

VIRTUAL SCIENTIFIC FORUM

TIDES ASIA

**Oligonucleotide &
Peptide Therapeutics**

MARCH 2-5, 2021 COMPLIMENTARY WEBINAR SERIES

Presented in Japanese Standard Time (JST)

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PEPTIDE, MRNA AND DRUG DELIVERY SPECTRUM**

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2021 SPEAKERS



Stanley T. Crooke, M.D., Ph.D.

Chairman and CEO, n-Lorem &
Executive Chairman,
Ionis Pharmaceuticals



Sudhir Agrawal, Ph.D.

Founder and President,
Arday Sciences



Patrick C. Reid Ph.D.

President, CEO and Chairman of the Board,
PeptiDream, Inc.



Yosuke (Oscar) Yamasaki

Marketing Department, Product Management
Unit Bioscience Division,
Tosoh corporation



Akihiro OOTA

General Manager of Manufacturing,
Ajinomoto Bio-Pharma Services Osaka



Brian Rivera

Senior Product Manager – Biologics
Phenomenex



Wolfgang Seufert, Ph.D.

Director of Production,
Bachem AG



Andreas Kuhn, Ph.D

Senior Vice President RNA Biochemistry &
Manufacturing
BioNTech RNA Pharmaceuticals GmbH



Jyothi Thundimadathil, Ph.D.

Associate Director,
Bachem Americas



Dr. Catherine McKeen

Site Operational Director & Head of Chemistry
LGC



Ivo Eggen

Section Lead,
Aspen API



Dr. Juergen Mueller

Site Operational Director & Head of Chemistry
LGC

AGENDA

DAY 1 – TUESDAY, MARCH 2, 2021

8:30am – 9:30am	How Antisense Drugs Work: Molecular Mechanisms of Cellular Pharmacokinetics, Pharmacodynamics and Toxicity Speaker: Stanley T. Crooke, M.D., Ph.D. Executive Chairman, Ionis Pharmaceuticals
9:30am – 10:00am	N-Lorem a New Charitable Foundation: Creating Individualized Treatments for Patients with Ultra-Rare Diseases using Antisense Technology. Speaker: Stanley T. Crooke, M.D., Ph.D., Chairman and CEO, n-Lorem
10:00am – 11:00am	Oligonucleotide Manufacturing from Small to Large Scale (Tentative) Speaker: Akihiro OOTA, General Manager of Manufacturing, Ajinomoto Bio-Pharma Services Osaka

DAY 2 – WEDNESDAY, MARCH 3, 2021

9:00am – 10:00am	A Strategic Vision for Therapeutic Development: The PeptiDream Story Speaker: Patrick C. Reid Ph.D., President, CEO and Chairman of the Board, PeptiDream, Inc
11:00am – 12:00pm	Solubility-directed Development of an LPPS Manufacturing Process for Trileucine Speaker: Wolfgang Seufert, Ph.D., Director of Production, Bachem AG
12:00pm	GC-LPPS: Aspen's Green Continuous Liquid Phase Peptide Synthesis – Green, Lean and Clean Speaker: Ivo Eggen, Section Lead, Aspen API

DAY 3 – THURSDAY, MARCH 4, 2021

9:00am – 10:00am	RNA Therapeutics: Importance of Nucleotide Sequence and Modifications on the Mechanism of Action Speaker: Sudhir Agrawal, Ph.D., Founder and President, Arny Sciences
10:00am – 11:00am	Preparative Separation of Oligonucleotide by Ion-Exchange and Hydrophobic Interaction Chromatography Speaker: Yosuke (Oscar) Yamasaki, Marketing Department, Product Management Unit, Bioscience Division, Tosoh corporation
11:00am – 12:00pm	Sample Preparation Strategies for Oligonucleotide Bioanalytical Workflows Speaker: Brian Rivera, Senior Product Manager – Biologics, Phenomenex

DAY 4 – FRIDAY, MARCH 5, 2021

9:00am – 10:00am	Usage of an mRNA Platform Technology for "Light Speed" Development of a Vaccine against SARS-CoV-2 Speaker: Andreas Kuhn, Ph.D., Senior Vice President RNA Biochemistry & Manufacturing, BioNTech RNA Pharmaceuticals GmbH
10:00am – 11:00am	LGC's Development of a Novel, Modular, GalNAc Access, and Conjugation Strategies for Nucleic Acid Medicines Speaker: Dr. Catherine McKeen, Site Operational Director & Head of Chemistry, LGC

All session times are listed in JST (Japanese Standard Time)

SESSION ABSTRACTS

How Antisense Drugs Work: Molecular Mechanisms of Cellular Pharmacokinetics, Pharmacodynamics and Toxicity

Describing the educational value of attending your webcast session Antisense technology is beginning to deliver on the broad therapeutic promise of three decades and the technology continues to advance at a rapid pace yielding ever better performing therapeutic agents. The technology has progressed to this exciting moment and continues to advance because of long term investment in basic research into the technology itself. Today, the molecular mechanisms by which PS ASOs enter and distribute in cells, the molecular mechanisms of action and molecular mechanisms of toxicity are well understood and provide the basic understanding to use medicinal chemistry to enhance their performance and versatility even more. In this presentation we focus not on what PS ASOs do, but how and why they do what they do and look to the future of the technology to be shaped by today's knowledge.

N-Lorem a New Charitable Foundation: Creating Individualized Treatments for Patients with Ultra-Rare Diseases using Antisense Technology.

Describing the educational value of attending your webcast session n-Lorem is a charitable

foundation that is bringing experimental ASO treatments to patients with ultra-rare diseases (n: 1-30 worldwide) for free for life. Though each patient may be unique or a member of a very small cohort of patients, in aggregate there are millions of such patients identified worldwide. To do this n-Lorem collaborates with Ionis Pharmaceuticals to create precision ASOs designed to address the specific mutation of each patient, one patient at a time. n-Lorem has systems that assure that patients are exposed to only prudent risks and importantly n-Lorem plans to publish the results of our efforts in scientific literature annually. n-Lorem currently focuses on patients with mutations that cause symptoms in the CNS (I.T. dosing), the liver or kidney (SC dosing), the lung (aerosol dosing) and the eye (Intravitreal dosing) as for these organs Ionis has substantial experience, the doses needed are low and there are substantial safety margins, for ASOs used in these organs. In our first year of operation, we have helped two investigators treat >10 patients with several neurological diseases with experimental ASOs and received more than 50 applications for treatment and proceeding to create experimental ASO treatments for >20 patients.

A Strategic Vision for Therapeutic Development: The PeptiDream Story

This presentation will provide an overview of the strategic vision and business philosophy that has guided PeptiDream's evolution into

one of the leading peptide drug discovery and development companies in the field.

Microspheres and Nanoparticles for Peptide Delivery

Delivery of peptides is a challenging task due to their poor stability toward proteolytic enzymes, their large size and poor penetration into cells. The great innovation in this field relies on the formulation of microspheres and nanoparticles to encapsulate the peptide in order to enhance its bioavailability and therapeutic efficacy. Microparticle formulation has been successfully applied in a number of commercial products containing peptides as active pharmaceutical ingredients. The use of nanoparticles for delivering proteins and peptides are still in early stages of development and is expected to mature in near future.

Solubility-directed Development of an LPPS Manufacturing Process for Trileucine

The excipient Trileucine is applied for improving aerosol performance and stability of spray-dried powders for inhalation. The peptide Trileucine can form different polymorphs, which show large variations in water solubility. In this presentation, the development of a new and straightforward manufacturing process in solution is shown. Based on the results of a polymorph screening study, Trileucine material

SESSION ABSTRACTS

with optimal solubility properties in water was obtained.

GC-LPPS: Aspen's Green Continuous Liquid Phase Peptide Synthesis – Green, Lean and Clean

Aspen API has developed a Green Continuous Liquid Phase Peptide Synthesis, which combines the advantages of classical solution-phase synthesis and solid-phase synthesis. The process consists of repetitive cycles of coupling and deprotection in a permanent green organic phase (ethyl acetate), in which the growing peptide is anchored due to the presence of hydrophobic protecting functions. It is further characterized by the fact that intermediates are not isolated. These distinctive features account for a significant reduction of organic waste streams with respect to the classical methods of peptide synthesis. Processes according to this highly efficient synthesis method are easy to scale up and yield products of reproducible high purity.

RNA Therapeutics: Importance of Nucleotide Sequence and Modifications on the Mechanism of Action

Describing the educational value of attending your webcast session RNA Therapeutics are finally taking their place as a main drug platform alongside small molecules and proteins. In this platform drug candidates are com-

posed of modified synthetic oligonucleotides. While the intended target of these compounds is RNA, they can also be recognized by pattern recognition receptors. I will discuss the twists and turns on their road to success and highlight areas of ongoing research.

Preparative Separation of Oligonucleotide by Ion-Exchange and Hydrophobic Interaction Chromatography

Production capacity for synthetic oligonucleotides are becoming more important and critical due to increase in global demand. While preparative separation of oligonucleotide is well applied by reversed-phase chromatography (RPC), RPC using organic solvents has problems in safety in production facility and environmental protection. To overcome these problems, ion-exchange chromatography (IEC) and hydrophobic interaction chromatography (HIC) would be considered with aqueous eluent and with less organic solvent. Recent application to oligonucleotide by IEC and HIC will be discussed.

Sample Preparation Strategies for Oligonucleotide Bioanalytical Workflows

In this presentation, we will give an overview of sample preparation strategies for synthetic oligonucleotides, including how to adjust experimental design depending on the oligo and sample matrix. Additionally, we will dis-

cuss approaches for other challenging oligos, such as peptide nucleic acids or morpholinos, which are unique in that they do not have the negatively charged backbone associated with other nucleic acid chemistries.

Usage of an mRNA Platform Technology for "Light Speed" Development of a Vaccine against SARS-CoV-2

Describing the educational value of attending your webcast session Messenger (m)RNA is increasingly investigated as a platform technology for multiple therapeutic applications including as a vaccine against infectious diseases. With respect to manufacturing, mRNA has several advantages compared to other biopharmaceuticals. Most importantly, one process can be essentially used to manufacture any RNA sequence, significantly shortening development time for a new project. With the recent outbreak of COVID-19, our mRNA platform was thus ideally suited to develop an mRNA-based vaccine in "light speed". In this talk, the approach that was used and the current status will be presented.

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