TIDES ASIA
Oligonucleotide & Peptide Therapeutics

19-21 March 2024
Westin Miyako Kyoto
Kyoto, Japan

The Only Event in Asia for Oligo, Peptide, mRNA, Genome Editing & Delivery Experts to Exchange Strategies to Accelerate Molecules to Market

Industry-Leading Experts Share New Science, Applications and Lessons Learned

Developing Next Generation Oligonucleotide Therapeutics

Shalini Andersson, Ph.D.,
Vice President Oligonucleotide Discovery, AstraZeneca, Sweden

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

Jennifer Lockridge, Ph.D.,
VP, Dicerna TRU Early Development, Novo Nordisk

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

Kousaku Ohinata, Ph.D.
Associate Professor, Kyoto University and Founder and Scientific Advisor, Viage Therapeutics

Editing for Atherosclerotic Cardiovascular Disease

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

Strategies for mRNA Analytics

Andreas Czech, Ph.D., Associate Director RNA Analytics, BioNTech SE, Germany

Discovery and Delivery of Oligonucleotide Therapeutics

Jayaprakash Nair, Ph.D.,
Vice President, Research, Chemistry and Delivery Science, Alnylam

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Plus Featured Presentations from:

Ionis Pharmaceuticals, Beam Therapeutics, WAVE, GlyTech, Shionogi, Prime Medicine, Acuitas, Stoke Therapeutics, Merck, Ethris and MANY MORE!

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Apply the Latest Innovations in Oligonucleotide, Peptide and mRNA Development

SCIENCE

Accelerate Your Product To Market

Hear CMC/manufacturing, discovery, delivery and clinical case studies, best practices and lessons learned from leading oligonucleotide, peptide and mRNA therapeutic developers. Ensure product approval by hearing regulatory guidance and roadmaps to successful IND/IMPD submissions from industry leaders.

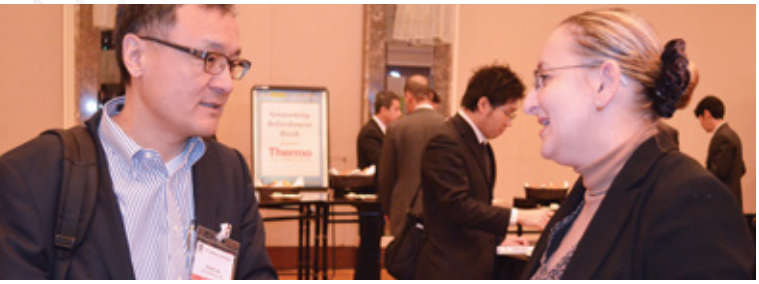
TECHNOLOGY

Evaluate New Technologies And Services

Improve your discovery, clinical, process development, analytical and manufacturing efforts by meeting with 20+ global technology leaders in the exhibit hall. The exhibit hall also features peer-submitted posters that contain new and unpublished research from global scientists working across all phases of oligonucleotide and peptide development.

NETWORKING

Meet Your Next Partner At TIDES ASIA



Connect with 250+ oligonucleotide, peptide and mRNA leaders across Asia, Europe and North America during networking lunches, poster sessions, dinners and cocktail receptions.

Tuesday, March 19, 2024

* OPTIONAL PRE-CONFERENCE WORKSHOPS * 9:00AM-12:45PM

8:00 Registration and Coffee

Workshop 1

Overcoming Manufacturing Challenges and Defining CMC Control Strategies of Synthetic RNA Therapeutics

Workshop Moderator:
Thomas Rupp, Owner and Principal, Thomas Rupp Consulting AG, Germany

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.

Workshop 2

Analytical Characterization and Bioanalysis of Peptides

9:00 Workshop Moderator's Opening Remarks

Bruce Morimoto, Ph.D., Vice President, Drug Development, Alto Neuroscience, USA

9:15 Synthetic Peptide Purity Method Development Challenges – Illustrated Case Studies

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.

Alaric Desmarchelier, Ph.D., Business Development Manager – Peptides, Almac Group, United Kingdom

10:00 Updates on Peptide and Protein Characterization Methodologies

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.

Michael McGinley, Director, Global Applications, Phenomenex, USA

10:45 Networking Refreshment Break

11:15 Solubility and Physical Stability Challenges in Peptide Formulation Development

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.

Juerg F. Tschopp, Ph.D., Principal Scientist, Stratum Medical Corporation

12:00 Bioanalytical Challenges, Best Practices and Case Studies

Speaker TBA

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.

12:45 Close of Workshop and Luncheon for Morning Workshop Attendees

Tuesday, March 19, 2024

* MAIN CONFERENCE PLENARY KEYNOTE SESSION * 2:00PM-5:00PM

1:55 Chairperson's Remarks

2:00 Developing Next Generation Oligonucleotide Therapeutics

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.

Shalini Andersson, Ph.D., Vice President Oligonucleotide Discovery, AstraZeneca, Sweden

2:30 Chemical Engineering of Oligonucleotides: Applications for RNA Therapeutic

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.

Sudhir Agrawal, D. Phil., President and Founder, Arnay Sciences, USA

3:00 Peptide and Protein Therapeutics Conjugated with Human N-Linked Glycans

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.

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

3:30 Networking Refreshment Break

4:00 RNAi Therapeutics in Japan: Needs and Solutions-An Alnylam Perspective

Yutaka Okada, President and Representative Director, Alnylam Japan KK, Japan

4:30 The Endosomal Escape Vehicle Platform of Cyclic Cell-Penetrating Peptides Enhances the Delivery of Oligonucleotides

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.

Leo Qian, Ph.D., Co-Founder and Vice President, Discovery Research, Entrada Therapeutics, USA

5:00 Realizing the Potential of RNA-Targeted Oligonucleotide Therapeutic; Pipeline Progress and Partnerships

Brett Monia, Ph.D., Chief Executive Officer, Ionis Pharmaceuticals

5:30 Close of Day 1

Wednesday, March 20, 2024

MAIN CONFERENCE SESSIONS

Plenary Session

7:45 Registration and Coffee

8:10 Chairperson's Remarks

8:15 Transforming the Care of Cardiovascular Disease Through Single-course Gene Editing Medicines

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

8:45 From Circles to Conjugates: Rethinking the Architecture of guide RNA

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.

Brian Cafferty, Ph.D., Director, Beam Therapeutics, USA

9:15 Improving Oligonucleotide Pharmacology Across Tissues and Modalities through Base, Sugar, and Backbone Modifications

Wave's PRISMTM 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.

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

9:45 Networking Refreshment Break with Poster and Exhibit Viewing

Track 1

10:15 Chairperson's Remarks

10:20 Bivalent Nucleic Acid Recognition and Its Applications in Targeting RNA Triplet-Repeats

This presentation highlights our ongoing efforts to develop a novel set of bivalent (Janus-type) nucleic acid recognition codes for targeting RNAs, primarily focusing on CUG-RNA repeats associated with Myotonic Dystrophy type 1 (DM1). The underlying molecular platform is quite versatile, as it can be tailored to bind with various RNA repeats in a highly specific and selective manner. The newly crafted "ligands," named as such due to their compact size—comprising only three units in length—bear a closer resemblance to small molecules than they are to traditional oligonucleotides. However, unlike the latter, they engage their targets in a sequence-specific and strand-selective fashion through bifacial H-bonding with the adjacent nucleobases in both strands of the RNA double helix. This work substantiates the proof-of-concept that such diminutive nucleic acid "ligands" can indeed be developed for the recognition of CUGexp-RNA transcripts, and potentially other members within this relatively large class of neuromuscular and neurodegenerative disorders.

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

Track 2

10:15 Chairperson's Remarks

10:20 Synthesizing Long Guide RNAs for In Vivo Prime Editing

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.

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

Wednesday, March 20, 2024

MAIN CONFERENCE SESSIONS

Track 1

10:50 **Amide-Modified Oligonucleotides for Chemical Control of Functional RNAs**

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.

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

11:20 **Chemical Approaches to Enhance siRNA Selectivity**

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.

Mehran Nikan, Ph.D., Research Fellow, Ionis Pharmaceuticals, USA

Track 2

10:50 **Addressing the Regulatory Expectations for Guide RNA Quality While Providing Manufacturing Capacity**

Blake Unterreiner, Associate Vice President, Business Development and Customer Relations, Agilent Technologies, USA

11:20 **Delivery of Genetic Medicine with Hydrophilic Nanoparticles**

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.

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

11:50 Networking Luncheon with Poster and Exhibit Viewing

Track 1

12:55 **Chairperson's Remarks**

1:00 **Comprehensive Analysis of Brain Distribution for Antisense Oligonucleotides Using Whole Tissue Imaging Technique**

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.

Syunsuke Yamamoto, Associate Director, Center of Excellence for Drug Metabolism, Pharmacokinetics and Modeling, Preclinical and Translational Sciences Research, Takeda Pharmaceutical Company, Japan

Track 2

12:55 **Chairperson's Remarks**

1:00 **Acceleration of Process Development for GMP Production of Peptides**

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

Dewey Sutton, Ph.D., Research and Development Supervisor, AmbioPharm, USA

1:30 **Recent Technological Innovation in Peptide Manufacturing**

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.

Yoshitaka Nemoto, General Manger R&D, PeptiStar, Japan

Wednesday, March 20, 2024

MAIN CONFERENCE SESSIONS

Track 1

1:30 **Synthetic Strategy for Ligand Conjugated Oligonucleotides via Diamine Modified Stable Phosphoroamidite**

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.

Mitsuaki Sekiguchi, Ph.D., Principal Scientist, Biopharmaceutical Research Division, Shionogi, & Co., Ltd., Japan

2:00 **Nucleic Acid Therapeutics: Process Optimization and Purification**

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.

Lulu Zhang, Ph.D., Field Application Scientist, Thermo Fisher Scientific

2:30 **Overcoming Oligonucleotide Manufacturing Challenges**

Loïc Cornelissen, Ph.D., Sales Manager, PolyPeptide Group, Belgium

Track 2

2:00 **De Novo Discovery of Natural Product-like Thiopeptides with Designed Biological Activities**

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.

Alexander Vinogradov, Ph.D., Project Assistant Professor, Department of Chemistry, The University of Tokyo, Japan

2:30 **Discovery of Macrocycles for Delivery of RNA and Targeted Radiopharmaceuticals**

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.

Ratmir Derda, Ph.D., Founder and Chief Scientific Officer, 48Hour Discovery, Canada

3:00 Networking Refreshment Break with Poster and Exhibit Viewing

Track 1

3:30 **Going Large-scale with Manufacturing of Oligonucleotides**

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.

Daniel Samson, Ph.D., Vice President, Head Oligonucleotides, Bachem AG, Switzerland

Track 2

3:30 **Macrocyclic Peptide Drug Development by Combining the Strengths of all Small Fragments, mRNA Display and AI Technologies**

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.

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

Wednesday, March 20, 2024

MAIN CONFERENCE SESSIONS

Track 1

4:00 Synthesis PAT: IR Spectroscopy for Real-Time Phosphoramidite Identification

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.

Spenser Pruett, Scientist II, Process Development, Nitto Avecia

4:30 Addressing Complex Oligonucleotide Therapeutics and Approach Towards Endotoxin Removal

Juergen Mueller, Ph.D., Vice President of Commercial Operations, LGC Axolabs, Germany

5:00 New Ligation Approach: Technology for High Quality Manufacturing of Over 150 mer RNA

Masato Sanosaka, Ph.D., Group Leader of Research & Process Development, Ajinomoto Biopharma Services

Track 2

4:00 From Discovery to the Clinic: Development of a Novel Bioactive Peptide that Improves Mood and Cognition by Engaging the Gut-Brain Axis

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.

Kousaku Ohinata, Ph.D. Associate Professor, Kyoto University and Founder and Scientific Advisor, Viage Therapeutics, Japan

4:30 A Purely Thermodynamic Anti-prionic Mode of Action for Protein-misfolding Diseases is Realized by All-D-peptides

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.

Dieter Willbold, Ph.D., Director and Full Professor, Forschungszentrum Jülich and Co-Founder, Priavoid GmbH, Germany

Wednesday, March 20, 2024

MAIN CONFERENCE SESSIONS

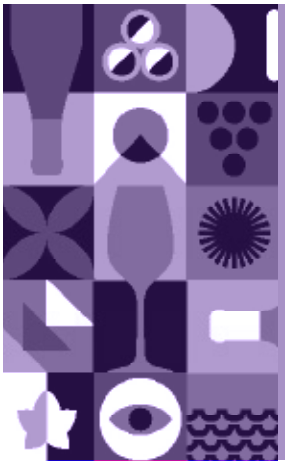
Track 1

Track 2

5:00 **In Cellulo Library-derived Peptide-based Inhibitors of Alpha-synuclein Aggregation and Toxicity**

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.

Jody Mason, Ph.D., Professor of Biochemistry, University of Bath, United Kingdom



5:30 Networking Cocktail Reception with Poster and Exhibit Viewing

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

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6:30 Close of Day 2

Thursday, March 21, 2024

MAIN CONFERENCE SESSIONS

Plenary Session

8:00 Registration and Coffee

8:10 Chairperson's Remarks

8:15 Modulation of Host Immunity in the Airways with Interferon Lambda Encoding mRNA

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.

Christian Plank, Ph.D., Chief Technology Officer, Ethris GmbH, Germany

8:45 Strategies for mRNA Analytics

Andreas Czech, Ph.D., Associate Director RNA Analytics, BioNTech SE, Germany

9:15 Fine Tuning a PCR based mRNA Manufacturing Platform for each mRNA Sequence

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.

Aditi Mehta, Ph.D., Associate Director, Head of mRNA Process & Delivery, Merck KGaA, Germany

9:45 Lipid Nanoparticles to Enable Clinical Development of mRNA-based Therapeutics

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.

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

10:15 Networking Refreshment Break with Poster and Exhibit Viewing

Track 1

10:55 Chairperson's Remarks

Track 2

10:55 Chairperson's Remarks

11:00 Protein-based Nano-capsules for Delivery of Therapeutic RNAs Across the Blood-Brain-Barrier

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.

Ekkehard Leberer, Ph.D., Scientific Advisor, Neuway Pharma, Germany

Thursday, March 21, 2024

MAIN CONFERENCE SESSIONS

Track 1

11:00 **Preclinical Data for STK-002, an Antisense Oligonucleotide Being Developed for the Treatment of Autosomal Dominant Optic Atrophy (ADOA)**

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.

Shobha Ravipaty, Ph.D., Director, Stoke Therapeutics, USA

11:30 **Discovery and Delivery of Oligonucleotide Therapeutics**

Jayaprakash Nair, Ph.D., Vice President, Research, Chemistry and Delivery Science, Alnylam, USA

12:00 **From Bench to Bedside: Development of a GalXC-Plus siRNA, DCR-STAT3, for Immunotherapy in Refractory Cancer Patients**

Jennifer Lockridge, Ph.D., VP, Dicerna TRU Early Development, Novo Nordisk, USA

Track 2

11:30 **Redefining Non-viral Delivery for Novel Genomic Medicines with Tissue-targeted Lipid Nanoparticle (ttLNP) Platform**

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.

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

12:00 **Tuning Lipid Nanoparticles for Specific Applications**

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.

Ed Yaworski, Chief Technology Officer, Genevant Sciences Corp, Canada

12:30 **Networking Luncheon with Poster and Exhibit Viewing**

Track 1

1:40 **Chairperson's Remarks**

1:45 **Examples of Antisense Oligonucleotides that Target the Immunosuppressive Tumor Microenvironment for Treatment of Cancer**

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.

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

Track 2

1:40 **Chairperson's Remarks**

1:45 **Gate2Brain Shuttles, Going Beyond the Transport of Small Molecules**

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.

Meritxell Teixidó, Ph.D., CEO and CSO, Gate2Brain, Spain

Thursday, March 21, 2024

MAIN CONFERENCE SESSIONS

Track 1

2:15 **CIVI 008: An Orally Active LNA Drug Against PCSK9**

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.

Henrik Oerum, Ph.D., Founder and Chief Scientific Officer, CiVi Biopharma Inc., Denmark

2:45 **Development of TTX-MC138, a First-in-Class miRNA-10b-Targeted Therapeutic Against Metastatic Cancers of Multiple Primary Disease Origins**

We have previously developed a therapeutic miRNA-10b inhibitor, named TTX-MC138, which can elicit life-long disease remissions in preclinical models of adenocarcinoma. To de-risk further clinical development of TTX-MC138, we have embarked on a Phase 0 clinical trial with Cu64-labeled TTX-MC138. The trial involves microdose injection of the agent into stage IV cancer patients, followed by positron emission tomography-magnetic resonance imaging (PET-MRI) to determine its pharmacokinetics and uptake in clinical metastases.

Zdravka Medarova, Ph.D., Founder and Chief Technology Officer, TransCode Therapeutics, USA

Track 2

2:15 **Control Strategy Set-Up for Efficient & Safe Large Scale Peptide Manufacturing Process**

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.

El Djouhar Rekai, Ph.D., Head of Business Unit Peptide Process Development & Manufacturing, PolyPeptide Group, Belgium

2:45 **In-line Monitoring Method Integrating Flow Chemistry for Peptide/Nucleotide Synthesis**

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.

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

3:15 Networking Refreshment Break with Poster and Exhibit Viewing

Track 1

3:45 **Chimeric PN-containing Oligonucleotides Yield Exon Skipping in Preclinical Models and Boys with Duchenne Muscular Dystrophy**

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.

Elizabeth Wagner, Ph.D., Director, Biology, Wave Life Sciences, USA

Track 2

3:45 **Flow SPPS – Towards Greener and More Efficient Peptide Manufacturing**

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.

Eike-Fabian Sachs, Ph.D., Head of Development Frankfurt, CordenPharma International GmbH, Germany

Thursday, March 21, 2024

MAIN CONFERENCE SESSIONS

Track 1

4:15 **Key Elements of a Well Composed Nonclinical Section of an IND Application**

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.

Emily Place, Ph.D., Senior Consultant Nonclinical, Biologics Consulting, USA

4:45 **GalAhead™: A Proprietary GalNAc-RNAi Therapeutic Platform to Downregulate Single and Multiple Genes**

Sirnaomics has developed the proprietary GalNAc-RNAi therapeutic delivery platform, GalAhead™, comprising two key technological components – mxRNA™ (miniaturized RNAi triggers) and muRNA™ (multi-unit RNAi triggers). mxRNAs are composed of single ~30nt oligonucleotides to downregulate individual genes, while muRNAs are comprised of 2 or more oligonucleotides to simultaneously silence two or more targets. We will present data validating these technologies, as well as developments with quickly expanding and progressing GalAhead™ therapeutic pipeline.

Jim Weterings, Ph.D. Senior Director, Head of Technology Development, Sirnaomics, USA

Track 2

4:15 **Biocatalysis for Green Manufacturing of Amino Acids**

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.

Christina Boville, Ph.D., Co-founder and CEO, Aralez Bio, USA

4:45 **Individualised Cancer Vaccines: Developing a High-throughput cGMP Manufacturing Philosophy**

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.

Alaric Desmarchelier, Ph.D., Business Development Manager – Peptides, Almac Group, United Kingdom

5:15 Close of Conference

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For more information on how you can connect with key buyers at TIDES 2024, contact:

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