential for oral absorption and accessibility to intracellular tough targets. In this presentation, the concept of our cyclic peptide drug discovery platform and as the first example from this technology, the discovery of a RAS inhibitory clinical compound (LUNA18) will be introduced.

Participants

Ryuji Hayashi, PhD - Group Head, Discovery Chemistry Department, Research Division, Chugai Pharmaceutical Co., Ltd.

Networking Refreshment Break

15:45 - 16:15
Main Conference Plenary Keynote Session

RNAi Therapeutics in Japan: Needs and Solutions-An Alnylam Perspective

16:15 - 16:45
Main Conference Plenary Keynote Session

Participants

Yutaka Okada - President and Representative Director, Alnylam Japan KK

The Endosomal Escape Vehicle Platform of Cyclic Cell-Penetrating Peptides Enhances the Delivery of Oligonucleotides

16:45 - 17:15
Main Conference Plenary Keynote Session

To overcome current limitations of oligonucleotide therapeutic delivery, we have designed a family of proprietary cyclic cell-penetrating peptides that form the core of our Endosomal Escape Vehicle (EEV™) technology and covalently conjugated it to oligonucleotides. Using preclinical models of Duchenne muscular dystrophy (DMD), we demonstrated the ability of our EEV platform technology to efficiently deliver oligonucleotides to skeletal and cardiac muscle, the primary sites of pathology in DMD.

Participants

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

Realizing the Potential of RNA-Targeted Oligonucleotide Therapeutic; Pipeline Progress and Partnerships

17:15 - 17:45
Main Conference Plenary Keynote Session

Participants

Brett Monia, PhD - Chief Executive Officer, Ionis Pharmaceuticals

Close of Day One

17:45 - 17:50

SCHEDULE

DAY 1 - 19/03/2024

19-21 March 2024
Westin Miyako Kyoto
Kyoto, Japan

TIME	WORKSHOP #1: OVERCOMING MANUFACTURING CHALLENGES AND DEFINING CMC CONTROL STRATEGIES OF SYNTHETIC RNA THERAPEUTICS	WORKSHOP #2: ANALYTICAL CHARACTERIZATION AND BIOANALYSIS OF PEPTIDES	MAIN CONFERENCE PLENARY KEYNOTE SESSION
08:00	**08:00** - Registration and Coffee	**08:00** - Registration and Coffee	**08:00** - Registration and Coffee
09:00	**09:00** - Workshop Moderator's Opening Remarks **09:15** - Workshop Agenda	**09:00** - Workshop Moderator's Opening Remarks **09:15** - Synthetic Peptide Purity Method Development Challenges – Illustrated Case Studies	
10:00		**10:00** - Updates on Peptide and Protein Characterization Methodologies **10:45** - Networking Refreshment Break	
11:00		**11:15** - Solubility and Physical Stability Challenges in Peptide Formulation Development	
12:00	**12:30** - Close of Workshop and Luncheon for Morning Workshop Attendees	**12:00** - Bioanalytical Challenges, Best Practices and Case Studies **12:30** - Close of Workshop and Luncheon for Morning Workshop Attendees	**12:30** - Close of Workshop and Luncheon for Morning Workshop Attendees
13:00			**13:40** - Chairperson's Remarks **13:45** - Developing Next Generation Oligonucleotide Therapeutics
14:00			**14:15** - Chemical Engineering of Oligonucleotides: Applications for RNA Therapeutic **14:45** - Peptide and Protein Therapeutics Conjugated with Human N-Linked Glycans
15:00			**15:15** - Development of Orally Bioavailable Peptides Targeting an Intra-cellular Protein: From a Hit to a Clinical KRAS Inhibitor **15:45** - Networking Refreshment Break
16:00			**16:15** - RNAi Therapeutics in Japan: Needs and Solutions-An Alnylam Perspective **16:45** - The Endosomal Escape Vehicle Platform of Cyclic Cell-Penetrating Peptides Enhances the Delivery of Oligonucleotides
17:00	**17:45** - Close of Day One	**17:45** - Close of Day One	**17:15** - Realizing the Potential of RNA-Targeted Oligonucleotide Therapeutic; Pipeline Progress and Partnerships **17:45** - Close of Day One

SESSIONS
DAY 2 - 20/03/2024

Registration and Coffee

07:30 - 08:00

Chairperson's Remarks

08:00 - 08:05
Plenary Session

Participants

Shiro Akinaga, PhD - President and CEO, NANO MRNA, Co., Ltd.

Transforming the Care of Cardiovascular Disease Through Single-course Gene Editing Medicines

08:05 - 08:35
Plenary Session

Participants

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

From Circles to Conjugates: Rethinking the Architecture of guide RNA

08:35 - 09:05
Plenary Session

High quality gRNA is needed for the effective application of CRISPR/Cas-based therapeutics. Several challenges face the development of therapeutic gRNA, including the need for more robust synthesis processes and higher potency (particularly, in vivo). Here I will describe two new gRNA architectures, circular gRNA and peptide-gRNA conjugates, and show how each, in part, addresses these challenges. I will also discuss why we should reconsider the most common gRNA design and review alternatives.

Participants

Brian Cafferty, PhD - Director, Beam Therapeutics

Improving Oligonucleotide Pharmacology Across Tissues and Modalities through Base, Sugar, and Backbone Modifications

09:05 - 09:35
Plenary Session

Wave's PRISM^{TM} platform enables synthesis of stereopure oligonucleotides with position-controlled chemistry and stereochemistry, facilitating optimization for the target, tissue, and modality. We describe our progress in improving the pharmacological properties of oligonucleotides for RNA interference (RNAi) and RNA editing in hepatic and extrahepatic tissues through base, sugar, and backbone modifications.

Participants

Michael Byrne, Ph.D. - Vice President, In Vivo and CNS Biology, Wave Life Sciences

Networking Refreshment Break with Poster and Exhibit Viewing

09:35 - 10:15

Chairperson's Remarks

10:15 - 10:20
Track 1

Participants

Shiro Akinaga, PhD - President and CEO, NANO MRNA, Co., Ltd.

Chairperson's Remarks

10:15 - 10:20
Track 2

Participants

Rowshon Alam, Ph.D. - Senior Director, Head of Process Chemistry, Prime Medicine

Amide-Modified Oligonucleotides for Chemical Control of Functional RNAs

10:20 - 10:50
Track 1

This presentation will discuss synthesis, structure, and RNAi and CRISPR activity and specificity of amide modified RNA. Amides are excellent mimics of the phosphodiester linkages in RNA. Amide modifications of siRNAs significantly reduced the off-target activity of guide and passenger strands. Amides did not interfere with CRISPR-Cas9 activity when placed in the protospacer adjacent motif distal region of crRNAs. Our results suggest that amides have strong potential to optimize biological and pharmacological properties of siRNAs and crRNAs for in vivo applications.

Participants

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

Synthesizing Long Guide RNAs for In Vivo Prime Editing

10:20 - 10:50
Track 2

Prime Editing Guide RNAs (pegRNA and nickRNA) are key components of Prime Editing (PE) Systems that require innovative and robust manufacturing processes and testing to generate guide RNAs suitable for in-life applications of PE. Various chemistries and methods that enable the generation of high-quality guide RNA from mg to gram scale supporting pre-clinical development of several *in vivo* programs will be described.

Participants

Rowshon Alam, Ph.D. - Senior Director, Head of Process Chemistry, Prime Medicine

Chemical Approaches to Enhance siRNA Selectivity

10:50 - 11:20
Track 1

Exogenous siRNAs do not always exhibit specificity, potentially resulting in unintended consequences on the expression of non-targeted genes, referred to as off-target effects. Among these off-target effects, the most common is the miRNA-like effect, which arises from either perfect or partial seed sequence complementarity with non-targeted mRNAs. Here, we report that placing a bulky alkyl phosphonate backbone in the seed region of the guide strand improves specificity of siRNA.

Participants

Mehran Nikan, Ph.D. - Research Fellow, Ionis Pharmaceuticals Inc

SESSIONS
DAY 2 - 20/03/2024

Addressing the Regulatory Expectations for Guide RNA Quality While Providing Manufacturing Capacity

10:50 - 11:20
Track 2

Participants

Blake Unterreiner - Associate Vice President, Business Development & Customer Relations, Agilent Technologies

Synthetic Strategy for Ligand Conjugated Oligonucleotides via Diamine Modified Stable Phosphoroamidite

11:20 - 11:50
Track 1

This presentation provides the methodologies for synthesis of ligand conjugated oligonucleotides along with developing a novel diamine modified stable phosphoroamidite, which has favorable physical characteristics that can help to specify and regulate quality attributes of oligonucleotides.

Participants

Mitsuaki Sekiguchi, Ph.D. - Principal Scientist, Biopharmaceutical Research Di, Shionogi & Co., Ltd.

Delivery of Genetic Medicine with Hydrophilic Nanoparticles

11:20 - 11:50
Track 2

Conventional delivery technologies for genetic medicine face challenges: off-target delivery, innate immune response, unable to repeat dose, or costly manufacturing. NanoGalaxy platform consists of a diverse library of hydrophilic polymers and, through systematic and iterative screening, has been used to identify NPs with selective delivery to the nervous system via intrathecal administration and to the innate immune system via intravenous administration. This presentation will introduce NanoGalaxy platform and share the delivery results of genetic medicine payloads.

Participants

Kunwoo Lee, PhD - Chief Executive Officer, GenEdit

Networking Luncheon with Poster and Exhibit Viewing

11:50 - 12:50

TIDES Talks: Exhibit Hall Luncheon Spotlight Presentations

12:15 - 12:30 WuXi TIDES

Amidite RSM Impurity Control and Impact on Oligonucleotide API Quality

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.

Yun Yang, Ph.D., Senior Director, Oligonucleotide Discovery, WuXi TIDES

12:30 - 12:45 Cytiva

Oligonucleotide Therapeutics; Manufacturing for Research and Beyond

The rise in approved oligo therapeutics has led to a growing demand on scale and optimization of oligonucleotide manufacturing. This change has created pressure on oligo manufacturers to focus on ways to secure their supply chain, decrease costs, and shrink process time. In this presentation, we illustrate our scalable oligo nucleotides manufacturing strategies from synthesis to fill finish. You can see how manufacturing process is intensified with our single use technology in downstream. The processes used for oligonucleotide synthesis create challenges for sustainability. Large volumes of hazardous solvents or reagents may be involved, and synthesis, purification, and isolation are energy-intensive processes. We also show that there are ways to reduce the environmental impact.

Midori Sasao, Field Application Specialist, Cytiva

Participants

Yun Yang, PhD - Senior Director, Oligonucleotide Discovery, WuXi TIDES

Midori Sasao - Field Application Specialist, Cytiva

Chairperson's Remarks

12:50 - 12:55
Track 2

Participants

Naoki Yamamoto, PhD - Head of Business Development, Asia Pacific, Bachem Japan K.K.

Chairperson's Remarks

12:55 - 13:00
Track 1

Participants

Hideaki Sato - President and CEO, Luxna Biotech

Acceleration of Process Development for GMP Production of Peptides

12:55 - 13:20
Track 2

This presentation will discuss high-throughput process development via microwave-assisted SPPS and optimizing the synthesis of a hydrophobic 19 amino acid peptide.

Participants

Dewey Sutton, PhD - Research and Development Supervisor, AmbioPharm

Comprehensive Analysis of Brain Distribution for Antisense Oligonucleotides Using Whole Tissue Imaging Technique

13:00 - 13:30
Track 1

A direct administration into the cerebrospinal fluid is conventionally employed in antisense oligonucleotides (ASOs) therapy for central nervous system diseases. However, the intra-brain behavior of ASOs after intrathecal / intracerebroventricular injection and have not been fully clarified. In this study, we aimed to figure out the brain distribution behavior of ASOs in mice, rats, and marmosets using whole-brain microscopic imaging with tissue clearing technique.

Participants

Syunsuke Yamamoto - Associate Director, Takeda Pharmaceutical Company

Recent Technological Innovation in Peptide Manufacturing

13:20 - 13:45
Track 2

Accelerating the development of peptides and oligonucleotides as pharmaceuticals requires technological development that disrupts conventional approaches and overcomes cost and quality challenges in all manufacturing processes, including synthesis, purification, and lyophilization. We will introduce specific examples of new technologies that can solve these issues using our model peptides.

Participants

Yoshitaka Nemoto - Vice President R&D, PeptiStar

SESSIONS
DAY 2 - 20/03/2024

In vivo Genome Editing: Translating Science from Bench to Bedside

13:30 - 14:00
Track 1

Here we describe our work developing a High-Throughput Evolution Platform for Discovery and Optimization of Novel Editors (HEPDONE) system, which have identified and optimized a repertoire of proprietary novel Cas enzymes and base editors that could be tailor-made for precisely targeting previously undruggable disease indications. In vivo preclinical studies in mice and NHPs have demonstrated several orders of magnitude higher levels of edits utilizing our proprietary lipid nanoparticle (LNP) mediated delivery systems targeting both liver and HSCs in the bone marrow. In addition, recent IIT clinical trial readouts from our ongoing in vivo genome editing programs in China will be discussed.

Participants

Zi Jun Emma Wang, PhD - Chief Technology Officer, YolTech Therapeutics

De Novo Discovery of Natural Product-like Thiopeptides with Designed Biological Activities

13:45 - 14:10
Track 2

The talk will discuss how an in vitro reconstituted and reengineered natural product biosynthesis pathway can be integrated into mRNA display, a powerful in vitro selection technique. The resulting platform enables de novo discovery of natural product-like macrocyclic peptides active against proteins of interest. The high potency, selectivity, proteolytic stability, and cell uptake of the discovered compounds highlight how the established platform can accelerate early drug discovery efforts.

Participants

Alexander Vinogradov, PhD - Project Assistant Professor, The University of Tokyo

Nucleic Acid Therapeutics: Process Optimization and Purification

14:00 - 14:30
Track 1

Innovative cell-free DNA template production, and the usage of Dynabeads for in vitro transcription and crude mRNA purification could help optimize current mRNA workflow and possibly become the alternative method for the oligo synthesis.

Participants

Lulu Zhang, PhD - Field Application Scientist, Thermo Fisher Scientific

Discovery of Macrocycles for Delivery of RNA and Targeted Radiopharmaceuticals

14:10 - 14:35
Track 2

The talk will describe genetically encoded and DNA encode pipeline used by 48Hour Discovery to discovery nonomolar and pioconolar lead compounds that bind to extra cellular receptors; we will discuss both internal pipeline of 48Hour discovery and overview partnership projects and discuss advance of these assets though the preclinical pipeline.

Participants

Ratmir Derda, PhD - Founder and CSO, 48Hour Discovery and Associate Professor, Department of Chemistry, University of Alberta

Overcoming Oligonucleotide Manufacturing Challenges

14:30 - 15:00
Track 1

Participants

Loic Cornelissen, PhD - Associate Director, Business Development, PolyPeptide Group

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

14:35 - 15:00
Track 2

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

Ping Ye - Senior Scientist II, UCB

Networking Refreshment Break with Poster and Exhibit Viewing

15:00 - 15:30

Going Large-scale with Manufacturing of Oligonucleotides

15:30 - 16:00
Track 1

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

Macrocyclic Peptide Drug Development by Combining the Strengths of all Small Fragments, mRNA Display and AI Technologies

15:30 - 16:00
Track 2

Quantum Intelligence Corporate (QIC) provides hit compound discovery and lead optimization for therapeutic peptides to pharmaceutical and biotech companies. Our advanced technology (QUEST) is based upon quantum mechanical electrostatic potential calculations and artificial intelligence such as neural networks and deep-learning techniques performed on GPU-powered High Performance Computing clusters. Our supernatural module predicts the optimal unnatural pharmacophore substitution of a template peptide that enhances the binding affinity to a target protein and increases the lipophilicity of the lead.

Participants

Hwanho Choi, M.D., Ph.D. - CEO and Founder, Quantum Intelligence Corporation

SESSIONS
DAY 2 - 20/03/2024

Synthesis PAT: IR Spectroscopy for Real-Time Phosphoramidite Identification

16:00 - 16:30
Track 1

The use of innovative PAT approaches reduces manufacturing risk and improves reliability. FTIR spectra collected by an in-line ATR probe can accurately identify amidites during synthesis operations. Software compares collection data against a spectral databank via cosine similarity and performs amidite solution identification in real time, with direct feedback to the synthesizer during priming setup and manufacturing operations.

Participants

Spenser Pruett - Scientist II, Process Development, Nitto Avecia

From Discovery to the Clinic: Development of a Novel Bioactive Peptide that Improves Mood and Cognition by Engaging the Gut-Brain Axis

16:00 - 16:30
Track 2

We have developed a peptide drug that improves neurologic impairment by acting through a novel gut-brain circuit. DGX-001 is a potent small peptide, orally administered with a mechanism that involves engagement of unique sensory cells in the gut lumen and activation of afferent vagal signals that reach the brain. In healthy human volunteers, DGX-001 was well-tolerated and following short term dosing led to measurable increases in cognitive function and mood that tracked with EEG changes in the brain.

Participants

Kousaku Ohinata, Ph.D. - Founder and Scientific Advisor, Viage Therapeutics

Addressing Complex Oligonucleotide Therapeutics and Approach Towards Endotoxin Removal

16:30 - 17:00
Track 1

Participants

Juergen Mueller, PhD - Vice President of Commercial Operations, LGC Axolabs

Xiaocen (Chris) Li, PhD - Supervisor, LGC Axolabs

A Purely Thermodynamic Anti-prionic Mode of Action for Protein-misfolding Diseases is Realized by All-D-peptides

16:30 - 17:00
Track 2

Thermodynamic stabilization of aggregation-prone proteins, like Aβ and α-synuclein, is not only inhibiting their aggregation, but also disassembling already existing aggregates into harmless monomers. We achieved thermodynamic stabilization of the monomers by all-D-peptides that are highly affin and specific for the protein species in its monomeric conformation, which is intrinsically disordered. The all-D-enantiomeric ligand for Aβ, RD2, demonstrated target engagement ex vivo and disassembled Aβ oligomers obtained from brain tissue of former AD patients. I will present the results of a clinical phase Ib, double-bind, placebo-controlled study with mild cognitively impaired (MCI) and mild AD patients treated once daily orally with RD2 or placebo for 4 weeks. A phase II study is scheduled.

Participants

Dieter Willbold, Ph.D. - Director and Full Professor, Forschungszentrum Jülich

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

17:00 - 17:30
Track 1

Participants

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

In Cellulo Library-derived Peptide-based Inhibitors of Alpha-synuclein Aggregation and Toxicity

17:00 - 17:30
Track 2

A major group focus is the design and selection of peptides that target amyloidogenic proteins involved in age-related diseases. Amyloid proteins are known to be important in a number of such diseases that include Alzheimer's, Parkinson's, Lewy Body Dementia, Huntington's, and CJD. We use a novel *in cellulo* library-screening platform to select peptides that can bind amyloidogenic target proteins to sequester and detoxify them. Utimising a Protein-fragment Complementation approach (PCA), we have identified both strand and helix-based peptide antagonists of α-synuclein proteins implicated in PD and related synucleinopathies. PCA is multiplexed, making no mechanistic assumptions about the target oligomeric state or conformer populated. Rather, library members must bind to and reduce associated toxicity to become selected. Those that either generate, or fail to prevent formation of a toxic species, result in cell death or retarded cell growth rates relative to effective binders. Library members that confer the most rapid bacterial growth are then selected from the PCA by increased stringency during further competition selection. Our antagonists have been characterised using a range of biophysical and cell-based approaches and been downsized / refined using truncation, alanine-scanning, and incorporation of structure-inducing constraints and non-natural sequences. Our work in this area is currently funded by an Alzheimer's Research UK Major Project Award.

Participants

Jody Mason, PhD - Professor of Biochemistry, University of Bath

Networking Cocktail Reception with Poster and Exhibit Viewing

17:30 - 18:30

Please join your fellow attendees in the exhibit hall for an evening of networking while enjoying beverages and appetizers.

Close of Day Two

18:30 - 18:35

SCHEDULE

DAY 2 - 20/03/2024

TIDES Asia: Oligonucleotide & Peptide Therapeutics Scientific Forum

19-21 March 2024
Westin Miyako Kyoto
Kyoto, Japan

TIME	PLENARY SESSION	TRACK 1	TRACK 2
07:00	**07:30** - Registration and Coffee	**07:30** - Registration and Coffee	**07:30** - Registration and Coffee
08:00	**08:00** - Chairperson's Remarks **08:05** - Transforming the Care of Cardiovascular Disease Through Single-course Gene Editing Medicines **08:35** - From Circles to Conjugates: Rethinking the Architecture of guide RNA		
09:00	**09:05** - Improving Oligonucleotide Pharmacology Across Tissues and Modalities through Base, Sugar, and Backbone Modifications **09:35** - Networking Refreshment Break with Poster and Exhibit Viewing	**09:35** - Networking Refreshment Break with Poster and Exhibit Viewing	**09:35** - Networking Refreshment Break with Poster and Exhibit Viewing
10:00		**10:15** - Chairperson's Remarks **10:20** - Amide-Modified Oligonucleotides for Chemical Control of Functional RNAs **10:50** - Chemical Approaches to Enhance siRNA Selectivity	**10:15** - Chairperson's Remarks **10:20** - Synthesizing Long Guide RNAs for In Vivo Prime Editing **10:50** - Addressing the Regulatory Expectations for Guide RNA Quality While Providing Manufacturing Capacity
11:00	**11:50** - Networking Luncheon with Poster and Exhibit Viewing	**11:20** - Synthetic Strategy for Ligand Conjugated Oligonucleotides via Diamine Modified Stable Phosphoroamidite **11:50** - Networking Luncheon with Poster and Exhibit Viewing	**11:20** - Delivery of Genetic Medicine with Hydrophilic Nanoparticles **11:50** - Networking Luncheon with Poster and Exhibit Viewing
12:00		**12:55** - Chairperson's Remarks	**12:50** - Chairperson's Remarks **12:55** - Acceleration of Process Development for GMP Production of Peptides
13:00		**13:00** - Comprehensive Analysis of Brain Distribution for Antisense Oligonucleotides Using Whole Tissue Imaging Technique **13:30** - In vivo Genome Editing: Translating Science from Bench to Bedside	**13:20** - Recent Technological Innovation in Peptide Manufacturing **13:45** - De Novo Discovery of Natural Product-like Thiopeptides with Designed Biological Activities
14:00		**14:00** - Nucleic Acid Therapeutics: Process Optimization and Purification **14:30** - Overcoming Oligonucleotide Manufacturing Challenges	**14:10** - Discovery of Macrocycles for Delivery of RNA and Targeted Radiopharmaceuticals **14:35** - Discovery of Zilucoplan: A Potent Macrocyclic Peptide Complement Component 5 (C5) Inhibitor in Acetylcholine Receptor Antibody-positive Generalized Myasthenia Gravis

SCHEDULE

DAY 2 - 20/03/2024

TIME	PLENARY SESSION	TRACK 1	TRACK 2
15:00	**15:00** - Networking Refreshment Break with Poster and Exhibit Viewing	**15:30** - Going Large-scale with Manufacturing of Oligonucleotides **15:00** - Networking Refreshment Break with Poster and Exhibit Viewing	**15:30** - Macrocyclic Peptide Drug Development by Combining the Strengths of all Small Fragments, mRNA Display and AI Technologies **15:00** - Networking Refreshment Break with Poster and Exhibit Viewing
16:00		**16:00** - Synthesis PAT: IR Spectroscopy for Real-Time Phosphoramidite Identification **16:30** - Addressing Complex Oligonucleotide Therapeutics and Approach Towards Endotoxin Removal	**16:00** - From Discovery to the Clinic: Development of a Novel Bioactive Peptide that Improves Mood and Cognition by Engaging the Gut-Brain Axis **16:30** - A Purely Thermodynamic Anti-prionic Mode of Action for Protein-misfolding Diseases is Realized by All-D-peptides
17:00	**17:30** - Networking Cocktail Reception with Poster and Exhibit Viewing	**17:00** - New Ligation Approach: Technology for High Quality Manufacturing of Over 150 mer RNA **17:30** - Networking Cocktail Reception with Poster and Exhibit Viewing	**17:00** - In Cellulo Library-derived Peptide-based Inhibitors of Alpha-synuclein Aggregation and Toxicity **17:30** - Networking Cocktail Reception with Poster and Exhibit Viewing
18:00	**18:30** - Close of Day Two	**18:30** - Close of Day Two	**18:30** - Close of Day Two

SESSIONS
DAY 3 - 21/03/2024

Registration and Coffee

08:00 - 08:10

Chairperson's Remarks

08:10 - 08:15
Plenary Sessions

Participants

Ed Yaworski - Chief Technology Officer, Genevant Sciences Corp

Modulation of Host Immunity in the Airways with Interferon Lambda Encoding mRNA

08:15 - 08:45
Plenary Sessions

Type III interferons play an important role in the innate antiviral, antifungal and antiprotozoal defences of mucosal barriers and enhance adaptive immune responses in the respiratory mucosa. Based on its proprietary Stabilized Non-Immunogenic mRNA (SNIM®RNA) and lipidoid nanoparticle delivery platforms, Ethris has developed interferon lambda encoding mRNA as a drug candidate for prophylactic and therapeutic administration to the airways for prevention and treatment of viral infections. In the presentation, formulation of mRNA suitable for administration to the airways as an aerosol will be discussed. Furthermore, preclinical proof of concept data will be presented, demonstrating the potency of interferon lambda endcoding mRNA in mouse and ferret influenza A and SARS-CoV-2 virus challenge models in mice and ferrets when administered to the airways.

Participants

Christian Plank, PhD - Chief Technology Officer, Ethris GmbH

Digital PCR for the Analysis of RNA Quality Parameters

08:45 - 09:15
Plenary Sessions

Digital PCR (dPCR) is a versatile tool for quality control analytics of RNA. In particular, RNA identity, RNA ratio in multivalent mixtures and RNA integrity can be assessed by this technique.

Participants

Andreas Czech, PhD - Associate Director RNA Analytics, BioNTech SE

Fine Tuning a PCR based mRNA Manufacturing Platform for each mRNA Sequence

09:15 - 09:45
Plenary Sessions

The increasing demand for mRNA therapeutics requires a flexible technology platform and a cost-effective manufacturing process with well-defined and characterized product quality attributes. Different mRNA sequences and lengths can impact the impurity profiles and create additional challenges which can impact the purification steps and challenges. We will discuss the approach we took to tackle these challenges and optimize/fine tune our process for such challenging mRNAs, by shedding light on a case study for a gene editor encoding mRNA.

Participants

Aditi Mehta, PhD - Associate Director, Head of mRNA Process & Deliver, Merck KGaA

Lipid Nanoparticles to Enable Clinical Development of mRNA-based Therapeutics

09:45 - 10:15
Plenary Sessions

Acuitas' is developing lipid nanoparticle (LNP) delivery systems to efficiently and safely deliver messenger RNA (mRNA). Through a combination of industry partnerships, academic collaborations and internal research, Acuitas is enabling mRNA-based medicines in a broad range of therapeutic areas. This presentation will focus on recent preclinical and analytical results from this research and development work, as these mRNA LNP medicines are rapidly translated into the clinic.

Participants

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

Networking Refreshment Break with Poster and Exhibit Viewing

10:15 - 10:55

Chairperson's Remarks

10:55 - 11:00
Track #1

Participants

Akiko Yanagiya, PhD - Scientist, Arcalis

Chairperson's Remarks

10:55 - 11:00
Track #2

Participants

Ed Yaworski - Chief Technology Officer, Genevant Sciences Corp

Preclinical Data for STK-002, an Antisense Oligonucleotide Being Developed for the Treatment of Autosomal Dominant Optic Atrophy (ADOA)

11:00 - 11:30
Track #1

Using the TANGO (Targeted Augmentation of Nuclear Gene Output) approach, we design ASOs that bind to pre-mRNA and help the target genes produce more protein. The initial application for this technology is haploinsufficient diseases in which one copy of a gene functions normally and the other is mutated. ADOA is a rare genetic disease characterized by severe and progressive visual decline due to loss of retinal ganglion cells. Most patients harbor loss-of-function mutations in the *OPA1* gene that codes for OPA1 protein. Stoke is developing STK-002 that reduces the inclusion of a non-productive event in *OPA1* gene resulting in increase in productive *OPA1* mRNA and protein. The preclinical data supporting the clinical development of STK-002 will be presented here.

Participants

Shobha Ravipaty, PhD - Director, Stoke Therapeutics

Protein-based Nano-capsules for Delivery of Therapeutic RNAs Across the Blood-Brain-Barrier

11:00 - 11:30
Track #2

The presentation will describe the generation and the use of protein-based nano-capsules to deliver therapeutic RNAs across the blood-brain-barrier to treat CNS diseases. The therapeutic potential of this delivery technology will be illustrated for the mRNA treatment of monogenetic CNS disorders such as metachromatic leukodystrophy (MLD), a lysosomal storage disease.

Participants

Ekkehard Leberer, PhD - Scientific Advisor, Neuway Pharma

SESSIONS
DAY 3 - 21/03/2024

Discovery and Delivery of Oligonucleotide Therapeutics

11:30 - 12:00
Track #1

Participants

Jayaprakash Nair, PhD - Vice President, Research, Chemistry and Delivery Science, Alnylam Pharmaceuticals

Redefining Non-viral Delivery for Novel Genomic Medicines with Tissue-targeted Lipid Nanoparticle (ttLNP) Platform

11:30 - 12:00
Track #2

We will present the In-depth characterization of tissue and cell tropism of LNP-mRNA which led us to achieve desired PK/PD profile of therapeutic drug candidates for pulmonary diseases. Beyond the targeted delivery to the tissue and cells of interest, we will also present how the diverse lipid chemistry library allows us to select right lipids to deliver the complex gene editing cargoes, including the mixture of RNA and pDNA for the therapeutic meaningful level of editing in vitro and in vivo.

Participants

Kate Zhang, Ph.D. - Chief Scientific Officer, Hopewell Therapeutics

From Bench to Bedside: Development of a GalXC-Plus siRNA, DCR-STAT3, for Immunotherapy in Refractory Cancer Patients

12:00 - 12:30
Track #1

Participants

Jennifer Lockridge, PhD - VP, Dicerna TRU Early Development, Novo Nordisk

Tuning Lipid Nanoparticles for Specific Applications

12:00 - 12:30
Track #2

Lipid Nanoparticles (LNP) are a well-established platform for delivery of nucleic acids (NA) to hepatocytes and for vaccine applications. However, many potential applications for diverse NA modalities exist outside of these areas. LNP with altered biodistribution can be achieved by changing route of administration, and modulating lipid composition accordingly. This presentation will describe the latest advances for hepatocyte delivery, as well as specialized LNP designed for extrahepatocyte use, including compositions targeting the hepatic stellate cell, lung, muscle and CNS.

Participants

Ed Yaworski - Chief Technology Officer, Genevant Sciences Corp

Networking Luncheon with Poster and Exhibit Viewing

12:30 - 13:40

TIDES Talks: Exhibit Hall Luncheon Spotlight Presentations

1:00-1:30 Waters Corporation

LC-MS for RNA Therapeutics to Ensure Product Quality and Process Consistency

Recent approvals of RNA modalities highlight the need for robust analytical methods to monitor critical quality attributes (CQAs), ensuring control of manufacturing processes and product quality. Oligonucleotide mapping via liquid chromatography coupled to mass spectrometry is effective for CQA assessment, however, limited tools for efficient data acquisition and processing present a time-consuming challenge. A workflow was developed to produce RNA fragment assignments for sgRNA and mRNA, measuring CQAs including ID, Structural Fidelity, 5'Capping efficiency and 3' PolyA Tail heterogeneity.

Nick Pittman, Marketing Manager, Global Biopharma Business, Waters Corporation

Participants

Nick Pittman - Marketing Manager, Global Biopharma Business, Waters Corporation

Chairperson's Remarks

13:40 - 13:45
Track #1

Participants

Akiko Yanagiya, PhD - Scientist, Arcalis

Chairperson's Remarks

13:40 - 13:45
Track #2

Participants

El Djouhar Rekaï, PhD - Head of Process Development & Manufacturing Life cycle Management, PolyPeptide Group

Examples of Antisense Oligonucleotides that Target the Immunosuppressive Tumor Microenvironment for Treatment of Cancer

13:45 - 14:15
Track #1

Secarna Pharmaceuticals is an antisense oligonucleotide (ASO) discovery company that has a diversified pre-clinical in house pipeline based on the locked nucleic acid (LNA) oligonucleotide platform LNAplus™. Our lead programs are within the areas of oncology and inflammatory / fibrotic diseases. We will present our immune-oncology strategy including data showing promising anti-tumor activity of ASOs targeting the multifunctional target neuropilin 1 (NRP1) in mouse tumor models.

Participants

Frank Jaschinski, Ph.D. - Chief Scientific Officer, Secarna Pharmaceuticals

Gate2Brain Shuttles, Going Beyond the Transport of Small Molecules

13:45 - 14:15
Track #2

Gate2Brain is a biotech company focused on the development of therapeutics that efficiently cross biological barriers such as the blood-brain barrier using a radically innovative peptide-based patented technology platform. The potential of Gate2Brain's peptide blood-brain barrier shuttles goes beyond the transport of small molecules but also drug-loaded nanoparticles and even antibodies can be delivered to the brain. These drug delivery systems could be considered a game-changer in the treatment of CNS diseases where there is a drug candidate that needs a better transport.

Participants

Meritxell Teixidó, PhD - CEO and CSO, Gate2Brain

CIVI 008: An Orally Active LNA Drug Against PCSK9

14:15 - 14:45
Track #1

CiVi Biopharma is developing an LNA oligo drug against PCSK9, which in early clinical studies has demonstrated robust, dose-dependent reduction in atherogenic lipoproteins when dosed subcutaneously. To provide a more convenient, patient friendly dosing format the company has developed an oral formulation of the drug, which is scheduled to soon commence clinical trials. The presentation will discuss the oral PCSK 9 program and the broader implication of CiVi Biopharma's oral platform for the delivery of oligonucleotide drugs in general.

Participants

Henrik Oerum, PhD - Founder and Chief Scientific Officer, CiVi Biopharma Inc.

Control Strategy Set-Up for Efficient & Safe Large Scale Peptide Manufacturing Process

14:15 - 14:45
Track #2

Process and analytical development in peptide pharmaceutical industries are the key foundations to build an in-depth process understanding allowing to set the best control strategy of peptide manufacturing processes. Throughout the scale-up manufacturing process, the robustness of the control strategy is consolidated and demonstrated in process validation batches. This presentation illustrates the journey to set an efficient, safe and robust commercial manufacturing process through an in-depth investment in the control strategy design and green chemistry application. Successful case studies will be shared.

Participants

El Djouhar Rekaï, PhD - Head of Process Development & Manufacturing Life cycle Management, PolyPeptide Group

Lymph Node Macrophages Drive Innate Immune Responses to Enhance the Anti-tumor Efficacy of mRNA Vaccines

14:45 - 15:15
Track #1

Herein, we identified a novel lipid nanoparticle (LNP) formulation, L17-F05, for mRNA vaccines by screening 34 ionizable lipids and 28 LNP formulations using human primary APCs*. Subcutaneous delivery of L17-F05 mRNA vaccine encoding Gp100 and Trp2 inhibited tumor growth and prolonged the survival of mice bearing B16F10 melanoma. Loss-of-function studies revealed that L17-F05 works as a self-adjuvant by activating the stimulator of interferon genes (STING) pathway in macrophages. Our findings provide strategies for further optimization of mRNA vaccines based on the innate immune response driven by LN macrophages.

Participants

Kenji Kubara - Scientist, Emerging Modality Generation, Eisai. Co., Ltd.

In-line Monitoring Method Integrating Flow Chemistry for Peptide/Nucleotide Synthesis

14:45 - 15:15
Track #2

Utilization of continuous flow method for peptide/nucleotides not only brings about faster and more efficient coupling/decoupling cycles, but also leads to less waste and less use of solvents/starting materials. However, as conventional method, in some cases, the failure of peptide synthesis reactions (e.g. low yield, generation of unwanted impurities) cannot be detected in a timely manner due to the lack of effective and high-precision in-line monitoring. Herein we present several case studies to demonstrate how different in-line spectroscopic measurements can significantly improve the results as well as describe the superiority and inferiority of these methods.

Participants

Pengyu Xu, Ph.D. - President and Representative Director, SynCrest Inc.

Networking Refreshment Break with Poster and Exhibit Viewing

15:15 - 15:45

Chimeric PN-containing Oligonucleotides Yield Exon Skipping in Preclinical Models and Boys with Duchenne Muscular Dystrophy

15:45 - 16:15
Track #1

Chimeric PN-containing oligonucleotides demonstrate high levels of muscle exposure, exon-skipping, and dystrophin restoration in various preclinical DMD models. Preliminary clinical data provide evidence that WVE-N531, being developed for patients with DMD amenable to exon 53 skipping, is leading to substantial accumulation and exon-skipping in the muscle after three biweekly doses.

Participants

Michael Byrne, Ph.D. - Vice President, In Vivo and CNS Biology, Wave Life Sciences

Flow SPPS – Towards Greener and More Efficient Peptide Manufacturing

15:45 - 16:15
Track #2

Traditional peptide synthesis relies on an orthogonal protecting group strategy, employing excess coupling reagents and generating significant waste. To enhance sustainability, our collaboration with PeptiSystems explores the potential of flow-through column technology. Initial experiments show promising results, reducing amino acid and coupling agent consumption, while maintaining comparable crude purities. In addition, coupling times were decreased, and the PMI significantly improved using high loaded resins without compromising the overall process. This initiative aligns with Corden Pharma's commitment to greener and more efficient manufacturing.

Participants

Eike-Fabian Sachs, PhD - Head of Development Frankfurt, CordenPharma International GmbH

Key Elements of a Well Composed Nonclinical Section of an IND Application

16:15 - 16:45
Track #1

New oligonucleotide therapeutics under investigation offer unique challenges relative to other pharmaceuticals. During preparation and submission of an investigational new drug (IND) application there are a number of regulatory gray areas due to the large heterogeneity in the type of oligonucleotide therapeutics and our knowledge of any particular subclass. Based on lessons learned, this talk will describe selected general considerations to a successful non-clinical IND submission.

Participants

Emily Place, PhD - Senior Consultant, Aclairo Pharmaceutical Development Group

Biocatalysis for Green Manufacturing of Amino Acids

16:15 - 16:45
Track #2

Aralez Bio uses biocatalysis to unlock noncanonical amino acids. Using directed evolution we develop enzymes to synthesize new amino acids needed to drive innovation in drug discovery and manufacturing. Our process is 10x cheaper, 10x faster, and 50x greener than conventional approaches, while simultaneously expanding amino acid chemical space 100x. Noncanonical amino acids are one of the fastest growing areas in peptide development, ushering in an era of therapeutics built on unique, low-cost, green building blocks.

Participants

Wendy Hartsock, PhD - Director of Strategic Partnerships, Aralez Bio

Clinically Effective Scar Treatment via a siRNA Transdermal Gene Silencing Technology: From Bench to Bedside and Beyond

16:45 - 17:15
Track #1

Breaking new ground in scar treatment, Singapore-based RNAscence Biotech unveils Renectin Anti-Scar, a Class A Medical Device registered under Singapore Health Sciences Authority (FDA Class 1 equivalent). Its siRNA transdermal gene silencing technology sets a new standard, achieving 35% better scar reduction compared to silicone patch demonstrated in a clinical study. Professor Timothy Tan will share his journey from ideation to the development of this first-in-class scar treatment product.

Participants

Timothy Tan, PhD - Associate Professor, Nanyang Technological University and Founder, RNAscence Biotech Pte Ltd.

Individualised Cancer Vaccines: Developing a High-throughput cGMP Manufacturing Philosophy

16:45 - 17:15
Track #2

Personalised neoantigen cancer vaccines are a revolutionary technology relying on an array of original molecules tailored to each patient's tumour profile. Creating dozens of unique peptides for each patient, with tight turnaround times, required a paradigm shift to a bespoke manufacturing mindset, facilities, and quality culture. We'll present how Almac successfully led the charge in this challenging field with recent case studies (*Pharmaceutics* **2022**, *14*(7), 151), and discuss the future of this approach in other applications.

Participants

Alaric Desmarchelier, PhD - Business Development Manager - Peptides, Almac Group

Close of Conference

17:15 - 17:20

SCHEDULE

DAY 3 - 21/03/2024

TIME	PLENARY SESSIONS	TRACK #1	TRACK #2	TRACK #2
08:00	**08:10** - Chairperson's Remarks **08:15** - Modulation of Host Immunity in the Airways with Interferon Lambda Encoding mRNA **08:45** - Digital PCR for the Analysis of RNA Quality Parameters **08:00** - Registration and Coffee	**08:00** - Registration and Coffee	**08:00** - Registration and Coffee	**08:00** - Registration and Coffee
09:00	**09:15** - Fine Tuning a PCR based mRNA Manufacturing Platform for each mRNA Sequence **09:45** - Lipid Nanoparticles to Enable Clinical Development of mRNA-based Therapeutics			
10:00	**10:15** - Networking Refreshment Break with Poster and Exhibit Viewing	**10:55** - Chairperson's Remarks **10:15** - Networking Refreshment Break with Poster and Exhibit Viewing	**10:55** - Chairperson's Remarks **10:15** - Networking Refreshment Break with Poster and Exhibit Viewing	**10:15** - Networking Refreshment Break with Poster and Exhibit Viewing
11:00		**11:00** - Preclinical Data for STK-002, an Antisense Oligonucleotide Being Developed for the Treatment of Autosomal Dominant Optic Atrophy (ADOA) **11:30** - Discovery and Delivery of Oligonucleotide Therapeutics	**11:00** - Protein-based Nano-capsules for Delivery of Therapeutic RNAs Across the Blood-Brain-Barrier **11:30** - Redefining Non-viral Delivery for Novel Genomic Medicines with Tissue-targeted Lipid Nanoparticle (ttLNP) Platform	
12:00	**12:30** - Networking Luncheon with Poster and Exhibit Viewing	**12:00** - From Bench to Bedside: Development of a GalXC-Plus siRNA, DCR-STAT3, for Immunotherapy in Refractory Cancer Patients **12:30** - Networking Luncheon with Poster and Exhibit Viewing	**12:00** - Tuning Lipid Nanoparticles for Specific Applications **12:30** - Networking Luncheon with Poster and Exhibit Viewing	**12:30** - Networking Luncheon with Poster and Exhibit Viewing

SCHEDULE

DAY 3 - 21/03/2024

TIME	PLENARY SESSIONS	TRACK #1	TRACK #2	TRACK #2
13:00		**13:40** - Chairperson's Remarks **13:45** - Examples of Antisense Oligonucleotides that Target the Immunosuppressive Tumor Microenvironment for Treatment of Cancer		**13:40** - Chairperson's Remarks **13:45** - Gate2Brain Shuttles, Going Beyond the Transport of Small Molecules
14:00		**14:15** - CIVI 008: An Orally Active LNA Drug Against PCSK9 **14:45** - Lymph Node Macrophages Drive Innate Immune Responses to Enhance the Anti-tumor Efficacy of mRNA Vaccines		**14:15** - Control Strategy Set-Up for Efficient & Safe Large Scale Peptide Manufacturing Process **14:45** - In-line Monitoring Method Integrating Flow Chemistry for Peptide/Nucleotide Synthesis
15:00	**15:15** - Networking Refreshment Break with Poster and Exhibit Viewing	**15:45** - Chimeric PN-containing Oligonucleotides Yield Exon Skipping in Preclinical Models and Boys with Duchenne Muscular Dystrophy **15:15** - Networking Refreshment Break with Poster and Exhibit Viewing	**15:15** - Networking Refreshment Break with Poster and Exhibit Viewing	**15:45** - Flow SPPS – Towards Greener and More Efficient Peptide Manufacturing **15:15** - Networking Refreshment Break with Poster and Exhibit Viewing
16:00		**16:15** - Key Elements of a Well Composed Nonclinical Section of an IND Application **16:45** - Clinically Effective Scar Treatment via a siRNA Transdermal Gene Silencing Technology: From Bench to Bedside and Beyond		**16:15** - Biocatalysis for Green Manufacturing of Amino Acids **16:45** - Individualised Cancer Vaccines: Developing a High-throughput cGMP Manufacturing Philosophy
17:00	**17:15** - Close of Conference	**17:15** - Close of Conference	**17:15** - Close of Conference	**17:15** - Close of Conference