

**TIDES ASIA**  
**Oligonucleotide &  
Peptide Therapeutics**

26-28 FEBRUARY, 2025  
KYOTO, JAPAN  
Westin Miyako Kyoto

# Accelerate Molecules to Market

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

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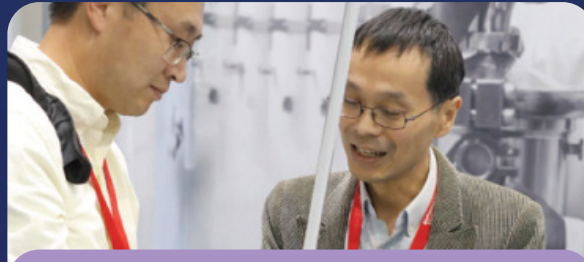
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# Industry-Leading Experts Share New Science, Applications and Lessons Learned



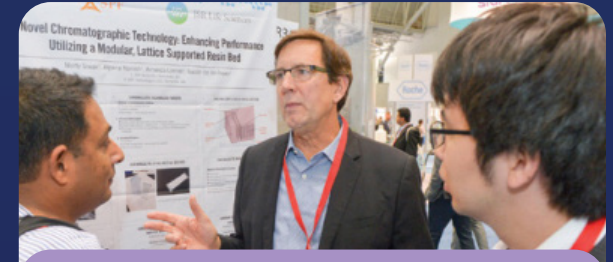
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## Industry-Leading Keynotes & Featured Speakers



### RaPID Display and Selection of Pseudo-natural Products

Hiroaki Suga, Ph.D., Professor of Chemistry, School of Science, University of Tokyo, Japan



### Biological Research with Thiomorpholino Oligonucleotides (TMOs)

Marvin Caruthers, Ph.D., Distinguished Professor, Biochemistry, University of Colorado, USA



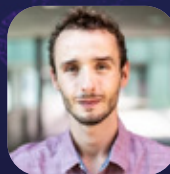
### RNA Therapeutics Modalities and Targeted Delivery Approaches for Developing Transformational Medicines for Patients

Tamar Grossman, Ph.D., VP, Global Head of RNA, Gene Therapy and Delivery, Johnson & Johnson Innovative Medicine, USA



### Cell Penetrating Properties of Cyclotides and Their Applications for Intracellular Targets

David Craik, Ph.D., Professor of Biomolecular Structure, Institute for Molecular Bioscience, University of Queensland, Australia



### State of the Art siRNA and Future Perspectives





Tomaz Einfalt, Ph.D., Principal Investigator II xRNA, Novartis Institutes for BioMedical Research, Switzerland



### Therapeutic Genome Editing with ePscas9 Using Lipid Nanoparticles

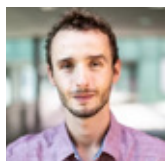
Grzegorz Sienski, Ph.D., Director and Project Leader, AstraZeneca, Sweden

8:00	Registration and Coffee	
	<b>Optional Pre-Conference Morning Workshops   09:00-12:30</b>	<b>Optional Pre-Conference Morning Workshops   09:00-12:30</b>
9:00	<p><b>Workshop #1: Oligonucleotide Manufacturing Insights At Large Scale</b></p> <p><b>9:00 Workshop Moderator's Opening Remarks</b> The workshop will provide insight into decisions made to enable a multi-product facility that can accept many oligonucleotide types/designs and scales, all the while ensuring proper alignment of large scale and downscale processes. Critical Quality Attributes are suspected to influence efficacy, safety, and quality of final products, therefore consideration of CQAs for large scale manufacturing will be discussed. Participants will gain an insight into Large Scale manufacturing of oligonucleotide API and to the design of a facility. Thomas Rupp, Managing Director, Axolabs Berlin GmbH, Germany</p> <p><b>9:10 Large Scale Manufacturing: Challenges and Solutions</b> Chris Oswald, Owner &amp; Principal, Coswald Consulting, USA</p> <p><b>9:55 Designing a Facility: Challenges and Solutions</b> Thomas Rupp, Managing Director, Axolabs Berlin GmbH, Germany</p> <p><b>10:40 Networking Refreshment Break</b></p> <p><b>11:15 Critical Quality Attributes (CQA): How to Maintain During Upscaling</b> Hagen Cramer, Ph.D., Chief Technology Officer, QurAlis Corporation, USA</p> <p><b>12:00 Panel Discussion and Q&amp;A with Workshop Speakers</b></p> <p><b>Who Should Attend?</b> Anyone interested in understanding the challenges that might exist if oligonucleotide manufacturing were to be build-up in-house rather than depending on a CDMO. Manufacturing personnel, quality assurance (QA), and project management (PM) would realize the most benefit out of this workshop.</p>	<p><b>Workshop #2: Innovations and Challenges in Peptide Manufacturing</b></p> <p><b>9:00 Workshop Co-Moderators' Opening Remarks</b> Bruce Morimoto, Ph.D., Vice President, Drug Development, Alto Neuroscience, USA Robert Hagopian, Director Business Development, PolyPeptide Group, USA</p> <p><b>9:10 Challenges of Raw Material Acquisition for Peptide Synthesis</b> The increased complexity of peptides leads to the usage of new building blocks and raw materials in manufacturing of peptides for clinical application. This leads to unanticipated challenges in qualification of the vendors as well as qualification of the raw materials used in the production process. Examples will be presented including potential solutions that would help overcome these challenges. Robert Hagopian, Director Business Development, PolyPeptide Group, USA</p> <p><b>9:50 Sustainability Challenge in Cyclic Peptide Synthesis: The Power of Crystallization and CSPS</b> Current peptide synthesis relies on chromatography and excess amino acids in solid-phase peptide synthesis (SPPS). For large-scale cyclic peptide production, crystallization's role in purifying and isolating active pharmaceutical ingredient, and the transition from SPPS to classical solution-phase peptide synthesis (CSPS) will be examined as a case study. These approaches aim to improve sustainability and process mass intensity in peptide manufacturing. Hiroshi Iwamura, Ph.D., Deputy Head of API Process Development Department (Chemical Process), Chugai Pharmaceutical Co., Ltd., Japan</p> <p><b>10:35 Networking Refreshment Break</b></p> <p><b>11:10 Crystallisation as a Purification and IP-extension) Strategy in Peptide Manufacturing</b> Alaric Desmarchelier, Ph.D., Business Development Manager – Peptides, Almac Group, United Kingdom</p> <p><b>11:50 Innovations in HPLC for Peptide Purification</b> Yvan Ruland, Ph.D., Technology Consultant Chromatography, Sartorius Stedim Singapore</p>
12:30	Close of Workshop and Luncheon for Morning Workshop Attendees Only	

Main Conference Plenary Keynote Session   1:45pm–5:30pm	
1:45	<p><b>Chairperson's Remarks</b> Yogesh Sanghvi, Ph.D., President, Rasayan, Inc., USA</p>
1:50	 <p><b>RaPID Display and Selection of Pseudo-natural Products</b> This lecture discusses a new development of display format for the discovery of pseudo-natural products against intracellular targets of protein. The technology enables us to access unexplored chemical space with cell membrane permeability of macrocycles. Hiroaki Suga, Ph.D., Professor of Chemistry, School of Science, University of Tokyo, Japan</p>
2:20	 <p><b>Biological Research with Thiomorpholino Oligonucleotides (TMOs)</b> Several biological studies have focused on using (TMOs) for studying genetic diseases. Targeted mouse studies have focused on DMD, NPC, DIPG, and Type II Diabetes with results superior to other chemistries. In various cell culture experiments using exon skipping or RNase H, we have shown significant biological activity targeting genes such as FUS, SLC6A1, ITGA4, PKM, STAT3, and others. Recently we have shown that TMOs are quite active as siRNAs and in CRISPR/CAS experiments. Marvin Caruthers, Ph.D., Distinguished Professor, Biochemistry, University of Colorado, USA</p>
2:50	 <p><b>Key Points in Oligonucleotide Formulation Development, AI/ML-Driven LNP Development, and Scale-Up: General Overview and Case Studies</b> This presentation will highlight key considerations (viscosity, etc.) in the formulation development of oligonucleotide therapeutics and the application of AI(Artificial Intelligence)/ML(Machine Learning) in lipid nanoparticle (LNP) development. It will also cover the scale-up of LNP drug product development. A general overview and case studies will be presented to illustrate these aspects. Tomomi Sato, Associate Director, Takeda Pharmaceutical Company Limited, Japan</p>
3:20	<p><b>Networking Refreshment Break</b></p>
4:00	 <p><b>Cell Penetrating Properties of Cyclotides and Their Applications for Intracellular Targets</b> Cyclotides are head-to-tail cyclic peptides of ~30 amino acids that incorporate three disulfide bonds arranged in a cystine knot topology, which makes them exceptionally stable. Their stability and compact structure makes cyclotides an attractive protein framework onto which bioactive peptide epitopes can be grafted to stabilize them. Some cyclotides are able to penetrate membranes and have the potential for oral administration and/or the ability to reach intracellular targets. This presentation will give an overview of the discovery and applications of cyclotides as drug leads for cancer, multiple sclerosis and pain as well as describing biophysical studies on their cell penetrating properties. David Craik, Ph.D., Professor of Biomolecular Structure, Institute for Molecular Bioscience, University of Queensland, Australia</p>
4:30	

## Main Conference Plenary Keynote Session | 1:45pm–5:30pm

4:30



### State of the Art siRNA and Future Perspectives

This presentation covers the pivotal role of advancements in chemical design variants and delivery systems in enhancing the state of the art of siRNA. It highlights these improvements' impact on siRNA's stability, specificity and efficacy, and explores how these breakthroughs could make siRNA a game-changer in the treatment of various diseases.

Tomaz Einfalt, Ph.D., Principal Investigator II xRNA, Novartis Institutes for BioMedical Research, Switzerland

5:00



### Therapeutic Genome Editing with ePsCas9 Using Lipid Nanoparticles


CRISPR-Cas technologies promise therapeutic genome editing, but only a few Cas enzymes show sufficient in vivo activity. Previously, we introduced *Parasutterella secunda* Cas9 (PsCas9), a Type II-B enzyme, as a high-fidelity editor (Degtev et al Nat Comms 2023). PsCas9 delivered via adenovirus induced gene editing comparable to *Streptococcus pyogenes* Cas9 (SpCas9), with minimal off-target effects and reduced chromosomal translocations. In this study, we investigated PsCas9 through cellular, structural, and biochemical analyses, optimizing it for therapeutic applications. We tested the engineered PsCas9 (ePsCas9) using lipid nanoparticle (LNP) delivery to disrupt the *Pcsk9* gene in the mouse liver, relevant for hypercholesterolemia treatment. ePsCas9 achieved high editing levels in the liver, reducing *Pcsk9* levels in blood plasma. Our latest in vivo editing data will be presented. This study presents ePsCas9 as an effective, precise genome editor and outlines a strategy for engineering other Cas9 orthologs. ePsCas9's enhanced efficiency, intrinsic fidelity, and compatibility with LNP delivery to expand the CRISPR toolkit for therapeutic genome editing.

Grzegorz Sienski, Ph.D., Director and Project Leader, AstraZeneca, Sweden

5:30

Close of Day 1

Main Conference Sessions	
7:45	Registration and Coffee
8:10	<p><b>Chairperson's Remarks</b></p> <p>Yogesh Sanghvi, Ph.D., President, Rasayan, Inc., USA</p>
8:15	<p><b>Morning Spotlight Presentation</b></p> <p><b>Isolation and Drying of Peptides and Oligonucleotides - Improved Production Capacities During Large Scale Manufacturing</b></p> <p>Manufacturing of TIDES has many bottlenecks and one of them is isolation and drying as currently used technologies pose many inherent challenges. Fluid Air's innovative technology, Electrostatic Drying (ESD), PolarDry, can improve the production capacity of oligonucleotides and peptides without sacrificing yield in large-scale production. ESD is a scalable, continuous process that uses electrostatic charge, for efficient drying producing dry powders that do not require post-processing. Impurity formation and activity loss is avoided with the additional benefit of particle engineering using high or low solids solutions and varying API loading. In this presentation, case studies on processing times, yield, solvent removal, and phase transitions will be presented.</p> <p>Keith Cronce, Chief Operating Officer, Fluid Air, A Division of Spraying Systems, USA</p>
mRNA Advances Track	
8:45	<p><b>Complete Chemical Synthesis of mRNA for Cancer Vaccine</b></p> <p>mRNA synthesis using transcription reactions with RNA polymerase makes it difficult to introduce site-specific chemical modifications. The use of site-specific chemical modifications can potentially enable an understanding of medicinal chemistry through structure-activity relationships. Therefore, we developed a complete chemical synthesis method for mRNA.</p> <p>Hiroshi Abe, Ph.D., Professor, Department of Chemistry, Graduate School, Nagoya University, Japan</p>


mRNA Advances Track		
9:15	<p><b>Developing the Next Generation RSV Vaccine</b></p> <p>Respiratory syncytial virus (RSV) is a significant cause of respiratory illness, particularly in infants, elderly adults, and immunocompromised individuals. Despite decades of research, an effective vaccine against RSV has remained elusive until recent advancements in mRNA technology. This presentation discusses the challenges in developing a next-generation RSV vaccine, focusing on viral variability, immunological immaturity in infants, and the potential risks of vaccine-enhanced disease. Promising candidates currently under investigation include mRNA vaccines developed by RNAimmune will be discussed, including the implications of receiving Investigational New Drug (IND) approvals from both the U.S. Food and Drug Administration (FDA) and China's Center for Drug Evaluation (CDE).</p> <p><b>Dong Shen, M.D., Ph.D., CEO, RNAimmune, USA</b></p>	
9:45	<p><b>mRNA Therapy for Asthma</b></p> <p>Based on its proprietary drug design and delivery platforms Ethris is developing ETH47, an mRNA-based drug product encoding type III interferon for local delivery to the respiratory tract. ETH47 is designed to restore innate immune dysfunction in patients with asthma with the goal to contribute to clinical remission by reducing asthma exacerbations. Results from the first-in human trial suggest excellent safety and tolerability of nasally delivered ETH47 along with desired target engagement.</p> <p><b>Thomas Langenickel, M.D., Ph.D., Chief Medical Officer, Ethris GmbH, Germany</b></p>	
10:15	<p><b>Networking Refreshment Break with Poster and Exhibit Viewing</b></p>	
10:20	<p><b>TIDES Talks: Exhibit Hall Stage Presentations</b></p> <p><b>Initiating CMO Operations at Axolabs Berlin: From Discovery to Commercial Therapeutic Oligonucleotides</b></p> <p>Thomas Rupp, Managing Director, Axolabs Berlin GmbH, Germany</p> <div style="text-align: right;">  </div>	
Oligonucleotide Track	Peptide Track	
10:55	<p><b>Chairperson's Remarks</b></p> <p>Sridhar Vaddeboina, Ph.D., Senior Vice President- CMC, Wave Life Sciences</p>	<p><b>Chairperson's Remarks</b></p> <p>El Djouhar Rekai, Ph.D., Head of Business Unit Peptide Process Development &amp; Manufacturing, PolyPeptide Group, Belgium</p>

	Oligonucleotide Track	Peptide Track
11:00	<p><b>CMC Strategies for the Characterization of Stereopure Platform Chemistry</b></p> <p>Traditional phosphorothioate (PS) oligonucleotide synthesis methods generate stereorandom mixtures comprising up to several hundred thousand molecules, each with distinct stereochemistry. Wave Life Sciences is pioneering the development and manufacture of oligonucleotides with control over the backbone chirality. We will outline the various analytical tools we have deployed to characterize the platform processes that generate stereopure oligonucleotides, and determine the structural composition, identity, and stability of stereopure oligonucleotides.</p> <p>Sridhar Vaddeboina, Ph.D., Senior Vice President- CMC, Wave Life Sciences, USA</p>	<p><b>Industry Perspective on the Selection of Regulatory Starting Materials for Synthetic Peptides</b></p> <p>The IQ Synthetic Peptide Working Group suggests a systematic strategy to define regulatory starting materials (RSMs) for peptide manufacture, considering larger peptide fragments. This allows flexibility, supports new synthesis technologies, and enhances manufacturing robustness and sustainability, aligning with ICH Q11 guidelines.</p> <p>John Lopez, Ph.D., Associate Director Science &amp; Technology, Novartis, Switzerland</p>
11:30	<p><b>Template-independent Enzymatic Synthesis of Natural and Fully Modified siRNA Oligonucleotides</b></p> <p>We have developed an automated, template-independent enzymatic RNA synthesis platform. Synthesis of RNA is made possible by the controlled incorporation of 3'-O blocked nucleotides using a poly(U) polymerase variant. We demonstrate high coupling efficiencies, which enable the synthesis of siRNA-length oligonucleotides. Both natural and fully modified oligos have been synthesized with our platform, including the synthesis of the antisense strand of LEQVIO™, an siRNA drug with a large patient population.</p> <p>Jonathan Rittichier, Ph.D., Chief Scientific Officer &amp; Co-Founder, EnPlusOne BioSciences, USA</p>	<p><b>Impurities in Peptide Drug Substance and Challenges Faced during Registration (in US, EU, Brazil and China Markets) and Commercial Phase</b></p> <p>Controlling impurities in complex drug substances is a crucial part of development and due to lack of harmonized approaches from Health authorities, the manufacturer faces many challenges in development, registration and even after commercialization. This presentation will focus on the challenges and discussion on possible ways forward. The presentation will include a discussion of the current regulatory scenario and expectations on peptide impurity control strategy and a reflection on different regional guidelines. It will describe the challenges faced in impurity control of longer chain peptides, different analytical and orthogonal tools to be employed for impurity investigation and possible ways forward. An advanced case study describing the complex situation (of registration and commercial phase) and managing regulatory compliance, supply reliability and operational continuity will be presented.</p> <p>Dinesh Parmar, Associate Director Regulatory Affairs, PolyPeptide Group, India</p>
12:00	<p><b>Empowering Oligonucleotide Therapeutics: From Raw Materials to Manufacturing, and Advanced Delivery Solutions</b></p> <p>Sirat Sikka, Senior Scientist, Applications &amp; Innovation, Thermo Fisher Scientific, USA</p>	<p><b>Statistical Design of Experiments in Peptide Process Development</b></p> <p>Alaric Desmarchelier, Ph.D., Business Development Manager – Peptides, Almac Group, United Kingdom</p>
12:30	<p><b>Networking Luncheon with Poster and Exhibit Viewing</b></p>	



	Oligonucleotide Track	Peptide Track
1:40	<p><b>Chairperson's Remarks</b></p> <p>Sridhar Vaddeboina, Ph.D., Senior Vice President- CMC, Wave Life Sciences</p>	<p><b>Chairperson's Remarks</b></p> <p>El Djouhar Rekai, Ph.D., Head of Business Unit Peptide Process Development &amp; Manufacturing, PolyPeptide Group, Belgium</p>
1:45	<p><b>Drug Substance and Drug Product CMC Strategies for Start-Ups and Smaller Companies</b></p> <p>Discussion of strategies for smaller companies related to process development and scaleup of the manufacturing process, establishment of analytical tests/controls and specifications, and control strategies for starting materials, process, and analytics will be provided. Options will be presented where fastest timeline, lowest cost, or risk reduction were the focus – allowing smaller companies to observe examples that may match their CMC program goals. The discussion will include example case studies for drug substance and drug product for differing oligonucleotide modalities.</p> <p>Chris Oswald, Owner &amp; Principal, Coswald Consulting, USA</p>	<p><b>Development of an Efficient and Scalable CSPS Process for a Mid-Sized Cyclic Peptide</b></p> <p>Cyclic peptides like LUNA18 demonstrate potential for oral absorption and intracellular targeting. In 2023, Chugai reported studies leading to LUNA18's discovery, now undergoing clinical trials. To manufacture LUNA18 API at large scale, liquid-phase peptide synthesis (LPPS) was developed over solid-phase synthesis. Challenges arose from unnatural amino acids causing low reactivity and side reactions and novel LPPS process technologies overcame these issues.</p> <p>Hiroshi Iwamura, Ph.D., Deputy Head of API Process Development Department (Chemical Process), Chugai Pharmaceutical Co., Ltd., Japan</p>
2:15	<p><b>NMA Manufacturing Process Development</b></p> <p>The presentation is an overview of process development work done to manufacture 2'-O-(N-methylacetamide) (NMA) oligonucleotides. This includes the methods used to overcome challenges faced during solid-phase synthesis, intermediate storage, and purification. It will then give an example of how the aforementioned process development work translated into a large-scale GMP manufacturing campaign. Finally, it will explore alternative routes that can further improve the NMA manufacturing process.</p> <p>Carter Cooperman, Research Associate, Ionis</p>	<p><b>Overcoming Aggregation Challenges in Peptide Manufacturing: Technical Approach and Case Studies</b></p> <p>Peptide-based therapeutics have emerged as a significant class of drugs due to their specificity and efficacy. However, the synthesis of peptide presents unique challenges, particularly with respect to aggregation which can occur at various stages of production and compromise product quality, yield and bioactivity. This presentation explores the critical issue of aggregation in peptide synthesis focusing on the influence of process parameters and methods to mitigate. Case studies on complex peptide sequences prone to aggregation will be shared.</p> <p>El Djouhar Rekai, Ph.D., Head of Business Unit Peptide Process Development &amp; Manufacturing, PolyPeptide Group, Belgium</p>

	Oligonucleotide Track	Peptide Track
2:45	<p><b>Advancements in Oligonucleotide Sequencing Capabilities</b></p> <p>Nitto Avecia is a leader in oligonucleotide contract manufacturing services. Our capabilities span the entire development process—from pre-clinical and commercial process development to analytical development, manufacturing, and quality control. The presentation will give an overview of the analytical platforms the company uses for sequencing oligonucleotides. The methods discussed will include liquid chromatography coupled with mass spectrometry (LC-MS) based and non-mass spectrometry-based sequencing approaches. There will be an overview of mass LC-MS sequencing of intact oligonucleotides using MS2 and pseudo MS3, as well as enzymatically digested samples. The enzymes discussed will include exonucleases and endonucleases including restriction enzymes. Lastly the implementation of Sanger Sequencing platforms as a non-mass spectrometry-based approach will be presented. The goal of the presentation will be to illustrate how Nitto Avecia has been able to adapt these approaches to meet the needs of our customers.</p> <p><b>Collin Wetzel, Ph.D., Associate Principal Scientist, Analytical Development, Nitto Avecia, USA</b></p>	<p><b>An Inline Monitoring Method Integrated Flow Chemistry for Peptide Synthesis and Manufacturing</b></p> <p>In-process monitoring is extremely important in today's peptide and nucleic acid synthesis, and the development of this technology is directly related to the improved quality of peptides and nucleic acids. In this presentation, we will demonstrate our recent development of in-line monitoring as well as the integration of flow chemistry for the better synthesis/manufacturing of TIDES.</p> <p><b>Pengyu Xu, Ph.D., President and Representative Director, SynCrest Inc., Japan</b></p>
3:15	<p><b>Networking Refreshment Break with Poster and Exhibit Viewing</b></p>	
3:45	<p><b>Development of a Kilogram-Scale Manufacturing Route for Guadecitabine: A Dinucleotide DNA Methyltransferase Inhibitor</b></p> <p>Guadecitabine (SGI-110) is a dinucleotide that is a prodrug of decitabine. This presentation will describe a kilo-scale GMP production of clinical grade API. SGI-110 is of interest as hypomethylating agent (DNA methylation inhibitor), replacing decitabine, to be used in combination treatment regimens in patients with myeloid malignancies.</p> <p><b>Yogesh Sanghvi, Ph.D., President, Rasayan, Inc., USA</b></p>	<p><b>Improving Robustness and Recovery in a GLP-1 Purification Process</b></p> <p>GLP-1 receptor agonists (RA's) have shown potential to treat diabetes and obesity, marking a significant breakthrough in peptide-based therapies. In this presentation, we will explore strategies to master GLP-1 RA's production, along with chromatography and membrane based proven technologies tailored to optimize their recovery yield and to address potential robustness challenges.</p> <p><b>Yvan Ruland, Ph.D., Technology Consultant Chromatography, Sartorius Stedim Singapore</b></p>
4:15	<p><b>Pioneering the Development of Highly Stable Site-Specific Antibody-Oligonucleotide Conjugates (AOC) Using Fc-Targeting Reagents</b></p> <p>Conventional AOC development struggled with uncontrollable modification sites and numbers, causing material loss and complex purification. Moreover, even after purification, non-site-selectively modified AOCs have varied stability and efficacy. We developed a reagent targeting the Fc region of antibodies, enabling site-selective introduction of reactive groups with over 90% yield. This method provides AOCs in three steps, requiring column purification only in the final step. Early studies indicate our AOCs exhibit higher stability compared to conventional ones.</p> <p><b>Tsubasa Aoki, Ph.D., Researcher, Ajinomoto Co., Japan</b></p>	<p><b>Liquid-phase Peptide Fragment Manufacturing Technologies and Workflows</b></p> <p>Liquid phase peptide synthesis (LPPS) offers an attractive alternative approach with dramatic reductions in PMI and the ability to use traditional batch reactors. Snapdragon has developed two distinct LPPS processes for the production of peptide fragments in standard batch reactor to support downstream solution-phase fragment coupling. These processes have now been demonstrated on multi-kilogram scale and provide an order of magnitude reduction in PMI compared to typical SPPS processes. This presentation will further describe ongoing developments at Cambrex for the LPPS process development workflow to make these approaches more accessible at an early stage of clinical development.</p> <p><b>Adrian Amador, Ph.D., Director of Process Chemistry, Cambrex, USA</b></p>

	Oligonucleotide Track	Peptide Track
4:45	<p><b>Oligonucleotide Purification and CMC Strategies – Increasing Efficiencies/Overcoming Challenges</b></p> <p>The purification of oligonucleotides has historically relied on single column purification using either anion exchange or reverse phase chromatography. This slow to change field is become increasing more complex with addition of new synthesis chemistries, ASO-conjugates, along with increasing patient demand/quantity. This talk focuses on how these challenges can be met with more state of the art purification that delivers drug substance of high purity, yield and productivity.</p> <p><b>Robert Gronke, Ph.D., Senior Principal Scientist, Technical Development, Biogen, USA</b></p>	<p><b>A Breakthrough for “TIDES” Researchers in CMC: Developing a State-of-the-Art Technology to Improve Upon Traditional Labor-Intensive Methods of Purification</b></p> <p>This presentation will describe a state-of-the art technology which improves upon traditionally labor-intensive methods for peptide purification. We developed an automated and seamless transition purification procedure from analytical to preparative scale by creating unique algorithms. The method of column selection and minimization, which are factors that have a great influence on separation, are also described. Remarkable time and labor savings in projects for several pharmaceutical companies have been observed and lessons learned from these programs will be presented.</p> <p><b>Katsuhiko Miwa, CEO, ChromaJean, Japan</b></p>
5:15	 <p><b>Networking Cocktail Reception with Poster and Exhibit Viewing</b></p> <p>Please join your fellow attendees in the exhibit hall for an evening of networking while enjoying beverages and appetizers.</p>	
	<p><b>Close of Day 2</b></p>	

Main Conference Sessions	
General Session – Innovative Drug Delivery for Therapeutics & Vaccines	
8:00	<b>Registration and Coffee</b>
8:10	<p><b>Chairperson's Remarks</b></p> <p>Yogesh Sanghvi, Ph.D., President, Rasayan, Inc., USA</p>
8:15	<p><b>Lipid Nanoparticles for mRNA Vaccine Delivery</b></p> <p>Lipid nanoparticles (LNPs) represent the leading delivery platform for mRNA vaccines with advantageous biocompatibility, scalability, and adjuvant activity. We investigated the physicochemical characteristics and adjuvanticity of four-component LNP formulations in mice. We demonstrate that modifications significantly impact the magnitude and quality of the vaccine-elicited immune responses.</p> <p>Mate Vadovics, Ph.D., Postdoctoral Fellow, Norbert Pardi Lab, University of Pennsylvania, USA</p>
8:45	<p><b>LEAD (Ligand- and Enhancer-Assisted Delivery) Enables Efficient siRNA Delivery to Immune Cells by Targeting a Cell Type-Specific Receptors</b></p> <p>Extrahepatic delivery of siRNA presents significant challenges, particularly in targeting immune cells. Sanegene's innovative LEAD™ (Ligand and Enhancer Assisted Delivery) technology facilitates precise siRNA delivery to specific immune cells, including macrophages and dendritic cells, by targeting cell-specific receptors. Our studies demonstrate that, under physiological conditions, the LEAD platform efficiently delivers siRNA molecules specifically to tissue resident macrophages in both rodent and non-human primates, robustly silencing the target genes. Furthermore, under pathological conditions, our platform shows specific and efficient delivery of siRNA to residential and infiltrating monocyte-derived macrophages in an obese mouse model, and tumor-associated macrophages in allograft tumor models. This advanced delivery technology significantly enhances the therapeutic potential of siRNA, paving the way for new therapeutic strategies.</p> <p>Weimin Wang, Ph.D., Founder &amp; CEO, Sanegene Bio, USA</p>
9:15	<p><b>Phase 1 Clinical Testing of a First-in-Class ASO Therapeutic against Metastatic Cancer</b></p> <p>For the past 20 years our research has focused on developing potential therapies targeting specific properties of tumor cells that have metastasized. In our earlier discovery efforts, miRNA-10b was identified as a master regulator of the viability of metastatic tumor cells and we developed a therapeutic strategy based on miR-10b inhibition. The specific inhibition of miR-10b was achieved using ASOs delivered to metastatic sites by TransCode's proprietary delivery system (termed TTX-MC138). On the path to clinical development of TTX-MC138, we received FDA authorization for a now completed Phase 0 and an ongoing Phase 1 clinical trial in patients with advanced metastatic cancer. Results from initial clinical data and lessons learned will be presented.</p> <p>Zdravka Medarova, Ph.D., Co-Founder and Chief Scientific Officer, TransCode Therapeutics, USA</p>
9:45	<p><b>Targeting lncRNA TUG1 for Treatment of Patients with GBM by Using a Novel DDS System for ASO</b></p> <p>Taurine Upregulated Gene 1 (TUG1) is a lncRNA overexpressed in several cancers including glioblastoma (GBM). Recent evidence shows TUG1 prevents the accumulation of potentially harmful R-loops in cancer cells, thereby inhibiting cell death, so that TUG1 silencing can induce cancer cells without toxicity to normal cells. We are developing anti-TUG1 ASO formulated in YBC polymer for the treatment of GBM patients, and have recently started a first-in-human study in Japan.</p> <p>Shiro Akinaga, Ph.D., President and CEO, NANO MRNA, Co., Ltd., Japan</p>
10:15	<b>Networking Refreshment Break with Poster and Exhibit Viewing</b>

	Oligonucleotide Track	Peptide Track
10:55	<p><b>Chairperson's Remarks</b></p> <p>Hiroaki Suzuki, Country Manager, Vetter Pharma International Japan</p>	<p><b>Chairperson's Remarks</b></p> <p>Xinxin Gao, Ph.D., Principal Scientific Manager, Peptide Therapeutics, Genentech, USA</p>
11:00	<p><b>Probing Chemical Space to Improve siRNA Therapeutic Profile</b></p> <p>Building on our previous research addressing siRNA off-target effects, we have developed novel chemical modification strategies to enhance siRNA specificity. Our latest findings reveal that the precise placement of these modifications within the siRNA duplex significantly enhances the target specificity while preserving on-target potency. This presentation will also explore ongoing efforts to expand these modifications, aiming further to improve the specificity and overall profile of RNA therapeutics.</p> <p>Mehran Nikan, Ph.D., Research Fellow, Ionis Pharmaceuticals, USA</p>	<p><b>Revolutionizing Healthcare: Pioneering Peptide Innovations by AI Computational Design</b></p> <p>We have entered the 2nd century of peptide therapeutics with a bang. Computational design using artificial intelligence has the potential to revolutionize peptide research and unlock intracellular targets and oral bioavailability. How can we best utilize these tools in peptide drug discovery and development?</p> <p>Kerry Blanchard, M.D. Ph.D., Chief Executive Officer, Perpetual Medicines, China</p>
11:30	<p><b>Development of Targeted RNA Therapeutics: Preclinical, Clinical Progress and Lessons Learned</b></p> <p>This presentation will discuss how we: 1) Derisk RNA sequences and novel chemical modifications with AI algorithm, in vitro and in vivo preclinical models; 2) Develop cellular and animal models with relevant disease readouts to demonstrate target engagement and 3) Identify AOCs for development candidate selection and clinical development.</p> <p>Hanhua Huang, Ph.D., Vice President, Biology, Avidity Biosciences, USA</p>	<p><b>Identification of Disulfide Constrained Peptide Based Binders against Membrane Bound E3 Ubiquitin Ligases</b></p> <p>Disulfide constrained peptides (DCPs) show great potential as templates for drug discovery. We developed DCPs binding to membrane bound E3 ubiquitin ligases. They can be used to develop strategies for targeted protein degradation at the plasma membrane (membrane PROTAC), or as potential therapeutic tools for tissue regeneration. These DCPs can be produced synthetically or recombinantly, providing great versatility compared with large biologics or small molecules.</p> <p>Xinxin Gao, Ph.D., Principal Scientific Manager, Peptide Therapeutics, Genentech, USA</p>
12:00	<p><b>Advanced Manufacturing Partnerships: Innovative Solutions for Oligo Programs</b></p> <p>The acquisition of BioVectra, a CDMO specializing in biologics and targeted therapeutics, has enabled Agilent to expand its biopharma capabilities, including sterile fill-finish services, pDNA and mRNA capabilities, and lipid nanoparticle formulation. Also it has also allowed to align manufacturing capabilities and resources to drive innovation and operational excellence. Learn how this change aims to help pharma and biopharma companies implement end-to-end solutions, accelerating drug development and manufacturing.</p> <p>Amanda Haas, Business Development Manager, Agilent Technologies, Agilent Technologies</p>	<p><b>First De-novo Designed Cyclic Peptides for SORT1 and CNS Delivery</b></p> <p>ProteinQure has designed a series of de-novo peptides including the first known cyclic binders to SORT1 using our proprietary computational platform. Sortilin (SORT1) is a member of the vacuolar protein sorting 10 protein (Vps10p). As a cell surface receptor, SORT1 is able to mediate efficient endocytosis of extracellular ligands to the lysosomal compartment. Numerous reports have identified enriched SORT1 expression in the brain. We sought to exploit SORT1-dependent internalization of peptides as a platform for rapid and specific siRNA delivery into CNS cells. Using PQStudio (our proprietary computation-enabled design capabilities), we generated high affinity SORT1 targeting peptides that exhibit efficient receptor-dependent internalization. Alternative computational approaches such as AlphaFold2 and large language models failed to recapitulate the peptide design. Peptide-siRNA conjugates molecules exhibit potent and durable knockdown in all regions of the brain in mouse models, thereby highlighting the potential of SORT1-engaging peptides for nucleotide delivery.</p> <p>Lucas Siow, CEO and Co-Founder, ProteinQure, Canada</p>

12:30		Networking Luncheon with Poster and Exhibit Viewing	
	Oligonucleotide Track		Peptide Track
1:40	<p><b>Chairperson's Remarks</b></p>	<p><b>Chairperson's Remarks</b></p> <p>Markus Muttenthaler, Ph.D., Associate Professor, Neuropeptide Research, The University of Queensland and University of Vienna, Austria</p>	
1:45	<p><b>Design and Selection of High-Affinity XenoAptamers for Diagnostic and Therapeutic Applications</b></p> <p>We have developed ExSELEX, an innovative method for generating high-affinity DNA aptamers (XenoAptamers) that bind to target proteins by introducing two new letters using genetic alphabet expansion technology. With sub-nanomolar Kd values and high specificity for their targets, XenoAptamers can be applied as next-generation antibodies in diagnostics and drug discovery.</p> <p>Michiko Kimoto, Ph.D., Chief Operating Officer, R&amp;D Director, Xenolis Pte. Ltd., Singapore</p>	<p><b>Program Update on JNJ-2113, The First Investigational Targeted Oral Peptide for Psoriasis</b></p> <p>Steven Fakharzadeh, M.D., Ph.D., Global Medical Affairs Leader Immunodermatology, Johnson &amp; Johnson, USA</p>	
2:15	<p><b>Nitto Bispecific siRNA Targeting YAP1/WWTR1 As A Novel Therapeutic Agent for Liver Fibrosis</b></p> <p>Whereas a paralog pair of proteins has been identified as a novel drug target, there are significant technical challenges in creating small molecule inhibitors. Here, we developed novel bispecific siRNA in which each single antisense oligonucleotide binds to both paralog mRNA specifically and induces RNA interference. In this presentation, preclinical data of Nitto bispecific siRNA targeting two paralogous transcriptional coactivators, YAP1/WWTR1 as a novel therapeutic agent for liver fibrosis will be introduced.</p> <p>Masayuki Sugimoto, Ph.D., Manager, Nucleic Acid Medicine Business Division, Nitto Denko Corporation, Japan</p>	<p><b>Oral Peptide Therapeutics for Gut Disorders</b></p> <p>Peptides have long been ignored as good drug leads for gastrointestinal disorders due to their intrinsic metabolic limitations. However, we argue that peptides could be ideal drug candidates for gut-specific treatments and have developed methods to render them gut-stable for oral treatment. We exemplify this new frontier with several examples, including the trefoil factor family, blockbuster linaclotide, oral oxytocin for abdominal pain, and new treatment opportunities for diseases associated with intestinal biofilms.</p> <p>Markus Muttenthaler, Ph.D., Associate Professor, Neuropeptide Research, The University of Queensland and University of Vienna, Austria</p>	
2:45	<p><b>Recent Progress of Luxna's Antisense Oligonucleotide Therapeutics Platform and Application to Neurological Diseases</b></p> <p>Luxna Biotech is a preclinical-stage company focused on developing antisense oligonucleotides (ASOs). We have established a robust optimization process for lead ASOs applied to central nervous system (CNS). In this presentation, we will share our latest findings on Gapmer ASOs and splice regulators, both of which are tailored for neurological diseases, maximizing therapeutic efficacy while minimizing neurotoxicity in vivo. Additionally, we will provide updates on our ongoing progress in drug discovery for neurological disease.</p> <p>Hideaki Sato, President and CEO, Luxna Biotech, Japan</p>	<p><b>Biodegradable Silica Composite Technology Enabling Long-Acting Controlled Release of GLP-1RAs</b></p> <p>The widespread use of glucagon-like peptide-1 receptor agonists (GLP-1RA) for the treatment of obesity and a growing number of new indications has highlighted critical challenges with the tolerability and efficacy of these agents. In this talk, we will explore the capabilities of biodegradable silica composites to prolong the release of these peptide agents and moderate the pharmacokinetic profile to optimize tolerability. Through both in vitro and in vivo controlled release formulation case studies, we demonstrate the ability of the technology to effectively manage burst release and significantly enhance the durability of these agents, promoting the maintenance of health benefits associated to GLP-1RA use.</p> <p>Frederic Dargelas, Ph.D., Senior Director, Head of Business Development and Alliance Management, DelSiTech Ltd., Finland</p>	

3:15	<b>Networking Refreshment Break with Poster and Exhibit Viewing</b>	
	<b>Oligonucleotide Track</b>	<b>Peptide Track</b>
3:45	<p><b>Reviving ANGPTL4 As a Drug Target with a GalNAc ASO Approach - A Case Study</b></p> <p>Using a liver specific ASO approach we have been able to circumvent previously known problems with the drug target ANGPTL4. Global ablation of ANGPTL4 was shown to give serious side effects which was avoided using a GalNAc ASO approach. This would be a case study of pre-clinical and clinical results.</p> <p><b>Stefan K. Nilsson, Ph.D., CEO, Lipigon, Sweden</b></p>	<p><b>Discovery of Macrocytic Peptide Inhibitors of SIRT7 and HDAC11</b></p> <p>Histone deacetylases (HDACs) are validated targets for treatment of certain cancers and play regulatory roles ranging from epigenetics to metabolism. My laboratory has developed numerous peptide-based inhibitors with varying potencies, selectivity profiles, and mechanisms of inhibition, to illuminate the function of HDACs and sirtuins in biology and medicine. In this presentation, I will focus on our advances in the selective targeting of SIRT7 and HDAC11, using two different strategies for discovering macrocytic peptide inhibitors.</p> <p><b>Christian A. Olsen, Ph.D., Professor, Drug Design and Pharmacology, University of Copenhagen, Denmark</b></p>
4:15	<p><b>Recent Progress in the Treatment of Myotonic Dystrophy Type 1 (DM1), The Most Common Adult-onset Muscular Dystrophy</b></p> <p>Myotonic Dystrophy Type 1 (DM1), is a multisystemic, autosomal dominant genetic disorder characterized by progressive muscle wasting and weakness, myotonia. From 2017, several clinical trials have been launched with different nucleic acid-based therapies leading valuable insights into the safety, efficacy, delivery challenges, and potential mechanisms of action of emerging therapies.</p> <p><b>Frederic Legros, Ph.D., Chairman and Chief Executive Officer, Arthex Biotech, Spain</b></p>	<p><b>(4:10) Preclinical Studies of Macrocytic Peptides, Targeted Radiopharmaceutical Agents for PDL-1, Developed with 48Hour Discovery Pipeline</b></p> <p>The talk will describe the use of DNA encoded platform based on chemically modified phage libraries for preclinical development of macrocytic peptides as delivery vectors for targeted radiopharmaceuticals. Combination of selection, maturation and machine learning approaches yielded preclinical assists in low nanomolar range and confirmed engagement with PDL on tumor cells. Identical pipeline is applied to a series of other targets in the preclinical development pipeline of 48Hour Discovery.</p> <p><b>Ratmir Derda, Ph.D., Founder and CSO, 48Hour Discovery and Associate Professor, Department of Chemistry, University of Alberta, Canada</b></p>
4:35	<p><b>Please Choose Another Track</b></p>	<p><b>Combining Advanced Peptide Screening Methods to Identify Macrocytic Peptide Candidates for Radiotherapy Development</b></p> <p>We have developed a cutting-edge peptide discovery platform that efficiently identifies macrocytic peptides for the development of peptide-radionuclide conjugates (RDCs). This platform integrates multiple advanced technologies, including Peptide Information Compression Technology (PICT), Disulfide-Rich Peptides (DRPs) phage display, animal toxin peptides, nano-macrocytic peptide libraries, and virtual peptide screening. The platform's ability to discover high-affinity and stable peptides makes it as a promising tool for targeted radiotherapy, paving the way for more effective and personalized cancer treatments.</p> <p><b>Weiliang Xu, Ph.D., Associate Director of Business Development, Peplib, USA</b></p>

# Agenda | Friday, February 28, 2025

	Oligonucleotide Track	Peptide Track
5:00	<b>Please Choose Another Track</b>	<b>Regeneration-Inducing Medicine™ - Harness the Power of Innate Stem Cells to Conquer Intractable Diseases</b> Hitoshi Kuroda, Ph.D., Business Development, StemRIM, Japan
5:25	Close of Conference	



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