DIA/FDA/Health Canada/AAPS/OTS

3rd Oligonucleotide-based Therapeutics Conference

Where Regulators and Industry Partner to Advance Oligonucleotide Science Together

March 23-25, 2010

DoubleTree Hotel and Executive Meeting Center
Bethesda, MD, USA

Co-sponsored by

FDA

Health Canada / Santé Canada

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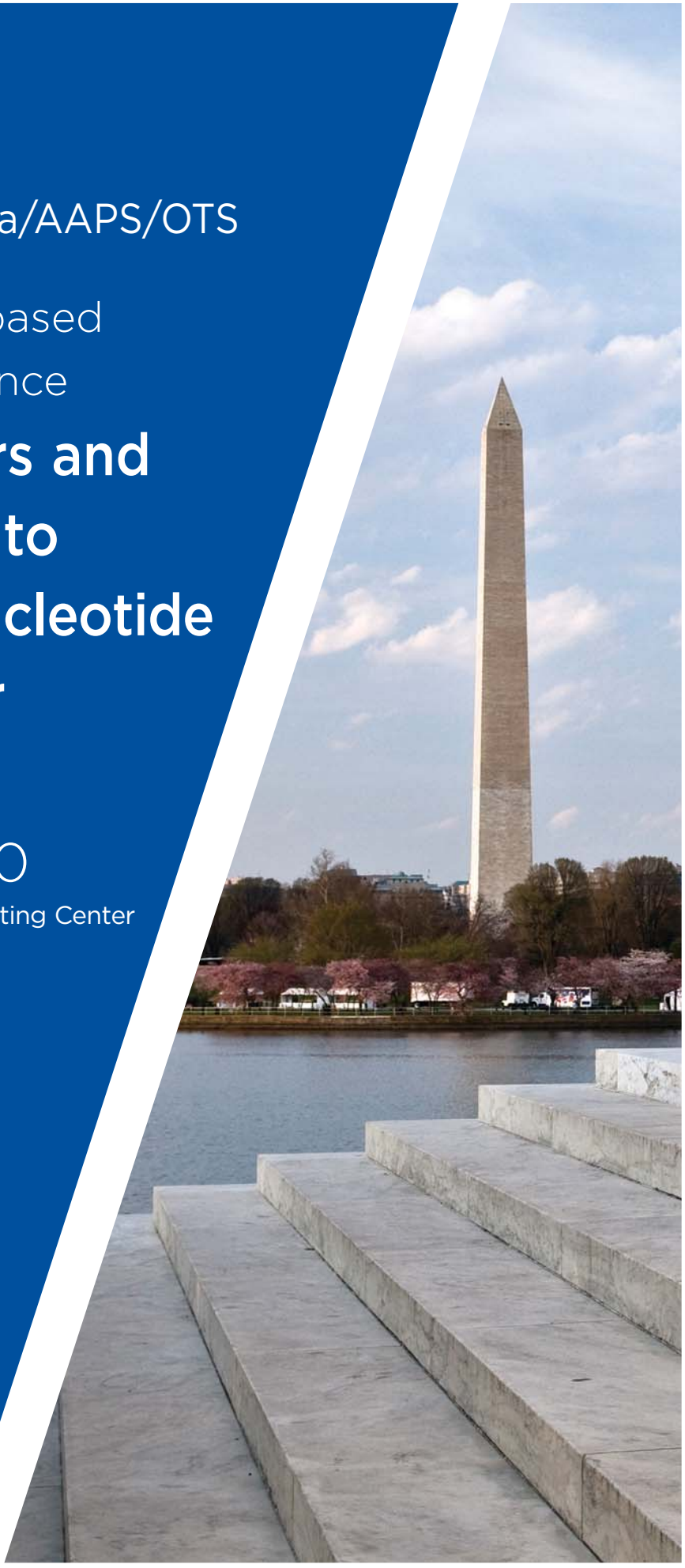

DIA/FDA/Health Canada/AAPS/OTS

3rd Oligonucleotide-based Therapeutics Conference: Where Regulators and Industry Partner to Advance Oligonucleotide Science Together

March 23-25, 2010

DoubleTree Hotel and Executive Meeting Center
Bethesda, MD, USA

Keynote Speakers

Mauro Ferrari, PhD
Professor and Chairman, Department of Nanomedicine and Biomedical Engineering, University of Texas Health Science Center at Houston

Dr. Mauro Ferrari serves as a Professor & Director, Center for NanoMedicine, Brown Foundation Institute of Molecular Medicine, Chairman, Department of Biomedical Engineering, University of Texas Health Science Center at Houston, Professor of Experimental Therapeutics, University of Texas M.D. Anderson Cancer Center, Professor of Bioengineering, Rice University, and President of the Alliance for NanoHealth, Houston TX. Dr. Ferrari received a PhD in Mechanical Engineering from U.C. Berkeley. Dr. Ferrari is a founder of biomedical nano/micro-technology in biomedical applications with more than 160 peer-reviewed journal articles, six books, more than 20 issued patents and about thirty more pending in the US and internationally. His career research and development portfolio totals over $50 million. Dr. Ferrari served as Special Expert on Nanotechnology at the National Cancer Institute in 2003-2005, providing leadership into the establishment of the NCI's Alliance for Nanotechnology.

Richard DiMarchi, PhD
Professor and Gill Chair in Biomolecular Science Indiana University

Dr. DiMarchi is former Group Vice President at Eli Lilly & Company where for more than two decades provided leadership in biotechnology, endocrine research and product development. Dr. DiMarchi is a co-founder of Ambrx, Inc. and Marcadia Biotech, and serves as a board member to Ambrx, Marcadia, Isis Pharmaceuticals, and Alba Therapeutics. He is also a scientific advisor to Epitome Biosciences, Kai Pharmaceuticals, Semafore Biotechnologies, and three venture funds; 5AM, TMP, and Twilight.

Dr. DiMarchi is readily recognized for discovery and development of rDNA-derived Humalog® (LisPro-human insulin). He has published more than one hundred papers and is a co-inventor on numerous patents. He recently received the 2005 AAPS Career Research Achievement Award in Biotechnology, the 2006 ACS Earle B. Barnes Award for Leadership in Chemical Research Management, the 2006 ACS Gustavus Esselen Award for Chemistry in the Service of Public Interest, and the 2007 Wallace Carothers Award for Excellence in Polymer Sciences.

Worldwide Headquarters
Drug Information Association, Inc.
800 Enterprise Road, Suite 200
Horsham, PA 19044, USA

Regional Offices
Basel, Switzerland Tokyo, Japan Mumbai, India Beijing, China

Join Industry and Regulatory Key Stakeholders from FDA and Health Canada to Share Product Development and Regulatory Information for Olignoculeotide-based Therapeutics.

CONFERENCE HIGHLIGHTS

***Opening Plenary Session:* The Challenges Faced and Accomplishments Made in the Development of Oligonucleotide-based Therapeutic Drugs**

High-level dialogue to address the current challenges and emerging product development for oligonucleotide-based therapeutics from FDA, EU, Health Canada and Industry perspectives.

Two Keynote Speaker Sessions:

Mauro Ferrari, PhD
Professor and Chairman, Department of Nanomedicine and Biomedical Engineering, University of Texas Health Science Center at Houston

Richard DiMarchi, PhD
Professor and Gill Chair in Biomolecular Science, Indiana University

Closing Plenary Session: Panel Discussion and Path Forward

Regulators and Industry Align for the Next Generation of Oligonucleotide-based Therapeutics 2010

This program was developed with the support of the Oligo Safety Working Group and the CMC Working Group.

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3rd Oligonucleotide-based Therapeutics Conference: Where Regulators and Industry Partner to Advance Oligonucleotide Science Together

March 23-25, 2010
DoubleTree Hotel and Executive Meeting Center
Bethesda, MD, USA

PROGRAM CO-CHAIRPERSONS

David H. Schubert
Vice President of Regulatory Affairs & Quality Assurance
Logical Therapeutics, Inc.

S. Leigh Verbois, PhD
Supervisory Pharmacologist, Division of Drug Oncology Products, CDER, FDA

SCIENTIFIC ADVISORY COMMITTEE

Daniel Capaldi, PhD
Vice President, Analytical and Process Development
Isis Pharmaceuticals, Inc.

Geoffrey Ebere, PhD
Assessment Officer, Clinical Group 1
Office of Clinical Trials, Therapeutic Products Directorate
Health Canada

Shwu-Luan Lee, PhD
Pharmacology/Toxicology Reviewer, CDER, FDA

Arthur A. Levin, PhD
Vice President and Chief Development Officer
Santaris Pharma a/s

James V. McArdle, PhD
McArdle and Associates, LLC

Sara V. Nochur
Vice President, Regulatory Affairs
Alnylam Pharmaceuticals, Inc.

Felix Omara, DVM, MSc, PhD
Senior Biologist/Clinical Reviewer, Health Canada

Ramesh Raghavachari, PhD
Chemist, FDA

Alan Sachs, MD, PhD
Vice President, RNA Therapeutics, Merck Research Laboratories

Dmitry Samarsky, PhD
Vice President, Technology Development
RXi Pharmaceuticals

James D. Thompson, PhD
Vice President, Pharmaceutical Development
Quark Pharmaceuticals, Inc.

René Thürmer, PhD
Deputy Head Unit Pharmaceutical Biotechnology BfArM – Federal Institute for Drugs and Medical Devices, Germany (Regulatory Agency)

Andrew M. Vick, PhD
Executive Vice President
Senior Director Pharmacokinetics, Dynamics and Metabolism, Seventh Wave Laboratories, LLC

CONFERENCE OVERVIEW

This conference will serve as a continuum for discussion among Industry and Health Authorities to inform, educate, and share oligonucleotide-based therapeutic product development and regulatory information in the areas of nonclinical, chemistry, manufacturing and control (CMC) and clinical development. These sessions will address targeted therapeutics and exploratory approaches of oligonucleotide-based therapeutic drugs, including bioinformatics, microRNAs, delivery, impurities and metabolism, thresholds and pre-clinical immune screenings prior to IND filing. Delivery technologies are of high priority to expand the therapeutic indications of oligonucleotide-based therapeutics and therefore this conference will also incorporate current dialogue to address RNA interference technologies and expand to the therapeutic segment to discuss emerging technologies for oligonucletoide science.

WHO SHOULD ATTEND

Chief Scientific Officers, Vice Presidents, Directors, Senior Management, Group/Team/Project Leaders, Scientists, Investigators and Researchers working in the following areas:

- Biotechnology
- Biologics
- Clinical research
- Chemistry, manufacturing, and control
- Clinical, regulatory, and business development
- Delivery technologies
- Drug discovery
- Preclinical
- Quality assurance
- RNAi
- Vaccines

CONTACT INFORMATION

Conference: Joanne Wallace, Program Manager, Phone 215.442.6180 / Fax 215.293.5931 / email Joanne.Wallace@diahome.org

CONFERENCE OBJECTIVES AND SESSION TOPICS

Nonclinical

The nonclinical sessions are designed to provide an opportunity to discuss the critical issues in oligonucleotide therapeutics and also to highlight efforts being made within the oligonucleotide community to address these issues. Some of the sessions are dedicated to technologies and other sessions provide a forum for discussing position papers prepared by scientists under the auspices of the Oligonucleotide Safety Working Group.

- Joint session to address CMC Issues and Impurities/theoretical discussion
- Off target effects and their assessment
- Delivery Issues and Oligonucleotides Therapeutics / Neuromuscular Indications
- New Technologies and Regulatory Requirements
- Oligonucleotide Safety Working Group (OSWG): Exaggerated Pharmacology Committee
- Immunostimulation Working Group
- Inhalation Working Group

Chemistry, Manufacturing, and Controls (CMC)

The CMC sessions will focus on a few of the issues that provide special challenges in development of synthetic oligonucleotides as compared to small molecules. Validation of analytical methods and the application of reporting, identification, and qualification thresholds to therapeutic oligonucleotides are prominent among these topics. We will hear about some of the latest developments in delivery technology, and we will consider if, after more than 20 years, we are ready for "sudden" success of therapeutic oligonucleotides. Many of the ideas from the first sessions will come together in a final session that looks in detail at drug substance specifications.

- Validation of Analytical Methods
- Novel Formulations
- Success
- Qualification Thresholds
- Reporting and ID Threshold Discussions
- Drug Substance Specifications

Clinical Development

The clinical sessions have been organized by disease indication to highlight the challenges faced, and accomplishments made, with oligonucleotide-based therapeutics to address unmet medical needs. Proof of concept data in humans using "naked" oligonucleotides will be presented in liver, hematologic, pulmonary, ophthalmic, oncology and neuromuscular diseases, while examples using delivery formulations will be provided in oncology and metabolic diseases. Talks will span programs ranging from early Phase I to post-NDA, and will cover synthetic siRNAs, antisense, aptamers, anti-miRs and immunostimulatory oligonucleotides.

- Liver/Metabolic Diseases
- Cardiorenal and Pulmonary
- Ophthalmics
- Oncology
- Vaccines
- Neuromuscular Indications

CONTINUING EDUCATION CREDITS

This activity has been planned and implemented in accordance with the Essential Areas and Policies of the Accreditation Council for Continuing Medical Education (ACCME) through the joint sponsorship of the Postgraduate Institute for Medicine and Drug Information Association. The Postgraduate Institute for Medicine is accredited by the ACCME to provide continuing medical education for physicians.

PIM Postgraduate Institute for Medicine

Postgraduate Institute for Medicine designates this educational activity for a maximum of 15.25 *AMA PRA Category 1 Credit(s)*™. Physicians should only claim credit commensurate with the extent of their participation in the activity.

Disclosure Policy: It is Drug Information Association policy that all faculty participating in continuing education activities must disclose to the program audience (1) any real or apparent conflict(s) of interest related to the content of their presentation and (2) discussions of unlabeled or unapproved uses of drugs or medical devices. Faculty disclosure will be included in the course materials.

To receive a certificate of credit you must complete both of the evaluation forms, both DIA and PIM

LEARNING OBJECTIVES At the conclusion of this meeting, participants should be able to:

- Identify oligonucleotide-based therapeutic product development challenges and the relevant FDA, EU and Health Canada regulations.
- Assess the challenges faced and the accomplishments made in the clinical development of oligonucleotide-based therapeutic drugs to meet unmet needs.
- Describe the critical issues in the nonclinical development of oligonucleotides.
- Outline efforts from industry and regulatory authorities to address critical issues in the nonclinical development of oligonucleotides.
- Differentiate the special challenges associated with the development of synthetic oligonucleotides as compared to small molecules.
- Assess special properties of oligonucleotide-based therapeutics that present special challenges in development including the scientific approaches to overcoming those challenges.
- Recognize achievements made in the field to date including the vision of potential benefits to patients.

MONDAY, MARCH 22, 2010

4:00-6:00 PM CONFERENCE REGISTRATION

DAY 1 | TUESDAY, MARCH 23, 2010

7:30-8:45 AM Conference Registration and Continental Breakfast

8:30-8:45 AM WELCOME AND OPENING REMARKS

David H. Schubert
Vice President of Regulatory Affairs & Quality Assurance
Logical Therapeutics, Inc.

8:45-10:00 AM SESSION 1: SPECIAL PLENARY SESSION

The Challenges Faced and Accomplishments Made in the Development of Oligonucleotide-based Therapeutic Drugs

PLENARY DISCUSSANTS:

S. Leigh Verbois, PhD
Supervisory Pharmacologist
Division of Drug Oncology Products
CDER, FDA

Sara V. Nochur
Vice President, Regulatory Affairs
Alnylam Pharmaceuticals, Inc.

Daniel Capaldi, PhD
Vice President
Analytical and Process Development
Isis Pharmaceuticals, Inc.

10:00-10:30 AM REFRESHMENT BREAK

10:30-12:00 PM SESSION 2

Keynote Address: Introduction

Alan Sachs, MD, PhD
Vice President, RNA Therapeutics
Merck Research Laboratories

Keynote Speaker Presentation:

Mauro Ferrari, PhD
Professor and Chairman, Department of Nanomedicine and Biomedical Engineering, University of Texas Health Science Center at Houston

12:00-1:30 PM LUNCHEON AND NETWORKING OPPORTUNITY

1:30-3:00 PM SESSION 3: CONCURRENT SESSIONS

SESSION 3A: NONCLINICAL TRACK

EP/Genotox

SESSION CHAIRPERSON

Robert Dorsam, PhD
Pharmacology/Toxicology Reviewer
CDER, FDA

Exaggerated Pharmacology Subcommittee Report
Douglas Kornbrust, PhD, DABT
President and Scientific Advisory Board Member
Preclinsight

Genotoxicity Subcommittee Report
Cindy Berman, PhD
Independent Consultant

Audience Questions and Discussion

SESSION 3B: CMC TRACK

Validation of Analytical Methods

SESSION CHAIRPERSON

Ramesh Raghavachari, PhD
Chemist
CDER, FDA

Regulatory Perspective on Analytical Oligonucleotide Separations
Linda Ng, PhD
CMC Lead
CDER,FDA

Challenges to Validation of Methods for Double-Stranded Oligonucleotide
Daren S. Levin, PhD
Investigator
Inhaled Product Development
GlaxoSmithKline

Validation of an HPLC-MS Impurities Method for LY2181308 Antisense Oligonucleotide
David Hollowell, PhD
Research Advisor
Eli Lilly and Company

Audience Questions and Discussion

SESSION 3C: CLINICAL TRACK

Liver/Metabolic Diseases

SESSION CHAIRPERSON

Diane Tribble, PhD
Vice President, Clinical Development
Isis Pharmaceuticals, Inc

Mipomersen: Phase 3 Results in FH Patients
Diane Tribble, PhD
Vice President, Clinical Development
Isis Pharmaceuticals

Phase I Evaluation of Stable Nucleic Acid Lipid Particles Containing Anti-ApoB siRNA
Ian MacLachlan, PhD
Executive Vice President and Chief Scientific Officer
Tekmira Pharmaceuticals Corporation

microRNA Therapeutics: SPC3649 as a Case Study
Arthur A. Levin PhD
Vice President and Chief Development Officer
Santaris Pharma a/s

Audience Questions and Discussion

3:00-3:30 PM REFRESHMENT BREAK

3:30-5:00 PM SESSION 4: CONCURRENT SESSIONS

SESSION 4A: NONCLINICAL TRACK

Emerging Technologies: Off-target Effects and Their Assessment

SESSION CHAIRPERSONS

Arthur A. Levin, PhD
Vice President and Chief Development Officer
Santaris Pharma a/s

Shwu-Luan Lee, PhD
Pharmacology/Toxicology Reviewer
CDER, FDA

Assessing off Target Effects of siRNAs
Arthur M. Krieg, MD
Chief Scientific Officer
Research Technology Center
Pfizer, Inc.

Bioinformatics Approaches to Understanding Off-Target Effects of Allele Specific Oligonucleotide (ASO) and MicroRNAs
Morten Lindow, PhD
Group Leader, Integrative Systems Biology
MicroRNA Research
Santaris Pharma A/S

Presentation of the Oligonucleotide Safety Working Group (OSWG) Committee on Off-Targets
Arthur A. Levin, PhD
Vice President and Chief Development Officer
Santaris Pharma a/s

Audience Questions and Discussion

SESSION 4B: CMC TRACK

Novel Formulations

SESSION CHAIRPERSON

Anastasia Khvorova, PhD
Chief Scientific Officer
RXi Pharmaceuticals

sd-rxRNA - Novel Class of Self-Delivering RNAi Compounds: Robust in vitro and in vivo Efficacy with Broad Clinical Potential
Anastasia Khvorova, PhD
Chief Scientific Officerr
RXi Pharmaceuticals

Making Drug Products Using Oligonucleotides
Keith Smith, PhD
Alliance Project Director
GlaxoSmithKline R&D
United Kingdom

Optimizing a Lipid-based Vehicle for siRNA Delivery to Liver
Paul A. Burke, PhD
Executive Director
RNA Therapeutics
Merck & Co., Inc.

Audience Questions and Discussion

SESSION 4C: CLINICAL TRACK

Cardiorenal and Pulmonary

SESSION CHAIRPERSON

Akshay Vaishnaw, PhD, MD
Senior Vice President, Clinical Research
Alnylam Pharmaceuticals, Inc.

Dose Escalation and Safety Study I5NP
Martin Polinsky, MD
Executive Medical Director
Cardio-Renal Program
Quark Pharmaceuticals, Inc.

Chris Rusconi, PhD
Senior Vice President Discovery/Preclinical Development and Chief Scientific Officer
Regado Biosciences

RSV
Development of an RNAi Therapeutic for RSV
Akshay Vaishnaw, PhD, MD
Senior Vice President, Clinical Research
Alnylam Pharmaceuticals

Audience Questions and Discussion

5:00-6:00 PM NETWORKING RECEPTION

Join your colleagues from the conference at The Restaurant Oz, located at the foot of the spiral staircase in the lobby of the DoubleTree Hotel and Executive Meeting Center.

DAY 2 | WEDNESDAY, MARCH 24, 2010

7:30-8:30 AM REGISTRATION AND CONTINENTAL BREAKFAST

7:30-8:15 AM OLIGONUCLEOTIDE CMC DISCUSSION FORUM *Will meet informally in Ballroom C. Please bring your breakfast and join us!*

8:30-10:00 AM SESSION 5: CONCURRENT TRACKS

SESSION 5A: NONCLINICAL TRACK

Issues in Drug Delivery of Oligos

SESSION CHAIRPERSONS

Ian MacLachlan, PhD
Executive Vice President and Chief Scientific Officer
Tekmira Pharmaceuticals Corporation

Understanding the Delivery Issues for siRNA – An Introduction
Ian MacLachlan, PhD
Executive Vice President and Chief Scientific Officer
Tekmira Pharmaceuticals Corporation

Pharmaceutical Development of a Novel Oligonucleotide Delivery Forumulation
Peter Jackson, PhD
Chief Operating Officer
Medesis Pharma SA
France
Patrick Maurel, PhD
Chief Scientific Officer
Medesis Pharma SA
France

Audience Questions and Discussion

SESSION 5B: CMC TRACK

Success

SESSION CHAIRPERSON

Stephen Sofen, PhD
Vice President
Operations Project/Product Management
Genzyme Corporation

Evolution of a Peptide Contract Manufacturer
Brian Gregg, MBA
Vice President
Regulatory Affairs and Quality
Bachem, Inc.

Coping with the 2008-9 Global Acetonitrile Shortage – Thoughts of a Heavy User
Peter McDonnell, PhD
Senior Technical Director
Genzyme Corporation

Attributes of Success: Development through Commercialization
Tracy TreDenick
Head of Quality and Regulatory
BioTechLogic, Inc.

Audience Questions and Discussion

SESSION 5C: CLINICAL TRACK

Ophthalmics

SESSION CHAIRPERSON

Nebojsa Janjic, PhD
Chief Scientific Officer
SomaLogic

Case History of the Development of Macugen
Nebojsa Janjic, PhD
Chief Scientific Officer
SomaLogic

Development of PF-4523655 siRNA for AMD and DME
Shai Erlich, PhD
Chief Medical Officer
Quark Pharmaceuticals

Development of QPI-1007 siRNA as a Neuroprotectant for NAION and Glaucoma
James D. Thompson, PhD
Vice President, Pharmaceutical Development
Quark Pharmaceuticals

Audience Questions and Discussion

10:30-11:15 AM HIGHLIGHTS FROM DAY 1

11:15 AM-12:00 PM SESSION 6

Keynote Address: Introduction

Andrew M. Vick, PhD
Executive Vice President
Senior Director Pharmacokinetics, Dynamics and Metabolism
Seventh Wave Laboratories, LLC

Keynote Speaker Presentation

The Emergence of Chemical Biotechnology as a means to Optimal Protein-based drugs

Richard DiMarchi, PhD
Professor and Gill Chair in Biomolecular Science
Indiana University

12:00-1:30PM LUNCHEON AND NETWORKING OPPORTUNITY

1:30-3:00 PM SESSION 7

SESSION 7A AND 7B: JOINT NONCLINICAL AND CMC TRACK

Qualification Thresholds: Joint CMC and Nonclinical Session

SESSION CHAIRPERSONS

Mamata De, PhD
Pharmacology/Toxicology Reviewer
CDER, FDA

Arthur A. Levin, PhD
Vice President and Chief Development Officer
Santaris Pharma a/s

Overview of Impurities in Synthetic Oligonucleotides
Daniel Capaldi, PhD
Vice President
Analytical and Process Development
Isis Pharmaceuticals, Inc.

Strategy for Evaluation and Qualification of Oligonucleotide Impurities: A Toxicologist's Perspective
Scott Henry, PhD, DABT
Vice President, PreClinical Development
Isis Pharmaceuticals, Inc

Moderator for Audience Questions and Discussion
James McArdle, PhD
McArdle and Associates, Inc.

SESSION 7C: CLINICAL TRACK

Oncology

SESSION CHAIRPERSON

Robert Justice, MD
Director, Division of Drug Oncology Products (DDOP)
Office of Oncology Drug Products, Office of New Drugs
CDER, FDA

ALN-VSP02 in Liver Cancer
Jared Gollob, MD
Senior Director, Clinical Research
Alnylam Pharmaceuticals

Status of the Clinical Development of our Oligonucleotide Candidate Trabedersen (AP 12009)
Hubert Heinrichs, MD
Chief Medical Officer
Antisense Pharma

Lessons from the Clinical Development of Genasense
Loretta Itri, MD
President Pharmaceutical Development and Chief Medical Officer
Genta

Audience Questions and Discussion

3:00-3:30 PM REFRESHMENT BREAK

3:30-5:00 PM SESSION 8: CONCURRENT TRACKS

SESSION 8A: NONCLINICAL TRACK

Immunostimulation Working Group

SESSION CHAIRPERSONS

Laine Peyton Myers, PhD
Pharmacology/Toxicology Reviewer
CDER, FDA

Rosanne M. Seguin, PhD
Associate Director Immunology and Development Support
Pharmaxis, Ltd

Discussion of the Complement Position Paper
Scott Henry, PhD, DABT
Vice President, PreClinical Development
Isis Pharmaceuticals, Inc.

Discussion of Toll-like Receptor (TLRS)
Arthur M. Krieg, MD
Chief Scientific Officer
Research Technology Center
Pfizer, Inc.

Investigations of Sequence Specific Toxicities of Antisense Oligonucleotides (ASOs)
Sebastien Burel, PhD
Associate Director, Preclinical Development
Isis Pharmaceuticals

Audience Questions and Discussion

SESSION 8B: CMC TRACK

Reporting and ID Threshold Discussions

SESSION CHAIRPERSON

James McArdle, PhD
McArdle and Associates, LLC.

PANEL DISCUSSANTS

Hüseyin Aygün, PhD
Chief Scientific Officer
BioSpring GmbH

Mamata De, PhD
Pharmacology/Toxicology Reviewers
CDER, FDA

Daniel Capaldi, PhD
Vice President
Analytical and Process Development
Isis Pharmaceuticals, Inc.

Audience Questions and Discussion

SESSION 8C: CLINICAL TRACK

Vaccines

SESSION CHAIRPERSON

Sukjoon Park, MD
Senior Director, Product Development
Emergent BioSolutions

Clinical experience with candidate malaria vaccines adjuvanted with CPG 7909 in US and Malian subjects
Ruth Ellis, MD, MPH
Head Clinical Group, Laboratory of Malaria, Immunology and Vaccinology
NIAID

Development of AV7909 next generation anthrax vaccine
Sukjoon Park, MD
Senior Director, Product Development
Emergent BioSolutions

Development of HEPLISAV, a 2-dose HBV caccine containing ISS1018 TLR9 adjuvant
Tyler Martin, MD
Chief Medical Officer
Dynavax Technologies

Audience Questions and Discussion

DAY 3 | THURSDAY, MARCH 25, 2010

7:30-8:30 AM REGISTRATION AND CONTINENTAL BREAKFAST

8:30-10:00 AM SESSION 9: CONCURRENT TRACKS

SESSION 9A: NONCLINICAL TRACK

Inhalation Working Group Discussion

SESSION CHAIRPERSONS

Luqi Pei, PhD
Pharmacology/Toxicology Reviewer
CDER, FDA

Topigen's Experience with Inhalation of Antisense Oligonucleotide Therapeutics
Paolo M. Renzi, MD, FCCP, FRCP
Chief Scientific Officer and Founder
Topigen Pharmaceutiacals, Inc (Pharmaxis).

Exploiting the Potential of Therapeutic siRNAs for Pulmonary Diseases
Mark R. Edbrooke, PhD
Director, Respiratory Centre of Excellence for Drug Discovery
GlaxoSmithKline R&D, United Kingdon

Inhalation Subcommittee Report
Nicolay Ferrari, PhD
Director, Pharmacology
Pharmaxis, Ltd

FDA Perspective
Luqi Pei, PhD
Pharmacology/Toxicology Reviewert
CDER, FDA

Audience Questions and Discussion

SESSION 9B: CMC TRACK

Drug Substance Specifications

SESSION CHAIRPERSON

RenéThûrmer, PhD
Pharmaceutical Expert, Unit Pharmaceutical Biotechnology, BfArM – Federal Institute for Drugs and Medical Devices, Germany

PANEL DISCUSSANTS:

Kathryn L. Ackley, PhD
Director of Project Management
Girindus America, Inc.

Daniel Capaldi, PhD
Vice President, Analytical and Process Development
Isis Pharmaceuticals, Inc.

Daren S. Levin, PhD, MS
Investigator, Inhaled Product Development
GlaxoSmithKline

Ipsita Roymoulik, PhD
Analytical Development Group Leader
Avecia Oligomedicines

Bob Sharma, PhD, MBA
Vice President, Process Development & Manufacturing, Oncology
Geron Corporation

Fran Wincott, PhD
President
Wincott & Associates, LLC

Rao Kambhampati, PhD
Review Scientist
CDER, FDA i

Audience Questions and Discussion

SESSION 9C: CLINICAL TRACK

Neuromuscular Indications

SESSION CHAIRPERSON

James Thompson, PhD
Vice President Pharmaceutical Development
Quark Pharmaceuticals

Barbara Wilcox, PhD
Pharmacolgoy/Toxicology Reviewer
CDER, FDA

PANEL DISCUSSANTS

Giles Campion, MD, PhD
Chief Medical Officer and Vice President for Research & Development
Prosensa

Exon Skipping in Duchene Muscular Dystrophy
Peter Sazani, PhD
Sr. Director Preclinical
AVI BioPharma, Inc.

Development of Oligonucleotide Therapeutics for the CNS using Intrathecal Administration
Scott Henry, PhD, DABT
Vice President, PreClinical Development
Isis Pharmaceuticals, Inc.

Audience Questions and Discussion

10:00-10:30 AM REFRESHMENT BREAK

10:30-11:30 AM PLENARY SESSION

Highlights from Day 2 and Entire Conference

11:30-1:00PM SESSION 10: PANEL DISCUSSION AND PATH FORWARD

Regulators and Industry Align for the Next Generation of Oligonucleotide-based Therapeutics 2010

This panel discussion will highlight the challenges and issues with the development of oligonucleotide-based products in general and as brought forth at this conference. The intention is to transform this discussion into action-oriented objectives to address the regulatory and industry issues and challenges affecting us all.

SESSION CO-MODERATORS

David H. Schubert
Vice President of Regulatory Affairs and Quality Assurance
Logical Therapeutics, Inc.

S. Leigh Verbois, PhD
Supervisory Pharmacologist, Division of Drug Oncology Products
CDER, FDA

PANELISTS

Paul Brown, PhD
ODE Associate Director for Pharmacology and Toxicology
CDER, FDA

Robert Kane, MD
Acting Deputy Director
Div of Medical Imaging and Hematology Products
CDER, FDA

Arthur A. Levin, PhD
Vice President and Chief Development Officer
Santaris Pharma a/s

Celia Lourenco, PhD
Manager Clinical Group I, Office of Clinical Trials
Therapeutic Products Directorate
Health Canada

James V. McArdle, PhD
McArdle and Associates, LLC

James D. Thompson, PhD
Vice President, Pharmaceutical Development
Quark Pharmaceuticals, Inc.

René Thürmer, PhD
Deputy Head Unit Pharmaceutical Biotechnology BfArM – Federal Institute for Drugs and Medical Devices, Germany

1:00 PM CONFERENCE ADJOURNED

REGISTRATION FORM
Register online or fax this page to +1.215.442.6199

DIA is a financially independent nonprofit, global, multidisciplinary association that provides a neutral forum for sharing information that optimizes the development and lifecycle management of biopharmaceutical and related products.

3rd Oligonucleotide-based Therapeutics Conference

Event #10010 • March 23-25, 2010

DoubleTree Hotel & Executive Meeting Center Bethesda, Bethesda, MD, USA

Contact Information

Event Information: Contact Joanne Wallace at the DIA office by telephone 215.442.6180, fax 215.293.5931 or email Joanne.Wallace@diahome.org.

Registration Fees

Registration fee includes refreshment breaks, luncheons, and reception (if applicable), and will be accepted by mail, fax, or online.

Industry Fee	US $1350 ❑
Join DIA now to save on future events and to receive all the benefits of membership. Visit www.diahome.org/Membership	**MEMBERSHIP US $140 ❑**
Discount Fees	
Government (Full-time)	US $ 405 ❑
Charitable Nonprofit/Academia (Full-time)	US $ 675 ❑

TRAVEL AND HOTEL The most convenient airport is Ronald Reagan National Airport and attendees should make airline reservations as early as possible to ensure availability. The DoubleTree Hotel & Executive Meeting Center Bethesda is holding a block of rooms at the reduced rate below until February 28, 2010, for the DIA event attendees. Room availability at this rate is guaranteed only until this date or until the block is filled.

Single $199 Double $199

Please contact the DoubleTree Hotel & Executive Meeting Center Bethesda by telephone at 301.664.7309 and mention the DIA event. The hotel is located at 8120 Wisconsin Avenue, Bethesda, MD 20814, USA.

CANCELLATION POLICY: On or before MARCH 16, 2010

Administrative fee that will be withheld from refund amount:
Member or Nonmember = $200
Government or Academia or Nonprofit (Member or Nonmember) = $100
Tutorial (if applicable) = $50

Cancellations must be in writing and be received by the cancellation date above. Registrants who do not cancel by that date and do not attend will be responsible for the full registration fee paid. Registrants are responsible for cancelling their own hotel and airline reservations. You may transfer your registration to a colleague at any time but membership is not transferable. Please notify DIA of any such substitutions as soon as possible. Substitute registrants will be responsible for nonmember fee, if applicable.

DIA reserves the right to alter the venue, if necessary. If an event is cancelled, DIA is not responsible for any airfare, hotel or other costs incurred by registrants.

PAYMENT OPTIONS: Register online at **www.diahome.org** or check payment method.

❑ **CREDIT CARD** number may be faxed to: +1.215.442.6199. You may prefer to pay by check or bank transfer since non-U.S. credit card payment will be subject to the currency conversion rate at the time of the charge.

☐ **Visa** ☐ **MC** ☐ **AMEX** Exp Date _____

Card # _____

Name (printed) _____

Signature _____

❑ **CHECK** drawn on a US bank payable to and mailed along with this form to: Drug Information Association Inc, P.O. Box 95000-1240, Philadelphia, PA 19195-1240, USA. Please include a copy of this registration form to facilitate identification of attendee.

❑ **BANK TRANSFER** When DIA completes your registration, an email will be sent to the address on the registration form with instructions on how to complete the Bank Transfer. Payment should be made in US dollars. Your name and company, as well as the Event I.D. # must be included on the transfer document to ensure payment to your account.

Participants with Disabilities: DIA event facilities and overnight accommodations are accessible to persons with disabilities. Services will be made available to sensory-impaired persons attending the event if requested at least 15 days prior to event. Contact the DIA office to indicate your needs.

Please check the applicable category:

❑ Academia ❑ Government ❑ Industry ❑ CSO ❑ Student
(Call for registration information)

Last Name

First Name M.I.

Degrees ❑ Dr. ❑ Mr. ❑ Ms.

Job Title

Company

Address (As required for postal delivery to your location) Mail Stop

City State Zip/Postal Country

email **Required for confirmation**

Phone Number Fax Number **Required for confirmation**