6th DRUG DISCOVERY FOR NEURODEGENERATION CONFERENCE: An Intensive Course on Translating Research into Drugs

February 12-14, 2012 • New York, NY

Presented by the Alzheimer's Drug Discovery Foundation

www.alzdiscovery.org
Mission

The Alzheimer’s Drug Discovery Foundation’s (ADDF) sole mission is to rapidly accelerate the discovery of drugs to prevent, treat and cure Alzheimer’s disease, related dementias and cognitive aging. Founded in 1998 by Co-Chairmen Leonard and Ronald Lauder, the ADDF awards grants to leading scientists conducting breakthrough drug discovery and early clinical research.

All of the ADDF’s overhead and administrative costs are covered by a private foundation, allowing 100% of funds raised to be used directly for Alzheimer’s drug research and related programs.

To date, the ADDF has granted more than $51 million to fund 370 Alzheimer’s drug discovery programs and clinical trials in academic centers and biotechnology companies in 18 countries. Subsequent to the ADDF’s critical initial seed funding, our grantees have received additional follow-on funding from government, pharmaceutical companies and venture capital firms in excess of $2 billion to further advance their drug research.

Our Annual Conferences

ADDF organizes two international scientific conferences yearly as part of our ongoing efforts to increase researchers’ knowledge about Alzheimer’s disease and the drug discovery process. The conferences promote networking to catalyze the exchange of ideas and foster alliances that accelerate the development of new treatments for AD.

Our annual International Conference for Alzheimer’s Drug Discovery, held in the fall, focuses on the discovery and development of drugs targeting Alzheimer’s disease and related dementias. The Drug Discovery for Neurodegeneration Conference, held in the winter, is designed to educate scientists on the process of translating basic neuroscience research into innovative therapies. The ADDF also plans smaller “catalyst conferences” that center around a relevant topic in the field of neurodegeneration.
On behalf of the Alzheimer's Drug Discovery Foundation (ADDF), I am pleased to welcome you to the 6th Drug Discovery for Neurodegeneration Conference: An Intensive Course on Translating Research into Drugs.

This course will advance the development of new drugs for neurodegenerative diseases by educating scientists on the principles and practice of drug discovery, including topics such as high throughput screening, lead optimization, preclinical proof-of-concept, and IND enabling and regulatory issues. The meeting will offer ample opportunities to network and form collaborations.

I would like to personally thank our scientific advisory committee, program chairs and speakers for their dedication and commitment to this meeting. Their expertise in the field and willingness to share lessons learned has helped to make this course possible.

We encourage you to visit the poster presentations by our talented Young Investigator Award and Scholarship winners. We are proud of their efforts and encourage them to continue pursuing their work in the neurodegeneration field.

We are pleased to host this year's meeting in New York City. Boasting the world's largest concentration of academic institutions and 60% of the nation's pharmaceutical industry in its vicinity, New York City is focusing its efforts on building a thriving bioscience industry. We are proud to be able to help accelerate these efforts.

Our meeting is made possible by the generous support of partners and sponsors: the National Institute on Aging, The Michael J. Fox Foundation for Parkinson's Research, Fast Forward LLC, National Multiple Sclerosis Society, JSW Life Sciences, BioFocus, Forest Laboratories, Inc., and reMYND. We would also like to thank our exhibitors and media partners for their commitment to making this meeting a success.

We are proud to welcome attendees from 20 countries, including Austria, Canada, Chile, China, France, Germany, India, Portugal, Saudi Arabia, Spain, U.K., and the U.S. Thank you for joining us.

Howard Fillit, MD
Executive Director
Alzheimer's Drug Discovery Foundation
## PROGRAM

### Sunday, February 12, 2012

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<tr>
<td>2:00 pm – 4:00</td>
<td>Registration</td>
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<tr>
<td>4:00 – 4:20</td>
<td><strong>Welcome &amp; Opening Remarks: Challenges and Opportunities in Academic Drug Discovery</strong>&lt;br&gt;Howard Fillit, MD, Alzheimer’s Drug Discovery Foundation</td>
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<tr>
<td>4:20 – 4:50</td>
<td><strong>Plenary: Where is Drug Discovery Going?</strong>&lt;br&gt;Christopher A. Lipinski, PhD, Melior Discovery, Inc.</td>
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<tr>
<td>4:50 – 5:00</td>
<td>Q&amp;A</td>
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<td>5:00 – 5:05</td>
<td><strong>Closing Remarks</strong>&lt;br&gt;Howard Fillit, MD, Alzheimer’s Drug Discovery Foundation</td>
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<td>5:05 – 7:00</td>
<td>Welcoming Reception</td>
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### Monday, February 13, 2012

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<td>Continental Breakfast</td>
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<td>8:30 – 8:40</td>
<td><strong>Welcome from New York City</strong>&lt;br&gt;Seth W. Pinsky, New York City Economic Development Corporation</td>
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<tr>
<td>I. Basics of Medicinal Chemistry</td>
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<td>Chair: D. Martin Watterson, PhD, Northwestern University</td>
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<tr>
<td>8:40 – 9:00</td>
<td><strong>Overview: Medicinal Chemistry Rules of Thumb, Myths and Realities in CNS Drug Discovery</strong>&lt;br&gt;D. Martin Watterson, PhD, Northwestern University</td>
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<td>9:00 – 9:10</td>
<td>Q&amp;A</td>
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<td>9:10 – 9:30</td>
<td><strong>Designing Small Molecules with Increased Potential for CNS Penetration</strong>&lt;br&gt;Laura Chico, PhD, Northwestern University</td>
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<td>9:30 – 9:40</td>
<td>Q&amp;A</td>
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<td>9:40 – 10:00</td>
<td><strong>Synthetic Chemistry Essentials for Biologists</strong>&lt;br&gt;Heather Behanna, PhD, JMP Securities LLC</td>
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<td>10:00 – 10:10</td>
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<td><strong>Natural Products as Drug Starting Points</strong>&lt;br&gt;Frank E. Koehn, PhD, Pfizer Inc.</td>
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<td>10:30 – 10:40</td>
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<td>10:40 – 11:10</td>
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<td>II. Early Phases of Drug Discovery</td>
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<td>Chair: Kurt R. Brunden, PhD, University of Pennsylvania</td>
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<td>11:10 – 11:15</td>
<td>Session Overview - Kurt R. Brunden, PhD, University of Pennsylvania</td>
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<td>11:15 – 11:35</td>
<td><strong>Basics of High Throughput Screening (HTS)</strong>&lt;br&gt;James Inglese, PhD, National Institutes of Health Chemical Genomics Center</td>
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<td>11:35 – 11:45</td>
<td>Q&amp;A</td>
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<td>11:45 – 12:05</td>
<td><strong>Compound Optimization after HTS: Beyond Potency</strong>&lt;br&gt;Kurt R. Brunden, PhD, University of Pennsylvania</td>
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<td>12:05 – 12:15</td>
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<td>12:15 – 12:35</td>
<td><strong>Importance of Toxicology</strong>&lt;br&gt;John E. Sagartz, DVM, PhD, DACVP, Seventh Wave Laboratories</td>
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<td>12:45 – 1:45</td>
<td>Lunch and Poster Session</td>
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<td>III. Pre-Clinical Proof-of-Concept and Development</td>
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<td>Chair: Edward G. Spack, PhD, Fast Forward, LLC</td>
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<td>1:45 – 1:50</td>
<td>Session Overview - Edward G. Spack, PhD, Fast Forward, LLC</td>
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<td>1:50 – 2:10</td>
<td><strong>What Makes a Clinical Candidate?</strong>&lt;br&gt;David Weiner, MD</td>
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<td>Q&amp;A</td>
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<td>2:20 – 2:40</td>
<td><strong>Requirements for an IND</strong>&lt;br&gt;Edward G. Spack, PhD, Fast Forward, LLC</td>
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<td>2:40 – 2:50</td>
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<td>2:50 – 3:20</td>
<td>Break</td>
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<tr>
<td>3:20 – 3:40</td>
<td><strong>Optimization and Characterization of Mouse Models of Neurodegeneration</strong>&lt;br&gt;Steve Perrin, PhD, ALS Therapy Development Institute</td>
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3:40 – 3:50 | Q&A
3:50 – 4:10 | **Value of Biomarkers in Preclinical Development: Translatable Endpoints**  
Barry Greenberg, PhD, Toronto Dementia Research Alliance
4:10 – 4:20 | Q&A
4:20 – 4:30 | **Closing Remarks and Announcement of Young Investigator Scholarship Winners**  
Diana Shineman, PhD, Alzheimer's Drug Discovery Foundation
4:30 – 6:30 | Networking Reception and Poster Session

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**Tuesday, February 14, 2012**

8:00 am – 8:30 | Continental Breakfast
8:30 – 8:35 | **Welcome & Opening Remarks**  
Suzana Petanceska, PhD, National Institute on Aging

**IV. What’s Druggable – Designing Drugs for CNS Target Classes**  
*Chair: Mark Frasier, PhD, Michael J. Fox Foundation for Parkinson’s Research*

8:35 – 8:40 | **Session Overview -** Mark Frasier, PhD, Michael J. Fox Foundation for Parkinson’s Research
8:40 – 9:00 | **Protein-Protein Interaction: A Growing Trend Towards Feasibility**  
Gérard Rossé, PhD, Dart Neuroscience
9:00 – 9:10 | Q&A
9:10 – 9:30 | **Challenges in Targeting Kinases for Neurodegenerative Diseases**  
Ravi G. Kurumbail, PhD, Pfizer Inc.
9:30 – 9:40 | Q&A
9:40 – 10:00 | **Druggability Considerations for GPCRs and Ion Channels**  
Shaun R. Stauffer, PhD, Vanderbilt University
10:00 – 10:10 | Q&A
10:10 – 10:30 | **Biologics for Challenging Targets: Unique Challenges and Lessons Learned**  
Guriq S. Basi, PhD, Elan Pharmaceuticals, Inc.
10:30 – 10:40 | Q&A
10:40 – 11:10 | Break

**V. Commercialization Strategies: Developing Science into Products**  
*Chair: Howard Fillit, MD, Alzheimer’s Drug Discovery Foundation*

11:10 – 11:15 | **Session Overview -** Howard Fillit, MD, Alzheimer’s Drug Discovery Foundation
11:15 – 11:30 | **Tech Transfer and Intellectual Property Management**  
Abram Goldfinger, MBA, New York University
11:30 – 11:45 | **An Early-Stage Venture Capitalist’s View of Neurodegeneration Research Opportunities**  
Geoffrey W. Smith, Ascent Biomedical Ventures
11:45 – 12:00 | **Pharmaceutical Companies: Licensing and Sponsored Research Agreements**  
Susan Rohrer, PhD, Merck Research Laboratories
12:00 – 12:15 | **Starting Your Own Biotech: Challenges and Pitfalls**  
Frank M. Longo, MD, PhD, Stanford University & PharmatrophiX
12:15 – 12:30 | **How Foundations Can Bridge the Gap**  
Sohini Chowdhury, MA, Michael J. Fox Foundation for Parkinson’s Research
12:30 – 1:00 | Open Discussion
1:00 – 1:15 | Lunch and Poster Session

**VI. Resources and Services For Advancing Drug Discovery**  
*Chair: Suzana Petanceska, PhD, National Institute on Aging*

1:45 – 1:50 | **Session Overview -** Suzana Petanceska, PhD, National Institute on Aging
1:50 – 2:10 | **An Academic Perspective on Drug Discovery Services: Centers & CROs**  
Marcie Glicksman, PhD, Harvard NeuroDiscovery Center
2:10 – 2:20 | Q&A
2:20 – 2:40 | **A Drug Discovery Services Perspective on Academic Collaborations**  
Bruce Molino, PhD, Albany Molecular Research, Inc.
2:40 – 2:50 | Q&A
2:50 – 3:10 | **Preclinical Therapeutics Development for Neurological Disorders: Funding & Resources**  
Rebecca Farkas, PhD, National Institute of Neurological Disorders and Stroke and  
Suzana Petanceska, PhD, National Institute on Aging
3:10 – 3:20 | Q&A
3:20 – 3:30 | **Closing Remarks -** Howard Fillit, MD, Alzheimer’s Drug Discovery Foundation
SCIENTIFIC ADVISORY COMMITTEE

Kurt R. Brunden, PhD, University of Pennsylvania
Neil Buckholtz, PhD, National Institute on Aging
Rebecca Farkas, PhD, National Institute of Neurological Disorders and Stroke
Howard Fillit, MD, Alzheimer's Drug Discovery Foundation
Brian Fiske, PhD, Michael J. Fox Foundation for Parkinson’s Research
Mark Frasier, PhD, Michael J. Fox Foundation for Parkinson’s Research
Abram Goldfinger, MBA, New York University
Lorenzo Refolo, PhD, National Institute on Aging
Suzana Petanceska, PhD, National Institute on Aging
Diana Shineman, PhD, Alzheimer's Drug Discovery Foundation
Edward G. Spack, PhD, Fast Forward, LLC
D. Martin Watterson, PhD, Northwestern University

CONTINUING MEDICAL EDUCATION

This activity has been planned and implemented in accordance with the Essential Areas and policies of the Accreditation Council for Continuing Medical Education through the joint sponsorship of CMI Education Institute, Inc. and Alzheimer's Drug Discovery Foundation (ADDF). CMI Education Institute, Inc. is accredited by the ACCME to provide continuing medical education for physicians.

AMA PRA Designation Statement
The CMI Education Institute, Inc. designates this live educational activity for a maximum of 12.5 AMA PRA Category 1 Credit(s)™. Physicians should only claim credit commensurate with the extent of their participation in the activity.

International CME
International Physicians are formally eligible for AMA PRA Category 1 Credit™.

CONFERENCE DELIVERABLES

A webcast of the entire conference will be made available on the ADDF website (www.alzdiscovery.org), where you may also access a webcast of last year's conference.

The ADDF will soon launch an interactive Drug Discovery Tutorial based on this conference. Stay tuned for the announcement of its release.
SPONSORS and EXHIBITORS

Funding for this conference was made possible in part by Cooperative Agreement U13AG031125-05 from the National Institute on Aging.

National Institute on Aging ★ ★ ★

The views expressed in written conference materials or publications and by speakers and moderators do not necessarily reflect the official policies of the Department of Health and Human Services; nor does mention by trade names, commercial practices, or organizations imply endorsement by the U.S. Government.

GENERAL MEETING SPONSORS

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### 2012 ADDF AWARDS and SCHOLARSHIPS

Congratulations to all of the 2012 ADDF Young Investigator Scholarship and Award winners. These scholarships recognize the early achievements of talented young investigators and seek to encourage the career development of the next generation of research scientists in the field of drug discovery for neurodegenerative diseases. All winners receive free conference registration and the opportunity to present a poster. Outstanding Young Investigators and Award Winners also receive a travel stipend.

#### 2012 ADDF OUTSTANDING YOUNG INVESTIGATOR AWARDS

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<thead>
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<th>Name</th>
<th>Institution</th>
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<tr>
<td>Karim Belarbi</td>
<td>University of California, San Francisco</td>
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<td>Gina Finan</td>
<td>Columbia University</td>
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<tr>
<td>Marguerite Prior</td>
<td>Salk Institute</td>
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<tr>
<td>Maria Telpoukhovskaia</td>
<td>University of British Columbia</td>
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<tr>
<td>Lawren VandeVrede</td>
<td>University of Illinois at Chicago</td>
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#### 2012 ADDF YOUNG INVESTIGATOR SCHOLARSHIPS

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<th>Name</th>
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<tr>
<td>Hazem Abdelkarim</td>
<td>University of Illinois at Chicago</td>
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<tr>
<td>Fatima Ali-Rahmani</td>
<td>Pennsylvania State University</td>
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<tr>
<td>Tari Awipi</td>
<td>F. Hoffmann-La Roche</td>
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<tr>
<td>Seema Bag</td>
<td>University of Massachusetts</td>
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<tr>
<td>Shireesha Boyapati</td>
<td>Vaagdevi College of Pharmacy</td>
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<td>Dwayne Brown</td>
<td>Howard University</td>
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<td>Daniel Curlik</td>
<td>Rutgers University</td>
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<tr>
<td>Alexandra Gaspar</td>
<td>Faculty of Sciences of University of Porto</td>
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<tr>
<td>James Kraus</td>
<td>Northwestern University</td>
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<tr>
<td>Cong Liu</td>
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<td>Katrina Paumier</td>
<td>Pfizer Inc.</td>
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<td>Joana Reis</td>
<td>Faculty of Sciences of University of Porto</td>
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<tr>
<td>Isaac Schiefer</td>
<td>University of Illinois at Chicago</td>
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<tr>
<td>Tiago Silva</td>
<td>Faculty of Sciences of University of Porto</td>
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<td>Neha Singla</td>
<td>Panjab University</td>
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<tr>
<td>Abha Sood</td>
<td>University of Massachusetts in Boston</td>
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<tr>
<td>Jose Teixeira</td>
<td>Faculty of Science of University of Porto</td>
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<tr>
<td>Aditya Vaidya</td>
<td>University of Illinois at Chicago</td>
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<td>Amit Verma</td>
<td>The Maharaja Sayajirao University of Baroda</td>
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<td>Meagan Wisniewski</td>
<td>University of North Carolina-Pembroke</td>
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Funding for these awards and scholarships was made possible in part by support from reMYND.
• JSW is one of the leading experts in transgenic disease models of neurodegenerative diseases.
• JSW maintains large colonies of well characterized mouse and rat models covering amyloid, tau and combined pathologies.
• JSW offers a unique in vivo drug screening platform to evaluate the effects of new chemical entities, biologics or gene therapies.
• A real “one stop shop” for your drug development program in AD.
  • large portfolio of behavioral paradigms
  • quantitative histological evaluation of drug effects
  • biochemical analyses of brain and CSF samples
  • wide spectrum of molecular biological methods
• JSW has tested hundreds of drug candidates and treatment approaches for AD in vivo models.
• Highest level of quality (GLP-certified) – Years of experience – Scientific excellence
• JSW gratefully acknowledges the support provided by the Alzheimer’s Drug Discovery Foundation (ADDF).

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**Lipophilic Mercaptans Break the Neuroinflammation Cycle**

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CHAIRS AND SPEAKERS

BIOS AND ABSTRACTS
Howard Fillit, MD, a geriatrician, neuroscientist and a leading expert in Alzheimer's disease, is the founding Executive Director of the Institute for the Study of Aging (ISOA), an Estée Lauder family foundation founded in 1998, and the Alzheimer's Drug Discovery Foundation (ADDF), an affiliated public charity founded in 2004. ISOA and ADDF share a common mission of accelerating drug discovery for Alzheimer's disease through venture philanthropy. Dr. Fillit has had a distinguished academic medical career at The Rockefeller University and The Mount Sinai School of Medicine where he is a clinical professor of geriatrics and medicine and professor of neurobiology. He was previously the Corporate Medical Director for Medicare at New York Life, responsible for over 125,000 Medicare managed care members in five regional markets. He is the author or co-author of over 250 scientific and clinical publications, and is the senior editor of the leading international Textbook of Geriatric Medicine and Gerontology. Dr. Fillit has received several awards and honors including the Rita Hayworth Award for Lifetime Achievement from the Alzheimer’s Association. He also serves as a consultant to pharmaceutical and biotechnology companies, health care organizations and philanthropies.
Dr. Christopher Lipinski learned his medicinal chemistry skills in a 32 year career at Pfizer in Groton, CT where he retired at the most senior scientific position. He is currently a Scientific Advisor to Melior Discovery a drug repurposing startup located in Exton, PA and carries out his medicinal chemistry consulting through Christopher A. Lipinski, PhD, LLC located in Waterford, CT. Chris serves on the scientific advisory boards for academic drug discovery efforts in Leuven, Belgium, Dundee Scotland and London UK. He is a conference committee member for the annual MIPTEC meeting in Basel, Switzerland which is now the largest early drug discovery meeting in Europe. He is a member of the American Chemical Society (ACS), the American Association of Pharmaceutical Sciences (AAPS) and the Society for Laboratory Automation Screening (SLAS). He is the author of the “rule of five” a widely used filter to select for acceptable drug oral absorption which is now the most highly cited paper in medicinal chemistry drug discovery. Chris is a member of the ACS “Medicinal Chemistry Hall of Fame”. In 2006 he received an honorary law degree from the University of Dundee and won the Society of Biomolecular Sciences Achievement Award. In 2005 he won the ACS E. B. Hershberg Award for Important Discoveries in Medicinally Active Substances and in 2004 won the ACS Division of Medicinal Chemistry Award. An adjunct faculty member in Biochemistry at the University of Massachusetts Amherst, Chris has over 250 publications and invited presentations and 18 issued US patents.

Plenary: Where is Drug Discovery Going?

Christopher A. Lipinski, PhD

Melior Discovery, Inc., Exton, PA

With respect to small molecules drug discovery changes today are more profound than the major changes of the early 90's involving combinatorial chemistry (combichem)and high throughput screening (HTS) and at the end of the 90's in the deciphering of the human genome (genomics). The common theme of combichem, HTS and genomics was that these technologies, especially in the early years, were enormously over hyped and despite a lot of beautiful science their contributions to drug discovery were charitably speaking modest and for genomics not even that much. Another common theme is that all these efforts shared a highly reductionist viewpoint. The search for a superbly selective ligand for a single target became the accepted method of drug discovery for almost two decades. We now know that the single most difficult problem in drug discovery is clinical efficacy. The superbly selective ligand for a single target works clinically at best 10% of the time and the overwhelming problem becomes one of target validation. If one employs the reductionist approach how does one find the validated target - the magic and elusive approach which actually helps patients? The reductionist approach is even worse in CNS diseases where there is actually very little evidence for reductionist success. Fortunately, as the limitations of one mind set became apparent, attractive alternatives beckoned. Today, we see a resurgence of phenotypic screening, much of it mechanistically unbiased. We see a rise of multi targeted drug discovery which all of the yeast data and genomic knockout models suggest is a better targeting approach. We see a phenomenal rise in drug repurposing with suggestions that finding a new use for an existing drug might actually be preferable in some situations to finding a new drug de novo. Finally we see a rise in collaborative drug discovery with attention being paid both to the technology of industry academic collaborations but also to the difficult cultural issue of the error inherent in preclinical hypothesis driven experimentation.
Seth W. Pinsky, New York City Economic Development Corporation

Seth W. Pinsky was appointed President of the New York City Economic Development Corporation (NYCEDC) by Mayor Michael R. Bloomberg in February 2008, seven months before the collapse of Lehman Brothers ushered in one of the most significant economic downturns in generations. Seth has worked to meet the challenge presented by the crisis by re-evaluating the agency’s strategy for expanding the City’s economy and redoubling existing efforts to position the City as the international center for innovation in the 21st century. NYCEDC’s agenda includes an aggressive slate of programs aimed at diversifying the City’s economy, helping legacy industries transition to 21st Century business models, and expanding entrepreneurship to ensure that the City is well-represented in the fields of tomorrow. Seth’s efforts have also included modernizing NYCEDC’s property management portfolio; overseeing $2.5 billion in capital investments ranging from basic infrastructure improvements to new parks and streetscapes across the City; and helping to negotiate and structure the City’s involvement in some of the most complex development projects in recent years, including the World Trade Center, Yankee Stadium, and Citifield.

An attorney by training, prior to joining NYCEDC, Seth was an associate at the law firm of Cleary Gottlieb, Steen & Hamilton in the Real Estate practice and a financial analyst at the Mergers & Acquisitions boutique, James D. Wolfensohn Incorporated. Seth is a graduate of Columbia College, where he majored in ancient history, and Harvard Law School.
SESSION I

Basics of Medicinal Chemistry

Chair — D. Martin Watterson, PhD, Northwestern University

Overview: Medicinal Chemistry Rules of Thumb, Myths and Realities in CNS Drug Discovery
  D. Martin Watterson, PhD, Northwestern University

Designing Small Molecules with Increased Potential for CNS Penetration
  Laura Chico, PhD, Northwestern University

Synthetic Chemistry Essentials for Biologists
  Heather Behanna, PhD, JMP Securities LLC

Natural Products as Drug Starting Points
  Frank E. Koehn, PhD, Pfizer Inc.
D. Martin Watterson, PhD, Northwestern University

Daniel Martin Watterson holds the John G. Searle Endowed Chair Professorship at Northwestern University where he is a Professor in the Department of Molecular Pharmacology & Biological Chemistry at the Feinberg School of Medicine. Dr. Watterson has worked successfully with major pharmaceutical and biotech companies in diverse areas of drug discovery, participated actively in bringing new drug candidates to clinical development, served on the Board of Directors for technology companies, founded profitable commercial enterprises with success in deliverables and timelines, and assisted colleagues and various government agencies with science and technology development. At Northwestern, he has served as a Department Chair, Co-Director of the Graduate Curriculum in Drug Discovery and Chemical Biology and a University Center, and founding director of the Drug Discovery Program. The Drug Discovery Program, founded in 1996 and now a university center, provides a supportive intellectual infrastructure to assist cooperative faculty participants in moving their basic science research toward preclinical drug discovery and eventual commercial development, mainly through out-license. Earlier small molecule deliverables by Northwestern faculty in the area of CNS drug discovery range from a mature blockbuster drug marketed by a major pharmaceutical company to potential disease-modifying novel candidates with potential for multiple indications that are currently in promising clinical trials.

The academic basic science research in Dr. Watterson’s laboratory continues to be on the elucidation of signal transduction pathways in eukaryotic organisms, examination of their role in physiology and disease states, and leveraging of the emergent knowledge of molecular and biological mechanisms to identify new points of potential therapeutic interventions. He has published in the areas of drug discovery, signal transduction, structural biology, pharmacology and medicinal chemistry, and previously developed diagnostic and research tools as well as novel small molecule therapeutic candidates licensed to industry.

Before moving to Northwestern University, Dr. Watterson held faculty positions at The Rockefeller University, where he was an Andrew Mellon Fellow, and at Vanderbilt University Medical Center, where he was Professor of Pharmacology and Howard Hughes Investigator. His doctoral training in chemical sciences was at Emory University, followed by postdoctoral training in biochemistry and bioorganic chemistry at Duke University Medical Center where he was supported by a National Research Service Award from the National Institutes of Health.

Medicinal Chemistry Rules of Thumb, Myths and Realities in CNS Drug Discovery

D. Martin Watterson, PhD

Northwestern University, Chicago, IL

This presentation will provide an introduction to the drug discovery and drug development continuum from the perspective of medicinal chemistry goals, and scientific background to concepts that will be covered in more detail by the three main lectures in this session.
Laura Chico, PhD, Northwestern University

Dr. Chico is currently a healthcare research analyst at Robert W. Baird & Company and holds an adjunct faculty position at Northwestern University. Previously, Dr. Chico was the founder and president of privately-held LKC Pharma Services, a consulting firm focused on the development of proprietary computational algorithms for pharmacological and chemical sciences to facilitate client “Go/NoGo” decisions or project prioritizations in drug discovery and early-stage product development, rendering significant time and cost savings in the short term and major risk reduction at later stages. She received her PhD in pharmacology and MS in computational biology from Northwestern University. Dr. Chico’s past research contributions facilitated advancement of novel small molecule drugs into late stage drug development for CNS disorders and the discovery of new classes of small molecule drug candidates for cancer therapeutics.

Designing Small Molecules with Increased Potential for CNS Penetration

Laura Chico, PhD

Northwestern University, Chicago, IL

This talk will provide an overview of the role of molecular properties in designing compounds with a higher probability of penetrating the CNS. Key areas to be covered include the application of molecular properties considerations during early drug discovery, discussion of available commercial tools for property calculations, and small-molecule, fragment-based screening case studies.

Learning objectives:

- Learn what makes a molecule “drug-like” and how molecular properties criteria can be used as prioritization tools.
- Discuss how molecular properties can influence pharmacokinetic outcomes, such as cytochrome P450 metabolism, and what matters most to CNS drug discovery campaigns.
- Become familiar with the unique properties requirements associated with the CNS, how these may change through the evolution of hits to clinical candidates, and how fragment-based screening can be applied.
Heather Behanna, PhD, JMP Securities LLC

Dr. Behanna is Biotechnology Research Associate at the JMP Securities LLC. Prior to that, Dr. Behanna was a Senior Scientist at the Astellas Research Institute of America (ARIA). She received her PhD in organic chemistry from the Department of Chemistry at Northwestern University, and did postdoctoral training at the Feinberg School of Medicine in pharmaceutical chemistry. Her past research has included novel compound discovery chemistry as well as developing a synthetic scheme for large scale production of clinical grade material under FDA regulated conditions.

Synthetic Chemistry Essentials for Biologists

Heather Behanna, PhD

JMP Securities LLC, Chicago, IL

This talk will provide an overview of the medicinal chemistry skill sets needed in the drug discovery process, including pattern recognition and synthetic chemistry planning. The use of these skill sets in the early drug discovery process, including choice of compounds for inclusion in a screening library and the recursive task of taking hits to lead compounds and then to clinical candidates, will be covered.

Learning objectives:

- Develop an appreciation for the key roles of synthetic chemistry in the multidisciplinary team and when to engage qualified assistance and collaboration.
- Learn what constitutes a "hit" versus a lead compound from a chemistry perspective.
- Become familiar with the linked but different goals for hit-to-lead refinement, lead optimization, and clinical material production.
Frank E. Koehn, PhD, Pfizer Inc.

Frank E. Koehn is Research Fellow and Head of the Natural Products Laboratory at Pfizer Worldwide R&D. Koehn obtained his BS degree in chemistry from Butler University, Indianapolis Indiana in 1977, and did his PhD research on marine red tide neurotoxins at the University of Wisconsin–Madison, USA. Following postdoctoral work in plant natural products at the University of Pennsylvania, he joined the Harbor Branch Oceanographic Institution in Fort Pierce, Florida, USA, where he spent the next decade identifying biologically active molecules from marine macro and microorganisms. Intrigued by the therapeutic potential of natural product-based drug candidates, Koehn joined the Natural Products and Analytical Chemistry program at Lederle Laboratories in 1994, which subsequently became Wyeth Research. In 2010 he joined Pfizer as Natural Products Laboratory head. At Pfizer Dr. Koehn’s research group is focussed on the discovery and application of microbial natural products to address unmet medical need.

Natural Products as Drug Starting Points

Frank E. Koehn, PhD

Pfizer Inc., Groton, CT

Natural products, compounds produced by microbes, plants, and animals have historically served as an unsurpassed direct source of new medicines, and an equally vast source of chemical inspiration for synthetic drug leads. In addition, natural products have been a primary means of discovering new drug targets. Over the past decade, evolution of the drug discovery landscape has simultaneously brought a reduction in traditional natural products methods and an emergence of new approaches based on genomics, chemical biology and biosynthesis. This talk will introduce how these new approaches play a role in modern lead generation, and how they impact the search for new medicines for neurodegenerative diseases.
SESSION II

Early Phases of Drug Discovery

Chair — Kurt R. Brunden, PhD, University of Pennsylvania

Session Overview
Kurt R. Brunden, PhD, University of Pennsylvania

Basics of High Throughput Screening (HTS)
James Inglese, PhD, National Institutes of Health Chemical Genomics Center

Compound Optimization after HTS: Beyond Potency
Kurt R. Brunden, PhD, University of Pennsylvania

Importance of Toxicology
John E. Sagartz, DVM, PhD, DACVP, Seventh Wave Laboratories
Dr. Inglese is currently establishing the Laboratory of Assay Development and Screening Technology focused on rare and neglected diseases in the newly formed NIH Center for Translational Therapeutics (NCTT). He is also co-founder of the NIH Chemical Genomics Center (NCGC) and Associate Investigator of the National Human Genome Research Institute (NHGRI). Dr. Inglese received his PhD in organic chemistry from the Pennsylvania State University and completed post-doctoral training in the laboratory of Prof. Robert J. Lefkowitz at Duke University Medical Center. Dr. Inglese has led research teams at the Princeton-based biotech Pharmacopeia and Merck Research Laboratories before coming to the NIH. Over the past two decades Dr. Inglese has contributed to over 150 publications and patents and has made major contributions to the early drug discovery process through the development of novel assay formats and high throughput screening paradigms. Dr. Inglese is the Founding Editor (2002) and Editor-in-Chief of the journal, ASSAY and Drug Development Technologies.

Basics of High Throughput Screening (HTS)

James Inglese, PhD

National Institutes of Health Chemical Genomics Center, Bethesda, MD

The rapid testing of chemical libraries for biological activity is the primary aim of high throughput screening (HTS). Advances in HTS have paralleled those in molecular biology, instrumentation and automation, and informatics, and the increased availability of arrayed compound libraries, sophisticated high sensitivity assays and the associated technologies required to implement these assays in HTS have been largely developed within the pharmaceutical industry for the identification of new chemical matter for drug development. However, HTS approaches are now widely available in academia to address broader questions within biological research and expand the disease portfolio for therapeutic development. In this presentation I will describe the components of HTS and provide examples of strategies used to identify novel chemotypes for specific biological targets or phenotypes using large and targeted chemical libraries. I will illustrate with specific case studies how this approach can be used in the identification of chemical modulators or probes for processes that form the basis of neurological disorders.
Dr. Kurt R. Brunden received his BS degree from Western Michigan University, with dual majors of Biology and Health Chemistry. He obtained his PhD in Biochemistry from Purdue University, and did a post-doctoral fellowship at the Mayo Clinic in Rochester, MN. Dr. Brunden subsequently spent 3 years as a faculty member within the Biochemistry department at the University of Mississippi Medical Center, with a research focus on the regulation of myelination. He was then recruited to the biotechnology sector, where he advanced to VP of Research at Gliatech, Inc. and later served as Sr. VP of Drug Discovery at Athersys, Inc. In these positions, he oversaw projects in Alzheimer's disease (AD), cognition, schizophrenia, inflammation and metabolic disease. The majority of these programs were ultimately partnered with or acquired by major pharmaceutical companies. In addition to his oversight of drug discovery programs, Dr. Brunden was responsible for the preparation and management of patents, as well as the preparation of Phase I clinical trial regulatory documents. In 2007, Dr. Brunden became a faculty member and Director of Drug Discovery in the Center for Neurodegenerative Disease Research at the University of Pennsylvania, where he oversees drug discovery programs in the areas of AD, frontotemporal lobar degeneration and Parkinson's disease. Dr. Brunden also serves as the chair of the NSD-C translational research study section for the National Institutes of Neurological Diseases and Stroke, is a reviewer for the Alzheimer's Drug Discovery Foundation and an ad hoc reviewer for several scientific journals. He has over 70 publications and a number of issued and pending patents.

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**Compound Optimization after HTS: Beyond Potency**

Kurt R. Brunden, PhD

*University of Pennsylvania, Philadelphia, PA*

The completion of high-throughput screening (HTS) of compound libraries often triggers a series of subsequent drug discovery activities that include secondary testing of initial HTS leads, selection of preferred chemotypes, and initiation of medicinal chemistry efforts. As chemical analogues are generated, they must be evaluated not only for their potency at the desired drug target, but also for key attributes such as aqueous solubility, pharmacokinetic behavior, pharmacological safety and, in the case of CNS drug targets, blood-brain barrier permeability. This session will provide examples of assays that can established and utilized in academic centers to gain a better understanding of these key compound characteristics before progressing to more advanced toxicological and efficacy testing in animals.
John E. Sagartz, DVM, PhD, DACVP, Seventh Wave Laboratories

John E. Sagartz, DVM, PhD, Diplomate, ACVP is President of Seventh Wave Laboratories, LLC. He received his Doctor of Philosophy from The Ohio State University, Department of Veterinary Biosciences, Columbus, OH, completed his Residency in Veterinary Pathology at Ohio State University, Department of Veterinary Pathobiology, Columbus, OH, Doctor of Veterinary Medicine from Kansas State University, College of Veterinary Medicine and his BS from Kansas State University, College of Agriculture in Manhattan, Kansas. From 1997 to 2003 he held positions as a Investigative Pathologist, Section Head, Assistant Director, Director and Site Head, Pharmacia Fellow Manager at Global Investigative Toxicology, St. Louis, MO. John has been the recipient of numerous honors and awards including recognition as a Pharmacia Fellow in 2003. The Pharmacia Fellow Program recognizes individuals who demonstrated outstanding proficiency and accomplishment in their field and a strong record of technical leadership. In addition, Dr. Sagartz received the W.E. Upjohn award in 2001 which is granted to individuals with sustained high impact contribution to the performance of Pharmacia Corporation. Further, Seventh Wave Laboratories was awarded the 2009 Outstanding Entrepreneur Award by the St. Louis County Economic Council. Past Professional Activities include Chair, American College of Veterinary Pathologists Endowment Committee, Endocrine Pathology Program Planning Committee, American College of Veterinary Pathologists annual meeting, 1999. Dr. Sagartz is a member of the Society of Toxicologic Pathologists, Society of Toxicology, American Society of Investigative Pathology, American College of Veterinary Pathologists, and American Veterinary Medical Association, and serves on the Board of Directors of the Missouri Biotechnology Association. He has authored and coauthored numerous publications related to experimental and spontaneous disease.

Importance of Toxicology

John E. Sagartz, DVM, PhD, DACVP

Seventh Wave Laboratories, Chesterfield, MO

Upon selection of a molecule for formal development, a series of safety studies must be conducted to enable dosing of human subjects, either healthy volunteers or patients. Although there is variation by world region and by therapeutic indication in the expectations of the supporting safety package, at a minimum, an evaluation of the potential for genetic toxicology, adverse pharmacology (cardiovascular, respiratory, CNS), and general toxicity is expected prior to the initiation of clinical trials. This session will provide an overview of the regulatory guidelines and specific studies for the evaluation of new chemical entities prior to first in human dosing.
SESSION III

Pre-Clinical Proof-of-Concept and Development

Chair — Edward G. Spack, PhD, Fast Forward, LLC

Session Overview
Edward G. Spack, PhD, Fast Forward, LLC

What Makes a Clinical Candidate?
David Weiner, MD

Requirements for an IND
Edward G. Spack, PhD, Fast Forward, LLC

Optimization and Characterization of Mouse Models of Neurodegeneration
Steve Perrin, PhD, ALS Therapy Development Institute

Value of Biomarkers in Preclinical Development: Translatable Endpoints
Barry Greenberg, PhD, Toronto Dementia Research Alliance
David Weiner, MD

Dr. Weiner received his medical degree from the State University of New York at Buffalo. He was a National Institutes of Health/Howard Hughes Medical Institute Research Scholar in the Laboratory of Molecular Biology at NINDS in Bethesda. He trained in neurology at Cornell New York Hospital/Memorial Sloan Kettering Cancer Center, and was a post-doctoral fellow in the Molecular Neuropharmacology Laboratories at the University of Vermont. He held an active clinical and teaching position as Adjunct Associate Clinical Professor in the Departments of Neurosciences and Psychiatry at the University of California at San Diego (1997-2007). Dr. Weiner has worked, in both the pre-clinical and clinical arena, on the discovery and development of novel small molecule and antibody therapeutics for human neuropsychiatric disease. He worked at ACADIA Pharmaceuticals Inc. from 1997-2006, where he held various positions including Head of Target Validation and Medical Director-CNS, and most recently was Vice President, and Head of Early Clinical Development in neurodegenerative disease at EMD Serono, where he had global responsibilities for early clinical development programs in Multiple Sclerosis and Parkinson’s Disease. Dr. Weiner is also a member of the scientific advisory board of the Michael J. Fox Foundation.

What Makes a Clinical Candidate?

David Weiner, MD

New Orleans, LA

The development of novel therapeutics for neurological disorders is a risk intensive and resource consumptive endeavor. The transition from pre-clinical development to first in human studies thru proof of clinical concept is an important, yet generally underappreciated, stage of the drug discovery process. The nomination of a specific clinical candidate, and the design of a rationale and milestone driven early clinical development plan are essential decisions that, if done well, can significantly increase the likelihood of later stage clinical success. Planning for clinical studies should begin relatively early in the development process, and target patient population, dose, duration of treatment, and early drug formulations are all important factors that need to be addressed far in advance of the initiation of an initial first clinical study. If possible, pharmacokinetic/pharmacodynamic relationships, and potential objective biological signatures of drug effect should be sought in early clinical studies. In addition, consideration of a target product profile should be explored and refined during early clinical development, so that trial design for proof of concept studies can be optimized to deliver data that inform the design of latter registration studies. This presentation will include a general discussion of the critical attributes of successful clinical candidates, as well as the milestones that are important to achieve in early clinical development of novel therapeutics for neurological disease.
Edward G. Spack, PhD, Fast Forward, LLC

Dr. Spack received his doctoral degree from The Johns Hopkins University and his postdoctoral fellowship in cellular immunology at Stanford University. He worked in Bay area biotech companies for 14 years developing therapies for autoimmunity, cancer, and infectious disease spanning discovery and preclinical development through Phase III trial support. At SRI International, Dr. Spack directed the PharmaSTART program, a consortium of SRI, Stanford, UC Berkeley, UCSD, and UCSF chartered to support translational development. His responsibilities included designing preclinical development plans for academic researchers, foundations, NIH programs, and start-up companies. He consulted with the NIH translational core services committee and several individual NIH institutes on preclinical development and served as an advisor on the NIH Regional Centers of Excellence for Biodefense and Emerging Infectious Diseases Produce Development Working Group. As Senior Director of Business Development for SRI’s Bioscience Division, Dr. Spack was responsible for alliance management, in-licensing, and out-licensing of drug candidates. He has served on scientific advisory boards, grant review boards, or national board of directors for several foundations, and is currently a managing director at Fast Forward LLC, supporting a translational development program advancing therapeutics for the prevention/reversal of neurodegeneration in multiple sclerosis in partnership with EMD Serono.

Requirements for an IND

Edward G. Spack, PhD

Fast Forward, LLC, San Francisco Bay, CA

In the United States, an Investigational New Drug (IND) application must be submitted to the FDA before a drug candidate can be tested in humans. An IND follows a proscribed format and documents the drug discovery and preclinical development activities that support the basis for testing in a specified therapeutic application, define the drug composition, and demonstrate the level of safety. A new IND is required for a new indication, change in route of drug administration or dosage, or change in patient population. Each IND includes information on three broad areas: animal pharmacology and toxicology studies; chemistry and manufacturing processes; clinical protocol and investigator information. Previous talks will cover studies of drug absorption, distribution, metabolism, and excretion (ADME); this presentation will include a discussion of Good Laboratory Practices (GLP) and the formal components of an IND Animal Pharmacology and Toxicology section. The Chemical, Manufacturing, and Control (CMC) section characterizes the chemical composition, manufacturing methods, potency, purity, stability, and controls used for manufacturing the drug substance and the drug product (active ingredient and excipients) performed according to Good Manufacturing Practices (GMP). The presentation will also discuss differences between investigator initiated and sponsor initiated INDs, pre-IND meetings, and other regulatory issues. Several commercial and government contract options are available for investigators seeking to advance their candidate(s) to an IND application. Government programs such as the Small Business Innovative Research (SBIR) and Small Business Technology Transfer (STTR) grants and the National Institutes of Health (NIH) Rapid Access to Interventional Development (RAID) Pilot Program provide funding and services to assist applicants in preparing the preclinical programs and documentation for their drugs. Increasingly, private foundations are also providing technical guidance and funding for preclinical work required for an IND submission.
Dr. Steven Perrin is currently the Chief Executive Officer and Chief Scientific Officer at the ALS Therapy Development Institute (ALS TDI) in Cambridge, MA. He earned his PhD at Boston University Medical Center studying the transcriptional regulation of genes during adipocyte and myocyte differentiation. Dr. Perrin moved into the pharmaceutical industry in 1997 holding positions at the Hoechst-Ariad Genomics Center, Aventis Pharmaceuticals and more recently as Director of Molecular Profiling at Biogen Idec. Dr. Perrin joined ALS TDI in 2007 as part of historical collaboration between the Muscular Dystrophy Association, Augie's Quest and ALS TDI to develop effective therapeutics for ALS patients. Since joining ALS TDI Steven has spearheaded the development of computational biology capabilities and information management systems to more clearly understand the molecular mechanisms associated with disease onset and progression in neurodegenerative diseases. He has expanded ALS TDI’s drug screening program to include expertise in the generation and assessment of gene therapy vectors and protein biologics in preclinical models of neurodegeneration. He has developed a business plan to facilitate pharmaceutical partnerships for rapid clinical development and commercialization of promising targets for ALS patients. Dr. Perrin is a frequent participant in international conferences in computational biology, genomics, drug development, and neurodegeneration.

Optimization and Characterization of Mouse Models of Neurodegeneration

Steven Perrin, PhD

ALS Therapy Development Institute, Cambridge, MA

The productivity of the pharmaceutical industry in the last decade has rapidly declined due to the increased cycle time and cost of preclinical and clinical development. The preclinical phase of drug development has a very high attrition rate and is challenging for complex disease indications such as neurodegenerative diseases. Reducing the cycle time and cost of preclinical studies will facilitate rapid GO/NOGO decisions in early phase clinical trials improving productivity across the value chain of the pharmaceutical industry. The Prp-TDP43A315T model is a commercially available model of neurodegeneration distributed by The Jackson Laboratories. The proper characterization of noise variables that influence disease onset and survival is critical to avoid the pitfalls that the ALS research community has encountered with irreproducible results in preclinical studies using the hSOD1G93Amouse model. The initial characterization of the Prp-TDP43A315T model warrants further characterization of the molecular mechanisms leading to neuronal loss, neuroinflammation, and muscle atrophy that has been described thus far. A comparison of these mechanisms to those evident in the hSOD1G93Amodel will facilitate the prioritization of druggable targets across multiple neurodegenerative disease indications.
Barry Greenberg has been involved in Alzheimer’s disease research and drug discovery since 1985. He has held a series of positions internationally in the US, Sweden and Canada within the biotechnology and pharmaceutical industries. Dr. Greenberg was the leader of a drug discovery project at AstraZeneca through lead optimization, involving up to 50 individuals from eight departments. Before joining UHN he was Senior Director of Pharmacology at Neurochem, responsible for the preclinical biology research program and a contributor to the analyses of the phase III Alzhemed trial. At UHN, he is currently co-directing the Toronto Dementia Research Alliance as Director of Strategy, a consortium involving academic research and the five memory clinics at hospitals affiliated with the University of Toronto to create a citywide dementia research center. He possesses a significant background in most aspects of the drug discovery process in neurological disease, with externally recognized expertise ranging from target identification and validation through preclinical and clinical development including issues of biomarker-based diagnosis and proof of concept. He has a strong international network in the Alzheimer field including industry, academia, government and the voluntary sector, plus previous involvement in multi-sector consortia. He has authored or co-authored 70 articles in peer-reviewed journals and 19 book chapters and reviews.

Value of Biomarkers in Preclinical Development: Translatable Endpoints

Barry Greenberg, PhD

*Toronto Dementia Research Alliance, Toronto, Canada*

In order to develop any therapeutic drug, standard issues of access to the intended target in vivo at appropriate concentrations for relevant periods of time must be addressed – issues of pharmacokinetics and pharmacodynamics. For CNS disease, there are additional challenges in accessing therapeutic targets in the brain. Moreover, as has become increasingly clear over the past several years, neurodegenerative and psychiatric diseases involve neural networks rather than merely single molecular targets. Commonly used translational animal models represent only aspects of the human disease, thereby providing pharmacodynamic tools for assessing target access, but fall short in terms of addressing the true translational effects into human disease. Closing the gap between the stage of the disease represented by the selected *in vivo* models, and the stage of the disease in the patient cohorts that will be treated with the selected therapeutic agent, remains a significant challenge that will likely need to be overcome in order to maximize the potential for successful therapeutic development in neurodegenerative disease.
Diana Shineman, PhD, is the Assistant Director, Scientific Affairs at the Alzheimer's Drug Discovery Foundation, where she is responsible for developing and managing all aspects of the Foundation's drug discovery and development research programs.

Dr. Shineman earned her PhD in Cell and Molecular Biology from the University of Pennsylvania (Penn). At Penn's Center for Neurodegenerative Disease Research led by Drs. Virginia Lee and John Trojanowski, she studied signal transduction pathways that alter amyloid generation in Alzheimer's disease. Dr. Shineman also worked with the Center's Drug Discovery Group to perform high-throughput screening using cell-based assays. In addition to her dissertation research, Dr. Shineman was an Editorial Intern for the Journal of Clinical Investigation and was an active member of the Penn Biotechnology Group.

Dr. Shineman received a BA in Biology with a Nutrition concentration from Cornell University, where she was named a Howard Hughes Undergraduate Research Scholar. She is also a member of the Society for Neuroscience, New York Academy of Sciences, and the Association for Women in Science, and has authored numerous peer-reviewed publications.
SESSION IV

What’s Druggable – Designing Drugs for CNS Target Classes

Chair — Mark Frasier, PhD, Michael J. Fox Foundation for Parkinson’s Research

Session Overview
Mark Frasier, PhD, Michael J. Fox Foundation for Parkinson’s Research

Protein-Protein Interaction: A Growing Trend Towards Feasibility
Gérard Rossé, PhD, Dart Neuroscience

Challenges in Targeting Kinases for Neurodegenerative Diseases
Ravi G. Kurumbail, PhD, Pfizer Inc.

Druggability Considerations for GPCRs and Ion Channels
Shaun R. Stauffer, PhD, Vanderbilt University

Biologics for Challenging Targets: Unique Challenges and Lessons Learned
Guriq S. Basi, PhD, Elan Pharmaceuticals, Inc.
Mark Frasier, PhD, joined the Michael J. Fox Foundation in March 2006. Dr. Frasier received his BS in biochemistry from the University of Dayton and his PhD in pharmacology from Loyola University Chicago. His dissertation was completed in the laboratory of Benjamin Wolozin and focused on understanding the biological function of proteins implicated in neurodegeneration such as alpha-synuclein and tau. Dr. Frasier completed his postdoctoral work in the Neuroscience Discovery Research Group at Eli Lilly, Inc., in Indianapolis, Indiana, where he worked on drug-discovery research in Parkinson's and Alzheimer's disease. At MJFF, Dr. Frasier co-leads a team of scientists responsible for allocating MJFF resources across Parkinson's disease research. He also leads the MJFF biomarker strategy and portfolio of projects.
Gérard Rossé, PhD, Dart Neuroscience

Dr. Rossé has been involved in drug discovery research and development for more than 15 years. He is currently Associate Director, Chemistry, at Dart Neuroscience and also serves as Adjunct Associate Professor at Drexel University as well as Professor in Medicinal Chemistry at the Pennsylvania Drug Discovery Institute. Prior he functioned in leadership and scientific positions in medicinal and high throughput chemistry with Cephalon, Sanofi Aventis and F. Hoffman-La Roche. During his industrial tenure, he led multidisciplinary teams and invented 3 pre-clinical candidates and 9 compound lead series spanning a wide range of therapeutic indications. These include CNS (cognition, schizophrenia, Alzheimer’s disease), Inflammation, Metabolism, Oncology, Cardiovascular disease and Antibacterial agents. Dr. Rossé’s career is also characterized by implementation of innovative high throughput technology platforms in the area of small molecules and peptides drug discovery, analytical chemistry and data management that increased productivity and accelerated the drug discovery process. Dr. Rossé is a distinguished speaker at business meetings in US and Europe and has authored 40+ patents and publications. He received the PhD degree in chemistry from the University of Basel in Switzerland and postdoctoral training at Stanford University.

Protein-Protein Interaction: A Growing Trend Towards Feasibility

Gérard Rossé, PhD

Dart Neuroscience, Doylestown, PA

Protein-protein interactions (PPIs) play a crucial role in many biological processes and represent a treasure trove of possible novel targets with huge therapeutic potential. Discovering small molecule drugs that disrupt PPIs is an enormous challenge due the large and featureless interfacial areas involved in PPIs. Despite the relative unsuitability of PPIs to serve as druggable targets, successful examples of small-molecule inhibitors of PPIs are beginning to accumulate. This presentation will highlight the current progress in the development of ligands interacting with PPIs that have applications in the treatment or study of central nervous system function and disease. In particular, novel approaches to design inhibitors of amyloid-β and α-synuclein aggregation will be discussed.
Ravi Kurumbail is currently a research fellow and a structural biology lab head at Pfizer Worldwide Research & Development at their Groton, Connecticut campus. He received a BS degree in chemistry from his home town college (Victoria College, Palakkad) and an MS degree in chemistry from the Indian Institute of Technology, Madras. Ravi moved to America in the early 80s and obtained his doctorate degree in chemistry from the Michigan State University where he worked on protein X-ray crystallographic studies of blood coagulation proteins in the lab of Alexander Tulinsky. During this time, Dr. Kurumbail also had an opportunity to collaborate with Professor Robert Huber at the Max-Planck Institute, Martinsried, Germany, which led to the structure determination of human ß-hrombin, one of the first structures of thrombin that were reported. He then worked with Johann Deisenhofer at the Howard Hughes Medical Institute and the Univ. of Texas Southwestern Medical center, Dallas and solved the structure of a bacterial cytochrome P450. Ravi then moved to St. Louis where he spent the next 13 years at Searle, Monsanto, Pharmacia and eventually Pfizer. While at St. Louis, Dr. Kurumbail elucidated the structure of the membrane protein cyclooxygenase-2 (COX-2) that is the target of non-steroidal anti-inflammatory drugs. He was an active member of the COX-2 team that discovered Celebrex™, Bextra™ and Dynastat™ and contributed to their mechanistic study and regulatory filings. Over the years, Ravi’s research interests have spanned proteases, protein kinases, hydrolases, nuclear hormone receptors, cyclooxygenases, cytochrome P450s and proteins involved in blood coagulation and fibrinolysis. Dr Kurumbail has been actively pursuing structure-based drug design over the past 15 years in the pharmaceutical industry. He has broad experience in evaluation of kinase inhibitors for Alzheimer’s and Parkinson’s disease and was the leader for a kinase discovery project for Alzheimer’s disease.

Challenges in Targeting Kinases for Neurodegenerative Diseases

Ravi G. Kurumbail, PhD

Pfizer Inc., Groton, CT

Over the last two decades, the pharmaceutical industry has enthusiastically pursued protein kinases as key targets for the discovery of novel therapeutics for several diseases such as cancer and inflammatory diseases. During this time, we have also witnessed the regulatory approval of over a dozen small molecule kinase inhibitors, mostly for oncology indications. The vast majority of these drugs are directed toward the highly druggable ATP site of kinases. The clinical success of kinase inhibitors has stimulated heightened activities focused on kinases for the treatment of neurological disorders. However, the physico-chemical properties that are required for efficiently targeting the ATP site of kinases present significant hurdles for brain-penetration. I will discuss the discovery of highly selective, brain-penetrant glycogen synthase kinase 3-ß inhibitors that are effective in modulating the levels of phosphorylated tau in vivo for the treatment of Alzheimer’s disease.
Dr. Stauffer is currently a Research Assistant Professor in the Departments of Pharmacology and Chemistry at Vanderbilt University and is Associate Director of Medicinal Chemistry within the Vanderbilt Center for Neuroscience Drug Discovery. Dr. Stauffer received his PhD in Organic Chemistry from the University of Illinois in the laboratory of John Katzenellenbogen developing combinatorial approaches towards selective estrogen receptor modulators (SERMs). Dr. Stauffer then conducted post-doctoral studies with Professor John Hartwig at Yale University as an NIH fellow where he developed a general high-throughput FRET-based assay for reaction discovery platforms. From 2001-2008 Dr. Stauffer pursued his industrial career at Merck & Co. where he was involved in several cardiovascular and CNS programs including targets for Alzheimer’s disease and chronic pain. While at Merck he was the recipient of the MRL 2007 Special Achievement Award and was a co-author and co-inventor on over 25 publications and patents.

At Vanderbilt Dr. Stauffer leads a team involved in an industry sponsored program focusing on the identification of preclinical candidates for the treatment of schizophrenia, a collaboration which is currently entering year four. In addition, as part of the Molecular Libraries Probe Production Centers Network (MLPCN) supported by the NIH, Dr. Stauffer leads several probe projects including 3C/PL protease inhibitors for SARS and PPI inhibitors for MLL-associated acute leukemias. Dr. Stauffer’s research interests lie in the areas of CNS disorders, glutamate signaling, protease structure-based inhibitor design, PPI inhibitors, organometallic chemistry and reaction discovery.

Druggability Considerations for GPCRs and Ion Channels
Shaun R. Stauffer, PhD
Vanderbilt University, Nashville, TN

This talk will focus on the challenges and opportunities regarding the druggability of GPCR and ion channel targets. Within each class of molecular targets, critical issues arise that govern the potential druggability of a given target. For GPCRs, understanding and quantifying receptor expression/density and receptor reserve in the primary cell lines driving discovery is crucial. As well, one must carefully weigh targeting the orthosteric or an allosteric site with a small molecule, as either approach brings with it unique obstacles and rewards. Here, we will showcase representative examples and lessons learned. For ion channels, a similar number of considerations must be contemplated. For instance, should the lead optimization program be driven via a surrogate reporter assay, or EP? When the HTS is bountiful, should one pursue a voltage-dependent or voltage independent ligand? What subunit make-up of a given channel is more druggable and likely to result in fewer AEs? For both GPCRS and ion channels, a major consideration is family-related ancillary pharmacology and the selection of appropriate counter-screens to drive the program. Finally, for druggability, development of a biomarker early on in the discovery process to assess target engagement in Phase I is very important.
Guriqbal S. Basi, PhD, Elan Pharmaceuticals, Inc.

Guriqbal (Guriq) S. Basi has served as Vice President of Extramural Research at Elan since June 2008. In this capacity, he is responsible for overseeing and providing input to relationships with academic laboratories and private enterprises engaged in translational research on neurological diseases. In May 2010, Dr. Basi was appointed Head of Pre-Clinical development at Neotope Biosciences Inc., a wholly owned subsidiary of Elan Corp specializing in discovery and development of biologics for treatment of diseases associated with protein misfolding. Dr. Basi conceived and spearheaded the humanization of bapineuzumab for immunotherapy of Alzheimer’s disease, and led Elan’s research efforts to discover a small molecule for arresting amyloid production in the brain, which culminated with the clinical development of a selective gamma-secretase inhibitor for the treatment of Alzheimer’s disease. Dr. Basi has been with Elan since 1992, and has also served as Senior Director of Discovery Research and Head of Molecular Biology. Prior to joining Elan, Dr. Basi was a staff scientist at Protein Design Labs, and conducted postdoctoral research in neurobiology at Stanford University. Dr. Basi received his PhD in biological chemistry from the University of Illinois at Chicago, and his BA in biochemistry from The Ohio State University.

Biologics for Challenging Targets: Unique Challenges and Lessons Learned

Guriqbal S. Basi, PhD

Elan Pharmaceuticals, Inc., San Francisco Bay, CA

The central nervous system has traditionally presented greater challenges for therapeutics to treat diseases associated with disorders of the brain, e.g. psychiatric, neurodegenerative, or trauma indications, than peripheral organs. These challenges are attributable to three factors: 1) The biological complexity of the organ and its associated diseases, which together make it difficult to fully reproduce all aspects of the disease in pre-clinical models for predictive drug testing; 2) The blood-brain barrier (BBB), which imposes additional physicochemical constraints beyond the traditional “Lipinski rule of five” for “drug-like properties” which small molecule candidates must meet in order to fulfill criteria of target engagement of intracellular or extra-cellular targets in the CNS; and 3) With the possible exception of certain psychiatric indications, the typically slow time-course of neurodegenerative diseases, or the variable degrees of spontaneous recovery associated with certain CNS traumas, e.g. stroke, present complications for design of clinical trials. Progress into biological pathways, and mechanistic insights into disease processes over the past 50 yrs. have greatly advanced our knowledge and understanding regarding diseases of the CNS. In addition, other lectures in this course have elaborated on medicinal chemistry strategies to overcome the BBB and endow small molecules with CNS penetrant drug like properties. Finally, advances with blood and CSF biomarkers, combined with brain imaging techniques, provide better definition of disease progress from early to late stages, and offer opportunities for earlier intervention with therapies against defined and predictive end-points entailing (hopefully) shorter duration clinical trials. Nevertheless, due to restrictions imposed by the BBB, CNS diseases were, until recently, considered the exclusive realm of small molecule based therapies. However, with demonstrations of efficacy from AD immunotherapy, and the cell-to-cell transmissibility of tauopathies and synucleinopathies, the potential for biologics with specific applications in CNS has grown. In-spite of success with the aforementioned examples, limitations to biologics as therapeutics remain, e.g. growth factor based therapies, and targets requiring saturation kinetics for pharmacologic effect e.g. cell surface receptors. Other targets remain beyond the scope of biologics, e.g. protein-protein interactions. Examples illustrating successes and limitations mentioned above will be reviewed during the lecture, with the objective of sharing lessons learned to guide future efforts.
SESSION V

Commercialization Strategies: Developing Science into Products

Chair — Howard Fillit, MD, Alzheimer’s Drug Discovery Foundation

Session Overview
Howard Fillit, MD, Alzheimer's Drug Discovery Foundation

Tech Transfer and Intellectual Property Management
Abram Goldfinger, MBA, New York University

An Early-Stage Venture Capitalist’s View of Neurodegeneration Research Opportunities
Geoffrey W. Smith, Ascent Biomedical Ventures

Pharmaceutical Companies: Licensing and Sponsored Research Agreements
Susan Rohrer, PhD, Merck Research Laboratories

Starting Your Own Biotech: Challenges and Pitfalls
Frank M. Longo, MD, PhD, Stanford University & PharmatrophiX

How Foundations Can Bridge the Gap
Sohini Chowdhury, MA, Michael J. Fox Foundation for Parkinson’s Research
**Abram Goldfinger, MBA, New York University**

Mr. Goldfinger is the Executive Director of the Office of Industrial Liaison at New York University, which is responsible for the commercialization of university technologies. He has been involved in academic technology transfer for over 20 years, and has negotiated over 500 license agreements with industry and helped to form 75 university spin-off companies. Prior to joining NYU, he was the Director of Technology Transfer at Thomas Jefferson University, an academic medical center in Philadelphia. He has been involved in research and development in both industry and academia, including work at the MIT Artificial Intelligence Laboratory, Raytheon Company’s Advanced Systems Laboratory, and several start-up companies. He has also provided consulting to large and small companies and venture capital firms, regarding market analysis, technology assessment, and business plan development. Mr. Goldfinger received a BS in electrical engineering from MIT and an MBA from the Wharton School. He has also passed the Patent Bar Exam and is registered to practice before the U.S. Patent and Trademark Office.

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**Tech Transfer and Intellectual Property Management**

Abram Goldfinger, MBA

*New York University, New York, NY*

The passage of the Bayh-Dole Act by Congress in 1980 has led to a significant increase in university technologies being developed to benefit the public, in particular biomedical technologies. This session will cover effective university technology commercialization strategies, including managing intellectual property to give industry partners the necessary incentive to invest in research and development, gap funding programs to increase the likelihood of attracting a commercial partner, outreach to existing biotechnology and pharmaceutical companies, structuring of win-win technology commercialization agreements, and new venture creation.
An Early-Stage Venture Capitalist's View of Neurodegeneration Research Opportunities

Geoffrey W. Smith

*Ascent Biomedical Ventures, New York, NY*

Venture Capital & Neurodegeneration will describe the current environment for life sciences venture capital investing with a special emphasis on investments in therapeutics to treat neurodegeneration. Particular attention will be given to the challenges faced by pre-clinical and early-stage development programs.
Susan Rohrer, PhD, Merck Research Laboratories

Susan Rohrer obtained her undergraduate degree in biology at the University of Michigan and her graduate degree in biochemistry at the University of Notre Dame. She joined the Merck Research Laboratories in 1987 to work on the biochemical isolation of the receptor for Merck's antiparasitic agent known as ivermectin. She subsequently contributed to the development of small molecule analogues of somatostatin as potential agents for the treatment of diabetes and diabetic retinopathy and to the identification of selective estrogen receptor modulators for use as safer alternatives to currently available hormone replacement therapies.

Susan joined External Scientific Affairs as liaison for Neuroscience Licensing in September 2004. She chairs Merck's Neuroscience Review and Licensing Committee and is responsible for identifying external licensing opportunities aligned with priorities and needs of the Neuroscience Franchise. Her recent major licensing deals include Neuromed (NMED-160 patent license and research collaboration), Gladstone Institute of Neurological Disease (patent license and research collaboration covering ApoE mechanisms involved in neurodegenerative diseases), Addex mGluR5 (patent license covering mGluR5 PAMs for schizophrenia) and Addex mGluR4 (patent license and research collaboration covering mGluR4 PAMs for Parkinson's disease).

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Pharmaceutical Companies: Licensing and Sponsored Research Agreements

Susan Rohrer, PhD

*Merck Research Laboratories, New York, NY*
Frank M. Longo, MD, PhD, Stanford University & PharmatrophiX

Dr. Longo received his MD in 1981 and PhD in Neurosciences in 1983 from the University of California, San Diego. Following an internship in medicine at NYU/VA, he trained as a resident in neurology and fellow in neurobiology at University of California, San Francisco. While at UCSF he created the Neurogenetics Clinic which was the first West Coast site in the U.S. to offer DNA testing for families with Huntington's disease. He also led the creation of a national referral center for deep brain stimulation for Parkinson's disease and contributed to the development of programs in dementia, epilepsy and other areas. At UCSF he became professor and vice chair of the Department of Neurology and in 2001 he was recruited to become chair of the Department of Neurology at the University of North Carolina, Chapel Hill. While at UNC, Dr. Longo launched or expanded programs for Alzheimer's disease and other dementias, stroke, epilepsy, sleep disorders, multiple sclerosis and Parkinson's disease. In January 2006, Dr. Longo became chair of the Department of Neurology and Neurological Sciences at Stanford where he is focused on building and expanding multidisciplinary programs in neurology and neuroscience. In 2006 he was named a Stanford Fellow. Dr. Longo's research team focuses on elucidating novel mechanisms that prevent neural degeneration and promote regeneration. He and his colleagues have pioneered the development of small, drug-like, molecules that target neurotrophin receptors to delay onset of or slow progression of Alzheimer's and other neurodegenerative disorders.

Starting Your Own Biotech: Challenges and Pitfalls

Frank M. Longo, MD, PhD

Stanford University & PharmatrophiX, Stanford, CA

Our team has developed small molecule ligands targeted to various neurotrophin receptors that demonstrate efficacy in Alzheimer's and other neurodegenerative disease mouse models. Compounds are now in IND-enabling studies. Development of novel small molecules in an academic setting, that demonstrate preclinical efficacy and safety, raises the possibilities of either working with the university to license the technology or founding a company which can then license the technology. In this session we will review the pros and cons of each approach. We will also review in a stepwise progression the options for how a startup company can be founded and funded and how it might interact with funding sources, contract research organizations and potential industry partners. The concepts of dilutive and non-dilutive funding and the process of due diligence without potential funders and partners will be highlighted.
Sohini Chowdhury, MA, Michael J. Fox Foundation for Parkinson’s Research

Sohini Chowdhury joined the Foundation after spending five years at the World Economic Forum in Geneva, Switzerland. As the Senior Community Manager of the Forum’s Technology Pioneers program, she was responsible for annually selecting and integrating innovative biotech, energy and IT technology companies into Forum activities. Ms. Chowdhury also worked directly for the Forum’s CEO, acting as his liaison with key Forum stakeholders and overseeing several in-house projects. Ms. Chowdhury graduated with an MA from Georgetown University, and holds a BA in international studies from Vassar College. As Vice President for Research Partnerships at the Michael J. Fox Foundation, Ms. Chowdhury oversees a team that focuses on three areas: 1) increasing engagement and developing partnerships with the for-profit sector, namely pharmaceutical and biotech companies; 2) developing and implementing strategies to improve recruitment for Parkinson’s disease trials; and 3) managing the Parkinson’s Progression Markers Initiative (PPMI), a $40-45 million clinical biomarker study.

How Foundations Can Bridge the Gap

Sohini Chowdhury, MA

Michael J. Fox Foundation for Parkinson’s Research, New York, NY

The Michael J. Fox Foundation for Parkinson’s Research (MJFF) stands outside the traditional biomedical research system, yet sits at the hub of global Parkinson’s research. Since its founding in 2000, MJFF has funded over $270 million in research. Acknowledging that the funds at its disposal are dwarfed by the resources available to government and industry, MJFF utilizes a de-risking strategy -- invest to lower research risk and encourage ongoing investment in Parkinson’s disease (PD) -- to get the ‘biggest bang for its buck’. MJFF reviews more PD-specific grant proposals (~800 per year) than any other funder and has extensive outreach with hundreds of the world's top PD experts. Leveraging information we compile, we are able to assess the state of PD science and identify critical research roadblocks that can hinder ongoing and/or new investment in PD. In recent years, MJFF has prioritized addressing the lack of critical research tools, such as biomarkers and animal models, and has built novel partnerships around tool development.

Unlike other stakeholders in the medical enterprise, MJFF has only one goal: find improved therapies or a cure for Parkinson’s disease. Our mission and business model allows us to assume greater risk than other actors. As will be discussed, by working closely industry, academia and government and patients, MJFF can leverage its neutral platform to support, catalyze and fund research that is synergistic and complementary to drug development efforts undertaken by other groups.
SESSION VI

Resources and Services For Advancing Drug Discovery

Chair — Suzana Petanceska, PhD, National Institute on Aging

Session Overview
Suzana Petanceska, PhD, National Institute on Aging

An Academic Perspective on Drug Discovery Services: Centers & CROs
Marcie Glicksman, PhD, Harvard NeuroDiscovery Center

A Drug Discovery Services Perspective on Academic Collaborations
Bruce Molino, PhD, Albany Molecular Research, Inc.

Preclinical Therapeutics Development for Neurological Disorders: Funding & Resources
Rebecca Farkas, PhD, National Institute of Neurological Disorders and Stroke
Suzana Petanceska, PhD, National Institute on Aging
Marcie Glicksman is Senior Director, Leads Discovery Group at LDDN. Dr. Glicksman has extensive experience in assay development, high throughput screening, chemical databases, animal pharmacology and preclinical development. Her bachelor’s degree is from Brown University and PhD from Washington University. Before joining LDDN in 2004, she had been in industry for thirteen years. Previously, she was at the start-up company, Descartes Therapeutics focused on imaging techniques. Before this, she was Director of Leads Discovery at Cubist. Before this, she was at DuPont-Merck and at Cephalon, Inc. She led the assay development and screening program for a cell-based protease project, and numerous G-protein coupled receptors, many of which were continued when Bristol Myers Squibb bought DuPont Pharmaceuticals. At Cephalon, she was co-inventor of CEP1347, a neuroprotective agent directed at a novel kinase, currently in Phase III clinical trials. She also consults for industry. She is a board member of the non-profit drug discovery organization Society for Biomolecular Screening and currently serves as the Chairman.

An Academic Perspective on Drug Discovery Services: Centers & CROs

Marcie Glicksman, PhD

Harvard NeuroDiscovery Center and Brigham and Women’s Hospital, Boston, MA

The model of drug discovery is changing. As the pharmaceutical industry looks for later stage projects to fill their pipeline and decrease their research efforts, there is a gap that has formed between basic research and the identification of drug candidates. The Laboratory for Drug Discovery in Neurodegeneration (LDDN) at the Harvard NeuroDiscovery Center works with academic labs around the world in a collaborative model of drug discovery. Our primary mission is to accelerate the development of therapeutics for neurodegenerative diseases. As a small group of fifteen that covers biology, medicinal chemistry, informatics and computational chemistry, we use some external resources for needs not covered internally. Unexpectedly, the vendor community is a rich source for collaboration that allows the academic community access to new technologies. Details will be provided on the academic models of drug discovery and the use of Contract Research services.
Bruce Molino, PhD, Albany Molecular Research, Inc.

Dr. Bruce Molino is Senior Director of Medicinal Chemistry at AMRI, a provider of contract R&D services with global facilities in the United States, Europe and Asia. His international experience and successful track record in drug discovery comes from working more than 25 years in pharmaceutical R&D. Dr. Molino has worked for nearly 14 years with AMRI, where he has successfully managed contract drug discovery teams in pursuit of novel drug molecules for the treatment of a wide range of diseases and therapeutic areas. Prior to AMRI, Dr. Molino was a Director in the Medicinal Chemistry Department of Rhone-Poulenc Rorer (known today as Sanofi-Aventis) for more than 10 years. Over his career, Dr. Molino has contributed to programs that have progressed compounds into human clinical trials for treatment of congestive heart failure, thrombosis, respiratory disorders and most recently major depressive disorder. He is currently a member of the ADDF scientific review board.

A Drug Discovery Services Perspective on Academic Collaborations

Bruce Molino, PhD

*Albany Molecular Research, Inc., Albany, NY*

There is a broad array of drug discovery contract services available to academic laboratories that can improve the chances for successful translation of academic innovation into therapeutically useful drugs. This lecture will discuss the rational for outsourcing parts of the drug discovery path vs. “going it alone.” The range of drug discovery services will be discussed along with perspective provided on costs for these services.
Dr. Rebecca Farkas is a Program Director in the Office of Translational Research (OTR) at the National Institute of Neurological Disorders and Stroke (NINDS). Dr. Farkas is one of the leaders of the NIH Blueprint Neurotherapeutics Network, a “virtual pharma” for advancing drug discovery projects from the validated hit stage through phase I clinical testing. She also oversees translational research training initiatives and programs for early-stage translational research. Dr. Farkas received her PhD in developmental biology at Stanford University School of Medicine and her BA in molecular biophysics and biochemistry from Yale University. She served as a science policy analyst in the NINDS Office of Science Policy and Planning for seven years before joining OTR.

Preclinical Therapeutics Development for Neurological Disorders: Funding & Resources

Rebecca Farkas, PhD

National Institute of Neurological Disorders and Stroke, Bethesda, MD

The translation of basic science into patient therapies is a critical mission of the National Institutes of Health. The Office of Translational Research (OTR), within the National Institute of Neurological Disorders and Stroke (NINDS), manages multiple programs in drug discovery and preclinical development of neurological therapeutics. These include grant programs for high throughput screening, medicinal chemistry optimization, and IND-directed studies and a “virtual pharma” network of contract resources for drug discovery. An overview of OTR activities in drug discovery will be presented, as well as tips for submitting a competitive application.
Suzana Petanceska, PhD, National Institute on Aging

Dr. Suzana Petanceska received a BS degree in molecular biology and physiology from the University of Belgrade, Yugoslavia and a PhD degree in Pharmacology from New York University. Following her postdoctoral training at Rockefeller University (1995-1998) and at the Nathan Kline Institute of NYU (1998-2000) she became an Assistant Professor of Psychiatry and Pharmacology at the Nathan Kline Institute of NYU (2001-2005). Her research focused on the role of disrupted sterol metabolism in the development of Alzheimer’s disease amyloidosis and the mechanisms by which estrogens and cholesterol-lowering drugs might exert neuroprotection. In 2005 she joined the Neuroscience and Neuropsychology of Aging Program at the National Institute on Aging where she serves as a Program Director covering research areas that address the role of metabolic and vascular factors in normal brain aging and in Alzheimer’s disease. She also facilitates the development of NIA’s drug discovery and preclinical drug development initiatives for AD, mild cognitive impairment and age-associated cognitive decline.
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