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Conference-at-a-Glance
Short Courses
Oligonucleotide Discovery and Delivery
Peptide Discovery and Development
Symposium 1: Oligonucleotides for Cancer Immunotherapy
Symposium 2: Peptides in Cancer Therapy
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Cambridge 25 YEARS HEALTHTECH Institute
A Division of Cambridge Innovation Institute

Cambridge Healthtech Institute's Second Annual

OPT Boston

Oligonucleotide & Peptide THERAPEUTICS

March 27 - 29, 2017 | Royal Sonesta Boston | Cambridge, MA

Advancing the Discovery, Development and Delivery of Oligonucleotide and Peptide Therapeutics

CONFERENCE PROGRAMS | MARCH 27 - 28, 2017

Oligonucleotide Discovery and Delivery

Peptide Discovery and Development

MARCH 29, 2017 | INAUGURAL

SYMPOSIUM 1:

Oligonucleotides for Cancer Immunotherapy

Advances in Immune-Modulatory Nucleic Acids

SYMPOSIUM 2:

Peptides in Cancer Therapy

Empowering Peptide Vaccines, Immunotherapies and Conjugates to Treat Cancer

FEATURED SPEAKERS



Melissa J. Moore, Ph.D.
CSO, mRNA Research Platform, Moderna



Tomi Sawyer, Ph.D.
Distinguished Scientist, Discovery Chemistry Modalities, Merck Research Laboratories



Muthiah (Mano) Manoharan, Ph.D.
Senior Vice President, Drug Discovery, Alnylam Pharmaceuticals, Inc.



Eric von Hofe, Ph.D.
President, Antigen Express

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Sponsorship, Exhibit, and Lead Generation Opportunities

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- Plated dinner with specific conversation focus

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Oligonucleotide & Peptide THERAPEUTICS CONFERENCE-AT-A-GLANCE

OPT Boston

Sunday, March 26th	Monday, March 27th	Tuesday, March 28th	Wednesday, March 29th
Pre-Conference Dinner Short Course	Track 1: **Oligonucleotide Discovery and Delivery**		Symposium 1: **Oligonucleotides for Cancer Immunotherapy**
	Track 2: **Peptide Discovery and Development**		Symposium 2: **Peptides in Cancer Therapy**
	Dinner Short Course		



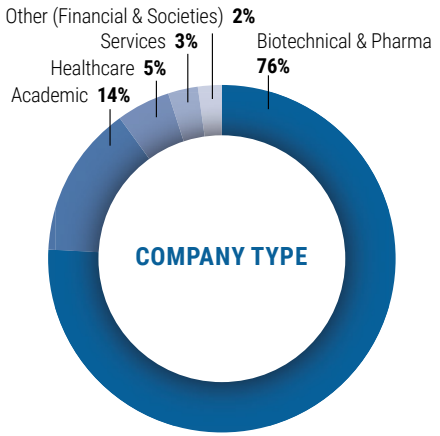
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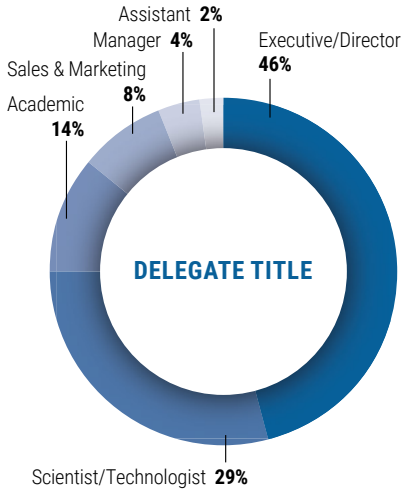
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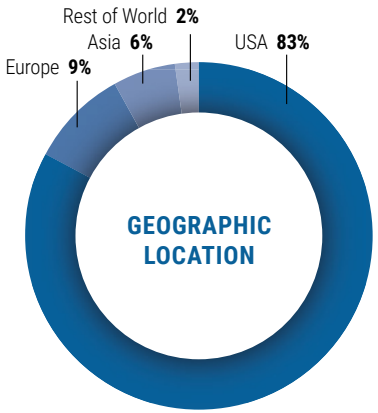
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2016 ATTENDEE DEMOGRAPHICS







USA Breakdown
East Coast **75%**
West Coast **14%**
Midwest **11%**

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SUNDAY, MARCH 26 | 5:00 - 8:00 PM

Pre-Conference Dinner Short Courses*

SC1: Oligonucleotide Therapeutics: From Discovery to Manufacturing

Instructor: Dmitry Samarsky, Ph.D., CSO, Silence Therapeutics

Additional Instructors to be Announced

RNAi/antisense oligonucleotides can target virtually any disease causing gene and promise to become a third major class of therapeutics (besides small molecules and biologics). For this to happen, however, several challenges have to be addressed. This course discusses considerations taken into account when selecting oligonucleotide therapeutic programs: from target and delivery selection to developmental and manufacturing particularities.

MONDAY, MARCH 27 | 6:30 - 9:30 PM

Dinner Short Courses*

SC3: CRISPR-Based Gene Editing for Targeted Therapies

Instructors: Clifford Steer, M.D., Professor of Medicine and Genetics, Cell Biology, and Development; Director, Molecular Gastroenterology Program, University of Minnesota Medical School

Ciro Bonetti, Ph.D., Scientist, Regeneron Pharmaceuticals

Hao Yin, Ph.D., Research Scientist, The David H. Koch Institute for Integrative Cancer Research, Massachusetts Institute of Technology

While the challenges and risks associated with oligonucleotide therapies still remain, there is a new and better understanding of how genes can be effectively manipulated and delivered. With the rise of gene editing tools and enhanced knowledge of targeted delivery, these therapeutic modalities are once again being embraced with renewed hope and enthusiasm. This course helps you understand how gene editing – particularly the one enabled by the CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats)/Cas9 system – works, and how it can be used to help develop targeted therapies with good efficacy and delivery.

** Separate registration required.*

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Oligonucleotide Discovery and Delivery

Advances in the Discovery, Development and Delivery of Aptamer, Antisense and RNA Therapeutics

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Recent advances in nucleic acid medicinal chemistry and delivery have led to the creation of a new generation of oligonucleotide therapies harnessing chemical modifications and conjugations to improve their stability, bioavailability, specificity and potency. These advances, along with a robust development landscape, and several late-stage clinical products poised for approval, have led to a sharp resurgence of interest in the discovery of oligonucleotide-based therapeutics.

Due to the remarkable success of the Inaugural event, Cambridge Healthtech Institute is delighted to host the Second Annual Oligonucleotide Discovery and Delivery conference, March 27-28, in Cambridge, MA. Join leading oligonucleotide developers and discovery scientists to discuss technological and scientific advances in nucleic acid synthesis, medicinal chemistry and delivery, as well as preclinical and clinical findings.

SUNDAY, MARCH 26

Recommended Pre-Conference Dinner Short Course*

5:00-8:00 pm **SC1: Oligonucleotide Therapeutics: From Discovery to Manufacturing**

** Separate registration required. See page 4 for details.*

MONDAY, MARCH 27

7:00 am **Registration and Morning Coffee**

ADVANCES IN OLIGONUCLEOTIDE THERAPEUTICS AND DELIVERY

8:00 **Welcome Remarks from Conference Director**
Kip Harry, Senior Conference Director, Cambridge Healthtech Institute

8:10 **Chairperson's Opening Remarks**
Muthiah (Mano) Manoharan, Ph.D., Senior Vice President, Drug Discovery, Alnylam Pharmaceuticals, Inc.

8:15 **Novel Phosphorodiamidate Oligomers (PMOs) for the Treatment of Genetic and Infectious Diseases**
Bruce Wentworth, Ph.D., Former Vice President, Biology, Sarepta Therapeutics
PMOs are being tested in advanced clinical trials for the treatment of patients with Duchenne muscular dystrophy (DMD), a rare, X-linked disease that results in progressive muscle loss and premature death. Research has shown that for other disorders, including viral and bacterial infection as well as rare diseases such as Pompe disease, modified PMOs may be more appropriate due to their potential for enhanced delivery and tissue targeting.

8:45 **Messenger RNA as a Novel Therapeutic Approach**
Melissa J. Moore, Ph.D., CSO, mRNA Research Platform, Moderna
The contemplation of mRNA as a therapeutic platform has historically been shunned owing to challenges in oligonucleotide delivery and, maybe more importantly, the perceived shortcomings of mRNA with regard to stability and immunogenicity. Significant advances in oligonucleotide delivery have been realized over the past decade thereby enabling mRNA therapeutics. Recent discoveries in mRNA chemistry further enhance the attractiveness of this platform by eliminating innate immune activation and maximizing protein expression.

9:15 **siRNA Therapeutics for Extrahepatic Indications: Quark's Case Study**
Elena Feinstein, M.D., Ph.D., CSO, Quark Pharmaceuticals
Quark is active in the field of discovery and development of siRNA therapeutics focusing on acute or subacute indications involving organs other than liver. An overview of advanced clinical (Phase III) and some of nonclinical programs will be provided.

9:45 **Sponsored Presentation** *(Opportunity Available)*

10:15 **Coffee Break in the Exhibit Hall with Poster Viewing**

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10:45 **Challenges in the Synthesis of Conjugated LNA Oligonucleotides**
Dennis Jul Hansen, MSc, Ph.D., Scientist, Oligo Synthesis, Discovery Operations, Roche Pharma Research and Early Development, Roche Innovation Center Copenhagen A/S
Recently there has been considerable focus in the conjugation of oligonucleotides with non-nucleotide moieties which, for example, enable targeting of therapeutic oligonucleotides to specific organs and tissues *in vivo*. The conjugation of different chemical moieties to oligonucleotides is often achieved by post-synthetic amide bond formation. This approach requires the synthesis of LNA-oligonucleotides with a primary amine the 5' end. During this work we noticed a (to us) new impurity with M+28. The post-synthetic conjugation reactions are traditionally performed in aqueous solvents and require several equivalents of conjugate group, thus increasing the cost of manufacture. We will here present the work to identify the M+28 impurity, how to remove it and ultimately how avoid its formation, together with the work on performing the conjugation reaction under nonaqueous conditions, which allows for drastic reduction of the amount of conjugate needed.

11:15 **Advances in Clinically Viable LNP Compositions for mRNA Delivery**
Michael Sofia, Ph.D., CSO, Arbutus Biopharma, Inc.
Arbutus' Lipid Nanoparticle (LNP) platform is the leading nucleic acid delivery technology platform enabling a number of early and late stage clinical trials. They are designed to deliver therapeutic nucleic acids (NA) to sites of disease, and have been used to target both viral and endogenous gene targets. We continuously seek to broaden the Therapeutic Index (TI) of the LNP platform, from both potency and tolerability perspectives.

11:45 **GalNAc-Conjugated siRNAs as a New Paradigm in Oligonucleotide Therapeutics**
Muthiah (Mano) Manoharan, Ph.D., Senior Vice President, Drug Discovery, Alnylam Pharmaceuticals, Inc.
During this presentation, I will discuss the progress in the advancement of RNAi therapeutics and review delivery of RNAi and where the field is going. I will also discuss conjugated delivery of oligonucleotides to the liver and combining novel chemical modifications with conjugation strategies.

12:15 pm **Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

EMERGING NUCLEIC ACID DEVELOPMENT PLATFORMS

1:55 **Chairperson's Remarks**
Thazha P. Prakash, Director, Medicinal Chemistry, Ionis Pharmaceuticals

2:00 **Approaches to Improve Potency of Antisense Oligonucleotides in Extrahepatic Tissues**
Thazha P. Prakash, Director, Medicinal Chemistry, Ionis Pharmaceuticals
Antisense oligonucleotide (ASO)-based drug development is evolving as an effective therapeutic modality. In order to fully realize the potential of this technology, it is necessary to improve the potency of ASOs in extrahepatic tissues. We investigated the effect of conjugating hydrophobic ligand capable of interacting with plasma proteins on productive uptake of ASO into extrahepatic tissues. Our results suggest that conjugation of hydrophobic ligands improved potency of ASOs in extrahepatic tissues.

2:30 **Development of SB 9200 that Activates RIG-I as Antiviral Agent**
R.P. (Kris) Iyer, Ph.D., Co-Founder & CSO, Spring Bank Pharmaceuticals
We have discovered SB 9200 as an oral broad-spectrum antiviral dinucleotide, that activates RIG-I and NOD2, thereby causing the induction of expression of intracellular Interferons, ISGs and cytokines. SB 9200 has entered global Phase II clinical trials against HBV following extensive preclinical studies. SB 9200 was shown to be an anti-HCV agent in the recently concluded Phase I clinical trials in HCV-infected patients.

3:00 **Third-Generation Antisense (3GA) Technology: Insights into Mechanism of Action**
Reina Improgo, Ph.D., Research Scientist, Idera Pharmaceuticals, Inc.
Development of the antisense oligonucleotide approach has been hampered by off-target effects and inflammatory responses. Based on the insights gained from our previous work, we have designed third-generation antisense (3GA) oligonucleotides with the goal of improving therapeutic index. The 3GA design consists of two antisense oligonucleotides linked together via their 5' end. We have conducted preclinical proof-of-concept studies in cell-based assays and *in vivo* mouse models using 3GAs against multiple gene targets.

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3:30 **Refreshment Break in the Exhibit Hall with Poster Viewing**

SYNTHESIS AND MEDICINAL CHEMISTRY

4:00 **High-Throughput Quantitative Bioanalysis and Metabolite Profiling Using LC-HRAM to Support Discovery and Development of Oligonucleotide Therapeutics**

Laixin Wang, Ph.D., Vice President, Bioanalytical, NovaBioAssays LLC

This presentation will discuss the challenges and solutions of sample preparations and LC-MS analysis of oligonucleotides in a variety of biological matrices, including cell culture, urine, plasma, and tissues. The pros and cons of these extraction methods and instrument platforms will be discussed with case studies for different structured oligonucleotides.

4:30 **Solid-Phase Purification of Synthetic DNA Sequences and/or Development of 2'-Hydroxy Protecting Groups for Ribonucleosides and Oligoribonucleotides**

Serge Beaucage, Ph.D., Chief, Laboratory of Biological Chemistry, CDER, FDA

Little has been done to develop high-throughput procedures for the purification of synthetic nucleic acid sequences. An efficient process for solid-phase purification of phosphorothioate and native DNA sequences will be presented. Furthermore, an innovative cleavage of 2'-hydroxy protecting groups from ribonucleosides and oligoribonucleotides through an intramolecular decarboxylative elimination process will also be discussed.

5:00 **Sponsored Presentation** *(Opportunity Available)*

5:30 **Welcome Reception in the Exhibit Hall with Poster Viewing**

Sponsored by OLIGO FACTORY

6:15 **Short Course Registration**

Recommended Dinner Short Course*

6:30-9:30 SC3: CRISPR-Based Gene Editing for Targeted Therapies

** Separate registration required. See page 4 for details.*

TUESDAY, MARCH 28

7:30 am **Breakfast Roundtable Discussions**

Grab a cup of coffee and join a roundtable discussion. These are moderated discussions with brainstorming and interactive problem solving, allowing participants from diverse backgrounds to exchange ideas and experiences and develop future collaborations around a focused topic.

For more details, please visit OPTcongress.com

RNA THERAPEUTICS AND DELIVERY

8:25 **Chairperson's Remarks**

Balkrishen (Bal) Bhat, Ph.D., Vice President, Chemistry, RaNA Therapeutics, Inc.

8:30 **Novel Strategies for Endogenous mRNA Upregulation**

Balkrishen (Bal) Bhat, Ph.D., Vice President, Chemistry, RaNA Therapeutics, Inc.

We are developing two gene upregulation platforms. The first approach targets lncRNAs (long non-coding RNA) with chemically modified oligonucleotides to block recruitment of PRC2 to the target gene of interest which results in significant upregulation of mRNA and protein. In the second approach, we selectively upregulate target mRNA and the corresponding protein by stabilizing identified regions of mRNA with chemically modified oligonucleotides.

9:00 **microRNA Targeted Therapies for Hematological Malignancies and Pathological Fibrosis: Translation from Basic Sciences to the Clinic**

William S. Marshall, Ph.D., President and CEO, miRagen Therapeutics

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9:30 **Development of Novel Breakthrough Cancer Therapies Based on the Unique Functions of miRNAs**
Roel Q.J. Schaapveld, Ph.D., MBA, CEO, InteRNA Technologies BV
To explore miRNAs as therapeutic agents for the treatment of cancer, InteRNA Technologies has performed functional screens in cell lines covering different types of cancer. Lead candidates are now advancing in preclinical development programs with a focus on hepatocellular cancer. This presentation will provide insights into the latest progress in the preclinical development of InteRNA's lead miRNA compounds.

10:00 **Coffee Break in the Exhibit Hall with Poster Viewing**

DELIVERY TO THE CNS

10:40 **Chairperson's Remarks**
Anastasia Khvorova, Ph.D., Professor, Molecular Medicine, RNA Therapeutics Institute, University of Massachusetts Medical School

10:45 **Exosome Mediated Delivery of Therapeutic Oligonucleotides for Treatment of Neurodegenerative Disorders**
Anastasia Khvorova, Ph.D., Professor, Molecular Medicine, RNA Therapeutics Institute, University of Massachusetts Medical School
Oligonucleotide therapeutics is a new class of drugs, the clinical utility of which has been limited by inefficient tissue distribution and cellular uptake. We have developed a novel methodology that enables the loading of hydrophobically modified oligonucleotides (hsiRNA) into exosomes. These hsiRNAs show efficient cellular uptake *in vitro* as well as broad brain distribution and *in vivo* efficacy. Exosome-formulated oligonucleotide therapeutics might be a solution for the development of novel therapeutics for the treatment of neurodegenerative disorders.

11:15 **nOligos: A Cell Specific Oligonucleotide Delivery Platform: Application to MDD and PD Treatments**
Andres Montefeltro, Ph.D., CEO, nLife Therapeutics, S.L.
nLife Therapeutics has developed different nucleic acid chemical modifications with the aim to optimize cell specific delivery capabilities to neurons. We have combined siRNAs and antisense oligonucleotides (ASOs) with some specific and potent small molecule ligands to neuronal receptors or transporters, named nOligos (neuronal specific oligonucleotides). These combinations proved to deliver the nucleic acid to the target neuron in an effective way.

11:45 **Sponsored Presentation** *(Opportunity Available)*

12:00 pm **Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

NOVEL AND EMERGING APPROACHES FOR *IN VIVO* DELIVERY

1:25 **Chairperson's Remarks**
Dmitry Samarsky, Ph.D., CSO, Silence Therapeutics

1:30 **Development of Lipid-Based Oligonucleotide Delivery Systems**
Steffen Schubert, Ph.D., Head, In Vitro Pharmacology – Drug Discovery, Silence Therapeutics
In vivo delivery is still one of the most challenging parts when it comes to the therapeutic application of nucleotides like siRNA and mRNA. Here we discuss how Silence Therapeutics has broadened its portfolio of lipid-based formulations, how lipid chemistry influences specificity and activity, and certain process parameters that led to new formulations characteristics.

2:00 **A Novel Nano-Medicine Platform for Oligonucleotide Discovery and Delivery**
Andy Geall, Ph.D., Vice President, Formulations and Chemistry, Avidity NanoMedicines
Despite the considerable promise, delivery has proven to be one of the central challenges of oligonucleotide-based therapeutics. Oligonucleotides are large, hydrophilic and highly negatively charged, so they don't cross cell membranes. We have pioneered the development of Precision NanoMedicines, which are targeted, polymeric nanoparticles encapsulating siRNA drug payloads for delivery to specific tumor types. These self-assembling nanoparticles can be decorated with antibodies, proteins, peptides and small molecules to bind to extracellular receptors and facilitate cellular uptake.

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2:30 **Intracellular Trafficking of Splice Switching LNA Oligonucleotides**
Cy A. Stein, M.D., Ph.D., Professor, Medicine and Experimental Therapeutics & Molecular and Cellular Biology, City of Hope National Medical Center
We have discovered that after egress from the late endsosome, LNA-SSOs become associated with a protein complex that includes nucleolin and Ago2, in addition to other shuttling proteins. This complex appears to be able to translocate to the nucleus, where it can be observed as "nuclear speckles." Factors that contribute to this translocation, and which synergize with each other as determined by the Chou-Talalay Combination Index method, will be discussed.

3:00 **Sponsored Presentation** *(Opportunity Available)*

3:15 **Refreshment Break in the Exhibit Hall with Poster Viewing**

3:45 **Development of Anti-Fibrosis siRNA Therapeutics Using HKP Polypeptide Nanoparticle Technology**
Patrick Y. Lu, Ph.D., President & CSO, Sirnaomics, Inc.
Using a proprietary and optimized polypeptide-based delivery technology, we have developed the novel anti-fibrotic therapeutics with siRNAs targeting both TGFß1 and Cox-2 simultaneously, for initial indication of skin hypertrophic scar followed with liver fibrosis and other fibrotic conditions. I will discuss the unique advantage of HKP polypeptide nanoparticle technology for efficient siRNA delivery, its pharmaceutical properties for manufacturing and its preclinical safety profile.

4:15 **Self-Delivering RNAi Compounds (sd-rxRNA™) for the Treatment of Ocular Disorders**
Pamela Pavco, Ph.D., Chief Development Officer, RXi Pharmaceuticals Corp.
RXi's proprietary sd-rxRNA® technology is perfectly suited for direct, local delivery to cells and tissues. This aspect has been exploited in our initial two program areas, dermatology and ophthalmology, to evaluate clinically the reduction of connective tissue growth factor as a potential way to decrease the progression of fibrosis in the skin and the eye. An exciting new program uses the sd-rxRNA technology to target checkpoints, singly or in combination, to augment cell therapy in the immuno-oncology space, as a possibility to treat both hematological and solid tumors. An overview of ongoing programs will be provided.

4:45 **Therapeutic Protein Expression *in vivo* Using Messenger RNA-Lipid Nanoparticles**
Thomas D. Madden, Ph.D., President and CEO, Acuitas Therapeutics
Acuitas is developing lipid nanoparticle systems (LNP) that allow the efficient delivery and expression of mRNA via different routes of administration. Biophysical characteristics that facilitate efficient mRNA delivery and provide a favorable safety profile will be discussed. In addition, we will present preclinical results illustrating the potential application of mRNA-LNP therapeutics in a several clinical areas.

5:15 **Close of Conference**

Peptide Discovery and Development

Creating the Next Generation of Peptide Therapeutics

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With over 150 peptide therapeutics in clinical trials and 500 in preclinical development, the peptide area is thriving with new chemistries, designs and discoveries. However, challenges still remain in delivering peptides into patients and, more importantly, keeping them there.

Cambridge Healthtech Institute's Peptide Discovery and Development meeting brings you the latest findings in peptide chemistry, design, delivery, formulation and development, with in-depth case studies and scientific updates from industry's leading players and new, upcoming biotechs, helping you build the next generation of peptide therapeutics.

MONDAY, MARCH 27

7:00 am **Registration and Morning Coffee**

CHALLENGES AND OPPORTUNITIES IN PEPTIDE DISCOVERY AND DEVELOPMENT

8:00 **Welcome Remarks from Conference Director**
Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute

8:10 **Chairperson's Opening Remarks**
Waleed Danho, Ph.D., Distinguished Research Leader and Consultant for Peptides, Danho Associates, Inc., USA

8:15 **FEATURED PRESENTATION: Peptide Drug Hunter: Breakthrough Medicines and Disruptive Innovation**
Tomi Sawyer, Ph.D., Distinguished Scientist, Discovery Chemistry Modalities, Merck Research Laboratories
A deeper understanding of peptide science and technology is driving a renaissance in peptide drug discovery. This presentation will highlight past achievements and future opportunities to advance novel peptide therapeutics in tackling intracellular target space and complex diseases thereof.

8:45 **Strategy for Highly Efficient Synthesis of the Insulin Superfamily Peptides**
Fa Liu, Ph.D., Director, Medicinal Chemistry, Novo Nordisk
Chemical synthesis of the insulin superfamily peptides presents unique challenges associated with peptide assembly and disulfide bond formation. We have established a general biomimetic strategy enabling high efficient synthesis exemplified by insulin, relaxin-2 and insulin-like peptide 5. The key steps involve a unique intermediary and temporary oxime-based ligation that enables high efficient formation of disulfide bonds, and two alkaline mediated degradative steps to yield native heterodimeric insulin-like peptides.

9:15 **DNA-Encoded Libraries of Peptidomimetic Compounds as a Source of Probe Molecules and Drug Leads**
Thomas Kodadek, Ph.D., Professor of Chemistry & Cancer Biology, Chair, Department of Cancer Biology, The Scripps Research Institute
We have collaborated with the laboratory of Brian Paegel at TSRI to develop efficient methods for the synthesis and production of novel DNA-encoded one bead one compound libraries. Unlike most DNA-encoded libraries, these are made by solid-phase synthesis on hydrophilic beads, which confers several advantages, including the ability to rigorously quality control the library. The synthesis and screening of these libraries will be described.

9:45 **Peptide-Based Nanodrugs: A Strategy to Improve Protein Nanoconjugate Efficacies**
Sponsored by PALL FortéBio
Paul Hubbarrd, Project Scientist, Department of Biomedical Sciences, Cedars-Sinai Medical Center
Reducing the size of conjugated nanotherapeutics is a simple strategy that aims to improve drug distribution and cellular uptake. We have taken a high-affinity trastuzumab-conjugated lead and reduced its size in part by exchanging the conjugated monoclonal antibody with a lower affinity 12-mer anti-HER2/neu peptide. Surface plasmon resonance shows that while conjugation reduces the peptide's affinity for recombinant Her2 receptor, the off-rate is relatively unaffected, and *in vivo* efficacy is comparable to the antibody-conjugated form.

10:15 **Coffee Break in the Exhibit Hall with Poster Viewing**

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PEPTIDE DESIGN AND DISCOVERY

10:45 **Discovery of Novel Ion Channel Modulators from Animal Venoms**
Laurent Bialy, Ph.D., Section Head, R&D IDD Medicinal Chemistry, Research & Development Integrated Drug Discovery Medicinal Chemistry, Sanofi
Animal venoms are potentially a valuable resource for finding new drug candidates. Despite several examples in recent literature, only a minute fraction of animal toxins has been discovered so far. The structure elucidation and synthesis of new ion channel modulators identified from an animal venom screening campaign will be presented. First *in vitro* and SAR results will be discussed in the context of a recent drug discovery project.

11:15 **Engineered Dengue EDIIIs as Broad Immunogens**
Jonathan R. Lai, Ph.D., Associate Professor, Department of Biochemistry, Albert Einstein College of Medicine
Dengue virus is a flavivirus that causes hundreds of millions of infections world-wide each year. There are four serotypes of Dengue (DENV1-4) that co-circulate in hyperendemic regions. Primary infection by a single serotype results in febrile illness, but secondary infections by heterotypic serotypes can lead to severe disease. We will discuss structure-based protein design and phage display approaches to develop novel broad immunogens (subunit vaccines) against all four DENV serotypes.

11:45 **Brain-Shuttle-BACE1 Peptide Inhibitor Conjugates Lower Brain Aβ Levels**
Eric Kitas, Ph.D., Senior Scientist, Roche

12:15 pm **Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

PEPTIDES FOR CNS AND CARDIOVASCULAR

1:55 **Chairperson's Remarks**
Soumitra S. Ghosh, Ph.D., CSO, Avexegen Therapeutics, Inc.

2:00 **The CS6253 ABCA1 Agonist Peptide Reverses apoE4 Alzheimer's Disease**
Jan Johansson, M.D., Ph.D., CEO & President, Artery Therapeutics, Inc.
Apolipoprotein E4 (apoE4) is the strongest risk factor for Alzheimer's disease (AD) and no treatment is available. This presentation will show how we contrasted CS6253 peptide effects in apoE4 and apoE3 astrocytes (cholesterol efflux) and target replacement (TR) mice (brain phenotype and cognition). ABCA1 agonist treatment by CS653 resulted in improved apoE4 lipidation in cells and in mice. Six weeks of treatment counteracted apoE4-driven AD with regard to both phenotype and cognition.

2:30 **NA-1: The Development of an Intracellular Peptide Therapeutic for Stroke from Animal Studies through the Late Stage Clinical Studies ESCAPE-NA1 and FRONTIER**
Dave Garman, Ph.D., CTO, NoNO, Inc.
NA-1 is a novel peptide therapeutic shown to reduce brain ischemia and improve neurological function in rodent and primate models of stroke. The FRONTIER trial is assessing the efficacy of administering NA-1 in the ambulance as soon as possible after the onset of stroke, and the ESCAPE-NA-1 trial is testing the effect of NA-1 in acute ischemic stroke patients who are candidates for endovascular reperfusion.

3:00 **Discovery and Development of KISS1R Agonists as Clinical Peptide Therapeutics**
Taiji Asami, Ph.D., Associate Director, Medicinal Chemistry Research Laboratories, Pharmaceutical Research Division, Takeda
Kisspeptin (Kp) and its receptor KISS1R play critical roles in the regulation of the hypothalamic–pituitary–gonadal (HPG) axis. Highly potent and stable nonapeptides TAK-683 and TAK-448 were rationally designed and synthesized as analogues of the N-terminally truncated decapeptide of Kp. Continuous dosing of TAK-683 and TAK-448 effectively suppressed the HPG axis, which indicates the potential of these investigational drugs for sex-hormone-dependent diseases.

3:30 **Refreshment Break in the Exhibit Hall with Poster Viewing**

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PEPTIDES FOR GASTROINTESTINAL DISORDERS

4:00 **A Novel Mucosal Healing Therapy for Inflammatory Gastrointestinal Disorders**
Soumitra S. Ghosh, Ph.D., CSO, Avexegen Therapeutics, Inc.
Avexegen Therapeutics is developing Neuregulin-4 (NRG-4), a naturally occurring constituent of human breast milk, as a novel mucosal healing peptide therapeutic for inflammatory disorders of the gastrointestinal tract. NRG-4 leverages physiological mechanisms to both directly protect the intestinal epithelium and to attenuate colonic macrophage-driven inflammatory cascade. These actions have been shown to promote mucosal healing and restoration of the damaged gut barrier in multiple experimental animal models of intestinal inflammation.

4:30 **Evolving Clinical Profile of Linaclotide: Driving Innovation for Patients with Functional GI Disorders**
Mark G. Currie, Ph.D., President and CSO, Ironwood Pharmaceuticals
Linaclotide is a synthetic 14-amino acid peptide that is approved for treatment of adults with chronic constipation and irritable bowel with constipation (IBS-C). In animal models, linaclotide acts through stimulation of guanylate cyclase-C to decrease abdominal pain. Based on these findings, and our experience with decreasing pain in the IBS-C patients, we continue to study these actions as we seek to advance and expand the clinical profile of this peptide.

5:00 **Sponsored Presentation** *(Opportunity Available)*

5:30 **Welcome Reception in the Exhibit Hall with Poster Viewing**

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6:15 **Short Course Registration**

TUESDAY, MARCH 28

7:30 am **Breakfast Roundtable Discussions**
Grab a cup of coffee and join a roundtable discussion. These are moderated discussions with brainstorming and interactive problem solving, allowing participants from diverse backgrounds to exchange ideas and experiences and develop future collaborations around a focused topic.
For more details, please visit OPTcongress.com

NON-INVASIVE PEPTIDE DELIVERY

8:25 **Chairperson's Remarks**
Ved Srivastava, Ph.D., Vice President, Peptide Chemistry, Intarcia Therapeutics

8:30 **FEATURED PRESENTATION: Delivery of Peptides by Non-Invasive Routes: Focus on Successful Oral Technologies Progressing in the Clinic and Future Challenges**
Joël Richard, Ph.D., Senior Vice President, Peptides Development, Ipsen
Injectable sustained-release peptide formulations based on biodegradable microparticles or implants have been very successful to enhance patient adherence and convenience, and increase safety and efficacy. They are likely to remain a significant and important part of the new peptide products coming to the market. However, the tremendous developments in alternative non-invasive routes of delivery are likely to result in more and more peptides being delivered by the transdermal, nasal, inhalation and oral routes. The main purpose of this talk will be to analyze and compare the various alternative non-invasive peptide delivery technologies progressing in the clinic, discussing the pros and cons of these technologies in regards to stability, bioavailability, safety/efficacy balance, impact on costs of goods and manufacturability.

Peptide Discovery and Development

Creating the Next Generation of Peptide Therapeutics

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9:00 **Oral Bioavailability beyond the Rule of 5: Attempts at Oral Delivery of a Series of Short Unnatural Peptides**
Claudio Mapelli, Ph.D., Principal Scientist, Discovery Chemistry Modalities, Merck Research Laboratories
Cell membrane permeability and oral bioavailability of peptides remain a challenge, in spite of recent advances in peptide drug design and formulation methods. This presentation will focus on a number of approaches investigated to orally deliver a new class of re-designed GLP-1 peptides with improved stability. Key findings will be presented on how bioavailability was modulated using structural modifications and formulation methodologies. Lessons learned will be reviewed and utilized to define some of the challenges in oral delivery of rule of 5 breakers.

9:30 **Oral Inhalation – A Simple Approach to Peptide Delivery**
John Freeman, Ph.D., Principal Scientist, Formulations Development Department, Mannkind Corporation
Oral inhalation, a viable option for peptide drug delivery, provides benefits such as rapid onset of action, avoidance of first pass metabolism, and elimination of injections. While oral inhalation is the standard for treating pulmonary diseases, only within the last decade has it been applied to therapies requiring systemic exposure. Development of orally inhaled dry powders will be exemplified with non-clinical data in the therapeutic areas of diabetes, obesity, and pain.

10:00 **Coffee Break in the Exhibit Hall with Poster Viewing**

IMPROVING PEPTIDE DELIVERY

10:40 **Chairperson's Remarks**
Ved Srivastava, Ph.D., Vice President, Peptide Chemistry, Intarcia Therapeutics

10:45 **Oral Insulin for Diabetes Treatment: Bypassing the Roadblock**
Miriam Kidron, Ph.D., CSO and Director, Oramed Pharmaceuticals, Inc.
A novel platform for preserved bioavailability and effective delivery of protein and peptide therapeutics will be described. Results demonstrating applicability of this platform toward delivery of insulin and exenatide to manage diabetes, as assessed in animal models, healthy volunteers and in both type 1 and type 2 diabetes patients, will be presented. In addition, the physiological benefits of oral versus subcutaneous insulin delivery will be discussed.

11:15 **Peptide Formulation: Challenges and Strategies**
Sunita Yadav, Ph.D., Senior Scientist, Chemical and Pharmaceutical Profiling, Novartis
Peptide therapeutics is becoming an important segment of the pharmaceutical industries. There have been tremendous advances in production of the active peptide sequences; however, delivery and formulation still remains as a challenging task. The relationship between the clinical administration, patient compliance, pharmacokinetics, toxicity and physicochemical properties of the drug must be considered and balanced to successfully develop a peptide drug.

11:45 **Sponsored Presentation** *(Opportunity Available)*

12:00 pm **Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

DELIVERING POTENT, STABLE AND LONG-LASTING PEPTIDES

1:25 **Chairperson's Remarks**
Joël Richard, Ph.D., Senior Vice President, Peptides Development, Ipsen

1:30 **FEATURED PRESENTATION: Strategies for Peptide Optimization and Sustain Delivery for Longer than Once a Week**
Ved Srivastava, Ph.D., Vice President, Peptide Chemistry, Intarcia Therapeutics
Peptide therapeutics continue to develop biopharmaceutical pipelines, and a number of GLP-1 analogs made remarkable commercial successes. Design and development strategies for a potent and stable peptide including longer duration of action are still challenging. In this presentation, we will discuss overcoming challenges from BID to once a year/half yearly delivery of GLP-1 agonist.

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2:00 **Harnessing Transthyretin to Enhance the Circulation Half-Life of Peptides**
Mamoun Alhamadsheh, Ph.D., Associate Professor, Department of Pharmaceutics & Medicinal Chemistry, University of the Pacific
The tremendous therapeutic potential of peptides has not yet been fulfilled. A major challenge impeding the widespread use of peptides as therapeutics is their poor pharmacokinetic profile, due to short *in vivo* half-life. In this talk, we will discuss our effort in developing a fundamentally new approach for enhancing the *in vivo* half-life of peptides without affecting its biological activity, which could decrease dosing frequency and improve patient compliance.

2:30 **Transdermal Approach to Peptide Delivery**
Alan Smith, Ph.D., Vice President and Co-Founder, 4P Therapeutics
Transdermal delivery offers a needle-free alternative to injection of peptides and avoids the disadvantages associated with the pulmonary/nasal and oral routes. Currently marketed transdermal products are limited to small lipophilic molecules that can penetrate the skin. Various approaches have been developed to overcome the skin barrier to enable peptide delivery at therapeutic levels in humans. An overview of the field will be presented and the limitations of the transdermal route and challenges for commercialization will be discussed.

3:00 **Sponsored Presentation** *(Opportunity Available)*

3:15 **Refreshment Break in the Exhibit Hall with Poster Viewing**

MANUFACTURING AND CMC STRATEGIES

3:40 **Chairperson's Remarks**
Waleed Danho, Ph.D., Distinguished Research Leader and Consultant for Peptides, Danho Associates, Inc., USA

3:45 **MedImmune's Peptide Development Strategy**
Lutz Jermutus, Ph.D., Senior Director, Product Development Team Leader, Cardiovascular & Metabolic Diseases iMED, MedImmune

4:15 **Automated Flow Peptide Synthesis: Toward Amide Bonds at Nature's Pace**
Bradley L. Pentelute, Ph.D., Professor Pfizer-Laubach Career Development, Department of Chemistry, Associate Member, Broad Institute of Harvard and MIT
This presentation describes a rapid flow solid phase peptide synthesis methodology that enables incorporation of an amino acid residue in 40 seconds with amide-bond formation taking only 5 seconds. To demonstrate the broad applicability of this method, it was employed to synthesize hundreds of peptides and proteins.

4:45 **Leveraging Biophysical Characterization in the Formulation Development of Multi-Dose Aggregation-Prone Peptides**
Jingtao Zhang, Ph.D., Principle Scientist, Merck
Synthetic peptides are conformationally more flexible than protein or other large biomacromolecules due to the relatively small size. As a result, the development of aggregation-prone peptides as a therapeutic could be highly challenging. The presentation will focus on the characterization of reversible and irreversible fibril aggregates in peptide formulations, biophysical assays that guide AME study design, and high resolution biophysical study to enable insights in formulation stabilization mechanism. Overall considerations on the development of multi-dose peptide formulations as well as strategies in overcoming the challenges will also be highlighted.

5:15 **Close of Conference**

Register by February 10
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Oligonucleotides for Cancer Immunotherapy

Advances in Immune-Modulatory Nucleic Acids

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As waves of novel immunomodulatory agents continue to progress into clinical studies, much has focused on the development of antibodies, bi-specifics, and chimeric receptors where toxicity can be a concern. Advances in the discovery and development of nucleic acids with the ability to overcome immunosuppressive mechanisms, increase tumor immunogenicity and provide agonistic costimulation are now providing a safer and effective approach to modulate the tumor immune response.

Cambridge Healthtech Institute is proud to introduce the Inaugural Oligonucleotides for Cancer Immunotherapy symposium, designed to convene immuno-oncology researchers and drug developers to discuss advances in the development of oligonucleotides with application to cancer immunotherapy.

WEDNESDAY, MARCH 29

7:45 am **Breakfast Presentation** *(Sponsorship Opportunity Available)* **or Morning Coffee**

TOLL-LIKE RECEPTOR (TLR) AGONISTS AND CHECKPOINT COMBINATIONS

8:25 **Chairperson's Opening Remarks**
Art Krieg, M.D., Founder and CEO, Checkmate Pharmaceuticals

8:30 **Creating a Beneficial Tumor Microenvironment for Effective Cancer Immunotherapy**
Sudhir Agrawal, D.Phil., FRSC, President, Research, Idera Pharmaceuticals
We are evaluating the role of endosomal Toll-like receptors 3, 7, 8, and 9, in the TME by administering intratumorally an agonist of these receptors. IMO-2125, a TLR9 agonist, has shown potent anti-tumor activity following i.t. administration and has led to an abscopal effect. These anti-tumor activities are associated with beneficial changes in the TME, which potentiated the anti-tumor activity of anti-CTLA4, anti-PD1, and IDO-1 inhibitor. A Phase I/II trial of intratumoral IMO-2125 in combination with ipilimumab in PD-1 refractory melanoma patients is in progress.

9:15 **FEATURED PRESENTATION: Making "Cold" Tumors "Hot" with Intratumoral Injection of CpG-A Oligonucleotide**
Art Krieg, M.D., Founder and CEO, Checkmate Pharmaceuticals
CMP-001 is a formulation of a CpG-A oligonucleotide, G10, within a virus-like particle, Qb. We are performing a Phase Ib dose escalation study of intratumoral CMP-001 given in combination with systemic pembrolizumab in advanced melanoma patients whose cancer has previously progressed on an anti-PD-1 Ab, or who have failed to respond to at least 12 weeks of such therapy.

10:00 **Networking Coffee Break**

NUCLEIC ACID DISCOVERY PLATFORMS FOR CANCER IMMUNOTHERAPY

10:30 **Immunomodulatory Spherical Nucleic Acids**
David Giljohann, Ph.D., CEO, Exicure
Immunomodulatory nucleic acids act by agonizing or antagonizing endosomal toll-like receptors (TLR3, TLR7/8, and TLR9), proteins involved in innate immune signaling. Immunomodulatory spherical nucleic acids (SNAs) that stimulate immunity by engaging TLRs have been designed, synthesized, and characterized. Given the clinical potential of SNAs due to their potency, defined chemical nature, and good tolerability, SNAs are attractive new modalities for developing immunotherapies. We will provide an update on our preclinical work and clinical plans for AST-008, a TLR9 agonist.

11:00 **Development of Pluripotent Stem Cell-Based Therapies for Neurologic and Oncologic Disorders**
Casey Case, Ph.D., Senior Vice President, Research and Nonclinical Development, Asterias Biotherapeutics
Our group has established protocols to produce oligodendrocyte progenitors that upon transplantation into animals with spinal cord injuries can remyelinate denuded axons, induce axonal sprouting, and improve locomotor activity. Extensive preclinical studies have been completed to examine the activity, biodistribution, dosing, delivery, and potential toxicity and tumorigenicity of the oligodendrocyte progenitors. In collaboration with Cancer Research UK, Asterias

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Oligonucleotides for Cancer Immunotherapy

Advances in Immune-Modulatory Nucleic Acids

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is preparing for a clinical trial using these hESC derived dendritic cells as a cancer immunotherapy in non-small cell lung carcinoma in the neoadjuvant setting.

11:30 **RNAi-Mediated β-Catenin Inhibition Promotes T-Cell Infiltration and Anti-Tumor Activity in Combination with Immune Checkpoint Blockade**

Shanthi Ganesh, Ph.D., Associate Director, Preclinical Oncology, Dicerna Pharmaceuticals, Inc.

Recent research strongly suggests an important role for Wnt/β-catenin signaling in mediating cancer immune evasion and resistance to immune checkpoint therapy. The mechanism by which Wnt/β-catenin signaling causes checkpoint resistance involves suppression of specific cytokines which trigger immune cell recruitment to the tumor, resulting in the phenomenon of T-cell exclusion and rendering the tumor to a non-inflamed state. Therefore, β-catenin inhibition might increase the low response rate to immunotherapy in numerous cancer populations. In this presentation, we will introduce DCR-BCAT, a novel RNAi-based experimental drug targeting β-catenin. DCR-BCAT is particularly effective at converting non-inflamed tumors to inflamed tumors in multiple, diverse preclinical models. Additionally, DCR-BCAT sensitized tumors to checkpoint inhibition in both syngeneic and transgenic tumor models. These data offer proof-of-concept for β-catenin inhibition as an effective strategy to improve responses to PD-1/PD-L1 therapy, and support clinical evaluation of this combination approach using a first-in-class RNAi-based agent.

12:00 pm **Sponsored Presentation** *(Opportunity Available)*

12:15 **Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

NOVEL IMMUNOMODULATORY NUCLEIC ACIDS

1:25 **Chairperson's Remarks**

Alexey Eliseev, Ph.D., Chief Business Officer, RXi Pharmaceuticals

1:30 **Targeting Immune Checkpoints Using Self-Deliverable RNAi Technology**

Alexey Eliseev, Ph.D., Chief Business Officer, RXi Pharmaceuticals

RNAi is a potent tool to regulate gene expression. Self-deliverable RNAi technology is based on chemical modification of siRNAs eliminating any need for transfection formulations or methods, emulating behavior of small molecule drugs. We have demonstrated efficient knockdown of multiple immune checkpoints in T cells and the increased antitumor efficacy of sdRNA-modified immune cells.

2:00 **Development of SB 11285, a Highly Potent STING Agonist for Application in Immune-Oncology**

R.P. (Kris) Iyer, Ph.D., Co-Founder & CSO, Spring Bank Pharmaceuticals

STING is an intracellular adapter protein that activates IFN and NF-KB pathways to induce potent innate and adaptive response against cancer. We have discovered the cyclic dinucleotide SB 11285 as a highly potent STING agonist for immuno-oncology applications for a variety of cancers. This talk will focus on the key aspects of development of this next-generation anticancer agent.

2:30 **FEATURED PRESENTATION: Selective Stimulation of RIG-1 with Novel Synthetic RNA Induces Strong Anti-Tumor Immunity in Mouse Tumor Models**

Jim Barsoum, Ph.D., Chair, Scientific Advisory Board, Rigontec GmbH

The cytosolic RNA receptor RIG-I recognizes double-stranded RNA bearing a 5'-triphosphate. RIG-I activation induces apoptosis preferentially in tumor cells and activates the immune system via type I interferon signaling. We developed an optimized, synthetic oligonucleotide, RGT100, as a RIG-I selective ligand. RGT100 is progressing toward clinical evaluation in cancer patients and will be evaluated for single-agent activity as well as in combination with checkpoint inhibitors.

3:00 **Networking Refreshment Break**

APTAMER-BASED CANCER IMMUNOTHERAPY

3:15 **Inducing Neoantigens in Therapeutic and "Prophylactic" Cancer Immunotherapy**

Brett Schrand, Ph.D., Postdoctoral Research Associate, Gilboa Lab, Leonard Miller School of Medicine, University of Miami

Tumor recurrence remains a major challenge in cancer therapy and individuals with premalignant lesions, chronic infections, or genetic predisposition are

Oligonucleotides for Cancer Immunotherapy

Advances in Immune-Modulatory Nucleic Acids

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at high risk of developing cancer. Whereas clinical experience with checkpoint blockade has underscored the importance of vaccinating against neoantigens, their identification on a patient-by-patient basis is facing significant hurdles. We have previously described an aptamer-targeted siRNA approach to induce neoantigens in tumor cells *in situ* that obviates the limitations of targeting endogenous neoantigens. Exploiting the ability to induce neoantigens *de novo*, here I will describe a vaccination strategy for patients in remission and for individuals at risk of developing cancer that will control the growth of the future tumor.

3:45 **Targeting Co-Stimulation with Bi-Specific Aptamers to Melanoma Cancer Stem Cells**

Fernando Pastor Rodríguez, Ph.D., Principal Investigator, Aptamers, Molecular Therapy, CIMA

We have developed an aptamer that binds to multidrug-resistant protein 1 (MRP1), which is not only expressed in tumor variants with higher resistance to chemotherapy but also in cancer stem cells. Anti-tumor effect with the systemic injection of the bi-specific-aptamer was proved in combination with peptide inhibition of Foxp3 in Treg or with CTLA4 blockade in an aggressive low-immunogenic melanoma tumor in mice. The bi-specific costimulatory aptamers could also be used to generate an *ex vivo* whole-cell cancer vaccine (Aptvax).

4:15 **Close of Symposium**

Peptides in Cancer Therapy

Empowering Peptide Vaccines, Immunotherapies and Conjugates to Treat Cancer

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The unique properties of peptides, namely their specificity, small size, low levels of toxicity, ease of synthesis and modification, are driving an increasing interest in peptides as an effective treatment of cancer, particularly in immuno-oncology.

Cambridge Healthtech Institute's Peptides in Cancer Therapy symposium provides a unique opportunity to discuss the latest developments in peptide-based cancer therapy with dedicated sessions on peptide cancer vaccines, immunotherapies and conjugates. Attention will also be paid to inducing immune responses, improving delivery and minimizing toxic side effects, supported by the latest scientific findings and company updates.

WEDNESDAY, MARCH 29

7:45 am **Breakfast Presentation** *(Sponsorship Opportunity Available)* **or Morning Coffee**

PEPTIDE-BASED VACCINES IN ONCOLOGY

8:25 **Chairperson's Opening Remarks**
Jesse Dong, Ph.D., Vice President, Peptide Chemistry, Neon Therapeutics

8:30 **FEATURED PRESENTATION: Advantages of a Peptide-Based Vaccine Designed to Activate CD4+ T Cells in Cancer Immunotherapy**
Eric von Hofe, Ph.D., President, Antigen Express
The low toxicity and high specificity of peptides make them attractive as drug development candidates for a variety of applications, including cancer vaccines. We have developed a modified HER2 peptide cancer vaccine that specifically activates both CD4+ and CD8+ T cells. In addition to long-lasting and specific CD4+ and CD8+ T cell responses, encouraging efficacy data in breast cancer patients has been observed in a controlled, randomized Phase II study.

9:00 **Epitope-Enhanced Peptide Vaccine for Prostate Cancer: Translation from Mice to Human Clinical Trials**
Jay A. Berzofsky, M.D., Ph.D., Branch Chief, Vaccine Branch, Center for Cancer Research, National Cancer Institute, NIH
We developed an epitope-enhanced-peptide prostate cancer vaccine using epitopes from TARP antigen. Epitope enhancement increased affinity for HLA-A2.1. Peptides induced CD8 T cell responses in HLA-transgenic mice and human T cells that killed human tumor cells. In a Phase I trial in stage D0 prostate cancer with rising PSA after tumor resection, 74% of vaccinated patients at one year had a decreased PSA slope (p=0.0004). A Phase II trial is underway.

9:30 **Development Strategies of Neo-Antigen-Based Personalized Cancer Vaccines**
Jesse Dong, Ph.D., Vice President, Peptide Chemistry, Neon Therapeutics
Neoantigens arise as a result of somatic mutations during the development and progression of tumors. They are inherently non-self and are seen as foreign by the immune system. Mounting evidence suggests that immune rejection of tumours, for example which is seen with checkpoint inhibitors, may be mediated by recognition of neoantigens. Neon's personalized cancer vaccine targets neoantigens unlocking the immune system to attack tumors.

10:00 **Networking Coffee Break**

PEPTIDE-BASED VACCINES IN ONCOLOGY

10:30 **Paradigm Shift in Oncology: Translating B Cell Epitope Peptide-Based Cancer Vaccines to the Clinic and Future Combination Immunotherapies**
Pravin T.P. Kaumaya, Ph.D., Professor, Division of Vaccine Development/Peptide & Protein Engineering Laboratory, Ohio State University
We have created and established a portfolio of validated peptide epitopes against multiple receptor tyrosine kinases and we have identified the most biologically effective combinations of EGFR (HER-1), HER-2, HER-3, VEGF and IGF-1R peptide vaccines/mimics to selectively inhibit multiple receptors and signaling pathways. This presentation will detail our ongoing studies based on the development of combinatorial immunotherapeutic strategies that act synergistically to enhance immune-mediated tumor killing aimed at addressing mechanisms of tumor resistance for several tumor types.

Peptides in Cancer Therapy

Empowering Peptide Vaccines, Immunotherapies and Conjugates to Treat Cancer

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11:00 **Inducing Immune Responses to Tumor-Associated Carbohydrate Antigens by a Carbohydrate Mimetic Peptide Vaccine: Clinical Experience in Phase I and Phase II Trials in Breast Cancer Patients**

Thomas Kieber-Emmons, Ph.D., Professor, Pathology, Winthrop P. Rockefeller Cancer Institute, University of Arkansas for Medical Sciences

We have developed a first-in-man peptide based vaccine that induces antibodies cross-reactive with Tumor Associated Carbohydrate Antigens (TACA). This peptide is a computer engineered pan mimic of TACAs. Immunization with this vaccine was found to be safe and tolerable in Phase I and II clinical trials, and induces functional antibodies that display a cell-death mediated therapeutic benefit. The data suggest that the vaccine-induced anti-tumor immune response in combination with standard of care chemotherapy may further improve clinical outcome.

11:30 **Vaccines for the Prevention of Breast Cancer**

Keith L. Knutson, Ph.D., Professor, Immunology, Director, Mayo Clinic Florida Cancer Research Program, Mayo Clinic Center for Immunology and Immune Therapies

Breast cancer causes 500,000 deaths each year. Rather than continue to add to the costs of treatment, we should advance promising approaches for prevention such as vaccination. One approach for a breast cancer vaccine is to target aberrantly expressed 'self' antigens. To produce such a vaccine, we're aiming to achieve key milestones, namely identifying peptide epitopes from cancer antigens and identifying systems that induce durable T cell responses.

12:00 pm **Sponsored Presentation** *(Opportunity Available)*

12:15 **Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

PEPTIDE-DRUG CONJUGATES

1:25 **Chairperson's Remarks**

Pravin T.P. Kaumaya, Ph.D., Professor, Division of Vaccine Development/Peptide & Protein Engineering Laboratory, Ohio State University

1:30 **Synthesis, Purification and Formulation of a New T Cell Vaccine Using 5 Novel Proteins**

John N. Bonfiglio, Ph.D, MBA, President and COO, TapImmune

TPIV 200 is a T cell vaccine targeting Folate Receptor Alpha-a receptor overexpressed in ovarian, triple negative breast and non-small cell lung cancers. After initial positive results in an investigator-sponsored Phase I study, it was essential to develop a stable, soluble formulation that could be used for Phase II and beyond. The work included solving some difficult purification problems and formulating the product to be compatible with other additives such as adjuvants.

2:00 **Evaluating Multivalent Peptidomimetic Conjugates as Therapeutics for Castration-Resistant Prostate Cancer**

Kent Kirshenbaum, Ph.D., Professor, Chemistry, NYC Chemistry

There is an urgent need for new strategies to block androgen receptor (AR) function in prostate cancer. We describe sequence-specific peptoid oligomers as scaffolds for constructing precise multivalent displays of AR ligands. The peptoid conjugates suppress proliferation of multiple AR-expressing prostate cancer cell lines including those that are resistant to enzalutamide and ARN509. The conjugates display attractive pharmacological characteristics and deter tumor growth in mouse xenograft models of prostate cancer.

2:30 **Development of BT1718, a Bicycle Peptide-Drug Conjugate (BDC) Targeting MT1-MMP for the Treatment of Solid Tumors**

Peter Parker, VP, Oncology R&D, Bicycle Therapeutics

MT1-MMP (MT1) is a cell surface membrane protease normally involved in tissue remodeling and overexpression of MT1 has been linked to cancer invasiveness and poor prognosis in many solid tumors. BT1718 is a Bicycle drug conjugate (BDC) containing the optimized MT1-binding peptide covalently linked through a hindered disulfide linker to the potent anti-tubulin agent DM1. BT1718 shows potent antitumor activity in human solid tumor xenograft models in mice, including models of fibrosarcoma, lung and breast cancer, at doses that are well tolerated, demonstrating the therapeutic potential of this small molecule targeting approach for the treatment of cancer.

3:00 **Networking Refreshment Break**

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PEPTIDES-DRUG CONJUGATES AND TARGETING MCL-1

3:15 **Peptide-Drug Conjugates for Targeting Breast Cancer**

Kamaljit Kaur, Ph.D., Associate Professor and Director, Center for Targeted Drug Delivery, Chapman University School of Pharmacy

Chemotherapeutic agents, like doxorubicin and paclitaxel, are potent cytotoxic agents that enter healthy tissues in the body with indiscriminate toxicity and do not preferentially accumulate at tumor sites. Chemotherapeutics can be conjugated to cancer-targeting peptides for specific delivery to cancer site. In this talk, our strategies for targeting breast cancer cells using cancer-targeting peptides and peptide-doxorubicin conjugates will be presented.

3:45 **Hydrocarbon Stapled Peptide Antagonists as Novel Agents for Targeting MCL-1**

Raheleh Rezaei-Araghi, Ph.D., Postdoctoral Associate, Biology Department, MIT

Mcl-1 has emerged as a widely expressed pathologic factor in human cancer. Previous studies indicate that hydrocarbon stapling can confer improved affinity for Mcl-1. The virtually unlimited structural variations within this design scheme present a challenge. To address this problem, we applied synthetic libraries, which enable the construction and screening of chemically modified repertoires of peptidic scaffolds. By applying this method, molecules with high affinity and selectivity were identified.

4:15 **Close of Symposium**

Oligonucleotide and Peptide Therapeutics BOSTON STUDENT FELLOWSHIP

Full-time graduate students and Ph.D. candidates are encouraged to apply for the Oligonucleotide and Peptide Therapeutics Boston Student Fellowship. Applications are due by January 27, 2017.

- Interested students must complete the application for the 2017 Student Fellowship which can found on OPTCongress.com.
- Fellows are required to present a scientific poster. A poster title and abstract are due at the time of the application.
- All applications will be reviewed by the scientific review committee and the accepted students will be notified no later than February 3, 2017 if they were accepted for the 2017 Student Fellowship.
- Accepted 2017 Student Fellows will receive a discounted conference rate of $195*, which must be paid in full by February 17, 2017. Credit card information is requested at the time of the application and will be charged upon application approval.
- This fellowship is limited to 10 students and is for the Best Value Package (includes access to entire 2-day Oligonucleotide and Peptide Therapeutics programs AND the 1-day Symposia), March 27-29, 2017.
- All accepted 2017 Student Fellows will be asked to help promote the conference onsite at their college, and throughout their social media networks.
- Students not accepted for the 2017 Student Fellowship, can register at a discounted rate $295*, and will not be required to present a poster.

Please visit https://chidb.com/reg/ops/reg_fellowship.asp for more information about the Student Fellowship.



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Pricing and Registration Information

SHORT COURSE PRICING

	Commercial	Academic, Government, Hospital-affiliated
Single Short Course	$699	$399
Double Short Course	$999	$699

March 26, 2017 5 - 8 pm	March 27, 2017 6:30 - 9:30 pm
SC1: Oligonucleotide Therapeutics: From Discovery to Manufacturing	SC3: CRISPR-Based Gene Editing for Targeted Therapies
~~SC2: Macrocyclic and Constrained Peptides~~	~~SC4: CMC and Scale-Up Strategies for Peptides~~

CONFERENCE PRICING

BEST VALUE REGISTRATION (Includes access to entire 2-day Oligonucleotide and Peptide Therapeutics programs, as well as 1-day Symposia. Does not include access to any short courses)

Advance Registration Discount until February 10, 2017	$2249	$1129
Registrations after February 10, 2017, and on-site	$2449	$1199

BASIC PACKAGE (Includes access to 2-day Oligonucleotide and Peptide Therapeutics programs. Does not include access to any short courses or symposia)

Advance Registration Discount until February 10, 2017	$1799	$879
Registrations after February 10, 2017, and on-site	$1999	$1029

SYMPOSIA REGISTRATION (Includes access to symposia. Does not include access to conference programs or short courses)

Symposia	$929	$549

March 27 - 28, 2017 (Concurrent Tracks)	March 29, 2017 (Concurrent Symposia)
Oligonucleotide Discovery and Delivery	Oligonucleotides for Cancer Immunotherapy
Peptide Discovery and Development	Peptides in Cancer Therapy

CONFERENCE DISCOUNTS

Poster Submission - **Discount ($50 Off)**: Poster abstracts are due by February 17, 2017. Once your registration has been fully processed, we will send an email containing a unique link allowing you to submit your poster abstract. If you do not receive your link within 5 business days, please contact jring@healthtech.com. *CHI reserves the right to publish your poster title and abstract in various marketing materials and products.

REGISTER 3 - 4th IS FREE: Individuals must register for the same conference or conference combination and submit completed registration form together for discount to apply.

Alumni Discount: Cambridge Healthtech Institute (CHI) appreciates your past participation at Oligonucleotide and Peptide Therapeutics Boston. As a result of the great loyalty you have shown us, we are pleased to extend to you the exclusive opportunity to save an additional 20% off the registration rate.

Group Discounts: Discounts are available for multiple attendees from the same organization. For more information on group rates contact Jeff Knight at +1-781-247-6264, jknight@healthtech.com.

If you are unable to attend but would like to purchase the Oligonucleotide and Peptide Therapeutics Boston (OPT Boston) 2017 CD for $750 (plus shipping), please visit OPTCongress.com. Massachusetts delivery will include sales tax.

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Each registration includes all conference sessions, posters and exhibits, food functions, and access to the conference proceedings link.

Handicapped Equal Access: In accordance with the ADA, Cambridge Healthtech Institute is pleased to arrange special accommodations for attendees with special needs. All requests for such assistance must be submitted in writing to CHI at least 30 days prior to the start of the meeting.

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