

**The only event in Asia with comprehensive updates on therapeutics in development
from major big pharma, biotech and academic scientists worldwide**

IBC's 4th Annual

AsiaTIDES

Oligonucleotide and Peptide®
Research, Technology and Product Development

February 28 – March 1, 2012

**Sheraton Miyako Hotel
Tokyo, Japan**

Keynote Presentations

Clinical Development of Mipomersen, an Antisense Second-Generation Oligonucleotide Targeting Apolipoprotein B

Tejdeep Singh, M.D.

Medical Director, Global Patient Safety and Risk Management, Genzyme Corporation, USA

Oligonucleotide and Delivery Platform Optimization Strategies for siRNA Drug Development



Laura Sepp-Lorenzino, Ph.D.

*Senior Director, RNA Therapeutics
Merck & Co., Inc., USA*

Recent Advances in Routes of Delivery for Peptide Pharmaceuticals



Nozer Mehta, Ph.D.

*Vice President, R&D
UniGene Laboratories, Inc., USA*

Overview of Peptide Therapeutics and Vaccines in Development



Yuji Heike, M.D., Ph.D.

*Chief of Medical Staff, Department of Medical Oncology
National Cancer Center Hospital, Japan*

Evolution and Development of GLP-1 Based Therapeutics



Andrew A. Young, M.D., Ph.D.

*Vice President, Head, Enterendocrine Biology
GlaxoSmithKline, USA*

A Pivotal Year for Oligonucleotide Therapeutics



Arthur A. Levin, Ph.D.

*Chief Development Officer
Santaris A/S, USA and Denmark*

RaPID Selection of a New Class of Peptide Drug Leads against Various Therapeutic Targets



Hiroaki Suga, Ph.D.

*Professor, Chemical Biology and Biotechnology Lab
The University of Tokyo, Japan*

- Regulatory Updates: Preclinical Safety Assessment of Oligonucleotides and Peptides including Reports from JPMA and the Oligonucleotide Safety Working Group on ICH S6 and Off Target Effects
- miRNA, Oligonucleotides, Peptides and Peptide Vaccines: Updates on therapeutic candidates in preclinical and clinical development
- Manufacturing and analytical development strategies to speed process development
- Formulation and delivery updates to help you gauge product marketability

In-Depth Tutorials to Increase your Knowledge:

- Technical and Regulatory Aspects of Peptide Specifications and Characterization: From Preclinical through to Market Application
- Strategies and Approaches for *in vivo* Delivery of Oligonucleotides

"My first time attending AsiaTIDES was very enjoyable. AsiaTIDES brought my company meaningful information widely. I was very happy meeting the delegates from American/Europe, and Asian companies... The balance was good in oligonucleotide and peptide. I enjoyed conference hopping."

*Dr. Yoshitsugu Akiyama, Senior Research Scientist,
NOF Corporation, Japan*

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The best forum to gain applied and basic knowledge, build your collaborative network, and learn skills to move your projects forward in one of today's hottest research areas

AsiaTIDES continues to be the premier forum to get a comprehensive update, meet key players and increase your knowledge of the oligonucleotide- and peptide-based therapeutics fields.

The faculty list for the 2012 program is the strongest yet, with more representation from big pharma than ever before. Hear new data and strategic perspectives from

Merck, Genzyme, and GlaxoSmithKline, including three presentations from Glaxo on a peptide project and their assessments of both the oligo and peptide landscape. Plus hear from Isis, Novo Nordisk, Ipsen, Quark, Alnylam, Santaris, Tekmira and a host of other important companies you need to follow to stay abreast of this field.

Past attendees congratulate AsiaTIDES

"The TIDES series of conferences is an indispensable catalyst for the industry, bringing expert people together to share their knowledge and address common issues, ensuring that we can grow our respective enterprises and help TIDES-based medicine flourish. A unique opportunity to hear the latest developments in the field across all disciplines and hear how industry colleagues are rising to the challenges. Over and above the informative presentations will be the invaluable networking with experienced and knowledgeable individuals."

– Allison L. Morgan, Vice President, Clinical Research and Development, Prosensa Therapeutics, The Netherlands

"At TIDES I have the opportunity to meet clients and industry collaborators, to educate myself enormously about current projects, new products and new potential therapies. I think we will be part of the breakthrough in taking medicine that step further in treating complicated and pernicious diseases which still remain today incurable. (I believe)... **the combination of peptides and oligonucleotides represent a combined ingredient class in therapeutics which will break the back of cancer and other diseases that have plagued mankind for all our history.** It's a special, special event. I always enjoy myself and find it incredibly enriching and rewarding."

– Lester Mills, Ph.D., MBA, Chief Marketing Officer, Bachem Holding AG, Switzerland

Pre-Conference Tutorials

Tuesday, February 28, 2012

8:30 Conference Registration Begins

Tutorial #1:

Technical and Regulatory Aspects of Peptide Specifications and Characterization: From Preclinical through to Market Application

Tutorial Leaders:

Robert Hagopian, Director, Business Development, PolyPeptide Laboratories, USA
Christopher P. Holmes, Ph.D., Executive Director, Chemistry, Affymax, Inc., USA
Bruce Morimoto, Ph.D., Vice President, Drug Development, Allon Therapeutics, Inc., Canada

9:00 **Introduction to the Tutorial: Scientific and Regulatory Framework for Peptide Characterization and Analytical Release Testing**

Bruce Morimoto, Ph.D., Vice President, Drug Development, Allon Therapeutics, Inc., Canada

9:30 **Analytical Requirements and Related Specifications Based on Various Phases of Clinical Trials (Drug Substance)**

Robert Hagopian, Director, Business Development, PolyPeptide Laboratories, USA

10:15 **What to Avoid and Include in your Filing: Common Deficiencies in Chemistry and Analytical Development in Preclinical and Early Clinical Phases (Drug Substance and Drug Product)**

Dr. René Thürmer, Deputy Head Unit Pharmaceutical Biotechnology, BfArM - Federal Institute for Drugs and Medical Devices, Germany

11:00 *Networking Refreshment Break*

11:30 **Regulatory Considerations on Setting Impurity Specification for Peptide Drug Products**

Duu-Gong Wu, Ph.D., Executive Director, Consulting Division, Pharmanet, USA

12:15 **Characterization of Hematide**

Christopher P. Holmes, Ph.D., Executive Director, Chemistry, Affymax, Inc., USA (invited)

1:00 *Close of Tutorial*

Tutorial #2:

Strategies and Approaches for *in vivo* Delivery of Oligonucleotides: How to Achieve Acceptable Therapeutic Indexes in Various Organs and Tissues Using Therapeutically Relevant Administration Routes

Tutorial Leader: Dmitry Samarsky, Ph.D., Executive Vice President, Technology Development, RiboBio, China

9:00 **Introduction to the Tutorial**

9:30 **UNPUBLISHED DATA Fundamentals of Developing Successful Oligonucleotide Delivery Systems**

Sujit K. Basu, Ph.D., Senior Director, Formulation, Dicerna Pharmaceuticals, Inc., USA

10:15 **Development of Lipid-Based Systems for Delivery of siRNA to the Liver**

James J. Cunningham, Ph.D., Associate Director, Pharmaceutical Sciences and Clinical Supplies, Merck Research Laboratories, USA

11:00 *Networking Refreshment Break*

11:30 **Transition of Systemic siRNA Delivery Systems into the Clinic: Practical Considerations**

Peter Lutwyche, Ph.D., Senior Vice President, Pharmaceutical Development, Tekmira Pharmaceuticals Corporation, Canada

12:15 **UNPUBLISHED DATA Evaluation of Antisense Pharmacology in Multiple Tissues by In-situ Hybridization**

A. Robert MacLeod, Ph.D., Executive Director, Discovery Biology, Isis Pharmaceuticals, Inc., USA

1:00 *Close of Tutorial*

Lunch will be served for tutorial attendees.

1:00 Registration for Main Conference Begins

1:55 **Chairperson's Remarks**

Laura Sepp-Lorenzino, Ph.D., Senior Director, RNA Therapeutics, Merck & Co., Inc., USA

2:00 **Oligonucleotide and Delivery Platform Optimization Strategies for siRNA Drug Development**

RNA interference is a powerful gene-targeting technology with the potential for becoming an important new therapeutic modality, offering a strategy for accessing disease targets previously considered un-druggable with traditional small molecule approaches. Despite recent progress in preclinical and clinical oligonucleotide delivery systems, safe and effective systemic delivery remains a hurdle in therapeutic siRNA drug development. The presentation will showcase case-studies from our lead identification and optimization of siRNAs and delivery vehicles.

Laura Sepp-Lorenzino, Ph.D., Senior Director, RNA Therapeutics, Merck & Co., Inc., USA

2:40 **Overview of Peptide Therapeutics and Vaccines in Development**

There are several cancer vaccine trials such as peptide based vaccines and dendritic cell based vaccines underway in Japan. The majority of them are in academia trials and some are in sponsored registration trials. Recently, the National Cancer Center started the strategic commitment for cancer vaccine development in Japan. I will summarize the current situation of cancer vaccine trials and describe the "Cancer Vaccine Core Facility" developed at the National Cancer Research Center.

Yuji Heike, M.D., Ph.D., Chief of Medical Staff, Department of Medical Oncology, National Cancer Center Hospital, Japan

3:20 **Evolution and Development of GLP-1 Based Therapeutics**

GLP-1 based therapeutics are currently represented in the marketplace by oral DPP-4 inhibitors and injected GLP-1 receptor agonists. The success of the latter in ameliorating metabolic disease has driven efforts to further enhance convenience and compliance via sustained-release technologies (including devices) and molecular size enhancement. New GLP-1 based therapies will include stimulators of L-cell peptide release via nutrient-sensing receptors (eg. GPR40, GPR120 and GPR119) and non-nutrient (eg bile salt based) mechanisms. With GLP-1 agonism in association with other peptide synergists, weight loss and antidiabetic efficacy may approach that of bariatric surgery.

Andrew A. Young, M.D., Ph.D., Vice President, Head, Enterendocrine Biology, GlaxoSmithKline, USA

4:00 **Networking Refreshment Break and Business Card Exchange**4:30 **Recent Advances in Routes of Delivery for Peptide Pharmaceuticals**

Peptide therapeutics have traditionally been delivered by injectable, or in a few instances by nasal routes of delivery. However, for certain peptides, several additional formulations such as oral, transdermal, buccal, ocular, pulmonary, vaginal and rectal have been developed. There are currently several peptides in advanced stages of clinical development with oral or transdermal technologies. The status of these studies and their future prospects will be discussed.

Nozer Mehta, Ph.D., Vice President, R&D, UniGene Laboratories, Inc., USA

5:10 **Clinical Development of Mipomersen, an Antisense Second-Generation Oligonucleotide Targeting Apolipoprotein B**

This presentation will provide an overview of the clinical development of mipomersen, an antisense second-generation oligonucleotide being investigated for patients with severely elevated cholesterol such as those with familial hypercholesterolemia, including patients with homozygous and severe heterozygous familial hypercholesterolemia. Mipomersen targets hepatic production of apolipoprotein B (Apo B), the structural core for particles such as LDL that carry cholesterol.

Tejdeep Singh, M.D., Medical Director, Global Patient Safety and Risk Management, Genzyme Corporation, USA

5:50 **Close of AsiaTIDES Day One**6:30 **Networking Dinner**

Join fellow attendees and speakers for a collegial dinner out in Tokyo. Space is limited and additional fee applies. Please indicate on the registration page if you plan to join the dinner.

8:00 **Registration and Coffee/Tea****Updates on Oligonucleotide-Based Therapeutics in Preclinical and Clinical Development****Updates on Oligonucleotide-Based Therapeutics in Preclinical Development**

Session Sponsor: **Avecia** **OligoMedicines**
A Nitto Denko Company

8:30 **Chairperson's Remarks**

William S. Marshall, Ph.D., President and Chief Executive Officer, miRagen Therapeutics, Inc.

Keynote Presentation8:35 **A Pivotal Year for Oligonucleotide Therapeutics**

The dream for oligonucleotide therapeutics is to use Watson and Crick base-pairing rules to design drugs that modulate the expression of disease-related RNAs based solely on the sequence of the target RNA. In the past 12 months advances in siRNA, antisense therapeutics, and miRNA targeting drugs have brought the dream closer to a reality.

Arthur A. Levin, Ph.D., Chief Development Officer, Santaris Pharma A/S, USA and Denmark

9:15 **Discovery and Development of Novel Cardiovascular Drug Candidates Based on microRNA Targeting**

Outcomes of studies in the context of microRNA genetic deletion that have been recapitulated by synthetic anti-miR dosing clearly demonstrate that the inhibition of specific microRNAs is sufficient to produce beneficial outcomes in models of cardiac dysfunction. Therapeutic targeting of these microRNAs with short, high-affinity oligonucleotides has demonstrated significant promise as the altered biophysical properties of the anti-miR appear to enhance its drug like properties.

William S. Marshall, Ph.D., President and Chief Executive Officer, miRagen Therapeutics, Inc., USA

Updates on Peptide-Based Therapeutics in Preclinical and Clinical Development**Updates on Peptide-Based Therapeutics in Preclinical Development**8:30 **Chairperson's Remarks**

Jesper Lau, Ph.D., Vice President, Diabetes Protein and Peptide Chemistry, Novo Nordisk, Denmark

Keynote Presentation8:35 **RaPID Selection of a New Class of Peptide Drug Leads against Various Therapeutic Targets**

We recently devised a new means to reprogram the genetic code, which allows us to express non-standard peptides containing multiple non-proteinogenic amino acids *in vitro*. This lecture will describe the most recent development in the genetic code reprogramming approach that enables us to express natural product-like non-standard peptides and screen them against various drug targets inexpensively, less laboriously, and very rapidly by a selection platform, referred to as RaPID (Random Peptide Integrated Discovery) system.

Hiroaki Suga, Ph.D., Professor, Chemical Biology and Biotechnology Lab, The University of Tokyo, Japan

9:15 **UNPUBLISHED DATA Albumin Binding PYY and Exendin-4 Synergistically Normalize Weight and Glucose in Obese and Diabetic Mice**

In an attempt to replicate the hormonal changes contributing to the success of surgical treatments for obesity we have generated albumin binding (AlbudAb) versions of Exendin-4 and PYY. When combined and administered for two weeks to mice these compounds normalized body weight to the level of lean animals with associated improvements in blood glucose control and serum chemistries.

Bruce J. Hamilton, Ph.D., Investigator, Targeted Biopharm Discovery Unit, GlaxoSmithKline, United Kingdom

Updates on Oligonucleotide-Based Therapeutics in Preclinical Development

- 9:45 **UNPUBLISHED DATA** **Therapeutic Applications of Tumor Suppressor miRNAs**
Systemically delivered mimics of tumor suppressor miRNAs inhibit the growth and metastasis of tumors in mice. A clinical candidate featuring the miR-34 sequence and a liposomal delivery formulation is being developed for cancer patients with solid tumors. Intriguingly, the miR-34-based therapeutic is particularly potent with cancer stem cells.
David Brown, Ph.D., Director of Research, Mirna Therapeutics, Inc., USA
- 10:15 *Grand Opening of Exhibit and Poster Hall with Networking Refreshment Break*
- 11:00 **Dicer Substrate siRNA Preclinical Development: Tumor Delivery and Efficacy Using LNPs**
Dicer substrate siRNAs (DsiRNAs) are being developed for oncology applications, analogous to other types of RNAi triggers. DsiRNAs are longer than other types of siRNA molecules and offer greater flexibility in formulation and conjugation, and significantly greater potency in most cases. Data against multiple oncology target genes and multiple models will be presented.
Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals, USA
- 11:30 **CASE STUDY UNPUBLISHED DATA** **A Novel Antithrombotic Strategy by Targeting Intrinsic Coagulation Pathway Factor XI Using Antisense Oligonucleotide**
There continues to be steady progress in developing therapeutic applications for antisense inhibitors. Anticoagulants are effective antithrombotic agents but have a narrow therapeutic window and may cause serious bleeding complications. Factor XI (FXI) may be an attractive target for anticoagulant therapy as recent work points to an important role in thrombosis and a relatively minor role in normal hemostasis.
Tae-Won Kim, Ph.D., Executive Director, Toxicology, Isis Pharmaceuticals, Inc., USA
- 12:00 **A New Trial of miRNA Therapeutics Targeting on the Tumor Initiating Cells of Osteosarcoma**
There is emerging evidence that cancer stem cells (CSCs) /tumor initiating cells (TICs) are under the regulation of molecular signaling including microRNAs. We identified several microRNAs regulating the phenotype of TICs of osteosarcoma, and their therapeutic effects with current chemotherapeutics. This presentation will show the possibility of microRNA-based therapeutics as a novel strategy against solid tumors.
Tomohiro Fujiwara, M.D., Division of Molecular and Cellular Medicine, National Cancer Center Research Institute, Japan
- 12:30 *Luncheon in Exhibit and Poster Hall*

Updates on Oligonucleotide-Based Therapeutics in Clinical Development

Session Sponsor:  **Agilent Technologies**

- 1:30 **Chairperson's Remarks**
James D. Thompson, Ph.D., Vice President, Pharmaceutical Development, Quark Pharmaceuticals, Inc., USA
- 1:35 **siDNA – A New Strategy Interfering with Tumor Cell's DNA Repair Signaling to Overcome Tumor's Resistance to Cancer Therapies: From Concept to Clinic**
Enhanced DNA repair activity in tumors confers resistance to treatment. A first-in-class oligonucleotide therapeutic that mimics DNA double-strand breaks has been developed. It acts by jamming DNA damage signaling and thereby inhibits DNA repair. This presentation will describe its mechanism of action and proofs of concept, animal toxicology and pharmacokinetics data, and preliminary data in the first-in-human trial of DT01.
Prof. Jian-Sheng Sun, Ph.D., CEO, DNA Therapeutics SA, France
- 2:05 **Update on Safety and Clinical Activity of Synthetic siRNAs for Ophthalmic and Renal Indications**
Quark's current development pipeline focuses on indications where chemically-modified siRNAs can be delivered in the absence of complex formulations. A Phase 2 study of PF-655 in patients with diabetic macular edema demonstrated an improvement in visual acuity over the standard of care following local intravitreal administration. Clinical study updates of QPI-1002 for the treatment of renal injury and of QPI-1007 for ocular neuroprotection will also be provided.
James D. Thompson, Ph.D., Vice President, Pharmaceutical Development, Quark Pharmaceuticals, Inc., USA

Updates on Peptide-Based Therapeutics in Preclinical Development

- 9:45 **CASE STUDY UNPUBLISHED DATA** **SKL-18287: A Long-Acting GLP-1 Analog**
SKL-18287 is a novel long-acting GLP-1 analog designed for DPP-IV resistance and is comprised of natural amino acids without any modifications. Its half-life in human plasma was more than 24 hours at 37°C. The glucose lowering effects of SKL-18287 in obese type 2 diabetes mice were sustained for 10 hours after 10 nmol/kg sc administration.
Masayuki Okamoto, Senior Researcher, Drug Discovery Laboratories, Sanwa Kagaku Kenkyusho CO., LTD, Japan
- 10:15 *Grand Opening of Exhibit and Poster Hall with Networking Refreshment Break*
- 11:00 **CASE STUDY UNPUBLISHED DATA** **Dual- or Triple-Acting Peptide Agonists for Treatment of Diabetes and Obesity**
Second and third generation incretin-based therapies are being explored to improve therapies in diabetes and obesity. GLP-1-Glucagon and GLP-1-Gastrin are the two first examples of Zealand Pharma's dedicated drug discovery. Our strategy is to selectively target not one but two or even three receptors for optimized efficacy and safety profiles of future cardio-metabolic medicines.
Christian Gröndahl, D.V.M., Ph.D., M.D., Executive Vice President and Chief Scientific Officer, Zealand Pharma, Denmark
- 11:30 **Modification of a Novel Angiogenic Peptide, AG30, for the Development of Novel Therapeutic Agents**
We previously identified a novel angiogenic peptide, AG30, with antibacterial effects that could serve as a foundation molecule for the design of wound healing drugs. Toward clinical application, in this study we have developed a modified version of the AG30 peptide characterized by improved antibacterial and angiogenic action.
Hironori Nakagami, M.D., Ph.D., Professor, Vascular Medicine and Epigenetics, Osaka University United Graduate School of Child Development, Japan
- 12:00 **A Novel Peptide Inhibiting Binding between C1q and Immunocomplex Suppresses Joint Destruction in Experimental Arthritis**
C1q is the first component of the classical pathway of complement activation and involved pathological process in inflammatory and autoimmune disorders. A novel peptide inhibiting binding between C1q and immunocomplex suppressed joint destruction in collagen-induced arthritis via suppression of osteoclastogenesis. In vitro, the peptide suppressed the production of inflammatory cytokines and MMPs by synovial cell derived from rheumatoid arthritis patients.
Tetsuya Tomita, M.D., Ph.D., Associate Professor, Orthopaedic Biomaterial Science, Graduate School of Medicine, Osaka University, Japan
- 12:30 *Luncheon in Exhibit and Poster Hall*

Updates on Peptide-Based Therapeutics in Clinical Development

- 1:30 **Chairperson's Remarks:**
Jesse Z. Dong, Ph.D., Vice President, IPSEN/Biomeasure, Incorporated, USA
- 1:35 **Pharmaceutical Protein and Peptide Engineering**
Protein engineering has emerged as the technology used to optimize the properties of native NBEs to more stable and efficacious drugs with improved pharmaceutical profiles. The *in vivo* parameters that often are being optimized includes renal clearance, liver elimination, and plasma elimination. Glucagon like peptide 1 and insulin as case stories will be discussed.
Jesper Lau, Ph.D., Vice President, Diabetes Protein and Peptide Chemistry, Novo Nordisk, Denmark
- 2:05 **CASE STUDY** **Design Considerations for DPX-Survivac, a Multi-Cancer Peptide-Based Vaccine**
DPX-Survivac is a peptide based cancer vaccine that targets the tumor-associated antigen survivin. Survivin is over-expressed in a number of solid tumors and hematological malignancies but not found in most normal tissues. The vaccine was designed to overcome the limitations of HLA restriction. The low immunogenicity of peptides and the immune evasions strategies of cancer were also addressed.
Marc Mansour, Ph.D., COO and CSO, Immunovaccine Inc., Canada

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Updates on Oligonucleotide-Based Therapeutics in Clinical Development

2:35 **PRO-044 and Other Exons: A Rare Opportunity**

A status update of current clinical programmes will be provided. Challenges of studying increasingly rare populations and the need to extrapolate and contextualize data from one programme to another will also be discussed. The hurdles of demonstrating safety and efficacy comparability within a portfolio of compounds will be highlighted, along with some rare opportunities for partnership and progress.

Allison L. Morgan, Vice President, Clinical Research and Development, Prosensa Therapeutics, The Netherlands

3:05 **Networking Refreshment Break in Exhibit and Poster Hall**3:50 **Spotlight Presentation**

IBC's Spotlight Presentations give supplier and service companies the opportunity to present product and service offers directly to the audience at the conference. For further information on sponsoring a Spotlight Presentation, please contact Sherry Johnson at (508) 614-1451 or sjohnson@ibcusa.com.

4:20 **UNPUBLISHED DATA Identification and Characterization of STAT3RX: A Potent Generation 2.5 ASO for Cancer**

Activation of STAT3 has been documented in a broad range of human cancers. In addition to its role in tumor cells it has important functions in tumor-associated stromal cells. We have developed highly potent Generation 2.5 STAT3 ASOs that inhibit STAT3 in both tumor cells and non-tumor stromal cells resulted in anti-tumor activity and dramatic reduction in circulating levels of pro-tumorigenic cytokines including IL-6 and IL-1b in cancer models. STAT3RX is anticipated to enter Phase I clinical evaluations in Q1 2012.

A. Robert MacLeod, Ph.D., Executive Director, Discovery Biology, Isis Pharmaceuticals, Inc., USA

Featured Presentation

4:50 **Update on RNAi Therapeutics in the Clinic**

This presentation will review the clinical progress made with several programs utilizing a lipid nanoparticle platform for systemic delivery of siRNAs.

Jared A. Gollob, M.D., Senior Director, Clinical Research, Alnylam Pharmaceuticals, Inc., USA

5:20 **Networking Reception in Exhibit and Poster Hall**

Co-sponsored by  **ChemGenes CORPORATION**

Updates on Peptide-Based Therapeutics in Clinical Development

2:35 **UNPUBLISHED DATA Speeding up Peptide-Based Vaccine Research with VesiVax® Conjugatable Adjuvant Lipid Vesicles (CALV)**

The VesiVax® CALV system combines a liposomal antigen delivery vehicle with the adjuvant properties of Toll-like receptor (TLR) agonists to stimulate potent immune responses. The VesiVax® CALV displays conjugatable moieties on the liposome surfaces for the attachment of different antigens of interest, thus providing a customizable liposome-based delivery system for a variety of immunological research and vaccine development applications.

Gary Fujii, Ph.D., President and CEO, Molecular Express, Inc., USA

3:05 **Networking Refreshment Break in Exhibit and Poster Hall**3:50 **Spotlight Presentation**

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4:20 **UNPUBLISHED DATA Update on Clinical Development of CurePeptin**

We have developed a novel anti-microbial peptide, CurePeptin, based on newly identified angiogenic anti-microbial peptide. Application of CurePeptin to Band-Aid, sanitary and cosmetics is now ongoing. In this lecture, the clinical development of CurePeptin will be introduced.

Ryuichi Morishita, M.D., Ph.D., Professor, Dept. of Clinical Gene Therapy, Graduate School of Medicine, Osaka University; Founder, Board Member, AnGes MG, Inc., Japan

4:50 **The Effect of Davunetide on Clinical and Biomarker Endpoints in a Pilot Study of Schizophrenia**

The neuroactive peptide davunetide was evaluated in a pilot clinical study of cognitive-impairment associated with schizophrenia (CIAS). In a 12 week treatment study, davunetide-treatment resulted in an improvement on the functional capacity measure, UPSA. Davunetide-treated resulted in an increase in N-acetyl aspartate, a biomarker for neuronal integrity in a substudy looking at imaging biomarkers.

Bruce Morimoto, Ph.D., Vice President, Drug Development, Allon Therapeutics, Inc., Canada

5:20 **Networking Reception in Exhibit and Poster Hall**

Co-sponsored by  **ChemGenes CORPORATION**

7:30 *Networking Coffee and Tea*

Manufacturing and Analytical Development for Peptides and Oligonucleotides

8:00 **Chairperson's Remarks**

Paula Lorence, Vice President, Business Development, Avecia OligoMedicines, USA

8:10 **CASE STUDY UNPUBLISHED DATA Hydrophobic Tag-assisted Multi-step Solution Phase Peptide Synthesis for Advanced Modifications and Large Scale Production**

Hydrophobic tag-assisted solution phase peptide synthesis technique has been investigated to apply for the synthesis of highly modified peptides and their large-scale productions. The sequential peptide synthesis was performed on the hydrophobic tags that enable reactions in homogeneous solutions followed by product separations by sedimentations. For example, disulfide bond formation has been well-combined for practical preparation of somatostatin.

Kazuhiro Chiba, Ph.D., Professor, Applied Biological Science, Tokyo University of Agriculture and Technology, Japan

8:35 **From R&D to Revolutionary Peptide Production**

Lonza developed and patented an evolution of conventional liquid phase peptide synthesis (LPPS) that combines the best characteristics of LPPS with the key benefits of solid phase peptide synthesis (SPPS). Lonza kept standard reagents and protected amino acids involved in SPPS for its new approach, in addition no special C-terminal anchor where found needed. The higher purity of crude peptide product created by Lonza lessens the purification load and helps reduce the development time normally associated with LPPS, while generating a greater yield of higher-purity peptides.

Matthieu Giraud, Ph.D., Director, Lonza Chemicals R&D Peptides, Lonza Ltd, Switzerland

9:00 **CASE STUDY UNPUBLISHED DATA Synthetic Manufacturing of Icatibant (Firazyr®)**

Firazyr® is approved for the treatment of acute hereditary angioedema in the US and Europe. The peptidomimetic API Icatibant is a bradykinin B2 receptor antagonist consisting of 10 amino acids (AA) including several non-proteinogenic AA. This industrial case study comprises supply chain consideration and upstream/downstream processing, demonstrating the power of state-of-the-art synthetic API manufacturing and current analytical technologies.

Thomas Meier, Vice President, Production, Bachem AG, Switzerland

Formulation and Delivery of Peptides and Oligonucleotides

8:30 **Chairperson's Remarks**

Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals, USA

8:35 **UNPUBLISHED DATA Combinatorial Development of Biomaterials and Synthetic siRNA Delivery Systems**

High throughput, combinatorial approaches have revolutionized small molecule drug discovery. Here we describe our work on high throughput methods for developing and characterizing biomaterials, and in particular siRNA delivery systems. Libraries of nanoparticles, degradable polymers and lipid-like materials have been synthesized, formulated and screened for their ability to deliver siRNA, both in vitro and in vivo. A number of siRNA delivery formulations have been developed with in vivo efficacy, and show potential therapeutic application for the treatment of genetic disease, viral infection, and cancer.

Daniel G. Anderson, Ph.D., Associate Professor, Chemical Engineering, Harvard-MIT Division of Health Sciences and Technology, David H. Koch Institute for Integrative Cancer Research, Massachusetts Institute of Technology, USA

9:15 **Progress in the Development of Lipid Nanoparticle siRNA-Based Drugs**

Abstract unavailable at press date. Please visit www.IBCLifeSciences.com/AsiaTIDES for program updates.

Ian MacLachlan, Ph.D., Executive Vice President & Chief Scientific Officer, Tekmira Pharmaceuticals Corp., Canada



Manufacturing and Analytical Development for Peptides and Oligonucleotides

9:25 **CASE STUDY UNPUBLISHED DATA** Application of AjiPhase™ to Large Scale Peptide Manufacturing

We have developed and reported a novel liquid phase peptide synthetic methodology AjiPhase™, which can overcome the disadvantages of traditional LPPS. AjiPhase™ retains the traditional benefits of lower cost, higher quality and easier scale-up compared to Solid Phase Peptide Synthesis (SPPS). We have already applied this technology to the synthesis of variety of peptides including greater than 40-mers in length, cyclized, and conjugated in high volumes. The significant reductions in production costs and purification load, compared to SPPS have been repeatedly demonstrated.

Daisuke Takahashi, Section Chief, Institute for Bioscience Products and Fine Chemicals, Ajinomoto Co., Inc., Japan

9:50 **A Novel High Loading Polymer Support for Therapeutic Oligonucleotide Synthesis**

Hear about the novel support for both DNA and RNA synthesis from small scale to large scale.

Yi Jin, Ph.D., Director of Biomedical Division, Nitto Denko Technical Corp., USA

10:15 *Networking Refreshment Break in Exhibit and Poster Hall*

10:45 **Spotlight Presentation**

Impact of Phosphoramidite Impurities on the Impurity Profile of Oligonucleotides

Abstract unavailable at press date. Please visit www.IBCLifeSciences.com/AsiaTIDES for program updates.

Dr. Hüseyin Aygün, CSO, Nucleic Acid Technologies, BioSpring GmbH, Germany

Sponsored by



Panel Discussion

11:10 **Controls and Specifications for Raw Materials**

The panel will address materials including PEG, amidites, solid support, and protected nucleosides. Raw materials used in oligonucleotide manufacture are routinely tested and controlled through specifications. Further diligence is provided to those that get incorporated structurally within the API or as formulation excipients within the DP. The panel will focus on this subset of materials like solid support, amidites/ protected nucleosides and linkers (PEG) for API and excipients utilized in novel DP formulations.

Moderator: Paula Lorence, Vice President, Business Development, Avecia OligoMedicines, USA

Panelists:

Sujit K. Basu, Ph.D., Senior Director, Formulation, Dicerna Pharmaceuticals, Inc., USA

Paul Metz, Senior Director, Operations, Nucleic Acids Solutions Division, Agilent Technologies, Inc., USA

Ipsita Roymoulik, Ph.D., Associate Director, Analytical Development, Avecia OligoMedicines, USA

Additional panelists to be announced

11:40 **CASE STUDY UNPUBLISHED DATA** Multi-Site Evaluation of a Validated HPLC Method for Phosphoramidites

Analytical method standardization across the industry, from amide manufacturers to drug sponsors and CMOs, is critical to ensuring quality and robustness of the amidite supply chain and for providing consistency in regulatory submissions. Presented will be results of a multi-site evaluation of an HPLC-UV/MS method for analysis of phosphoramidites. Methods have been developed and optimized for each class of amidites. Validated methods and reports are being shared with the industry in a move that is aimed at catalyzing standardization.

Paul Metz, Senior Director, Operations, Nucleic Acids Solutions Division, Agilent Technologies, Inc., USA

12:05 **Spotlight Presentation** Considerations for Design of a Large-Scale Oligonucleotide Manufacturing Facility

Many sponsors are developing oligonucleotides for indications requiring over 100 kilograms post approval. A sponsor must determine if commercial manufacturing should be performed internally or outsourced. The various design requirements for a large scale oligonucleotide manufacturing facility will be presented including selection of synthesis and purification equipment for various types of oligonucleotides and for multi product and single product facilities.

Thomas M. Rupp, Technical Advisor, Oligonucleotide Manufacturing, Girindus, USA

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12:30 **Presentation title to be announced**

Presenter to be announced, Avecia OligoMedicines, USA

12:55 *Networking Luncheon in Exhibit and Poster Hall*

Formulation and Delivery of Peptides and Oligonucleotides

9:45 **Strategies for the Delivery of RNAi Therapeutics**

The key to fulfilling the potential of RNAi as a new therapeutic modality is the safe and efficacious delivery of short interfering RNAs (siRNAs), the molecules that mediate RNAi. In recent years, significant progress has been made to overcome the obstacles associated with *in vivo* delivery of siRNA through the utilization of both conjugation and lipid nanoparticle formulation strategies.

Akin Akinc, Ph.D., Associate Director, Research, Alnylam Pharmaceuticals, Inc., USA

10:15 *Networking Refreshment Break in Exhibit and Poster Hall*

11:00 **Therapeutic Gene Silencing in Liver by Enteral siRNA Delivery**

We developed a method for trans-intestinal delivery of siRNA using the postprandial pathway of vitamin E transport. The α -tocopherol-conjugated siRNA passes along with fatty acids across the colorectal epithelium, followed by binding to chylomicrons in the thoracic lymph duct through 'in vivo incubation,' and then into hepatocytes by a receptor-mediated mechanism. Consequently, a significant reduction of serum low-density lipoprotein (LDL)-cholesterol was achieved by silencing the apolipoprotein B (apoB) gene in mouse liver.

Takanori Yokota, M.D., Ph.D., Professor, Department of Neurology and Neurological Science, Tokyo Medical and Dental University, Japan

11:30 **Novel Approaches to Address Therapeutic Oligonucleotide Delivery**

In vivo delivery of the oligonucleotides resulting in efficient and safe down-regulation of targeted genes in the tissues and cell types of choice poses major challenge for developers of the drugs belonging to this group of molecules. We will introduce and discuss various strategies and approaches recruited for the oligonucleotide delivery, including formulation with delivery vehicles and chemical modifications of the molecules.

Dmitry Samarsky, Ph.D., Executive Vice President, Technology Development, RiboBio, China

12:00 **Clinical Development of a Solid Dosage Oral Formulation for the 32 Amino Acid Peptide Salmon Calcitonin**

Unigene's oral delivery technology was used in a phase 3 clinical study for salmon calcitonin successfully completed by Unigene's partner Tarsa Therapeutics. The development of the oral technology, as well as early and late phase clinical results will be presented. This clinical program demonstrated the advantages of Unigene's oral delivery technology, including an improved variability profile and limited food effects on the biological activity of the peptide.

Christopher Meenan, Director, Business Development Operations, UniGene Laboratories, Inc., USA

12:30 **Spotlight Presentation**

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1:00 *Networking Luncheon in Exhibit and Poster Hall*

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Conference Language

The conference will be conducted in English without translation.

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