NINTH ANNUAL
microRNA
Targets & Tools for Therapeutic Development

March 4-5 2013
Boston Marriott Cambridge
Cambridge, MA

20+ SCIENTIFIC PRESENTATIONS COVERING

• Therapeutic Potential in Inflammation, Cancer and Pain
• New Technological Strategies
• microRNAs as Biomarkers and in Diagnostics
• Tissue-Based Analysis
• Circulating miRNAs

AGENDA HIGHLIGHTS

microRNAs in Diagnostics and Targeting Rheumatic Disease
Steffen Gay, M.D., Professor, Experimental Rheumatology, University of Zurich

Causes and Consequences of microRNA Dysregulation in Cancer
Carlo M. Croce, M.D., Professor and Chair, Department of Molecular Virology, Immunology and Medical Genetics; Director, Institute of Genetics, Ohio State University

Pre-Clinical Development of a Tumor Suppressor miRNA
David Brown, Ph.D., Director, Research, Mima Therapeutics

microRNAs: Novel Target Class for Small Molecule Drug Discovery?
Pramod Pandey, Ph.D., Senior Research Investigator, Center for Proteomic Chemistry, Integrated Lead Discovery, Novartis Institute for Biomedical Research, Inc.

Circulating miRNAs as Biomarkers: How Far Have We Made It?
Igor Mikaelian, M.D., D.V.M., MSc, DACVP, Research Leader, Hoffman-La Roche, Inc.

Extracting Contextual microRNA Information for Cancer Diagnostics
Lorenzo F. Sempere, Ph.D., Research Assistant Professor, Department of Medicine, Geisel School of Medicine at Dartmouth

SHORT COURSE:
Sunday, March 3
Computational Aspects of microRNA

CORPORATE SPONSORS
**SUNDAY, MARCH 3**

1:30 pm Registration for Pre-Conference Short Course

2:00 – 5:00 pm Pre-Conference Short Course

Computational Aspects of microRNA

**Computational Analysis of Noncoding RNA**

Stefan Washietl, Ph.D., Scientist, Computational Biology, Massachusetts Institute of Technology

**Using a Bioinformatics Resource Manager**

Susan C. Tilton, Ph.D., Scientist, Computational Biology & Bioinformatics, Pacific Northwest National Laboratory

**BioVLAB-MMIA: A Cloud Environment for microRNA and mRNA**

Integrated Analysis (MMIA) on Amazon EC2

Hyungro Lee, Ph.D., Pervasive Technology Institute, School of Informatics and Computing, Indiana University

(Separate Registration Required)

**MONDAY, MARCH 4**

7:30 am Registration & Morning Coffee

8:10 Chairperson’s Opening Remarks

**Therapeutic Potential**

Inflammation

8:15 Presentation to be Announced

8:45 MiR-181b-Mediated Control of NF-kB in Acute and Chronic Inflammation

Mark W. Feinberg, M.D., Assistant Professor of Medicine, Cardiology, Brigham and Women’s Hospital

Endothelial activation and dysfunction have been linked to a variety of vascular inflammatory disease states including sepsis, atherosclerosis, diabetes, and rheumatoid arthritis, among others. Activation of NF-kB signaling has been implicated in physiological and pathological processes. This talk will highlight both published and unpublished findings on the role of a microRNA, miR-181b, that we identified as a critical regulator of NF-kB signaling in acute (sepsis) and chronic (atherosclerosis and asthma) inflammation disease models and in human subjects. Mechanistic studies will reveal how we uncovered miR-181b target gene, importin-alpha3 that regulates NF-kB nuclear accumulation. Innovative in vivo delivery strategies for miR-181b mimetics will be highlighted to target the vascular endothelium.

9:15 microRNAs in Diagnostics and Targeting Rheumatic Disease

Steffen Gay, M.D., Professor, Experimental Rheumatology, University of Zurich

By studying the pivotal role of synovial fibroblasts (SF) in rheumatoid arthritis (RA), we detected miR-145 and 155 to be expressed in RASF and monocytes and eventually to be used with miR 223 for diagnostics in very early disease. In related work we could not induce arthritis in miR 155 ko mice. We reported also that miR-203 is a strong regulator of IL6. Most interesting was that IL6 was neither regulated by TLR-signaling nor proinflammatory cytokines such as IL-1 and TNFα, but by methylation in the promoter of the miR-203. Moreover, we further found that miR 18a enhances the IL6 mediated production of the acute-phase proteins fibrinogen and haptoglobin in human hepatocytes. However, most promising is the targeting of miR 323 for the induction of TIMP-3 as an inhibitor of MMPs as well as IL6 and TNFα.

9:45 Coffee Break in the Exhibit Hall with Poster Viewing

10:15 Streamlined microRNA Profiling for the Clinical Setting

Daniel C. Pregibon, Ph.D., CTO, Firefly BioWorks

Clinical adoption of microRNA biomarkers necessitates a robust method to profile large sample cohorts. Firefly BioWorks has developed FirePlex™, a high-throughput method of microRNA analysis for research and clinical settings. We use FirePlex™ to demonstrate extraction-free microRNA detection in crude cell lysates, fresh tissues, FFPE, and blood-based specimens.

10:30 Sponsored Presentation (Opportunity Available)

10:45 Digital Multiplexed miRNA Quantitation for Translational Research Studies Using nCounter Technology

Joseph M. Beechem, Senior Vice President, Research & Development, NanoString Technologies

NanoString’s miRNA expression tools allow researchers to digitally count hundreds of miRNAs from Human, Mouse, Rat and Drosophila genomes. Compatible with challenging sample types (e.g., FFPE, plasma, serum) nCounter miRNA Expression Assays can be utilized for biomarker discovery, validation, and retrospective-prospective studies. NanoString’s miRGE-technology, enables highly-multiplexed detection of both miRNAs and miRNAs simultaneously: a single tube expression assay for small RNAs and their regulated targets. Translational research studies with miRNAs will be highlighted.

11:15 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

Cancer

12:55 pm Chairperson’s Remarks

1:00 Creating Unique Mass Signatures for Label-Free and Quantitative Analysis of MicroRNA Biomarkers

Norman Chiu, Ph.D., Associate Professor, Chemistry and Biochemistry, University of North Carolina at Greensboro

With microRNAs unique ability to post-transcriptionally regulate gene expression, microRNAs have been clinically associated with cancer and many other diseases. More than 1,700 human microRNAs have been reported. With their relative small sizes (19-25 nt), the analysis of specific microRNA poses a big challenge to the current methods for measuring nucleic acids. In this presentation, the development of a new mass spectrometric method that improves the accuracy for measuring specific microRNA biomarkers for glioblastoma is discussed.

1:30 P63/P73-Regulated microRNAs Control the Metastatic Dissemination

Benjamin Orly, Ph.D., Associate Professor, Therapy Primary Bone Tumor, Nantes Medical School, INSERM U967

Expression profiling allowed us to identify p63/p73-regulated miRs potentially targeting the TGFβ pathway. These miRs repress TGFRII and Smad4 principally and thereby interfere with metastasis dissemination. Preliminary data demonstrate that these miRs are regulated by both p63 and p73, and that they are able to repress the activation of the TGFβ pathway in vitro, and to regulate tumor cells dissemination in vivo. Taken together, these findings support the hypothesis that a p63/p73-regulated miR program mediates metastasis dissemination, in different type of cancer, through the regulation of the TGFβ pathway.

2:00 Causes and Consequences of microRNA Dysregulation in Cancer

Carlos M. Croce, M.D., Professor and Chair, Department of Molecular Virology, Immunology and Medical Genetics; Director, Institute of Genetics, Ohio State University

2:30 Refreshment Break in the Exhibit Hall with Poster Viewing

3:10 microRNA Regulation of Cell Viability and Drug Sensitivity in Lung Cancer

Alexander Pertsemidis, Ph.D., Associate Professor, Pediatrics and Cellular & Structural Biology, UT Health Science Center at San Antonio

Lung cancer is the leading cause of cancer-related deaths, with the majority of deaths due to failed therapy from tumor drug resistance. Third-generation chemotherapeutic agents represent the standard first-line treatment for advanced small cell (SCLC) and non-small cell (NSCLC) lung cancer patients. Response rates are poor (20-40%) with a median survival of 8–10 months. In an unbiased and comprehensive approach, we have combined a high-throughput screening platform with a library of chemically synthesized microRNA mimics and inhibitors. We have used this platform to identify mimics and inhibitors that reduce cell viability in general, and those that specifically sensitize cells to taxanes, which add novel therapeutic tools for the treatment of lung cancer.
Initially epithelial ovarian cancer is responsive to chemotherapy, however, eventually patients relapse because of the development of drug resistance. We identified and functionally characterized miRNAs that affect the sensitivity for cisplatin. By modulating the expression levels of these miRNAs in epithelial ovarian cancer cells present in the ascites fluid of chemo-resistant ovarian cancer patients, we re-sensitize tumor cells to cis/carboplatin.

A complete quality-controlled pipeline for detection of miR-371-3 and miR-302/367 in serum of germ cell cancer patients has been developed, based on magnetic bead-based purification and qPCR quantification. This Targeted Serum miRNA (TSmiR) test was applied to four independent serum sample series. TSMiR demonstrated a consistent and significant increase for miR-371-3 and 367 in serum of seminoma and nonseminoma patients. Compared to AFP and hCG, the TSMiR test performed better. Application of a combined AFP/hCG-TSMiR test correctly identified all patient serum samples with GCC, with only one false-negative case based on the TSMiR-test alone. In conclusion, a highly reproducible and informative serum test for patients with a GCC is described, suitable to be prospectively tested for diagnosis and follow-up of GCC patients.

Cumulative evidence now suggests that specific miRNAs and genetic variations interfering with miRNA function (miRNA polymorphisms) are involved in the prognosis and progression of a variety of diseases and can serve as biomarkers to predict drug response. Over expression or down regulation of a miRNA, and loss or gain of miRNA function due to miRNA polymorphisms can potentially affect expression of hundreds of genes and related pathways contributing to a drug resistant phenotype. Detection of prognostic-miRNAs and miRNA polymorphisms can potentially improve diagnosis, treatment and prognosis in patients and has profound implications in the fields of pharmacogenomics and personalized medicine.

4:45 microRNA Polymorphisms and the Future of Personalized Medicine
Prasun Mishra, Ph.D., Earl Stadtman Investigator Candidate, Center for Cancer Research, National Cancer Institute, NIH

Implementing microRNA Biomarkers for Non-invasive Diagnoses of Diseases
Thomas Brefort, Ph.D., Vice President, Biomarker Development & Services, Comprehensive Biomarker Center

microRNAs as Biomarkers and in Diagnostics
Tissue Based Analysis

3:40 miRNAs Modulating Anticancer Drug Sensitivity in Ovarian Cancer Cells: Possible Therapeutic Applications
Erik Wiemer, Ph.D., Associate Professor, Medical Oncology, Erasmus University Medical Center/Daniel den Hoed Cancer Center

4:10 A miR-371-3/367 Serum Test for the Diagnosis and Follow-Up of (Testicular) Germ Cell Cancer Patients
Leendert Looijenga, Ph.D., Head, Research Lab, Pathology, Erasmus University

10:10 microRNAs: Novel Target Class for Small Molecule Drug Discovery?
Pramod Pandey, Ph.D., Senior Research Investigator, Center for Proteomic Chemistry, Integrated Lead Discovery, Novartis Institute for Biomedical Research, Inc.

9:30 Coffee Break in the Exhibit Hall with Poster Viewing

4:45 microRNA Polymorphisms and the Future of Personalized Medicine
Prasun Mishra, Ph.D., Earl Stadtman Investigator Candidate, Center for Cancer Research, National Cancer Institute, NIH

Cumulative evidence now suggests that specific miRNAs and genetic variations interfering with miRNA function (miRNA polymorphisms) are involved in the prognosis and progression of a variety of diseases and can serve as biomarkers to predict drug response. Over expression or down regulation of a miRNA, and loss or gain of miRNA function due to miRNA polymorphisms can potentially affect expression of hundreds of genes and related pathways contributing to a drug resistant phenotype. Detection of prognostic-miRNAs and miRNA polymorphisms can potentially improve diagnosis, treatment and prognosis in patients and has profound implications in the fields of pharmacogenomics and personalized medicine.

5:15 Moderated Breakout Discussion Groups
6:15 Welcome Reception in the Exhibit Hall with Poster Viewing
7:15 End of Day

TUESDAY, MARCH 5

7:45 am Breakfast Presentation (Sponsorship Opportunity Available) or Morning Coffee
8:25 Chairperson’s Remarks

8:30 Exosome-Mediated miRNA Transfer and Its Role in Pain
Seena K. Aijit, Ph.D., Assistant Professor, Pharmacology and Physiology, Drexel University College of Medicine

To decipher the role of differentially expressed circulating miRNAs in blood samples from patients with Complex Regional Pain Syndrome, we investigated exosome-mediated information transfer. We characterized the exosomes obtained from cell culture media with and without inflammatory stimulus and extended the studies to patient samples to investigate changes in exosomal content in response to inflammation and chronic pain. In addition to miRNAs, alterations in exosomal mRNA and proteins were also studied.

9:00 Talk Title to be Announced
Henrik Ørum, Ph.D., MSc, CSO & Vice President, Business Development, Santaris Pharma A/S

12:10 pm Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own
miRNAs inhibit gene expression by annealing to complementary elements in a target mRNA. Therefore, sequence variants can alter miRNA function by creating or destroying target sites, leading to a myriad of diseases. Here we will discuss miRNA-target site polymorphisms identified by our laboratory that associate with an increased risk for developing cancer and appear to govern tumor biology and confer altered response to therapy.

2:45 Presentation to be Announced
Speaker to be Announced

3:15 Refreshment Break in the Exhibit Hall with Poster Viewing
Blood Based Analysis

3:50 Direct Detection of Circulating miRNAs
Sumedha Jayasena, Ph.D., Vice President, Technology & Therapeutic Development, Therapeutics, SomaGenics

SomaGenics has developed a new method called miR-IDirect that allows the direct release, enrichment and detection of miRNAs from plasma without prior isolation of total RNA. This novel technology will have an impact on the development and implementation of circulating miRNA-based biomarkers in various disease settings, including cancer.

4:20 Circulating miRNAs as Biomarkers: How Far Have We Made It?
Igor Mikaelian, M.D., D.V.M., MSc, DACVP Research Leader, Hoffman-La Roche, Inc.

Quantification of miRNAs in body fluids is the latest biomarker frontier in drug safety assessment. The presentation will review the microRNAs that have been proposed as biomarkers of organ toxicity, with a special emphasis on vascular, cardiac and hepatic toxicity. The discussion will focus on biomarker identification strategies and the pre-clinical validation plans for clinical implementation.

4:50 Enzymatic and Non-Enzymatic Quantitation of Circulating MicroRNAs
Dominik M. Duelli, Ph.D., Assistant Professor, Department of Cellular and Molecular Pharmacology, Department of Cell Biology and Anatomy, Chicago Medical School, Rosalind Franklin University of Medicine and Science

Accurate enzyme-based quantitation of miRNAs is compromised by inhibitors that copurify with RNA. We evaluated approaches for blood collection, extraction, and enzyme choice to promote exact quantitation. Alternatively, we introduce the use of a non-enzymatic method that employs SmartFlareTM RNA Detection probes for the specific and sensitive quantitation of plasma miRNAs requiring minimal processing within an hour of venipuncture.

5:20 End of Conference

HOTEL & TRAVEL INFORMATION

Conference Venue and Hotel:
Boston Marriott Cambridge
Two Cambridge Ctr
50 Broadway
Cambridge, MA 02142
Phone: 617-494-6600
Discounted Room Rate: $159 s/d
Discounted Room Cut-off Date: February 4, 2013

Please visit our conference website healthtech.com/MRN or call the hotel directly to reserve your sleeping accommodations. You will need to identify yourself as a Cambridge Healthtech Institute conference attendee to receive the discounted room rate with the host hotel. Reservations made after the cut-off date or after the group room block has been filled (whichever comes first) will be accepted on a space-and-rate-availability basis. Rooms are limited, so please book early.

Car Rental Discounts:
Special discount rentals have been established with Hertz for this conference. Please use one of the following methods:
• Call Hertz 1-800-654-3131 and use our Hertz Convention Number (CV): 04KL004
• Go online www.hertz.com and use our Hertz Convention Number (CV): 04KL004

Flight Discounts:
Special discounts have been established with American Airlines for this conference.
• Call American Airlines 1-800-433-1790 and use Conference code 5633BV.
• Go to www.aa.com/group and enter Conference code 5633BV in promotion discount box.
• Contact our dedicated travel agent, Wendy Levine at 1-877-559-5549 or wendy.levine@protravelinc.com.

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SPONSORSHIP & EXHIBIT INFORMATION

CHI offers comprehensive sponsorship packages which include presentation opportunities, exhibit space and branding, as well as the use of the pre and post-show delegate lists. Customizable sponsorship packages allow you to achieve your objectives before, during, and long after the event. Signing on early will allow you to maximize exposure to hard-to-reach decision makers!

Agenda Presentations
Showcase your solutions to a guaranteed, highly-targeted audience. Package includes a 15 or 30-minute podium presentation within the scientific agenda, exhibit space, on-site branding, and access to cooperative marketing efforts by CHI.

Breakfast & Luncheon Presentations
Opportunity includes a 30-minute podium presentation. Boxed lunches are delivered into the main session room, which guarantees audience attendance and participation. A limited number of presentations are available for sponsorship and they will sell out quickly. Sign on early to secure your talk!

Invitation-Only VIP Dinner/Hospitality Suite
Sponsors will select their top prospects from the conference pre-registration list for an evening of networking at the hotel or a choice local venue. CHI will extend invitations and deliver prospects. Evening will be customized according to sponsor’s objectives:

- Purely social
- Focus group
- Reception style
- Plated dinner with specific conversation focus

Exhibit
Exhibitors will enjoy facilitated networking opportunities with high-level conference delegates. Speak face-to-face with prospective clients and showcase your latest product, service, or solution.

*Inquire about additional branding opportunities!

Looking for additional ways to drive leads to your sales team?

CHI can Help!

We offer clients numerous options for custom lead generation programs to address their marketing and sales needs, including:

- Live Webinars
- White Papers
- Market Surveys
- Podcasts and More!

Benefits of working with Cambridge Healthtech Institute for your lead generation needs:

- Your campaign will receive targeted promotion to Cambridge Healthtech Institute’s unparalleled database of over 800,000 individuals, all of which are involved in all sectors of the life sciences – lists can be segmented based on geography, research area, title and industry.
- All custom lead generation programs are promoted through our experienced marketing team that will develop and drive targeted campaigns to drive awareness and leads to your lead generation program.
- For our webinar programs, we offer assistance in procuring speakers for your web symposia through our extensive roster of industry recognized speakers across multiple disciplines within life sciences, as well as provide an experienced moderator and dedicated operations team will coordinate all efforts.
- If choosing a white paper program, we can offer editorial experience and provide an industry recognized author to write your white paper.

CHI also offers market surveys, podcasts, and more!

To discuss the various ways your company can participate as a sponsor or exhibitor, please contact:

Tim McLucas
Business Development Manager
Phone: 781-972-1342
Email: tmclucas@healthtech.com
**Pricing and Registration Information**

**SHORT COURSES**

(Includes access to short course only)

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<tr>
<td>Single short course</td>
<td>$695</td>
<td>$395</td>
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**CONFERENCE PRICING**

(Includes access to a two-day conference, excludes short courses)

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<tr>
<td>Advance Registration Discount until January 25, 2013</td>
<td>$1695</td>
<td>$845</td>
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<tr>
<td>Registrations after January 25, 2013, and on-site</td>
<td>$1895</td>
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**CONFERENCE DISCOUNTS**

**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.

For more information on group rates contact David Cunningham at 781-972-5472.

**Alumni Discount-SAVE 20%**

Cambridge Healthtech Institute (CHI) appreciates your past participation at microRNA. 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. Please note: Our records must indicate you were an attendee of microRNA in the past in order to qualify. This discount cannot be combined with other discount offers.

**Poster Submission-Discount ($50 Off)**

Present a poster at the Ninth Annual microRNA Conference to showcase your research to leaders from top pharmaceutical, biotech, academic and government institutes. Your poster abstract will be displayed to our international delegation and published in conference materials.

**How to submit:** 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 jrring@healthtech.com. “CHI reserves the right to publish your poster title and abstract in various marketing materials and products. Poster abstracts are due by February 6, 2013.”

If you are unable to attend but would like to purchase the MicroRNA CD for $350 (plus shipping), please visit healthtech.com/MRN. Massachusetts delivery will include sales tax.

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How to Register: healthtech.com/MRN

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Please use keycode MRN F when registering!