

RNAi as a Therapeutic Modality: siRNA and miRNA

Wednesday, June 3, 2009 • 9:30 am - 3:00 pm

9:30 RNAi Therapy: Is it Too Early?

There is no doubt that siRNA is a powerful weapon to down-regulate target genes. We have checked siRNA sequences categorized as siRNA therapy in over 1,000 publications to evaluate siRNA sequence effectiveness to the target gene for potential therapy and to evaluate off-target effects. Prof. K. Saigo and others have made progress on basic science for RNAi mechanism governing its therapy. Their progress lets us challenge RNAi therapy. This presentation will discuss our latest results supported with actual demonstration of siRNA design and selection.

Yukikazu Natori, *Professor, Tokyo Institute of Technology; Executive Consultant, RNAi Co., Japan*



10:00 Development of a Novel RNAi Therapeutics Platform

The design and effective delivery of synthetic RNAi compounds are important factors for therapeutic applications. We will present data obtained with our proprietary rxRNA™ compounds, which can be up to 100 times more potent than conventional siRNAs, demonstrate nuclease resistance, and are potentially more specific for their intended targets. We will also discuss the possible mechanism of incorporation of chemically modified molecules into the RNAi machinery.

Dmitry Samarsky, Ph.D., *Vice President, Technology Development, RXi Pharmaceuticals, USA*



10:30 Networking Refreshment Break

11:00 Atu027, a Liposomal siRNA Formulation Targeting PKN3, Inhibits Cancer Progression

We show that systemic administration of Atu027 by repeated bolus injections or infusions in mice, rats and non-human primates results in specific, RNAi-mediated silencing of PKN3 expression. We demonstrate efficacy of Atu027 in orthotopic mouse models for prostate and pancreas cancer with significant inhibition of tumor growth and lymph node metastasis formation. A prospective, open label, single-centre, dose finding phase I study with Atu027 in subjects with advanced solid tumors will commence in March 2009.

Klaus Giese, Ph.D., *Chief Scientific Officer and Vice President, R&D, Silence Therapeutics plc, Germany*



11:30 Developing Multi-Targeted siRNA Therapeutics with Nanoparticle-Enhanced Delivery

Using siRNA cocktail to silence multiple disease genes is truly realizing the advantage of siRNA-based drugs. We have developed siRNA cocktails using the proprietary algorithm and “Tri-Blocker™” platform, as the active pharmaceutical ingredient (API). Those siRNA cocktails were further tested and validated in several disease relevant animal models using either polymer based or liposome-based nanoparticle delivery systems.

Patrick Y. Lu, Ph.D., *President and CEO, Sirnaomics, Inc., USA*

12:00 Benefits of A Comprehensive Approach to RNAi-Based Therapeutics

Successful RNAi-based therapeutics will require effective oligonucleotide construct and delivery options. MDRNA constructs include Dicer substrate (D-siRNA), meroDuplex (three-stranded) and UNA (unlocked nucleic acid) siRNAs. Constructs tailored to target gene provide high potency while minimizing undesired effects such as off-target events and cytokines. Delivery includes liposomes based on our novel DiLA2 platform, peptide-based formulations, or conjugates. These approaches have proven efficacious for viral, metabolic, and oncology targets.

Barry Polisky, Ph.D., *Chief Scientific Officer, MDRNA, Inc., USA*

12:30 Networking Luncheon

1:30 LNA Oligonucleotides – Within Reach of Potent and Safe Oligonucleotide Therapeutics?

Short, single stranded oligonucleotides based on Locked nucleic acids (LNAs) display unprecedented and long lasting potency in a range of tissues in experimental animals upon intravenous or subcutaneous administration of naked molecules at very low doses. To date 5 different LNA oligonucleotides have completed IND enabling tox against mRNA or miRNA targets and three are currently being examined in human clinical trials. The presentation will provide an update on the unique features of LNA oligonucleotides and their use in human therapeutics.

Henrik Ørum, Ph.D., *Chief Scientific Officer, Santaris Pharma, Denmark*



2:00 Identification and Therapeutic Application of Tumor Suppressor miRNAs

The miR-15/16, miR-34, let-7, and several other miRNAs have been shown to regulate the expression of key oncogenes and the activities of important cancer-related pathways. Reduced expression of these important miRNAs appears to contribute to tumorigenesis and metastasis. We have developed synthetic mimics for six tumor suppressor miRNAs and evaluated the capacity of the small RNAs to inhibit tumor development and metastasis following IV injections in mouse models of cancer. Intriguingly, several of the miRNA mimics have proven to be very effective in restricting the growth and spread of cancer. We are now proceeding with the development of miRNA-based therapies that can be tested for safety and efficacy in advanced animal models.

David Brown, Ph.D., *Director of Research, Mirna Therapeutics, Inc., USA*

2:30 Panel Discussion: Is RNAi a Viable Drug Modality?

Moderator: Dmitry Samarsky, Ph.D., Vice President, Technology Development, RXi Pharmaceuticals, USA

- in vivo delivery challenges
- Specificity issues associated with miRNA pathways and interferon induction
- Complexity and controversy of the IP landscape
- Regulatory aspects of RNAi drug development

3:00 Close of Session

RNAi as a Therapeutic Modality: siRNA and miRNA

Registration Form

Part of
IBC's 4th Annual International Conference
DRUG DISCOVERY & DEVELOPMENT JAPAN
www.IBCLifeSciences.com/Japan

5 EASY WAYS TO REGISTER

Mail the attached registration form with your cheque to **IBC Asia (S) Pte Ltd**, No. 1 Grange Road, #08-02, Orchard Building, Singapore 239693.
Customer Service Hotline: (65) 6514 3180
FAX: (65) 6733 5087 or (65) 6736 4312
E-MAIL: register@ibcasia.com.sg
WEB: www.IBCLifeSciences.com/Japan

Registration Fees	On or before March 6, 2009	On or before April 3, 2009	On or before May 1, 2009	Standard Rate After May 1, 2009
RNAi as a Therapeutic Modality Only (June 3)				<input type="checkbox"/> USD750
3 Day Conference (June 1-3)	<input type="checkbox"/> USD1499	<input type="checkbox"/> USD1599	<input type="checkbox"/> USD1699	<input type="checkbox"/> USD1799
2 Day Conference (June 1-2)	<input type="checkbox"/> USD1199	<input type="checkbox"/> USD1299	<input type="checkbox"/> USD1399	<input type="checkbox"/> USD1499
Immunogenicity Tutorial Only (June 1)				<input type="checkbox"/> USD750
Present a Poster <input type="checkbox"/> USD100 - Commercial <input type="checkbox"/> FREE - Academic				

Attend Networking Dinner USD100 (June 2, 5:45pm)

Academia/Government Discounts

Academic and government employees are eligible for over 40% savings off the above registration packages. Visit the registration page at www.IBCLifeSciences.com/Japan for academic packages and discounted pricing information. Rate extended to full-time employees of government, universities and university-affiliated hospitals who have NO affiliation to a for profit entity.

Please tell us which sessions you will primarily attend (You have access to both sessions on days you are registered)

June 1: R&D Strategies Antibody/Protein Summit **June 2:** From Discovery to POC Antibody/Protein Summit

Yes! I/We will attend **Drug Discovery & Development of Innovative Therapeutics JAPAN • Keio Plaza Hotel • Tokyo, Japan**

R9162RNAI

Name: Dr/Mr/Ms _____

E-Mail _____

Job Title _____

Mobile no _____

Department _____

Company _____

Address _____

Post Code _____

Tel _____

Name & Title of Approving Manager _____

Name & Title of Training Manager _____

Main Business/Activity _____

Please tick: I enclose my Cheque/Draft payable to IBC Asia (S) Pte Ltd

I am paying by bank transfer (copy attached)

Payment by Credit Card: Amex Visa Mastercard

Card Holder _____

Signature _____

Card Number _____ Expiry Date _____

I cannot attend this event but

I would like to purchase the conference documentation @ US\$399/S\$618

Please put me on your mailing list.

Please note: The invoice you receive for your registration will list the Singapore dollar equivalent of the listed registration fees since billing is done by IBC's Singapore office.

Fee includes luncheons, cocktail refreshments and complete set of documentation. It does not include the cost of accommodation and travel.

CANCELLATIONS/SUBSTITUTIONS:

If you are unable to attend, a substitute delegate will be very welcome in your place. If this is not suitable, a 10% service charge will be payable. Registrations cancelled less than seven days before the event must be paid in full.

Speakers are subject to change without notice.

Venue & Hotel Information

Keio Plaza Hotel Shinjuku

2-2-1 Nishi-Shinjuku • Shinjuku-ku, Tokyo • 160-8330 Japan

Tel: +81-3-3344-0111 • Fax: +81-3-3345-8269 • www.keioplaza.com

Make Your Hotel Reservation Online:

Step 1: Go to <http://www.keioplaza.com/index.html>

Step 2: Click on "Corporate Login" at the bottom of the hotel home page

Step 3: Enter Account Name: ibc0601 and Password: ibc0601

Step 4: The booking form will appear to make your reservations at the discounted IBC rates of JPY20,000 (May 31-June 5) and JPY22,000 (May 30 and June 6)

Conference Registration Details

TEAM DISCOUNTS: Register 3 at the Standard Rate and the 4th goes FREE! Save up to USD1799. When three members from the same company register and pay for the conference at the same time, the fourth attends for FREE. This discount is valid only based on the standard rate (after May 1, 2009). For more information on the group rates or to register your group, please contact customer service at register@ibcasia.com.sg

Payment

Payments should be made in US dollars

• Payments by USD bank draft or cheque should be made payable to "IBC Asia (S) Pte Ltd".

• Payment by telegraphic transfer in USD must be made to:

IBC Asia (S) Pte Ltd
A/C No.:260-457866-178 (USD)
The Hongkong and Shanghai
Banking Corporation Limited
21 Collyer Quay, HSBC Building,
Singapore 049320

Important Note

Please quote the name of the delegate and event title on the remittance advice when remitting payment. Bank charges are to be deducted from participating organisations own accounts.

Attendance will only be permitted upon receipt of full payment. Participants wishing to register at the door are responsible to ensure all details are as published. IBC Asia will not be responsible for any event re-scheduled or cancelled.

General Information

DATA PROTECTION:

The personal information entered during your registration/order, or provided by you, will be held on a database and may be shared with companies in the Informa Group in the UK and internationally. Sometimes your details may be obtained from or shared with external companies for marketing purposes. If you do not wish your details to be used for this purpose, please contact the Database Manager Catherine Shen on catherine.shen@ibcasia.com.sg, Ph: +65 6835 5141 or Fax: +65 6734 4053.