Third Annual
Encouraging Development of Therapeutics for
Neglected Diseases
March 12-13, 2012 Crowne Plaza Philadelphia Downtown Hotel | Philadelphia, PA

Coverage Includes:
- Promising Therapeutic Targets and Strategies
- Case Studies of Successful Collaborations
- Drug Repositioning
- Screening for Novel Therapeutic Possibilities
- Targeting Leishmania
- Open-Access Resources
- Success and Progress in NDs, Including Malaria and TB

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Educational Grants Supported by

organized by:
Cambridge Healthtech Institute

healthtech.com/NGL
MONDAY, MARCH 12

7:30 am  Registration and Morning Coffee
8:30  Chairperson’s Opening Remarks
8:35  Facilitating R&D for Neglected Diseases: Fostering Collaboration and Navigating the Complex Interface among Diverse Stakeholders

Jacqueline B. Fine, Ph.D., CLPTM, Associate Director, Global Outlicensing and Asset Management, Merck Research Laboratories

The challenges we face toward significantly impacting global health, particularly in the developing world, are too complex for any single entity to solve alone. Partnership, as a critical pillar of Merck’s global access strategy will be highlighted, with emphasis on depictions of how robust collaboration among individuals and organizations with diverse backgrounds and capabilities joined together by the mutual goal of improving human health can lead to true innovation. The route to achieving mutual goals may vary from partnership to partnership with respect to specific strategies, targets and needs of the parties; however, we believe the agreements supporting these collaborations should be aligned with current and evolving best practices and policies that include ensuring access on a sustainable basis. A spectrum of activities will be described, from licenses facilitating development of Merck assets for neglected disease indications by public-private partnerships, to a first-of-its-kind joint venture designed to be a sustainable R&D organization that operates like a business – but with a not-for-profit model – aimed at developing new vaccines in areas of unmet need and optimizing existing vaccines for resource-limited settings.

9:05  Drug Discovery for Neglected Diseases: Molecular Target-Based and Phenotypic Approaches
Ian Gilbert, Ph.D., Professor, Drug Discovery Unit, University of Dundee

I will describe the setting up of a fully integrated drug discovery unit within an academic sector, and how we are applying this for drug discovery for neglected diseases. The focus of the talk will be on human African trypanosomiasis, where we have used both molecular target-based and phenotypic approaches to discover starting points for drug discovery; I will discuss the relative merits of these approaches.

Jean-Pierre Paccaud, Director, Business Development, Drugs for Neglected Diseases Initiative

The emergence of PDPs developing new treatments and vaccines against neglected tropical diseases provides hope for millions of patients awaiting life saving therapeutics the industry failed to develop because of the lack of a market. Although it took PDPs several years to succeed in involving industry into their activities, today most pharma companies advertise their relationship with one or several PDPs. Most of the collaborations are still at the discovery or pre-clinical stage, where pharma involvement does not generate significant costs for the industry. PDPs have demonstrated various achievements in R&D, developing products or vaccines with the help of their industrial collaborations, but they may face difficult challenges when it comes to industrial manufacturing, registration and distribution if they are not backed up by a strong industrial partner. At this stage, will industry be willing to engage in insuring sustainable drug/vaccine delivery at conditions enabling the neglected patients to access the drug? What can be the alternatives? What framework would be favorable for continued private sector involvement?

10:05  SCYNEXIS Integrated Parasitology Screening: Focusing Biotechnology Innovation To Achieve Neglected Disease Solutions

Yves Ribeill, Ph.D., President & CEO, SCYNEXIS, Inc.

Current strategies to identify new drugs for Neglected Tropical Diseases may overlook a rich source of innovation: compound collections from biotechnology companies. By evaluating these compound collections for properties that are aligned with the Target Product Profiles of Neglected Diseases they can quickly be focused towards optimization. The SCYNEXIS Integrated Parasitology Screening platform provides the rapid assessment of compounds against over thirty parasite species relevant to Global Health.

10:35  Coffee Break in the Exhibit Hall with Poster Viewing

11:05  Blockade of iNKT Cells as a Therapeutic Strategy for Sickle Cell Disease
Robert Mashal, Ph.D., CEO, Office of the President, NKT Therapeutics

Blockade of iNKT cells is a promising new therapeutic strategy for the treatment of SCD. NKT Therapeutics is developing NKTT120, a novel antibody which specifically blocks iNKT cell function.

11:35  Distributed Drug Discovery (D3): Addressing Large Neglected Disease Challenges with Multiple Small-Scale and Open-Access Resources

William L. Scott, Ph.D., Research Professor, Department of Chemistry and Chemical Biology, Indiana University Purdue University Indianapolis (IUPUI)
Distributed Drug Discovery (D3) capitalizes on the powerful potential that comes from linking worldwide educational resources in an open-access framework. It creates synthetic procedures and equipment to enable students in distributed global locations to reproducibly make new neglected disease drug-lead candidates selected from large D3 virtual catalogs. With the freedom that comes from open-access it simultaneously harnesses the expertise of synthetic, computational and biological researchers while educating the next generation of drug discovery scientists.

12:05 pm  Challenges in the New Drug Discovery for Tuberculosis - A TMC207 Perspective and Update
Nacer Lounis, Ph.D., Scientist, Department of Microbiology, Johnson & Johnson Pharmaceuticals

- Discovery and developmental challenges for identifying new TB drugs.
- Update on TMC207: A new investigation drug in clinical studies for MDR-TB.
- Energy metabolism as a new target pathway for mycobacterial drug research

12:30 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

1:55 Chairperson’s Remarks

2:00 Recent Advances in Visceral Leishmaniasis Vaccine Development Using Genetically Altered Live Attenuated Parasites
Hira Nakhasi, Director, Division of Emerging and Transfusion Transmitted Diseases, OBRR/CBER/FDA
Leishmaniasis is a major Neglected Tropical Disease. There are no vaccines against Leishmania. Strategies such as killed parasites with or without adjuvants, subunit vaccines (recombinant proteins or DNA vaccines) have not resulted in a viable Leishmania vaccine. However, people who have recovered from infection develop a protective immunity against future infections. Hence we have used the strategy of developing live attenuated parasites which do not cause disease but develop protective immunity against various species of Leishmania.

2:30 Discover New Biologies for Protection Against Infection by Different and New Viruses
Grace Wong, Ph.D., CEO, (former Genentech, Millennium, AstraZeneca and Serono), ActoKine Therapeutics
We have identified ActoKine-2 (AK-2) which protects cells against infection by a broad spectrum of viruses. AK-2 was highly active against different viruses including West Nile viruses, bird flu, Rift Valley fever virus, Punta Toro phlebovirus, HIV, Yellow Fever Virus, Tacaribe virus, and Venezuelan equine encephalomyelitis (VEE) virus. AK-2 could be administered sub-lingually or nasally to prevent infection through the nose or mouth or intravaginally to prevent against sexually transmitted diseases. These novel routes of administration can be safer, cheaper, and logistically easier than traditional injections for disease prevention.

3:00 An End-to-End Corporate Commitment to NTDs: The Merck Model
Ken Gustavsen, Director, Global Health Partnerships, Merck & Co., Inc.
Neglected Tropical Diseases (NTDs) are a group of 20 diseases that disproportionately affect the poorest segment of the global population, thriving in areas with limited healthcare infrastructure. More than 1 billion people worldwide are infected or at risk of infection by one or more NTDs. Existing therapies can treat, control and eliminate some NTDs; for others, there is a need for the discovery and development of new therapies and for improvements in delivery methods to reach affected populations. As a global healthcare company, Merck has a broad range of resources to address NTDs. For decades we have been directly engaged with NTDs through program implementation, R&D and policy and advocacy efforts. Given our leadership position in global NTD programs, we aim to build on that foundation in the coming years through a variety of in-house activities and strategic external partnerships. Merck’s investments in global NTD partnerships and programs have yielded great progress in recent years and promise important advances in the near future. To secure those advances, continued collaboration and investment in both implementation and R&D will be critical. Merck continues to work in partnership with others to build on our portfolio of current programs and leverage our planned future investments to address the most critical needs in the fight against NTDs.

3:30 Refreshment Break in the Exhibit Hall with Poster Viewing

4:15 Thousands of Chemical Starting Points for Antimalarial Lead Identification
James Brown, Ph.D., Director, Computational Biology, GlaxoSmithKline

4:45 Focused Breakout Discussions

6:00 End of Day

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.

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 neuopathy, progressive multiple sclerosis and others.

10:00 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 Wyllie, 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 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 End of Conference
HOTEL & TRAVEL Information

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. You will need to 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.

Flight Discounts:
Special discount rentals have been established with American Airlines for this conference.
• Call 1-800-433-1790, use Conference code 1732AZ
• Go to www.aa.com/group, enter Conference code 1732AZ in promotion discount box
• Contact our designated travel agents at 1-877-559-5549 or chi@protravelinc.com.

Car Rental Discounts:
Special discount rentals have been established with Hertz for this conference. Please use one of the following methods:
• Call Hertz, 800-654-3131, use our Hertz Convention Number (CV): 04KL0003
• Go online www.hertz.com, use our Hertz Convention Number (CV): 04KL0003

EDUCATIONAL GRANT Opportunities

CHI has developed several Educational Grant opportunities for your company to participate and contribute to the conference. The options are designed to underwrite some speakers coming from overseas, as well as sponsoring selected delegates to be able to attend the event. Sponsoring companies who provide an educational grant will not only be promoted for providing assistance but will also have the option for a 25 minute sponsored podium presentation as part of the conference agenda. Your company will be branded on the conference brochure denoting Educational Grant support. Help the Cambridge Healthtech Institute bring awareness to this important cause by taking part in these opportunities.

Educational Grants also include:
• Educational Grant will be used to underwrite select speakers coming from overseas
• Company providing grant will have the opportunity of a 25-minute (including Q&A) presentation within the conference program
• Corporate logo on cover of conference brochure, denoting Educational Grant support (contingent upon CHI marketing deadlines)
• Company biography or Humane Program listing in program guide distributed on-site (50-word maximum)
• Total of 2 full-conference registrations (speaker does not have separate registration)
• Additional passes can be acquired at our discounted rate of 50%
• One-time use of delegate list — for one pre and one post-show mailing. Must sign Mailing List Agreement
• On-site signage denoting your company as an Educational Grant Supporter
• Corporate logo and URL link on the conference website denoting Educational Grant support.
• Corporate logo and URL link will be placed in conference documentation package delivered electronically, approximately 1 week prior to and 1-2 weeks post conference to all registered participants. Package includes: conference agenda, speaker slides, poster abstracts, sponsor logos with URL link, and exhibitor list with URL link.

To explore the various ways your company can participate, please contact:
Joseph Vacca
Manager, Business Development
T: 781-972-5431 | C: 781-687-9400 | E: jvacca@healthtech.com
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>
<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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<tr>
<td>Registrations after January 27, 2012, and on-site</td>
<td>$2325</td>
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**Individual Event Pricing - Encouraging Development of Therapeutics for Neglected Diseases**

| 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 |

**CONFERENCE DISCOUNTS**

- **Poster Submission** - Discount ($50 Off)
  - Poster abstracts are due by February 15, 2012. 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 jring@healthtech.com. *CHI reserves the right to publish your poster title and abstract in various marketing materials and products.

- **15% discount for SAPA members**

- **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. Please reproduce this registration form as needed.

- **GROUP DISCOUNTS AVAILABLE!** Special rates are available for multiple attendees from the same organization. For more information on group discounts contact David Cunningham at +1-781-972-5472.

If you are unable to attend but would like to purchase the Encouraging Development of Therapeutics for Neglected Diseases 2012 conference CD for $350 (plus shipping), please visit healthtech.com/NGL. Massachusetts delivery will include sales tax.

How to Register: [healthtech.com/NGL](http://healthtech.com/NGL)

Please use [keycode NGLF](#) when registering!

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