Indications Discovery & Drug Repositioning Summit
Finding New Tricks for Old Drugs
March 13-14, 2012
Crowne Plaza Philadelphia Downtown Hotel
Philadelphia, PA

Coverage Includes:
Data Analysis
Pre-Clinical Testing in Disease Models
Targeting Metabolic Pathways
Meeting Current Regulatory Standards
Technologies for Indication Discovery
Turning “Off Target Effects” of Current Drugs into Novel Targets
Screening in Disease Models
Computational Approaches
Drug Repositioning for Neglected Diseases
Successful Case Studies for Drug Repositioning

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TUESDAY, MARCH 13

7:30 am  Breakfast Presentation  (Sponsorship Opportunity Available) or Morning Coffee

8:25  Chairperson’s Opening Remarks
Aris Persidis, Ph.D., President, Biovista, Inc.

DRUG REPOSITIONING FOR NEGLECTED DISEASES

8:30  Repurposing Kinase-Targeted Medicinal Chemistry for Neglected Tropical Disease
Drug Discovery
Mike Pollastri, Ph.D., Associate Professor, Chemistry & Chemical Biology, Northeastern University
This presentation will discuss the target purposing approach for neglected disease drug discovery, highlighted by some examples in my lab where we have found anti-parasitic activities in classes of human kinase inhibitors.

9:00  Sponsored Presentation  (Opportunity Available)

9:30  Systematic Drug Repositioning for Neglected Diseases and Not Only
Aris Persidis, Ph.D., President, Biovista, Inc.
Drug repositioning is emerging as a major strategy to accelerate the development of drugs in rare or neglected diseases and beyond. The potential for innovation and novelty is significant and a number of case studies will be discussed, including Friedrich’s Ataxia, Leber’s neuropathy, progressive multiple sclerosis and others.

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

10:40  Drug Repositioning: An Opportunistic Approach to Identifying New Drugs for Malaria?
Julie Lotharius, Ph.D., Associate Director, Translational Medicine, Medicines for Malaria Venture
Medicines for Malaria Venture has tested a vast number of approved, investigational and discontinued drugs in an in vitro assay of human Plasmodium falciparum blood stage infection. Several hits with low micromolar activity and acceptable human safety and pharmacokinetic profiles were then followed up by in vivo testing in a humanized mouse model of P. falciparum infection. In addition, target and genome-based searches comparing the mechanism of action of known drugs to pathways responsible for parasite survival and propagation have been employed to identify compounds from the clinical development pipeline that may have anti-malarial activity. Data generated from these activities will be presented.

11:10  Repurposing Drug Candidates - Fexinidazole as a Promising Treatment for Visceral Leishmaniasis
Susan Wylie, Senior Research Associate and Lecturer, Division of Biological Chemistry and Drug Discovery, University of Dundee
We report the potential for therapeutic switching of fexinidazole, currently in phase I clinical trials for treatment of human African trypanosomiasis, as a novel oral treatment for Visceral Leishmaniasis. This 2-substituted 5-nitroimidazole is rapidly oxidized in vivo to sulfoxide and sulfone metabolites. These metabolites were both active against Leishmania donovani amastigotes grown in macrophages, whereas fexinidazole itself is inactive. A once daily regimen for 5 days at 200mg kg-1 caused a 98.4% suppression of infection in a VL mouse model, superior to the clinical drugs miltefosine or pentostam. In African trypanosomes, the mode of action of nitrodrugs involves reductive activation via an NADH-dependent bacterial-like nitroreductase. Overexpression of the leishmania homologue in L. donovani increased sensitivity to fexinidazole by 19-fold indicating that a similar mechanism is involved in these parasites. These findings illustrate the potential of fexinidazole as a much needed oral treatment for VL.

11:40  Repurposing Drugs for Tropical Diseases: Case Studies and Open-Source Screening Initiatives
Curtis R. Chong, M.D., Ph.D., M.Phil., Partners Cancer Care Hematology & Oncology Fellow, Dana Farber, Brigham and Women’s Hospital, Massachusetts General Hospital

12:10 pm  Luncheon Presentation  (Sponsorship Opportunity Available) or Lunch on Your Own

1:40  Chairperson’s Remarks

COMPUTATIONAL APPROACHES

1:45  Applying Computational Methods for Drug Repositioning
Pankaj Agarwal, Ph.D., Director, Computational Biology, Molecular Discovery & Development, GlaxoSmithKline
2:15 Computational Drug Repurposing Using Compendia of Public Molecular Data
Joel Dudley, Ph.D., Director, Informatics and Co-Founder, NuMedii, Inc.
Public molecular data repositories, such as the NCBI Gene Expression Omnibus (GEO), contain billions of molecular measurements, many of which are relevant to human disease. Although these repositories are often intended to serve as data archives, it is now possible to apply large-scale integrative informatics approaches to leverage the aggregate molecular data in these repositories towards evaluating new types of biomedical hypotheses. In this talk, I will present our recent work in developing and applying a systematic computational approach to identify novel drug indication relationships using public gene expression profiles, which recently led to the identification and pre-clinical validation of novel drug repositioning candidates for lung cancer and inflammatory bowel disease.

2:45 What is 505(b)(2) and the Growing Importance of it Today in Drug Development?
Ken Phelps, President and CEO, Camargo Pharmaceutical Services
In the fiscal year 2006, approximately 20% of new small-molecule drugs were approved through the 505(b)(2) process; two years later, more than half of the small-molecule new drugs approved in the United States were based on this strategy. Judging from this rate it is expected that the percentage of 505(b)(2) approvals will be greater than 80% within the next few years. We will walk through and explain the reasons behind the remarkable success of the 505(b)(2) process.

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

3:45 Repositioning Two Ways: Integrating Large-Scale Molecular Docking and High-Throughput Screening
Yvonne Li, Ph.D., Scientist, Canada’s Michael Smith Genome Science Center
We have developed a virtual screening platform to predict novel binding interactions between existing drugs and drug targets, with multiple levels of false positive filtering. We present an example of this approach for the breast cancer target p90RSK and validated our results using both high-throughput screening and cellular assays.

4:15 Repurposing of NSAIDs as Anticancer Chemopreventive Agents
Jie Zheng, Ph.D., Associate Member, Department of Structural Biology, St. Jude Children’s Research Hospital

4:45 Focused Breakout Discussions
In this interactive session, several topics will be offered for discussions and delegates are invited to choose a breakout topic of interest and join the moderated discussion at hand. In this informal setting, participants are encouraged to share examples from their work, vet ideas with peers and be part of a group problem-solving endeavor. We emphasize that this discussion is an informal exchange amongst scientists and is not meant to be, in any way, a product discussion.

6:00 End of Day

WEDNESDAY, MARCH 14

7:15 am Breakfast Presentation (Sponsorship Opportunity Available) or Morning Coffee

STRATEGIES AND CASE STUDIES

8:30 Chairperson’s Remarks

8:35 Combination Therapy for Rescuing Targeted Erlotinib Therapy in Oral Cancers: From in silico Models to Clinical Trial
Yves A. Lussier, M.D., Professor of Medicine and Engineering; Director, Institute for Interventional Health Informatics, University of Illinois at Chicago

9:05 Synopsis of Breakout Discussions

9:35 Talk Title to be Announced
Stephen Wong, Ph.D., P.E., Founding Chairman for Department, Systems Medicine and Bioengineering, The Methodist Hospital Research Institute; John S Dunn Distinguished Endowed Chair in Biomedical Engineering, The Methodist Hospital; Director, NCI Center for Modeling Cancer Development

10:05 Networking Coffee Break in the Exhibit Hall with Poster Viewing

10:45 Striking Gold: Pre-Clinical and Clinical Development of Auranofin for Relapsed Lymphocytic Leukemia
G. Sitta Sittampalam, Ph.D., Senior Scientific Officer, Therapies for Rare & Neglected Diseases and the Learning Collaborative, NIH Center for Translational Therapeutics, National Institutes of Health
In this presentation we will discuss the NIH Chemical Genomics Center (NCGC) Pharmaceutical Collection of approved drugs, and its application in repositioning auranofin for the treatment of relapsed CLL, progressing from screens of patient samples to the clinic in 18 months.

11:15 In silico Repositioning of Approved Drugs and Collaboration for Rare and Neglected Diseases
Sean Ekins, Ph.D., Collaborations Director, Collaborative Drug Discovery, Inc.

11:45 Panel Discussion: Turning Off-Target into “On” Target Drugs – Lessons to be Learned

12:15 pm End of Conference
Conference Hotel:
Crowne Plaza Philadelphia Downtown
1800 Market Street
Philadelphia, PA 19103
Phone: 215.561.7500
Discounted Room Rate: $179 s/d
Discounted Cut-Off Date: February 13, 2012

Please visit our conference website to make your reservations online or call the hotel directly to reserve your sleeping accommodations. Identify yourself as a Cambridge Healthtech Institute conference attendee to receive the reduced room rate. 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.

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C: 781-687-9400
E: jvacca@healthtech.com
# Indications Discovery & Drug Repositioning Summit

**Finding New Tricks for Old Drugs**

**Pricing and Registration Information**

**Conference Pricing**

<table>
<thead>
<tr>
<th>Premium Package</th>
<th>BEST VALUE</th>
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<tbody>
<tr>
<td>Includes access to two conferences: Encouraging Development of Therapeutics for Neglected Diseases (March 12-13) and the Indications Discovery &amp; Drug Repositioning Summit (March 13-14)</td>
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<td>Commercial</td>
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<tr>
<td>Early Registration Discount until December 16, 2011</td>
<td>$1975</td>
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<tr>
<td>Advance Registration Discount until January 27, 2012</td>
<td>$2125</td>
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<td>Registrations after January 27, 2012, and on-site</td>
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**Individual Event Pricing - Indications Discovery & Drug Repositioning Summit**

| Early Registration Discount until December 16, 2011 | $1295 | $585 |
| Advance Registration Discount until January 27, 2012 | $1445 | $665 |
| Registrations after January 27, 2012, and on-site | $1695 | $745 |

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