# New York City Drug Discovery: An Educational Course on Translating Research into Drugs

**New York City, NY • February 12, 2016**

Presented By:

Alzheimer's Drug Discovery Foundation

ALEXANDRIA®

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# WELCOME!

On behalf of the Alzheimer's Drug Discovery Foundation (ADDF) and the Alexandria Center, I am pleased to welcome you to the *1st New York City Drug Discovery: An Educational Course on Translating Research into Drugs*.

This meeting is based on the ADDF's 2.5 day annual *Drug Discovery for Neurodegeneration* conference (http://worldeventsforum.com/addf/drugdiscovery/), which was established in 2007 to meet the following objectives: (1) Train a cadre of interdisciplinary scientists in the principles of drug discovery; (2) Provide a platform to exchange ideas, knowledge and resources about drug discovery; (3) Stimulate preclinical research in the discovery and testing of novel compounds aimed at prevention and treatment; and (4) Build public-private partnerships that will accelerate drug discovery. We are excited to bring this condensed version to New York City, but for more information on our upcoming 2.5 day course in Miami Beach, FL on March 6-8, 2016, please see pages 17-18 or visit the registration desk.

This didactic conference will educate scientists on the processes of translating basic research into novel therapies and will leave participants with a knowledge base and relevant resources to address the associated barriers and challenges in developing a drug.

I would like to personally thank the 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 are pleased to host this meeting in New York City. There is a growing momentum for drug discovery in New York, which boasts the world's largest concentration of academic institutions and 60% of the nation's pharmaceutical industry in its vicinity. New York is focusing its efforts on building a bioscience industry and we are proud to be able to help accelerate these efforts, educate academic scientists, and stimulate new partnerships.

We are proud to welcome every attendee and are looking forward to a stimulating and educational day.

Thank you for joining us!

Howard

Howard Fillit, MD
*Founding Executive Director*
*Chief Science Officer*
Alzheimer's Drug Discovery Foundation

# ABOUT ALZHEIMER'S DRUG DISCOVERY FOUNDATION

Alzheimer's
Drug Discovery
Foundation

**CONQUERING ALZHEIMER'S THROUGH DRUG DISCOVERY**

## OUR MISSION:
## TO 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. The ultimate goal of our unique organization is to support the science that will drive the development of drug therapies for Alzheimer's.

### WHAT WE'VE ACCOMPLISHED

- The ADDF has granted more than **$87 million to fund 519 Alzheimer's drug discovery programs and clinical trials** in academic centers and biotechnology companies in **18 countries**.
- As a measure of success, programs funded by the ADDF have gone on to receive commitments of nearly **$2 billion** in follow-on commitments from the government, pharmaceutical companies and venture capital firms.
- In 2015, the ADDF raised **~$22million** to support preclinical drug discovery and clinical development programs. 100% of funds raised went directly to drug research and related scientific programs, thanks to the generosity of a private Lauder Family Foundation that covered all administrative and operational expenses.

### OUR CONFERENCES

The Alzheimer's Drug Discovery Foundation organizes two annual scientific conferences as part of our ongoing efforts to increase researchers' knowledge about Alzheimer's disease and the drug discovery process. For more information on our conferences in please see the end of the program.

**Drug Discovery for Neurodegeneration Conference**
**March 6-8, 2016 • Miami Beach, FL**

Designed as a comprehensive course on the drug discovery process, from target validation through to clinical development, the annual *Drug Discovery for Neurodegeneration* conference provides participants with the fundamental knowledge and resources to translate their research into new drugs to treat and prevent Alzheimer's disease and related neurodegenerative diseases. Attendees from academia and industry will also learn from specific case study examples and will have an opportunity to engage in interactive discussions on securing partnerships.

**International Conference on Alzheimer's Drug Discovery**
**September 12-13, 2016 • Jersey City, NJ**

This annual conference brings together academic and industry scientists intent on accelerating the development of innovative treatments for Alzheimer's disease and related dementias. Top-level scientists the field and the ADDF's funded investigators will present on their current research progress and stimulate discussion.

# ABOUT ALEXANDRIA CENTER

ALEXANDRIA®

**AN INTERNATIONAL HUB FOR COLLABORATION AND INNOVATION**

The Alexandria Center for Life Science – New York City is designed to foster unique and innovative collaborations among New York's world-renowned academic and medical institutions, preeminent scientific talent, top-tier investment capital, and the broad and diverse commercial life science industry, and to speed the translation of promising new life science discoveries "from bench to bedside." It is New York's first and only premier life science park in New York City and addresses New York's need for state-of-the-art commercial laboratory space, allowing the city to capitalize on its unparalleled top-tier science, talent and capital.

## ABOUT ALEXANDRIA

Alexandria Real Estate Equities, Inc., Landlord of Choice to the Life Science Industry®, is the largest REIT focused principally on providing high-quality real estate for the life science industry. Located in the top life science markets around the globe, Alexandria's world-class properties, highly specialized operations, and expansive network assist the life science industry in translating promising discoveries into important, safe, and effective products that benefit humankind.

## ALEXANDRIA'S NEWEST CLUSTER LOCATION: NEW YORK CITY

New York City is one of the strongest innovation locations in the world, marked by its renowned clinical and research institutions, energized collaborative culture and internationally acclaimed position as a home to leaders in science, technology, capital and talent.

### LOCATION

Alexandria is developing an integrative and collaborative first-in-class, life science and technology campus in an ideal location, proximate to innovative research and development, technology and finance.

### SCIENCE

New York City is at the crossroads of advanced life science technologies. It is the city from which over 118 Nobel laureates draw inspiration and in which approximately two dozen life science companies are started each year.

### CAPITAL

As a global hub for finance, New York City provides the necessary resources to effectively translate world-class science and technology into successful entrepreneurial organizations and human healthcare products.

### TALENT

New York City's vibrant international culture and renowned academic/medical institutions provide the ideal backdrop for recruiting and retaining extraordinary talent from within the city and around the world.

| FRIDAY, FEBRUARY 12, 2016 | |
|---|---|
| 8:30am | Registration Opens |
| 9:00–9:30 | **BREAKFAST** |
| 9:30–9:45 | **Welcome & Opening Remarks: Challenges and Opportunities in Drug Discovery**<br>Howard Fillit, MD—Alzheimer's Drug Discovery Foundation<br>Jenna Foger—Alexandria Real Estate Equities, Inc |
| 9:45–10:10 | **Embarking on a Drug Discovery Campaign**<br>David Bleakman, PhD—Eli Lilly and Company |
| 10:10–10:20 | Q&A |
| 10:20–10:45 | **Assay Development/Screening Considerations**<br>J.Fraser Glickman, PhD—The Rockefeller University |
| 10:45–10:55 | Q&A |
| 10:55–11:20 | **Medicinal Chemistry Basics**<br>Masanori Kawasaki, PhD—Tri-Institutional Therapeutics Discovery Institute |
| 11:20–11:30 | Q&A |
| 11:30–12:10 | **LUNCH** |
| 12:10–12:35 | **Drug Discovery: From Lead to Clinical Candidate**<br>Robert DeVita, PhD—Icahn School of Medicine at Mount Sinai |
| 12:35–12:45 | Q&A |
| 12:45–1:10 | **IND/Working with the FDA Throughout the Drug Development Process**<br>Sarah Schlesinger, MD—The Rockefeller University |
| 1:10–1:20 | Q&A |
| | **Lessons Learned in Drug Discovery and Development–Successful Case Study Stories in NYC**<br>**Moderator:** Lauren Friedman, PhD—Alzheimer's Drug Discovery Foundation |
| 1:20–1:40 | Case Study One: Building Intra-Cellular Therapies<br>Allen Fienberg, PhD—Intra-Cellular Therapies |
| 1:40–1:50 | Q&A |
| 1:50–2:10 | Case Study Two: BioAccelerate NYC<br>Shari Coulter Ford, MBA—Partnership Fund for New York City |
| 2:10–2:20 | Q&A |
| 2:20–2:40 | **BREAK** |
| 2:40–3:40 | **Commercialization Strategies: Developing Science into Products Panel**<br>**Moderator:** Howard Fillit, MD—ADDF<br>Sadhana Chitale, PhD, MBA—NYU Office of Industrial Liaison<br>Maria Gotsch, MBA—Partnership Fund for New York City<br>David McElligott, PhD—Accelerator Corporation<br>Martin Vogelbaum—Celgene Corporation |
| 3:40–3:50pm | **Closing Remarks:** Howard Fillit, MD—Alzheimer's Drug Discovery Foundation |

# BIOS AND ABSTRACTS

## HOWARD FILLIT, MD, ALZHEIMER'S DRUG DISCOVERY FOUNDATION

Howard Fillit, MD is an internationally recognized physician-scientist (a geriatrician and neuroscientist) and non-profit executive. He is a leading expert in Alzheimer's disease with extensive experience in academia, philanthropy and industry. Dr. Fillit is the founding Executive Director and member of the Board of Directors of the Institute for the Study of Aging (ISOA), a private foundation established by Leonard and Ronald Lauder in 1998. Since 2004, he has also been the Founding Executive Director and Chief Science Officer of the Alzheimer's Drug Discovery Foundation (ADDF), a public charity and affiliate of ISOA. ISOA and ADDF are dedicated to accelerating drug discovery and development for Alzheimer's disease. Since 1998, these organizations have provided over $80MM to over 450 academic and biotechnology drug discovery and development programs in 19 countries. Dr. Fillit currently holds the title of clinical professor of geriatrics and palliative medicine, medicine, and neuroscience at The Icahn School of Medicine at Mount Sinai (NY). He is also a Physician at The Rockefeller University Hospital. He received his BA in neurobiology *cum laude* from Cornell University, and his MD from the SUNY-Upstate Medical University. From 1995-1998, Dr. Fillit was the Corporate Medical Director for Medicare at NYLCare Health Plans (one of the largest national managed care organizations in the US at the time, a division of New York Life acquired by Aetna), providing leadership for program and policy in the provision of managed care to over 125,000 elderly individuals in several regional US markets. Dr. Fillit has served as a member of the Board of Directors for several biotechnology companies, and has been a consultant to, member, or Chair of Scientific and Clinical Advisory Boards for numerous pharmaceutical, biotechnology and health care companies.

## JENNA FOGER, ALEXANDRIA REAL ESTATE EQUITIES, INC

Jenna Foger, MA is Principal, Science and Technology at Alexandria Real Estate Equities, Inc. and Alexandria Venture Investments. She is responsible for providing scientific expertise and industry insights to support the company's real estate operations, venture investments, business development, and thought leadership initiatives, including tenant underwriting and relationship building across Alexandria's New York City life science cluster. Prior to this role, Ms. Foger worked as an Associate at Windham Venture Partners, a boutique healthcare venture capital firm focused on growth-stage medical device, diagnostic, and digital health investments. In this role, she assisted in growing the fund's life science investment practice. Before joining Windham, she was a Senior Consultant at Navigant Consulting (formerly Easton Associates), a leading NYC-based healthcare consulting firm specializing in new product evaluation and strategic planning for companies across the life science industry. Ms. Foger obtained extensive laboratory experience as a neurobiology Research Associate at The Rockefeller University. She earned her Master's degree in Biotechnology from Columbia University, and graduated Phi Beta Kappa summa cum laude from the University of Pennsylvania with a Bachelor's degree in Cognitive Neuroscience and Psychology.

## DAVID BLEAKMAN, PHD, ELI LILLY AND COMPANY

David Bleakman, PhD received his degree in Physiology from King's College, University of London. As a post-doctoral fellow at the University of Chicago, he studied neuropeptide modulation of voltage-dependent calcium channels and the role of glutamate receptors in neuronal function. He joined Eli Lilly and Company in 1993 in the United Kingdom as a scientist in the Neuroscience Division where his work focused on the discovery and development of novel therapeutic agents for the treatment of neurological and psychiatric disorders. In 1998 he moved to Lilly in Indianapolis where he continued to develop clinical candidate molecules for pain, migraine, Alzheimer's disease, Parkinson's diseases, schizophrenia and depression. In 2000 he was appointed Director of the Neuroscience Division, and in 2004, he was appointed Chief Scientific Officer overseeing pain and migraine research and development from hypothesis generation through to clinical proof of concept. In 2007 he was appointed Chief Scientific Officer of Psychiatric Disorders and Executive Director in the Neuroscience Division and in 2014 he was appointed as the site leader for Lilly Neuroscience in New York. As part of senior leadership in the Neuroscience Division, Dr. Bleakman has chaired multiple external research alliances with biotechnology companies and has formed and chaired precompetitive consortia with other pharmaceutical companies. Dr. Bleakman has published over 100 peer reviewed manuscripts, reviews and book chapters.

***Embarking on a Drug Discovery Campaign***

Within a drug discovery campaign, multiple functional partners work as a team in order to progress from a hypothesis, to a molecule that can be evaluated as a therapeutic in man. This presentation will describe the key elements that are required. A logical starting point is the identification of a druggable target that is linked to the disease process, through either empirical or genetic data for example. Experience indicates that published data must be validated to strengthen the link between the target and disease. Early on, considerations of assay development are needed to ensure that screening and SAR data are robust, reproducible and have appropriate throughput in order to support a sometimes long and challenging chemistry/bio-molecule development campaign. In addition, a molecule testing paradigm will be established with clear progression criteria. Also discussed are the criteria that establish a 'hit' a 'lead' and a 'candidate' in the drug discovery process.

## J.FRASER GLICKMAN, PHD, THE ROCKEFELLER UNIVERSITY

J. Fraser Glickman, PhD has 25 years of chemical lead discovery and optimization experience. Prior to taking the directorship of the Center at the Rockefeller University in 2008, Fraser spent 14 years understanding various drug targets, developing and miniaturizing drug target assays, evaluating novel assay technologies, managing high throughput screening laboratories and leading drug discovery project teams at Burroughs Welcome Co., Pharmacopeia Inc. and Novartis Pharmaceuticals Inc. He is an author of 41 research papers and review articles and holder of several patents on drug candidates and assay technologies. He also serves on the editorial board of *Assay and Drug Development Technologies*.

***Assay Development/Screening Considerations***

High Throughput screening of random, "medicinal quality" compound libraries has become the major technological approach for identifying small molecule drug leads for pre-clinical development. Although the breadth of miniaturized assay technologies that one can utilize for addressing a particular drug target is impressive, it is important to meticulously control and optimize screening assays such that they are statistically "robust", and physiologically relevant. In addition to this often difficult challenge, one must develop the capacity to rigorously evaluate hit compounds coming out of a screen. This effort post-screening effort also requires high throughput technology and significant resources. This talk will offer a brief overview of the considerations and technologies involved and the resources needed to successfully identify medicinally relevant lead compounds for further pre-clinical optimization.

## MASANORI KAWASAKI, PHD, TRI-INSTITUTIONAL THERAPEUTICS DISCOVERY INSTITUTE

Masanori Kawasaki, PhD is a Medicinal Chemistry Leader at the Tri-Institutional Therapeutics Discovery Institute (Tri-I TDI), a non-profit organization established by Weill Cornell Medicine, The Rockefeller University, and Memorial Sloan Kettering Cancer Center. Dr. Kawasaki received his PhD in Chemistry from Osaka City University in 2005. He was awarded a postdoctoral fellowship from the Uehara Memorial Foundation in 2004. After graduation, Dr. Kawasaki conducted postdoctoral research in the Department of Chemistry at the University of Chicago under the supervision of Hisashi Yamamoto. In 2008, he joined Takeda Pharmaceutical Company Ltd. as a Medicinal Chemist. He was transferred to Tri-I TDI in 2014.

***Medicinal Chemistry Strategies for CNS Drug Discovery***

In drug discovery, medicinal chemists are responsible for designing and synthesizing drug candidates as well as tool compounds, which are used to validate target proteins. Medicinal chemists convert hit compounds into lead compounds and identify drug candidates through the Lead Optimization Process. Several approaches – including structure-based drug design (SBDD), fragment-based drug design (FBDD), and ligand-based drug design (LBDD) – are applied to the design of lead compounds. In order to increase the productivity of drug discovery and the likelihood of approval (LOA), many benchmarks are used by medicinal chemists to assess the quality of a compound, such as the Rule of Five. In this presentation, basic medicinal chemistry approaches, benchmarks for drug discovery, and examples for CNS drug design will be introduced.

## ROBERT DEVITA, PHD, ICAHN SCHOOL OF MEDICINE AT MOUNT SINAI

Robert DeVita, PhD is a Professor at the Icahn School of Medicine at Mt. Sinai in the Departments of Pharmacology and Systems Therapeutics and Structural and Chemical Biology. He is also the Director of Medicinal Chemistry for the Experimental Therapeutics Institute (ETI). Prior to joining Mt. Sinai in 2014, Dr. DeVita gained expertise managing multi-disciplinary teams that delivered on key program objectives for complex molecular targets. He has over 25 years' experience working in biotech (VP of Chemistry at Agios) and the pharmaceutical

industry at Merck Research Laboratories where he was a director of medicinal chemistry from 2004-2012. He was trained as an organic synthetic chemist earning his PhD at University of Rochester followed by a N.S.F. Postdoctoral at the University of Geneva. He started his professional career at Merck in 1990. Dr. DeVita's work has spanned the drug discovery paradigm from target ID to PII, including leadership of drug development teams. In collaboration with multi-disciplinary discovery teams, he has identified numerous development candidates including two PII clinical compounds for Central Nervous System ($NK_1$) and Cardiovascular (NPC1L1) targets. Dr. DeVita has drug discovery experience within a broad range of therapeutic areas including: CNS, pain/inflammation, diabetes, cardiovascular, hypertension, obesity, endocrinology, urology and oncology. He has developed, in collaboration with his teams, orally active, brain penetrant, peripheral and GI-tract drug targeting strategies. He also has experience in the discovery and development of PET imaging agents and translational biomarkers for CNS targets. Dr. DeVita has been an active member of the Medicinal Chemistry Division of the American Chemical Society serving on the Long Range Planning Committee and on the organizing committees for National and International Medicinal Chemistry Meetings. He has served as an Ad Hoc Reviewer for the National Institutes of Health Study Section for Synthetic and Biological Chemistry (Section B) and National Institute of Diabetes, Digestive and Kidney Diseases. He also consults for academic, biotech, legal and venture capital clients.

***Drug Discovery: From Lead to Clinical Candidate (including biomarkers) for FAAH Inhibitors***

Fatty acid amide hydrolase (FAAH) in a peripheral and central nervous system membrane-bound enzyme responsible for the breakdown of fatty acid ethanol amide (FAEs) endocannibinoids, which are thought to suppress pain transmission thru a variety of receptors. FAAH has been pursued recently by biotech and pharma companies for a variety of CNS indications including pain, Multiple Sclerosis, Tourette's Syndrome and addiction. The lead optimization process, applied to the identification of a novel, non-covalent inhibitor for FAAH from Merck, will be presented. The discovery of a novel PET ligand for FAAH and its use as a target engagement biomarker using PET imaging will also be discussed.

## SARAH SCHLESINGER, MD, THE ROCKEFELLER UNIVERSITY

Sarah Schlesinger, MD is currently an Associate Professor of Clinical Investigation at The Rockefeller University and the Clinical Director of The Laboratory of Molecular Immunology and Senior Physician at The Rockefeller University Hospital. Sarah was graduated with honors from Wellesley College and then obtained her medical doctorate from Rush Medical College in Chicago, Illinois. Dr Schlesinger then trained in Anatomic Pathology at the New York Hospital/Cornell Medical Center, where she was chief resident in Anatomic Pathology and then joined the faculty. In 1994, Dr Schlesinger moved to Washington DC and led The Dendritic Cell section of The Division of Retrovirology at the Walter Reed Army Institute of Research ("WRAIR"). Dr Schlesinger's responsibilities at WRAIR included participation in the design and development of HIV vaccines. She was also a member of the Division of Infectious and Parasitic Disease Pathology at The Armed Forces Institute of Pathology ("AFIP"). In 2002, Dr Schlesinger returned to live in New York City. Since rejoining the Rockefeller University in 2002, Dr Schlesinger has led the clinical efforts to bring new HIV vaccine candidates developed at Rockefeller into the clinic. In this time, eleven phase I studies have been completed to evaluate the safety and immunogenicity of HIV vaccine candidates and adjuvants and monoclonal antibodies. She has personally held the IND for these candidates. Dr Schlesinger is working on the pre-clinical and regulatory plans to ensure a substantial pipeline of innovative HIV vaccine candidates and vaccine adjuvants for future testing**.** Sarah is also an

advocate for HIV prevention and serves on the board two non-profit organizations : AVAC The AIDS Vaccines Advocacy Coalition and Global Viral. She is also a corporate director of Ariad Pharmaceuticals.

***IND/Working with the FDA throughout the Drug Development Process***

A brief introduction to working with the US Food and Drug Administration (FDA) as a partner to bring a novel therapeutic from an academic lab into the clinic will be presented. The structure of regulatory filings associated with an Investigational New Drug application (IND) and the development and sequencing of the related pre-clinical work and clinical trials will be discussed.

## LAUREN FRIEDMAN, PHD, ALZHEIMER'S DRUG DISCOVERY FOUNDATION

Lauren Friedman, PhD, supports the management of our drug discovery portfolio by providing scientific and strategic review of preclinical drug discovery proposals and tracking program progress. Dr. Friedman also manages the ADDF ACCESS program, which provides a virtual network of contract research organizations (CRO) and consultants and offers educational resources on drug discovery and CRO selection and management. Dr. Friedman completed her postdoctoral training at Columbia University, where she studied modulators of autophagy in Alzheimer's disease. She earned a doctorate in neuroscience at the Icahn School of Medicine at Mount Sinai, where she focused on molecular mechanisms underlying the development and degeneration of brain circuits involved in autism and Parkinson's disease. She received a bachelor's degree in biopsychology from Tufts University. Dr. Friedman has authored numerous peer-reviewed publications and is a member of the Society for Neuroscience, New York Academy of Sciences and the Association for Women in Science.

## ALLEN FIENBERG, PHD, INTRA-CELLULAR THERAPIES

Allen Fienberg, PhD has served as Vice President of Business Development of ITI since June 2002. He co-founded ITI in May 2002. Dr. Fienberg received his A.B. degree in Genetics from the University of California, Berkeley and his Ph.D. in Human Genetics from Yale University. He completed post-doctoral studies at The Rockefeller University under the direction of Dr. Paul Greengard from 1991-1999. From 1999-2001, Dr. Fienberg was a staff scientist at the Genomics Institute of the Novartis Research Foundation and was appointed a Research Assistant Professor at The Rockefeller University from 2001-2002.

***Building Intra-Cellular Therapies, a CNS Focused Biotech in New York City***

Intra-Cellular Therapies is a biopharmaceutical company focused on the discovery and clinical development of innovative, small molecule drugs that address underserved medical needs in neuropsychiatric and neurological disorders by targeting intracellular signaling mechanisms within the central nervous system, or CNS. The Company is developing its lead drug candidate, ITI-007, for the treatment of schizophrenia, behavioral disturbances in dementia, bipolar disorder and other neuropsychiatric and neurological disorders. Dr. Fienberg, was one of the co-founders of Intra-Cellular Therapies, along with Sharon Mates, Paul Greengard (Nobel laureate 2000) and Moshe Alafi. He will discuss the history of the company and provide some first-hand observations of the challenges and advantages of being a biotech in New York City.

## SHARI COULTER FORD, MBA, PARTNERSHIP FUND FOR NEW YORK CITY

Shari Coulter Ford, MBA leads the Partnership Fund's investments in healthcare, including the BioAccelerate program and the New York Digital Health Accelerator. Previously, Shari launched NYC Tech Connect, a Partnership Fund initiative focused on catalyzing the NYC entrepreneurial bioscience community through a series of programs, including Riverside Chats Speakers Series and NYC Emerging Technologies Summit. Previously, Shari was President and CEO of New York Technology Accelerators LLC, a Partnership Fund portfolio company, and New York City's first university-based incubator. Shari spent 13 years with PR Newswire, a division of London-based United Business Media where she held several executive positions. Her last role was President of PR Newswire International, a startup business unit where she completed two acquisitions and opened offices in five countries in three years. Shari is a graduate of the Wharton Management program at the Wharton School, University of Pennsylvania and has a bachelor of journalism degree from the University of Missouri-Columbia.

***BioAccelerate NYC: Enabling Entrepreneurs and Startups***

The BioAccelerate program aims to increase the number of biomedical commercial spin outs coming out of New York's world class academic institutions and to spur business and job creation in the bioscience sector. The program fills a critical gap in proof-of-concept and early stage funding for the sector in New York City. A panel of experienced VC judges approves investments and each researcher/company that receives funding is paired with an industry mentor who provides guidance on commercialization and company formation. A total of $15 million is committed to this program, which includes $5 million for Phase I and $10 million for Phase II. Phase I provides up to $250,000 per researcher to complete proof-of-concept research. Funding is expected to be the last capital required to prove the commercial potential of the technology and spin out a company. To date, we have received ~210 applications and awarded $5M to 20 scientists. Phase II investments are up to $1.5M to help launch an early stage company, structured as equity or a hybrid investment. To date, we have invested $6.1 million in 9 companies.

## SADHANA CHITALE, PHD, MBA, NYU OFFICE OF INDUSTRIAL LIAISON

Sadhana Chitale, PhD, MBA is the Director of Life Sciences/Technology Transfer. Sadhana joined the Office of Industrial Liaison at New York University in 2001. Her primary area of responsibility is the management of intellectual property and technology transfer matters arising from New York University's Langone Medical Center and the downtown campus. Sadhana received her PhD from the University of Mumbai and an MBA from the University of Pittsburgh. She is a Certified Licensing Professional and a registered patent agent. Prior to joining NYU, Sadhana was a Licensing Manager at the Weill Cornell Medical School. She has been an invited speaker and moderator on technology transfer and intellectual property management matters at local and national meetings. Sadhana is an active member of the Annual Meeting Planning Committee of AUTM.

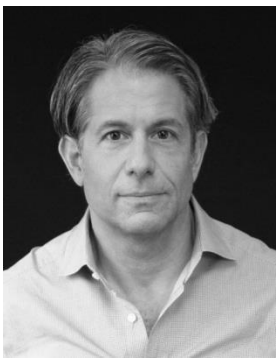
## MARIA GOTSCH, MBA, PARTNERSHIP FUND FOR NEW YORK CITY

Maria Gotsch, MBA is the President and CEO at the Partnership Fund for New York City, which is the investment arm of the Partnership for New York City. In addition to leading the Fund's operations, Maria has spearheaded the creation and operation of a number of the Fund's strategic initiatives, including: FinTech Innovation Lab; New York Digital Health Accelerator; Fashion Tech Lab; NYCSeed (seed financing for IT/digital media companies); BioAccelerate Prize NYC (proof-of-concept funding for university-based biomedical research); Arts Entrepreneurial Loan Fund (low cost loans for mid-size arts groups); and ReStart Central and Financial Recovery Fund (assistance and funding for small businesses impacted by 9/11/01). Prior to joining the Fund in 1999, Maria was a Managing Director at BT Wolfensohn (now part of Deutsche Bank), providing strategic and financial advice related to mergers, acquisitions, dispositions, joint ventures and the development of business strategies. Before starting with Wolfensohn, Maria worked at LaSalle Partners in the New York area and for Merrill Lynch Capital Markets in New York and London. Maria has an MBA from Harvard Business School and a B.A. from Wellesley College. She was also the recipient of a Fulbright Fellowship to study international relations at the Institut Universitaire de Hautes Etudes Internationales in Geneva, Switzerland.

## DAVID MCELLIGOTT, PHD, ACCELERATOR CORPORATION

David McElligott, PhD joined Accelerator in March of 2012 as its Chief Scientific Officer. Previously, he served as the founding Chief Scientific Officer at Groove Biopharma, a microRNA therapeutics company, where he was responsible for leading the spin-out of the technology from Nanogen (now part of ELITech Group). Prior to founding Groove Biopharma, Dr. McElligott served as Senior Director of Biological Sciences at ICOS Corp. where he led numerous preclinical drug discovery projects in oncology, inflammation, and urology. Several of those projects led to successful IND filings and clinical development programs. Before joining ICOS in 1997, Dr. McElligott held academic appointments in the Department of Immunology at The Scripps Research Institute and The Salk Institute for Biological Sciences. Dr. McElligott received his PhD and B.S degrees in Microbiology/Immunology from the University of Iowa. He also serves on the advisory boards of the NYU Innovation Venture Fund and Rockefeller University's newly-established Robertson Therapeutic Development Fund.

## MARTIN VOGELBAUM, CELGENE CORPORATION



Martin Vogelbaum is a Corporate Vice President, Business Development at Celgene Corporation. Prior to joining Celgene in 2015, he spent 22 years as venture capitalist, focused on the life sciences sector, most recently as a Partner at Rho Ventures. Mr. Vogelbaum joined Rho in 2005 where he invested in private and publicly traded life sciences companies at all stages of development including Gloucester Pharmaceuticals (acquired by Celgene), SARcode Biosciences (acquired by Shire), AqueSys (acquired by Allergan), Cara Therapeutics (NASDAQ: CARA) and Inotek Pharmaceuticals (NASDAQ: ITEK). Prior to Rho, Mr. Vogelbaum was a General Partner at

Apple Tree Partners where he founded or co-founded several companies including Gloucester Pharmaceuticals, Aileron Therapeutics and Tokai Pharmaceuticals (NASDAQ: TKAI). Mr. Vogelbaum began his venture career at Oxford Bioscience Partners where he served as a General Partner. Prior to venture capital, Mr. Vogelbaum was a Research Associate in the Bone Marrow Transplant Unit at Memorial Sloan Kettering, under the direction of Dr. Richard O'Reilly, where he conducted research in graft vs. host disease. Mr. Vogelbaum has an AB from Columbia University.

# KEY ABBREVIATIONS

- ADMET (absorption, distribution, metabolism, excretion)
    - Absorption-ability of drug to penetrate the GI tract to the circulatory system
    - Distribution-solubility of drug in blood, binding to plasma proteins
    - Metabolism -chemical modifications of drug (e.g. by cytochrome P), amount available to reach target
    - Excretion-mechanisms of drug elimination from the body
    - Toxicity
- API-Active pharmaceutical ingredient
- BBB-Blood brain barrier
- CMC-Chemistry, manufacturing, control
- CNS-Central nervous system
- CRO-Contract research organization
- CSF-Cerebral spinal fluid
- CYP450-Cytochrome P450 enzyme family
- FDA-Food and Drug Administration
- EMA-European Medicines Agency
- FBLD-Fragment based lead discovery
- FTE-Full time employee
- FIH-First-in-humans
- GCP-Good clinical practice
- GLP-Good laboratory practice
- GMP-Good manufacturing practices (cGMP)
- HCS-High content screening
- hERG-Human ether-a-go-go gene
- HTS-High throughput screening
- IND-Investigational new drug
- IRB-Institutional review board
- LC-MS/MS-Liquid chromatography coupled with tandem mass spectrometry
- LOEL-Lowest observed effect level
- logP-Octanol-water partition coefficient
- MW-Molecular weight
- NCE-New chemical entity
- NDA-New drug application
- NIA-National Institute of Aging
- NIH-National Institute of Health
- NINDS-National Institute of Neurological Diseases and Stroke
- NOAEL-No observable adverse effect level
- NOEL-No observable effect level
- MOA-Mechanism of action
- MTD-Maximum tolerated or minimally toxic dosePD-Pharmacodynamics
- PK-Pharmacokinetics
- POC-Proof of concept
- PSA-Polar surface area
- QSAR-Quantitative structure activity relationship
- SAR-Structure-activity relationship
- SBIR-Small Business Innovation Research Award
- SOP-Standard operating procedure
- STTR-Small Business Technology Transfer
- TI-Therapeutic index, ratio between the dose that produces toxic effects to the dose needed for therapeutic response.
- Toxicokinetic parameters:
    - AUC = area under the plasma concentration vs. time curve
    - $C_{max}$ = maximum plasma concentration
    - $T_{max}$ = time to achieve maximum plasma concentration
    - $T_{1/2}$ = elimination half-life
    - F = percent bioavailability
- TPP-Target product profile

*The Alzheimer's Drug Discovery Foundation Presents:*

# 10th ANNUAL DRUG DISCOVERY FOR NEURODEGENERATION:

## An Educational Course on Translating Research into Drugs

***March 6-8, 2016 • Miami Beach, FL***

### COURSE OBJECTIVES

1. Train a cadre of interdisciplinary scientists in the principles of drug discovery for neurdegenerative disease;
2. Provide a platform to exchange ideas, knowledge and re-sources about drug discovery for neurodegenerative disease;
3. Stimulate pre-clinical research in the discovery and testing of novel compounds aimed at the prevention and treatment of neurodegenerative disease; and
4. Build public-private partnerships that will accelerate drug discovery for neurodegenerative disease.

### TARGET AUDIENCE

- Academic and industry scientists engaged in drug discovery research for neurodegenerative disease or CNS
- Business development & licensing professionals
- Alliance management professionals
- Young investigators and graduate students

### GROUP DISCOUNTS

Discounts are offered for groups of three or more from the same organziation.

### WHAT ATTENDEES WILL LEARN

1. Challenges and opportunities in academic drug discovery
2. Fundamentals of medicinal chemistry relevant to drug discovery for neurodegenerative diseases
3. Newest trends in assay development and high throughput screening (HTS)
4. Go-no-go criteria for preclinical development, including pharmacokinetic behavior of candidate compounds, aqueous solubility, blood-brain barrier permeability, preliminary safety, and manufacturing issues
5. Study design considerations for animal model experiments
6. Biologics for challenging CNS targets and strategies to optimize brain delivery
7. Requirements for an Investigational New Drug (IND) application
8. Commercialization strategies for developing science into products
9. Best practices for working with tech transfer offices, managing intellectual property, and the role of funding organizations
10. Funding and resources for preclinical therapeutics development for neurological disorders.

## Register Online Today

http://worldeventsforum.com/addf/drugdiscovery/registration/

The
***Drug Discovery for Neurodegeneration***
conference
advances drug discovery for neurodegenerative diseases by educating scientists on the process of translating basic research into novel therapies.

The course is designed to give participants knowledge and relevant resources about this field of scientific investigation and address the associated barriers and challenges. The program will focus on Alzheimer's disease, Parkinson's disease, and Multiple Sclerosis.

Alzheimer's **Drug Discovery** Foundation

*For more details, please visit the ADDF website or call Sara Classen, Assistant Director, Scientific Events, at +1.212.901.8009 or email sclassen@alzdiscovery.org.*

Alzheimer's Drug Discovery Foundation

# 10th ANNUAL DRUG DISCOVERY FOR NEURODEGENERATION:
## An Educational Course on Translating Research into Drugs

# REGISTRATION

*March 6-8, 2016 • Miami, FL*

REGISTRATION FOR THE CONFERENCE INCLUDES:

- Access to the conference sessions, poster presentations and exhibits
- Program book inclusive of abstracts and speaker bios
- Continental breakfast and lunch (March 7 and March 8)
- Coffee breaks (March 7 and March 8)
- Access to the Welcome Reception (March 6)
- Access to the Networking and Poster Reception (March 7)

** Proof of academic status is required; please submit copy of student ID*
*** A start-up biotechnology company is defined as an organization less than three years old and with 20 or fewer employees. All such registrations require prior approval from the Conference Secretariat.*
**** Guest access to the two receptions only (guest must be accompanied by the registrant).*
***** Proof of affiliation will be required prior to the event.*

| SINGLE REGISTRATION *(all fees in US Dollars)* | EARLY BIRD (Received by February 5, 2016) | STANDARD (Received after February 5, 2016) | AT DOOR | SELECT |
|---|---|---|---|---|
| Post-Doctoral / Graduate Student* | $200 | $225 | $275 | |
| Academia /Government / Non-Profit Organization or Start-up Biotechnology Company** | $225 | $250 | $300 | |
| Industry and Private Practice | $600 | $650 | $700 | |
| Welcome & Networking Reception Guest*** | $55 | $55 | $55 | |
| Media**** | $0 | $0 | $0 | |

**TOTAL AMOUNT DUE $**

CHANGES TO THE PROGRAM:
Although great care has been taken in preparing and updating the meeting program, the organizers cannot be held responsible or accept any liability for inaccuracies or omissions and cannot be held responsible for any damage, loss or costs resulting from the compiled information.

LIABILITY:
The meeting organizers and the secretariat will not accept liability for any personal injury, damage or loss that may occur during or directly arising from this meeting. In addition, the meeting organizers reserve the right to change the contents, venue and/or time as necessary.

CANCELLATIONS:
Cancellations must be made in writing. A full refund minus a $35 processing fee is available through February 5, 2016. No partial refunds will be made available. Fax requests for cancellation to Sara Classen at +1.212.901.8010.

SUBSTITUTIONS:
Changes to attendee information or registration substitutions must be made no later than February 22, 2016.

**FAX COMPLETED FORM TO:**
**+1.212.901.8010**

**or**

**MAIL TO:**
**Sara Classen**
*Assistant Director, Scientific Events*
**Alzheimer's Drug Discovery Foundation**
**57 West 57th Street, Suite 904**
**New York, NY 10019—USA**

Questions about registration should be addressed to:

**Sara Classen**

*Assistant Director, Scientific Events*
Alzheimer's Drug Discovery Foundation
57 West 57th Street, Suite 904
New York, NY 10019—USA
T +1.212.901.8009 • F +1.212.901.8010
sclassen@alzdiscovery.org

**Register online at:**
http://worldeventsforum.com/addf/drugdiscovery/registration/

## REGISTRANT INFORMATION

**REGISTRANT 1 (all fields marked * are required)**

Registrant's First Name* | Registrant's Last Name* | Registrant's Middle Initial

Registrant's Name (as it should appear on the badge)* | Suffix* (PhD, MD, MD/PhD, MS, etc.)

Registrant's Organization* | Registrant's Department

Registrant's Address*

Registrant's City, State/Province, Zip Code* | Registrant's Country*

Registrant's Email Address* | Registrant's Telephone*

Special Requirements (accessibility, TDD, diet, etc.)

**REGISTRANT 2**

Registrant's Name (as it should appear on the badge) | Registrant's Position/Title

Registrant's Organization | Registrant's Department

Registrant's Email Address | Registrant's Telephone

Special Requirements (accessibility, TDD, diet, etc.)

**RECEPTION GUEST**

Guest's Name (as it should appear on the badge)

## METHOD OF PAYMENT

☐ **Check** payable to *Alzheimer's Drug Discovery Foundation (checks should be drawn ONLY on a US bank)*

☐ Visa ☐ MasterCard ☐ Discover ☐ AmEx

Name as it appears on the Credit Card

Credit Card Number | Billing Address (if different from above)

Expiration Date | Billing Address (cont.)

Security Code *(last 3 digits on the back of your card if Visa/MC/D, 4 digits on the front of your card if AmEx)* | City/State or Province/Zip Code or Postal Code

Signature | Country

# SAVE THE DATE

# 17TH INTERNATIONAL CONFERENCE ON ALZHEIMER'S DRUG DISCOVERY

September 12-13, 2016 • Jersey City, NJ *(across the Hudson River from NYC)*

*Presented by the Alzheimer's Drug Discovery Foundation*

## GENERAL INFORMATION

This annual conference brings together academic and industry scientists intent on accelerating the development of innovative treatments for Alzheimer's disease and related dementias. The ADDF's funded investigators and top level scientists in the field will present their current research, provide progress updates and stimulate discussion. The conference offers ample opportunities for collaboration and partnering.

Alzheimer's **Drug Discovery** Foundation

## TARGET AUDIENCE

The conference generally attracts 150 attendees from around the globe. Attendees include:

- Academic and industry scientists engaged in drug discovery research for Alzheimer's disease
- Business development and licensing professionals
- Alliance management professionals
- Venture capitalists and other investors


## OBJECTIVES

- Highlight scientific progress on drug discovery programs aimed at treating Alzheimer's disease and related dementias.
- Increase networking opportunities for scientists to share information and resources.
- Foster interdisciplinary and public-private partnerships and alliances.

## SCHOLARSHIPS

The Alzheimer's Drug Discovery Foundation invites applications for the 2016 ADDF Young Investigator Scholarships. Ten scholarships will be awarded. These prestigious scholarships recognize early achievements and seek to encourage the career development of the next generation of research scientists.

Questions about
the conference should be addressed to:

**Sara Classen**
*Assistant Director, Scientific Events*
Alzheimer's Drug Discovery Foundation
57 West 57th Street, Suite 904
New York, NY 10019
Tel: +1.212.901.8009 | sclassen@alzdiscovery.org

**www.alzdiscovery.org/events/conferences**