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

March 6-8, 2016 • Miami FL

Presented by the Alzheimer's Drug Discovery Foundation

www.alzdiscovery.org
#CNSDrugCourse

Mobile app:
http://my.yapp.us/ADDFDD4N
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LIST OF 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
- **MOA** - Mechanism of action
- **MTD** - Maximum tolerated or minimally toxic dose
- **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
- **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\text{max}** = maximum plasma concentration
  - **T\text{max}** = time to achieve maximum plasma concentration
  - **T\text{1/2}** = elimination half-life
  - **F** = percent bioavailability
- **TPP** - Target product profile
ABOUT THE 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 Alzheimer’s Drug Discovery Foundation (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. The ADDF also plans smaller “catalyst conferences” that center around a relevant topic in the field of neurodegeneration.

Our International Conference on Alzheimer’s Drug Discovery is held on September 12-13, 2016 in Jersey City, NJ. The 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 in the field and the ADDF’s funded investigators will present on their current research progress and stimulate discussion.

Our Drug Discovery for Neurodegeneration Conference will be held next year, March 2017, in San Diego, CA. This conference is 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. Finally, the European Drug Discovery for Neurodegeneration Conference will debut later this spring.
SCIENTIFIC ADVISORY COMMITTEE

Kurt Brunden, PhD, University of Pennsylvania

Howard Fillit, MD, Alzheimer’s Drug Discovery Foundation

Lauren Friedman, PhD, Alzheimer’s Drug Discovery Foundation

Marcie Glicksman, PhD, Orig3n, Inc.

Frank Longo, MD, PhD, Stanford University

Suzana Petanceska, PhD, National Institute on Aging

Diana Shineman, PhD, Alzheimer’s Drug Discovery Foundation

Edward Spack, PhD, Vector Preclinical Consulting

D. Martin Watterson, PhD, Northwestern University

CONFERENCE DELIVERABLES

A webcast of the entire conference will be made available on the Alzheimer’s Drug Discovery Foundation Conference website (http://worldeventsforum.com/addf/drugdiscovery/videocasts), where you may also access a webcast of last year’s conference.
On behalf of the Alzheimer's Drug Discovery Foundation (ADDF), I am pleased to welcome you to the 10th Drug Discovery for Neurodegeneration Conference: An Educational Course on Translating Research into Drugs.

Designed as a comprehensive course on the drug discovery process, from target validation through to clinical development, this 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.

I would like to personally thank our scientific advisory committee, session chairs, and speakers for their dedication and commitment to this meeting. Your 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 which showcases our talented Young Investigator Award winners and other presentations by individuals in academia. Poster presentations are scheduled for the last 30-40 minutes during the lunch breaks.

Our meeting is made possible by the generous support of our sponsors: National Institute on Aging, Eli Lilly, Taub Institute for Research on Alzheimer's Disease and the Aging Brain, Harrington Discovery Institute, and our exhibitors: Bachem Americas Inc, Brains On-Line, EMD Millipore, InviCRO/mCi, InterVivo Solutions Inc., OnDeckBiotech, Piramal Discovery Solutions, PsychoGenics Inc, and Renovo Neural Inc. We would also like to thank our media partners for their commitment to making this meeting a success.

This year, we are pleased to host the meeting in Miami, Florida, which boasts over 100 biotechnology companies, 90 pharmaceutical & biopharmaceutical companies, and 400 medical device manufacturing companies. It is a top 5 state for bioscience employment, and bioscience job growth in Florida is 5% greater than the national average. We are thrilled to be able to participate in this community and bring our conference to this progressive state.

We are proud to welcome attendees from all over the world and are looking forward to a stimulating and educational two and half days. Thank you for joining us!

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

Lectures will be held in the Regency Ballroom, Lobby Level.
Exhibits and Poster Sessions will be held in the Grande Promenade Ballroom, Lobby Level.
Partnering Sessions will be held in the Spanish Suites, Mezzanine.
Coffee breaks and meals will be served in the Grande Promenade Ballroom, Lobby Level.

Sunday, March 6, 2016

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<td>2:00pm–4:00</td>
<td>Registration (Regency Conference Room, Lobby Level)</td>
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<td>4:00–4:20</td>
<td>Welcome &amp; Opening Remarks: Challenges and Opportunities in Academic Drug Discovery Howard Fillit, MD—Alzheimer’s Drug Discovery Foundation</td>
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<tr>
<td>4:20–5:05</td>
<td>Global Collaboration: Essential to Accelerate the Identification, Validation and Development of New Targets and Treatments for Alzheimer’s Disease and other Neurodegenerative Disorders Luc Truyen, MD, PhD—Janssen Research &amp; Development, LLC.</td>
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<td>5:05–5:15</td>
<td>Q&amp;A</td>
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<td>5:15–7:00</td>
<td>Welcoming Reception (OceanView Room/Poolside)</td>
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Monday, March 7, 2016

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<td>7:30am–8:30</td>
<td>Continental Breakfast</td>
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<td>8:30–8:40</td>
<td>Welcome &amp; Opening Remarks</td>
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<td>8:40–8:50</td>
<td>National Institute on Aging Alzheimer’s Disease Translational Research Program Lorenzo Refolo, PhD—National Institute on Aging</td>
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<td>8:50–9:00</td>
<td>NINDS Opportunities for Translational Research Funding Amir Tamiz, PhD—National Institute of Neurological Disorders and Stroke</td>
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I. Embarking on a Drug Discovery Campaign
Chair: Marcie Glicksman, PhD—Orig3n, Inc.

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<td>9:00–9:05</td>
<td>Session Overview: Marcie Glicksman, PhD—Orig3n, Inc.</td>
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<td>9:05–9:25</td>
<td>Assembling the Right Interdisciplinary Team from the Beginning Julie Frearson, PhD—Charles River Laboratories</td>
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<td>9:25–9:35</td>
<td>Q&amp;A</td>
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<tr>
<td>9:35–9:55</td>
<td>Quantitative Systems Pharmacology for Supporting Drug Discovery in Alzheimer’s Disease Hugo Geerts, PhD—In Silico Biosciences</td>
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<td>9:55–10:05</td>
<td>Q&amp;A</td>
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<tr>
<td>10:05–10:25</td>
<td>New Trends and Technology in Assay Development Marcie Glicksman, PhD—Orig3n, Inc.</td>
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<td>10:25–10:35</td>
<td>Q&amp;A</td>
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<tr>
<td>10:35–11:05</td>
<td>Exhibitor Session and Break</td>
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II. Starting at the End: The Pharmacology—Chemistry Interface in Preclinical Drug Development
Chair: D. Martin Watterson, PhD—Northwestern University

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<td>11:05–11:10</td>
<td>Session Overview D. Martin Watterson, PhD—Northwestern University</td>
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<td>11:10–11:30</td>
<td>Fragment-based Drug Discovery Daniel Erlanson, PhD—Carmot Therapeutics, Inc.</td>
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<td>11:30–11:40</td>
<td>Q&amp;A</td>
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<tr>
<td>11:40–12:00</td>
<td>A Medicinal Chemistry Perspective on Picking the Right Screening Strategy David Swinney, PhD—Institute for Rare and Neglected Diseases Drug Discovery (iRND3)</td>
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<tr>
<td>12:00–12:10pm</td>
<td>Q&amp;A</td>
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<tr>
<td>12:10–12:30</td>
<td>Pharmacology Driven Optimization in Candidate Development D. Martin Watterson, PhD—Northwestern University</td>
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<td>12:30–12:40</td>
<td>Q&amp;A</td>
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<td>12:40–1:40</td>
<td>Lunch and Poster Session - All Poster Presenters Should Stand by their Posters from 1:05 to 1:40pm</td>
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III. Drug Discovery: From Lead to Clinical Candidate
Chair: Edward Spack, PhD—Vector Preclinical Consulting

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<td>1:40–1:45</td>
<td>Session Overview Edward Spack, PhD—Vector Preclinical Consulting</td>
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<td>1:45–2:05</td>
<td>PK/PD in Preclinical Development Barry Greenberg, PhD—University Health Network</td>
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<td>2:05–2:15</td>
<td>Q&amp;A</td>
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<tr>
<td>2:15–2:35</td>
<td>Overcoming Challenges in Formulation and Delivery Paul Skuitety, PhD—Xcelience</td>
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<tr>
<td>2:35–2:45</td>
<td>Q&amp;A</td>
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### Requirements for an IND
Edward Spack, PhD—Vector Preclinical Consulting

### How to Work Best with the FDA Throughout the Drug Development Process
Stephen Arneric, PhD—Critical Path Institute

### ADDF ACCESS & Young Investigator Scholarship Awards Presentation
Lauren Friedman, PhD—Alzheimer’s Drug Discovery Foundation

### Partnering Session / Mentoring Session *pre-registration required

### Networking Reception (Starlight Ballroom, 18th Floor)

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### Partnering Session / Mentoring Session *pre-registration required

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### Welcome & Opening Remarks
Diana Shineman, PhD—Alzheimer’s Drug Discovery Foundation

### Gene Therapy Strategies for Treating or Preventing Alzheimer’s Disease and Related Neurodegenerative Disorders
Steven Paul, MD—Voyager Therapeutics, Inc.

### Development of Tau Immunotherapy: Unique Considerations for Biologics
Tim West, PhD—C2N Diagnostics

### Targeting P2X7 to Block Neuroinflammation: HTS to Lead Discovery
Paolo Pevarello, PhD—Axxam SpA

### A Novel CB2 Agonist: Preparing for an IND
Joseph Foss, MD—NeuroTherapia Inc. & Cleveland Clinic

### Development and Regulatory Considerations for the Clinical Development of AGB101 (low dose levetiracetam) for aMCI Due to AD
Sharon Rosenzweig-Lipson, PhD—AgeneBio

### Lunch and Poster Session - All Poster Presenters Should Stand by their Posters from 12:45 to 1:10 pm

### Commercialization Strategies: Developing Science into Products
Chair: Frank Longo, MD, PhD—Stanford University and PharmatrophiX

### Intellectual Property Considerations for Early Stage Life Science Companies
Laurie McNamara, JD, PhD & Maya Skubatch, JD—Wilson Sonsini Goodrich & Rosati

### Lessons Learned in Drug Development from an Academic and Small Biotech Perspective
Frank Longo, MD, PhD—Stanford University & PharmatrophiX

### Strategies for Securing Private Investment
Melissa Krauth, MBA—Angel Investor

### Closing Remarks:
Howard Fillit, MD—Alzheimer’s Drug Discovery Foundation
1st EUROPEAN DRUG DISCOVERY FOR NEURODEGENERATION CONFERENCE

15-17 May, 2016 – Budapest, Hungary

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

The program will also highlight case studies of small molecule and biologics programs at different stages of development aimed at tackling challenging CNS targets relevant to neurodegenerative diseases.

AUDIENCE
The audience generally includes academic and industry scientists engaged in drug discovery research for neurodegenerative disease or CNS, business development and licensing professionals, alliance management professionals and young investigators and graduate students.

WHAT YOU WILL LEARN
- Challenges and opportunities in academic drug discovery
- Fundamentals of relevant medicinal chemistry
- Newest trends in assay development and high throughput screening (HTS)
- Go-no-go criteria for preclinical development (PK behavior of candidate compounds, aqueous solubility, BBB permeability, and manufacturing issues)
- Study design considerations for animal model experiments
- Biologics for challenging CNS targets
- Applying for an Investigational New Drug (IND)
- Commercialization strategies
- Best practices for working with tech transfer offices, managing intellectual property, and the role of funding organizations
- Funding & resources for preclinical therapeutics development for neurological disorders

TARGET AUDIENCE

This annual Alzheimer’s Drug Discovery Foundation (ADDF) 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 on their current research progress and stimulate discussion. This international conference offers ample opportunities for collaboration and partnering.

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

SCHOLARSHIPS
The Alzheimer’s Drug Discovery Foundation invites applications for the 2016 ADDF Young Investigator Scholarships. Review application details at the conference website.

SPONSORSHIP/EXHIBIT
The conference offers a variety of co-sponsorship and exhibition packages, customized to meet your company’s needs. Contact us today to secure your place at this year’s conference!

www.alzdiscovery.org

17th INTERNATIONAL CONFERENCE ON ALZHEIMER'S DRUG DISCOVERY

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

This annual Alzheimer’s Drug Discovery Foundation (ADDF) 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 on their current research progress and stimulate discussion. This international conference offers ample opportunities for collaboration and partnering.

TARGET AUDIENCE
The 17th edition of this conference attracted attendees from around the world which included:
- Academic and industry scientists engaged in drug discovery research for neurodegenerative disease or CNS
- Business development and licensing professionals
- Alliance management professionals
- Young investigators and graduate students

www.alzdiscovery.org
Funding for this conference was made possible, in part by Cooperative Agreement 1U13AG052268-01 from the 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.
Congratulations to all of the 2016 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.

OUTSTANDING YOUNG INVESTIGATOR AWARDS

Katherine Castor, PhD, HMRI
Nihar Kinarivala, PhD (cand.), Texas Tech University Health Science Center
Kevin Nash, PhD (cand.), The University of Toledo
Chaahat Singh, PhD (cand.), Michael Smith Laboratories
Nina Weishaupt, PhD, University of Western Ontario

YOUNG INVESTIGATOR AWARDS

Daniel Apicco, PhD (cand.), Boston University School of Medicine
Bryce Blankenfeld, PhD (cand.), University of Kansas
Audrey Branch, PhD, Johns Hopkins University
Izaskun Buendia Abaitua, PhD, Instituto Teófilo Hernando, Universidad Autónoma de Madrid
Dustin Chernick, BS, University of Minnesota
Priyanka Desirazu, BS, Johns Hopkins University School of Medicine
Serge Alain Fobofou, PhD (cand.), Leibniz Institute of Plant Biochemistry
Saurabh Gagangras, BS, University of Utah
Holly Hunsberger, MS, West Virginia University
Nan Jiang, PhD (cand.), Forschungszentrum Juelich
Apra Manral, PhD (cand.), Dr. B.R. Ambedkar Centre for Biomedical Research, University of Delhi
Poonam Meena, PhD, Dr. B. R. Ambedkar Centre for Biomedical Research
Patrycja Michalska, PhD (cand.), Universidad Autónoma de Madrid
Ronak Patel, Texas Tech, University Health Science Center
Bindu Raveendra, PhD, The Scripps Research Institute
Supriya Swarnkar, PhD, The Scripps Research Institute
Ella Zeldich, PhD, Boston University
Tamar Ziehm, PhD (cand.), Research Center Juelich

POSTER PRESENTERS

Dongming Cai MD, PhD, Icahn School of Medicine at Mount Sinai
Shaohua Xu, PhD, Florida Institute of Technology
CHAIRS AND SPEAKERS

BIOS AND ABSTRACTS
Howard Fillit, MD, Alzheimer’s Drug Discovery Foundation

Dr. Howard Fillit, 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.
**Global Collaboration: Essential to Accelerate the Identification, Validation and Development of New Targets and Treatments for Alzheimer’s Disease and other Neurodegenerative Disorders**

Luc Truyen

*Janssen Research & Development LLC., Titusville, NJ, USA*

Spurred on by

1) an ageing population driving significant increases in the prevalence of neurodegenerative disorders

2) increasing complexity of the science and

3) the poor track record of individual innovators’ efforts, the last decade has seen a significant increase in collaborative initiatives focused on better understanding the course of the disease(s) like ADNI and sharing of data like CAMD, etc.

After a further global call to action in 2013, today’s pre-competitive collaborations cover the entire spectrum from target identification, lead development, translational sciences, biomarker development to clinical trial frameworks. Utilizing a couple of examples, I will show how the paradigm of public–private partnerships is an essential component of getting answers for patients.
DAY 2: NIA Opportunities for Translational Research Funding

Lorenzo Refolo, PhD, National Institute on Aging

Dr. Lorenzo Refolo is the Program Director for Alzheimer’s Disease Drug Discovery and Development at the National Institute on Aging (NIA). He received his PhD in Molecular Genetics from the University of Medicine and Dentistry of New Jersey. He received postdoctoral training in the Department of Psychiatry at Mount Sinai School of Medicine and the Laboratory of Neurobiology at Rockefeller University.

Immediately before coming to the NIA, Dr. Refolo was a Program Director at the National Institute of Neurological Disorders and Stroke, managing a portfolio of basic, clinical, and translational research that was focused on neurodegenerative disorders. At the NIA, Dr. Refolo directs the Alzheimer’s Drug Development Program within the Dementias of Aging Branch. Dr. Refolo also has been the Scientific Director at the Institute for the Study of Aging and a Research Scientist at the Nathan Kline Institute for Psychiatric Research at New York University. He has held the position of Assistant Professor at the Mayo Clinic, Jacksonville, Florida, and at Mount Sinai School of Medicine, New York. His own research career focused for the most part on the molecular-cell biology of Alzheimer’s disease.

National Institute on Aging Alzheimer’s Disease Translational Research Program

Lorenzo Refolo

National Institute on Aging, Bethesda, MD, USA

The presentation will focus on:

- Overarching goals for the NIA Translational Research Program
- Active funding opportunities at NIA for Translational Research
- New NIA funded infrastructure for Translational Research.
DAY 2: NIA Opportunities for Translational Research Funding

Amir Tamiz, PhD, National Institute for Neurological Diseases and Stroke

Dr. Amir Tamiz is a Program Director at the National Institute of Neurological Disorders and Stroke (NINDS), Office of Translational Research (OTR). In this capacity Dr. Tamiz oversees NIH Blueprint Neurotherapeutics network (BPN) and Innovation Grants to Nurture Initial Translational Efforts (IGNITE).

Prior to joining NIH in 2012, Dr. Tamiz had held scientific and management positions in research and development of therapeutic programs at Corvas International (acquired by Dendreon), CovX (now part of Pfizer), and Alba Therapeutics.

Dr. Tamiz received his PhD at University of Oregon and conducted postdoctoral research at the Department of Neuroscience at Georgetown University Medical Center.

NINDS Opportunities for Translational Research Funding

Amir Tamiz

National Institute for Neurological Diseases and Stroke, Bethesda, MD, USA

The mission of the Office of Translational Research (OTR) within the National Institute of Neurological Disorders and Stroke (NINDS) is to accelerate the preclinical discovery and development of new therapeutic interventions for neurological disorders. The NINDS is part of the National Institutes of Health (NIH), the leading supporter of biomedical research in the world.

The presentation will cover funding opportunities at NINDS and provide examples for best practices for converting basic research discoveries into therapeutic modalities for treatment of neurological disorders.
SESSION I

Embarking on a Drug Discovery Campaign

Chair: Marcie Glicksman, PhD—Orig3n, Inc.

Session Overview
Marcie Glicksman, PhD—Orig3n, Inc.

Assembling the Right Interdisciplinary Team from the Beginning
Julie Frearson, PhD—Charles River Laboratories

Quantitative Systems Pharmacology for supporting Drug Discovery in Alzheimer’s Disease
Hugo Geerts, PhD—In Silico Biosciences

New Trends and Technology in Assay Development
Marcie Glicksman, PhD—Orig3n, Inc.
SESSION I: Embarking on a Drug Discovery Campaign

CHAIR

Marcie Glicksman, PhD, Orig3n, Inc.

Dr. Marcie Glicksman, is the Vice President, Biology at Orig3n, Inc.; a new biotech start-up company focused on stem cell models and regenerative medicine. Previously, Dr. Glicksman was co-Director of the Laboratory for Drug Discovery in Neurodegeneration (LDDN), which is focused on accelerating the identification of new therapeutics. Dr. Glicksman has been in the field of drug discovery for more than 20 years, thirteen years in the pharmaceutical industry and then the most recent ten years at LDDN. Previously, she was at the company, Descartes Therapeutics focused on pain therapeutics and Cubist focused on anti-infectives. Prior to these positions, she was at DuPont-Merck and at Cephalon, Inc.

She has led multiple advanced programs for neurodegenerative diseases including co-inventorship of CEP1347, a drug candidate directed at a kinase that has been in Phase III clinical trials. She has also been part of the team to prepare an IND for a drug for neuropathic pain that has just completed Phase II clinical trials.

She was elected (2005-2009) to the Board of Directors and served as Chairman of the Board for the Society for Biomolecular Sciences (now Society for Laboratory Automation and Screening). She is on the science advisory board for the Alzheimer’s Drug Discovery Foundation (ADDF) and the California Institute for Regenerative Medicine (CIRM), and reviews grants for NIH, Department of Defense, SBIR, the Michael J Fox, Alzheimer’s Association, the Canadian Cancer Society, and Rett Foundation.

Dr. Glicksman co-founded the Academic Drug Discovery Consortium with three colleagues as a way to build a collaborative network for the academic drug discovery community. Dr. Glicksman co-designed and developed an annual drug discovery course supported by NIH. She also regularly consults and this has included filing an Investigational New Drug application with the FDA, as well as projects involving the development of new technologies. Dr. Glicksman received a bachelor’s degree from Brown University and a PhD in Neuroscience from Washington University.

New Trends and Technology in Assay Development

Marcie Glicksman

Orig3n, Inc., Boston, MA, USA

Neurodegenerative diseases are challenging from a drug discovery perspective with no disease modifying agents available on the market. The lack of success is at least partly due to the poor disease models that have been available for a biologically complex system as the brain. Much of the success in drug discovery is dependent on assay development strategies, and assay development trends have moved towards more physiologically-based assays. This presentation will discuss choices that need to be made in selecting the best assay. For example, the pros and cons for target-based versus phenotypic-based assays, the role of patient-derived induced pluripotent stem cells and what other technologies are available for creating better disease models that can be used for drug discovery.
Quantitative Systems Pharmacology for Supporting Drug Discovery in Alzheimer’s Disease

Hugo Geerts

In Silico Biosciences, Lexington, MA, USA

While preclinical animal models in Alzheimer’s disease (AD) are essential to generate information about biological processes, they notoriously lack translation ability to the clinical situation. Possible reasons include the differential pharmacology of the clinical candidate for human vs rodent targets, different pharmacokinetics and metabolites, the absence of human genotypes and co-medications and the incomplete implementation of the neuropathology.

As a possible alternative, similar to other engineering industries with shorter cycle-times, we developed a mechanism-based computer platform of relevant and biophysically realistic neuronal circuits, based on formalized CNS domain expertise. This Quantitative Systems Pharmacology approach has shown value in pharmaceutical R&D by being able to predict blindly an unexpected clinical outcome for novel AD and schizophrenia drugs.

We will show how the platform can be applied to:

1. support current projects in beta-amyloid modulation
2. drug discovery on neuropsychiatric problems and
3. outline a new initiative around modeling tau pathology.

Such a platform can become a live dynamic knowledge repository integrating different information modalities for supporting the discovery and development of new drugs.
SESSION I: Embarking on a Drug Discovery Campaign

Julie Frearson, PhD, Charles River Laboratories

Dr. Julie Frearson studied signal transduction in vascular endothelial cells during her PhD at King’s College London. Her post-doctoral training was conducted at the Babraham Institute, Cambridge UK dissecting the role of tyrosine phosphatases in T-cell signalling. Dr. Frearson then joined Cambridge Drug Discovery where she led the cell signalling group and engaged in hit discovery programs across a range of enzyme classes. She became part of BioFocus in 2001 and after two years of leading a department engaging in hit discovery and hit-to-lead programs for kinases and ion channels, became Director of Biology. In this role she was responsible for both the scientific and commercial leadership of 30 staff engaging in molecular pharmacology and in vitro ADME across a range of early stage drug discovery projects.

In 2005 Dr. Frearson joined the University of Dundee (UK) to enable their translational drug discovery ambitions. As Professor of Molecular Pharmacology, Julie co-founded and raised >$30m to develop operations at The Drug Discovery Unit at Dundee. She directed translational drug discovery portfolios across a range of neglected diseases and for novel targets addressing oncology, rare genetic disease and stem cell fate. Julie is a strong advocate of early translational drug discovery from academic concepts and has served as SULSA Director of Translational Biology for Scotland and on MRC and Wellcome Trust Committees. Dr. Frearson is co-author of more than 60 peer-reviewed publications.

Following a move to the USA, Dr. Frearson joined BioFocus (now Charles River) in 2011 in a Scientific Alliances role. She now leads the Discovery group’s venture into new partnerships within all life sciences sectors pharma, biotech, academic institutions and patient foundations in USA & Asia Pacific.

Assembling the Right Interdisciplinary Team from the Beginning

Julie Frearson

Charles River Laboratories, Willmington, MA, USA

Logic dictates that the extreme challenges of drug discovery - designing and developing a single, simple organic molecule to be efficacious and safe across a patient population - will inevitably require the hearts and minds of a team effort. This session will be targeted at academic scientists who wish to pressure-test and translate their therapeutic hypothesis and will describe the ideal profile of the multitude of scientific disciplines involved from early target validation through to nomination of a candidate for non-clinical development. We will discuss the science, processes and cultural mix needed for a successful drug discovery effort and, critically, provide practical guidance on accessing the appropriate expertise.
SESSION II

Starting at the End: The Pharmacology—Chemistry Interface in Preclinical Drug Development

Chair: D. Martin Watterson, PhD—Northwestern University

Session Overview
D. Martin Watterson, PhD—Northwestern University

Fragment-based Drug Discovery
Daniel Erlanson, PhD—Carmot Therapeutics, Inc.

A Medicinal Chemistry Perspective on Picking the Right Screening Strategy
David Swinney, PhD—Institute for Rare and Neglected Diseases Drug Discovery (iRND3)

Pharmacology Driven Optimization in Candidate Development
D. Martin Watterson, PhD—Northwestern University
Session II: Starting at the End: The Pharmacology—Chemistry Interface in Preclinical Drug Development

Chair

D. Martin Watterson, PhD, Northwestern University

Dr. Daniel Martin Watterson serves in an advisory role to pharmaceutical and biotechnology companies in the areas of process and risk analysis. In addition to industry consulting, Dr. Watterson serves on advisory boards for small business start-ups, biotechnology companies, and non-profit organizations in the area of CNS drug discovery and development. His personal CNS drug development experience includes the discovery and preclinical development of novel small molecule therapeutic candidates that attenuate disease related to synaptic dysfunction, as well as participation in development of protein replacement therapeutics.

Dr. Watterson’s academic affiliation is the G.D. Searle Endowed Chair Professorship at Northwestern University, where he is also Professor of Pharmacology in the Feinberg School of Medicine. Previous relevant activities at Northwestern include the founding of an academic drug discovery research and training program characterized by the generation of multiple CNS drug candidates taken into preclinical and clinical development through the leveraged use of Foundation and NIH funding. He also served in various administrative positions, including Department Chair, University Center Director, and Curriculum Co-Director. Prior to Northwestern, he 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 an Investigator in the Howard Hughes Medical Institute.

Dr. Watterson’s academic research has focused on elucidation of the molecular basis of calcium signal transduction in CNS function and pathway responses to injury or disease. Examples include:

- co-discovery of calmodulin using an early proteomic approach to brain signal transduction,
- development of calmodulin bio-affinity methods for signaling partner identification,
- pioneering identification of a physiologically relevant calmodulin: enzyme recognition site and its role as a molecular mechanism for signaling cross-talk,
- elucidation of the relief-of-autoinhibition mechanism for calmodulin activation of protein kinases, and
- the development of unique genetic knock-out mouse strains for calmodulin-mediated protein kinases being used to identify new drug discovery targets related to stress and disease susceptibility.
Daniel Erlanson, PhD, Carmot Therapeutics, Inc.

Dr. Daniel Erlanson is the co-founder and president of Carmot Therapeutics, Inc. (www.carmot.us). Using a proprietary technology called Chemotype Evolution, Carmot is addressing unmet chemical needs in drug discovery to tackle challenging therapeutic targets.

Prior to Carmot, Dr. Erlanson spent a decade developing technologies and leading medicinal chemistry efforts in oncology and in metabolic and inflammatory diseases at Sunesis Pharmaceuticals, which he joined at the company’s inception.

Before Sunesis, he was an NIH postdoctoral fellow with James A. Wells at Genentech. Dr. Erlanson earned his PhD in chemistry from Harvard University in the laboratory of Gregory L. Verdine and his BA in chemistry from Carleton College.

As well as co-editing two books on fragment-based drug discovery, Dr. Erlanson is an inventor or author on more than forty patents and scientific publications. He is also editor of a blog devoted to fragment-based drug discovery, Practical Fragments (http://practicalfragments.blogspot.com/).

Fragment-Based Drug Discovery

Daniel Erlanson

Carmot Therapeutics, Inc., San Francisco, CA, USA

Faced with the need to deliver drugs and tool compounds against increasingly difficult targets, many academics and companies have turned to fragment-based drug discovery as an alternative to traditional high-throughput screening. Rather than screening millions of drug-sized compounds, fragment-based drug discovery starts with libraries of just a few thousand very small molecules, or fragments. This enables a more thorough exploration of chemical space to find better starting points for lead optimization. It also allows greater attention to physicochemical properties, which are particularly important for drugs targeting the central nervous system.
Dr. David Swinney has over 25 years of industrial drug discovery experience (Roche, Syntex, iRND3) working to identify promising leads, clinical candidates and effective mechanisms of drug action that address unmet medical needs. He has a PhD in medicinal chemistry from the University of Washington, Seattle, and expertise in drug discovery, drug discovery strategies, assay development and screening, pharmacology, enzymology and binding kinetics.

Dr. Swinney is currently at the non-profit Institute for Rare and Neglected Diseases Drug Discovery (iRND3) in Mountain View, CA, (www.irnd3.org) working to apply new knowledge of successful drug discovery to rare and neglected diseases. iRND3 has programs for neglect infectious diseases and cancers. They also provide fee for service work to help understand molecular mechanisms of action of drug candidates.

A Medicinal Chemistry Perspective on Picking the Right Screening Strategy

David Swinney

Institute for Rare and Neglected Diseases Drug Discovery (iRND3), Mountain View, CA, USA

The goal of drug discovery is to identify medicines that can benefit a patient at a safe dose. Two drug discovery strategies to address this are:

1) target-based drug discovery (TDD) and
2) phenotypic drug discovery (PDD).

These strategies differ in how they identify molecular mechanisms of action (MMOAs) that provide therapeutically useful efficacy and safety. These MMOAs can be considered ‘pharmacological hot spots’ that include the target and the molecular mechanism through which the target provides a safe, therapeutically useful response. The strategies differ in that PDD will empirically identify an MMOA, whereas with TDD target validation drives the strategy and MMOA is rarely considered. A strength of TDD is a rational approach to translate genetic information into clinical development and patient care, however its weakness is the inability to predict a priori an effective MMOA. PDD can help compensate for this weakness. Ultimately, the strengths and weaknesses of these two approaches are complementary. Drug discovery strategies that combine both TDD and PDD will have a greater chance for success.
SESSION III

Drug Discovery: From Lead to Clinical Candidate

Chair: Edward Spack, PhD—Vector Preclinical Consulting

Session Overview
Edward Spack, PhD—Vector Preclinical Consulting

PK/PD in Preclinical Development Practices
Barry Greenberg, PhD—University Health Network

Overcoming Challenges in Formulation and Delivery
Paul Skultety, PhD—Xcelience

Requirement for an IND
Edward Spack, PhD—Vector Preclinical Consulting

How to Work Best with the FDA Throughout the Drug Development Process
Stephen Arneric, PhD—Critical Path Institute

ADDF ACCESS & Young Investigator Scholarship Awards Presentation
Lauren Friedman, PhD—Alzheimer’s Drug Discovery Foundation
SESSION III: Drug Discovery — From Lead to Clinical Candidate

CHAIR

Edward Spack, PhD, Vector Preclinical Consulting

Dr. Edward Spack received his doctoral degree from The Johns Hopkins University and his postdoctoral fellowship in cellular immunology at Stanford University. He worked in San Francisco Bay area biotech companies and private research institutes for over 20 years developing therapies for neurodegenerative diseases, as well as for cancer and infectious diseases, focusing on the transition from discovery to clinical trial.

His translational experience includes leading preclinical development of drug candidates for multiple sclerosis, nosocomial infection, and botulism poisoning. At SRI International, Dr. Spack directed the PharmaSTART program, a consortium of SRI, Stanford, UC Berkeley, UC San Diego, and UC San Francisco chartered to support translational development of academic projects. He consulted with the NIH translational core services committee and several NIH institutes on preclinical development and serves on several study sections, including the NIA Alzheimer’s Disease Drug Development review panel and the NIH/CSR Drug Discovery for the Nervous System review panel.

Dr. Spack is currently a consultant on preclinical development for U.S. and international entrepreneurs in academia and start-up biotech companies, including the BayBio FAST program and SRI International Innovation workshops.

Requirements for an IND

Edward Spack

Vector Preclinical Consulting, San Francisco, CA, USA

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) that lay the groundwork for IND-enabling studies; 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. Preparing for an IND is not simply a matter of following a recipe or coloring within the lines- there are strategic considerations that should be part of the earliest planning for a drug candidate. A Target Product Profile (TPP) provides a good guideline for drug optimization and testing. As part of this early stage, in addition to proof of concept, the TPP disciplines drug developers to consider proof of relevance, focusing on clinical need and competing alternative approaches. Coordinating the safety testing and manufacturing, as well as the models that inform dosing and other aspects of clinical trial design requires careful project management. In summary, developing and executing IND-enabling studies and moving a discovery to clinical trial requires problem solving and teamwork, and the most important early consideration is to begin with the end in mind.
SESSION III: Drug Discovery — From Lead to Clinical Candidate

Barry Greenberg, PhD, University Health Network

Dr. 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 as Director, Neuroscience Drug Discovery and Development, 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, Dr. Greenberg is also Strategy Director of the Toronto Dementia Research Alliance, 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. Dr. Greenberg was a member of the committee charged with drafting the scientific strategic recommendations to inform the National Alzheimer Plan in the US in 2012, and was Chair of the Recommendations Writing Committee for the follow-up Alzheimer Research Summit at the NIA in February 2015. He has authored or co-authored 70 articles in peer-reviewed journals and 19 book chapters and reviews.

PK/PD in Preclinical Development

Barry Greenberg

University Health Network, Toronto, Ontario, Canada

In preclinical drug development, it is critical to ascertain that an exploratory therapeutic compound reaches its molecular target, or its target tissue in the case of phenotypic screens, at appropriate concentrations and with sufficient longevity to mediate its intended biological effect. In vitro experiments are static with respect to target and compound, while in vivo experiments are dynamic, with time-dependent impacts on compound concentration, distribution, and target disposition. High potency alone in prior in vitro assays is insufficient and often irrelevant. Certain attributes should be ascertained in advance of exposing experimental animals to exploratory compounds, such as physicochemical properties suitable for CNS exposure, and stability in microsomal assays. Subsequent in vivo studies must include pharmacokinetic (PK) and pharmacodynamic (PD) assessments to determine whether the compound exposure is sufficient to interact with its target or organ of interest. This is critical information in guiding administration routes and dosing to deliver informative outcomes. Depending on whether a study is exploratory for target engagement or therapeutic for outcomes analyses, the degree to which absorption, distribution, metabolism, excretion, and toxicity (ADMET) are profiled should be prospectively considered as part of the study design. Toxicity considerations are particularly critical in this context to minimize potential off-target phenotypic impacts on outcome measures. Moreover, in vivo studies should be conducted at doses well below those at which adverse events occur, not merely “just below” a toxic exposure level, as slightly sub-toxic systemic doses may also have an impact on phenotypic outcomes. Therapeutic studies should also include considerations of distribution/exposure of both the parent compound and its metabolites, some of which may have unanticipated activities that impact on outcomes. In summary, PK, PD and ADMET are important to consider in the strategic design of in vivo experiments for translational research, as all human clinical studies are in vivo experiments.
Dr. Paul Skultety joined the management team at Xcelience in 2008, bringing extensive experience in contract development and innovator companies. His broad drug development expertise and successful track record of developing new chemical entities from pre-IND to commercialization have made him an industry leader in the field of drug development and clinical solutions. Dr. Skultety has nine U.S. formulation and composition patents and is author of numerous publications.

Overcoming Challenges in Formulation and Delivery

Paul Skultety

Xcelience, Tampa Bay, FL, USA

Now more than ever, pharmaceutical companies are struggling to do more with less. Demands for accelerated timelines and financial pressures drive pharmaceutical companies to achieve critical milestones faster in spite of challenging properties associated with their candidates. Often this is done with limited quantities of active pharmaceutical ingredient (API). There are, however, a number of formulation development options to consider that enable speed to first-in-human studies while conserving API and other resources. Each option has relative advantages and disadvantages. Some approaches eliminate the need for excipients and therefore enable companies to bypass formulation development. These approaches allow rapid progression into human clinical trials and are valid options for companies with limited API. With a defined program, API into Capsule projects can, on average, be completed 45 percent faster than traditional formulation efforts, and in some specific cases can save as much as 17 weeks in the development timeline. Finally, some companies still prefer traditional formulation development of solid oral dosage forms, even for Phase I studies. This approach may be initiated for more challenging formulations with poorly water soluble compounds or for formulations that require use of a controlled-release technology. Although not all options are capable of reaching commercial scale, all are capable of supporting early clinical studies where speed and cost-effectiveness are the most important.
SESSION III: Drug Discovery — From Lead to Clinical Candidate

Stephen Arneric, PhD, Critical Path Institute

Dr. Stephen Arneric joined the Critical Path Institute in June 2015, and is Executive Director of the Coalition Against Major Diseases (CAMD), a consortium focused on developing Drug Development Tools for advancing innovative treatments of Alzheimer's disease and related dementias.

Previously he was VP Research/ Preclinical Development at Neuromed Pharmaceuticals, Chief Scientific Officer of the Pain/Migraine Drug Hunting Team at Lilly, and held senior management positions at Pfizer, Pharmacia, DuPont Pharmaceuticals, and Abbott. He has extensive leadership and scientific expertise in the areas of neurology, pain, psychiatry and urology, and over the last 25 years his teams have delivered more than 30+ drug candidates into clinical development.

Dr. Arneric has also had late stage product experience with Mirapex™, Exalgo™, Lyrica™, Cymbalta™ and Detrol™. As a medical educator he has experience teaching medical students, graduate students, and neuroscience medical liaisons. Currently he is Adjunct Professor of Pharmacology, SIU School of Medicine, and Research Professor of Medicine, University of Arizona. Dr. Arneric earned his Bachelor of Science degree in Physical Sciences (Lyman Briggs College, Michigan State University), his PhD in Pharmacology (University of Iowa) and Post-doctoral training at Cornell Medical College (New York Hospital). He is an accomplished author with 145 peer-reviewed articles, 190 abstracts, 17 chapters, one book, numerous IND submissions, as well as a co-inventor of 15 patents. He is also President of Horizons Pharma Consulting, LLC.

How to Work Best with the FDA Throughout the Drug Development Process

Stephen Arneric

Critical Path Institute, Tucson, AZ, USA

Many scientists incorrectly view regulatory agencies as static, bureaucratic organizations that impede the drug discovery process. This presentation highlights the alternative perspective that regulatory agencies such as the FDA are working to increase their partnership with various healthcare stakeholders to accelerate the advancement of innovative Drug Development Tools, and the supporting regulatory science required to do so, that will enable more rapid access of novel therapeutics to the patients in need. Specific examples will be given how public-private partnerships such as the Coalition Against Major Disease (CAMD) at the Critical Path Institute have done so for the Alzheimer and Parkinson Disease community, as well as outline future plans to expand this process through the sharing and standardization of data. Scientifically-based consensus on the standardized way to record, structure and report data will:

1) enable the integration of various data sources to quantify the predictive accuracy, utility and reliability assessments across clinical trials;

2) enable the prospective collection of data in standardized format in both clinical trials and observational studies; and

3) expedite regulatory submissions to FDA, EMA and other regulatory authorities.
Lauren Friedman, PhD, Alzheimer's Drug Discovery Foundation

Dr. Lauren Friedman, is the Assistant Director of Scientific Programs at the Alzheimer's Drug Discovery Foundation (ADDF) where she supports the management of the ADDF's drug discovery portfolio by providing scientific and strategic review of preclinical drug discovery proposals and tracking program progress. Additionally, she 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 PhD in Neuroscience at the Icahn School of Medicine at Mount Sinai where she studied molecular mechanisms underlying the development and degeneration of brain circuits involved in autism and Parkinson’s disease.

Dr. Friedman received a BS in Biopsychology from Tufts University. She 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.
DAY 3: Welcome Notes

Diana Shineman, PhD, Alzheimer’s Drug Discovery Foundation

Dr. Diana Shineman, is the Director for Scientific Affairs at the Alzheimer’s Drug Discovery Foundation, where she develops and manages the Foundation’s drug discovery and development grant programs and strategic initiatives. Combining scientific and business expertise, the ADDF manages its research funding portfolio to balance risk, stage of development, and drug target mechanism of action, ensuring that grants meet key milestones before securing follow-on funding. As a measure of success, projects funded by the ADDF have gone on to garner nearly $2 billion in follow-on funding.

The ADDF also works strategically with foundations, government and industry partners to tackle unmet needs in the field. As an example of such an initiative, Dr. Shineman led an interdisciplinary effort to standardize animal model study design to improve research efficiency and translatability. She joined the ADDF in 2008.

Dr. Shineman earned a PhD in Cell and Molecular Biology from the University of Pennsylvania working in the Center for Neurodegenerative Disease Research led by Drs. Virginia Lee and John Trojanowski. She also worked as 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. In addition to maintaining various professional memberships, she has also authored numerous articles and peer reviewed publications.
DAY 3: Keynote Lecture

**Steven Paul, MD, Voyager Therapeutics, Inc.**

Dr. Steven M. Paul, is currently the President and CEO at Voyager Therapeutics, Inc. as well as Venture Partner at Third Rock Ventures. He was formerly the Founding Director of the Helen and Robert Appel Alzheimer’s Disease Research Institute and the Burton P. and Judith B. Resnick Distinguished Professor in Neurodegenerative Diseases as well as a DeWitt Senior Scholar and Professor of Neuroscience (Brain and Mind Research Institute), Psychiatry and Pharmacology at Weill Cornell Medical College. Dr. Paul was also formerly the Executive Vice President of Science and Technology and President of the Lilly Research Laboratories (LRL) of Eli Lilly and Company, overseeing the development of several of Lilly’s largest products including Zyprexa® and Cymbalta®. Prior to this, Dr. Paul served as Scientific Director of the National Institute of Mental Health (NIMH/NIH) in Bethesda, Maryland.

Dr. Paul is the recipient of many honors and scientific recognitions, including: The Distinguished Service Medal of the USPHS and the Chief Scientific Officer of the Year Award. In 1997, Dr. Paul was elected to membership in the National Academy of Medicine of the National Academy of Sciences and in 2004 Dr. Paul was elected a Fellow of the American Association for the Advancement of Science (AAAS).

Dr. Paul has authored or co-authored over 500 papers and invited book chapters and was listed as one of the most highly cited scientists in the world (top 50 in Neuroscience) (1980-2000) by the Institute for Scientific Information (I.S.I.) in Philadelphia. He holds 9 patents on inventions made both at NIH and Lilly. His current work has focused on the role of apoE in the pathogenesis of Alzheimer’s disease. He is also a co-inventor of solanezumab, a humanized anti-Aβ monoclonal antibody currently in late-stage clinical testing by Lilly. Dr. Paul is on the boards of several biopharmaceutical companies including Alnylam Pharmaceuticals, SAGE Therapeutics and formerly the Sigma Aldrich Company and is also a founder of Sage Therapeutics, Voyager Therapeutics, and Tal Medical.

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**Gene Therapy Strategies for Treating or Preventing Alzheimer’s Disease and Related Neurodegenerative Disorders**

Steven Paul

*Voyager Therapeutics, Inc., Cambridge, MA, USA*

We have explored two novel gene therapy strategies for potentially treating or preventing Alzheimer’s disease (AD) and related neurodegenerative disorders. The first involves the direct intracerebral delivery of the well-recognized AD protective allele / gene APOE2 using an adeno-associated viral (AAV) vector. In our earlier published work we observed marked reductions in brain amyloid B-peptide (Aβ) and amyloid burden following direct intracerebral administration of APOE2 to mutant APP transgenic mice using either a lentiviral or AAV vector. Given the advantages of AAV vectors for CNS gene therapy we have now completed a series of studies exploring various AAV capsids, routes of administration and vector doses to deliver APOE2 in two APP transgenic mouse models of AD-related CNS amyloidosis, including a triple transgenic mouse (APP,PS1 / TRE4 mice) where the development of brain Aβ / amyloid pathology is almost completely dependent on APOE4 expression. Our data demonstrates that a rather widespread reduction of brain Aβ / amyloid burden can be achieved in either model following a single injection of vector. However, the extent of the “therapeutic benefit” depends on the exact site of vector administration, vector dose and level of APOE2 expression as well as the extent of pre-existing Aβ / amyloid pathology. Importantly, our results demonstrate that AAV-mediated gene delivery of APOE2 rescues the detrimental effects of APOE4 (the most common and robust genetic risk factor for late-onset AD) on brain amyloid pathology and thus may represent a viable therapeutic approach for treating or preventing AD, especially if sufficient brain APOE2 levels can be achieved early in the course of disease. More recently, we have used AAV vectors to deliver anti-tau monoclonal antibodies directly to the brain. Passive immunization of mutant (P301S) tau transgenic mice with the phospho-tau-specific monoclonal antibody PHF1 has been shown by several laboratories to reduce tau pathology (including NFTs) when administered systemically (IV) every week for several months. Modest but significant (40-50%) reductions in tau pathology have been reported following passive immunization of P301S mice with PHF1. We have now used an AAVrh.10 vector to deliver both the heavy and light chains of PHF1 directly to the hippocampus of P301S mice. In contrast to traditional passive immunization with this same antibody, we observed a marked (~90%) reduction in hippocampal tau pathology including pathological phospho-tau species and NFTs. Vectored intracerebral immunization with anti-tau monoclonal antibodies may represent a viable therapeutic approach to treating or preventing tauopathies such as FTD or AD. My presentation will cover recent data on both gene therapy strategies highlighting both the opportunities and challenges they offer for therapeutic intervention.
SESSION IV

Strategies for Challenging CNS Targets—Case Study Examples

Chair: Kurt Brunden, PhD—University of Pennsylvania

Session Overview
Chair: Kurt Brunden, PhD—University of Pennsylvania

Microtubule Stabilizing Compounds for Neurodegenerative
Kurt Brunden, PhD—University of Pennsylvania

Development of Tau Immunotherapy: Unique Considerations for Biologics
Tim West, PhD—C2N Diagnostics

Targeting P2X7 to Block Neuroinflammation: HTS to Lead Discovery
Paolo Pevarello, PhD—Axxam SpA

A Novel CB2 Agonist: Preparing for an IND
Joseph Foss, MD—NeuroTherapia Inc. & Cleveland Clinic

Development and Regulatory Considerations for the Clinical Development of
AGB101 (low dose levetiracetam) for aMCI Due to AD
Sharon Rosenzweig-Lipson, PhD—AgeneBio
Dr. Kurt Brunden is Director of Drug Discovery and Research Professor in the Center for Neurodegenerative Disease Research (CNDR) at the University of Pennsylvania, where he oversees drug discovery programs in the areas of Alzheimer’s disease (AD), frontotemporal lobar degeneration and Parkinson’s disease. Prior to joining CNDR in 2007, Dr. Brunden was an executive in the biotechnology sector, where he served as VP of Research at Gliatech, Inc. and later as Sr. VP of Drug Discovery at Athersys, Inc. In these positions, he initiated and managed drug discovery programs in AD, cognitive enhancement, schizophrenia, inflammation, metabolic disease and cancer.

Prior to his time in industry, Dr. Brunden was an NIH-funded faculty member within the Biochemistry Department at the University of Mississippi Medical Center, with a research focus on the regulation of myelination. He obtained his BS degree from Western Michigan University, with dual majors of Biology and Health Chemistry, and his PhD in Biochemistry from Purdue University, with a post-doctoral fellowship at the Mayo Clinic.

Dr. Brunden has over 90 scientific publications, and multiple issued and pending U.S. and PCT patents.

**Microtubule Stabilizing Compounds for Neurodegenerative Diseases**

Kurt Brunden

*Center for Neurodegenerative Disease Research, Perelman School of Medicine, University of Pennsylvania, Philadelphia, PA, USA*

Alzheimer’s disease (AD) and a number of related neurodegenerative disorders, including frontotemporal lobar degeneration, progressive supranuclear palsy, corticobasal syndrome and Pick’s disease, are characterized by the accumulation within central nervous system neurons of inclusions comprised of hyperphosphorylated forms of the microtubule-associated protein, tau. Tau normally stabilizes axonal microtubules and helps regulate axonal transport, and the disengagement of hyperphosphorylated tau from microtubules in neurodegenerative tauopathies is believed to affect axonal transport, with consequent impairment of neuronal function. Accordingly, small molecule microtubule-stabilizing agents, such as those used in the treatment of cancer, might have utility in the treatment of neurodegenerative tauopathies. We have previously identified and characterized a number of small molecules that elicited microtubule stabilization in the brain, including the natural product epothilone D, which was shown to enhance microtubule density, increase axonal transport, and reduce neuronal death in transgenic mouse models of tauopathy. Epothilone D has since advanced to clinical testing in AD patients. We have recently identified a number of additional non-natural product microtubule-stabilizing compounds that readily enter the brain and enhance microtubule stabilization. A summary of our studies supporting microtubule-stabilization as a therapeutic strategy for tauopathies will be presented.
SESSION IV: Strategies for Challenging CNS Targets—Case Study Examples

Tim West, PhD, C2N Diagnostics

Dr. Tim West is the vice president of Research and Development at C2N Diagnostics, in Saint Louis, MO. He has been with the company since its inception in 2008 and has been responsible for oversight of multiple clinical studies using the stable isotope labeling kinetics platform for measuring the effects of drugs on amyloid beta metabolism in humans. He is the principal investigator on multiple grants from institutions such as the NIH, Alzheimer’s Drug Discovery Foundation, Alzheimer’s Association, and the Michael J Fox Foundation. Dr. West oversaw part of the humanization and development of the anti-tau antibody C2N-8E12.

Dr. West obtained his PhD in molecular cell biology and neuroscience from Washington University. He conducted his graduate thesis research and post-doctoral training in the laboratory of Dr. David Holtzman, one of C2N’s scientific founders. In those capacities, Dr. West led a group of scientists and technicians studying neonatal stroke and hypoxic-ischemic injury. During 2006 and 2007, he served as Staff Scientist and Assistant Director of Technology Development for the Hope Center for Neurological Disorders at the Washington University School of Medicine. Dr. West was a recipient of a Kauffman Fellowship for Bio-Entrepreneurship. He received his BSc Honors in Molecular Biology at University College London and performed a laboratory internship at the Ludwig Institute for Cancer Research.

Development of Tau Immunotherapy: Unique Considerations for Biologics

Tim West

C2N Diagnostics, St. Louis, MO, USA

C2N-8E12 is a humanized anti-tau antibody developed by C2N Diagnostics. Preclinical data showed that both central and peripheral administration the precursor for C2N-8E12 lower tau pathology. Based on this strong preclinical data, the decision was made to create a humanized clinical candidate. This talk will go through some of the early and late stage decisions that are faced by a small company embarking on this type of endeavor.
Targeting P2X7 to Block Neuroinflammation: HTS to Lead Discovery

Paolo Pevarello

Axxam SpA, Milan, Italy

Many neurodegenerative CNS diseases, such as Alzheimer's Disease, Parkinson's Disease, Multiple Sclerosis and Amyotrophic Lateral Sclerosis, are characterized by a neuroinflammatory component.

P2X7 is a primary target to counteract neuroinflammation and much efforts are currently directed at finding an efficient antagonist, able to permeate the Blood-Brain Barrier.

In recent times we have been running a project aimed at finding novel P2X7 antagonists for neurodegenerative diseases.

We will present our P2X7 project experience, from HTS to in vivo Pharmacokinetics studies, focusing on how to plan a CNS drug discovery project and flexibly adapt it to cope with available resources and emerging results.
A Novel CB2 Agonist: Preparing for an IND

Joseph Foss

NeuroTherapia, Cleveland, OH, USA

The Investigational New Drug (IND) application allows an investigator to begin clinical trials in the United States. The process is well described in FDA Guidance Documents and numerous publications. There are, however, hundreds of "real world" decisions that need to be made in the execution of an IND campaign. This talk will outline the approach one small virtual company is using to progress a novel compound through the IND process.

Learner Objectives:

1) Get an overview of the IND process

2) Review the functional areas required for the submission and how to utilize external resources for preparation of the IND

3) Understand how to strategically approach designing an IND enabling program.
Sharon Rosenzweig-Lipson, PhD, AgeneBio

Dr. Sharon Rosenzweig-Lipson, is President of IVS Pharma Consulting. In 2011, Dr. Rosenzweig-Lipson founded IVS Pharma Consulting to bring her expertise in screening strategies, in vivo models, translation and early clinical development strategy to the neuroscience scientific community in pharma, biotech and academia.

She has over 20 years’ experience developing compounds for psychiatric and neurologic indications in the pharmaceutical industry. She has successfully led teams from the earliest exploratory studies through to Phase II Proof of Concept Trials.

Prior to her current positions, Dr. Rosenzweig-Lipson held the roles of Head of Translational Neuroscience and In-Vivo Head of Psychiatry at Wyeth Research. Dr. Rosenzweig-Lipson received her BA in Biological Basis of Behavior from the University of Pennsylvania and her PhD in Behavioral Neuroscience from Harvard University.

Development and Regulatory Considerations for the Clinical Development of AGB101 (low dose levetiracetam) for aMCI Due to AD

Sharon Rosenzweig-Lipson

AgeneBio, Baltimore, MD, USA

AGB101 (220 mg; low dose levetiracetam) is poised to enter Phase III clinical development for the treatment of aMCI due to AD (HOPE4MCI) trial. AGB101 is hypothesized to slow progression of aMCI due to AD by restoring entorhinal/hippocampal network balance. During this phase of the disease, fMRI studies show hippocampal overactivity and entorhinal cortex under activity. As shown in a Phase II study, AGB101 restores this network balance by attenuating hippocampal overactivity and restoring entorhinal cortex activity. Hippocampal overactivity predicts progression on CDR-SB (primary endpoint) and on entorhinal cortex atrophy (secondary endpoint). By restoring network balance, AGB101 is hypothesized to improve cognitive function (CDR-SB) and reduce neuronal injury (EC atrophy).

Keppra (1000-3000 mg; high dose levetiracetam) has been shown to be safe and efficacious for the treatment of epilepsy since 1999. This talk will address the advantages and challenges associated with repositioning a low dose of a generic compound for a new therapeutic indication and will highlight key aspects of novel formulation development and regulatory planning (pIND, IND, EOP2) under a 505(b)(2) regulatory pathway.
SESSION V

Commercialization Strategies: Developing Science into Products

Chair: Frank Longo, MD, PhD—Stanford University and PharmatrophiX

Session Overview
Frank Longo, MD, PhD—Stanford University & PharmatrophiX

Intellectual Property Considerations for Early Stage Life Science Companies
Laurie McNamara, JD, PhD & Maya Skubatch, JD—Wilson Sonsini Goodrich & Rosati

Lessons Learned in Drug Development from an Academic and Small Biotech Perspective
Frank Longo, MD, PhD—Stanford University & PharmatrophiX

Strategies for Securing Private Investment
Melissa Krauth, MBA—Angel Investor
SESSION V: Commercialization Strategies: Developing Science into Products

CHAIR

Frank Longo, MD, PhD, Stanford University & PharmatrophiX

Dr. Frank Longo is Professor and Chairman, Department of Neurology and Neurological Sciences at Stanford University. He received his MD in 1981 and PhD in Neurosciences in 1983 from UC San Diego. He completed his neurology and fellowship training in the Department of Neurology at UC San Francisco where he was then recruited as an assistant professor and promoted to professor and vice chair. From 2001 to 2005 Dr. Longo was chair of the Department of Neurology at the University of North Carolina-Chapel Hill and since 2006 has served as Chair of the Department of Neurology and Neurological Sciences at Stanford.

With support from the Alzheimer’s Drug Discovery Foundation (ADDF), Alzheimer’s Association, and the NIH, he and his team have pioneered small molecule treatment strategies for Alzheimer’s and other neurodegenerative diseases. In 2005, while at UNC, he founded PharmatrophiX, a company focused on the commercial development of these therapies. A lead candidate compound for Alzheimer’s disease has completed phase 1 safety trials and is slated for a phase 2a trial in Alzheimer’s patients.

In 2015, Dr. Longo was the recipient of the inaugural Melvin R. Goodes Prize for Excellence in Alzheimer’s Drug Discovery from the ADDF in recognition of his work creating Alzheimer’s disease therapies.

Lessons Learned in Drug Development from an Academic and Small Biotech Perspective

Frank Longo

PharmatrophiX, Menlo Park, CA, USA

Important drug candidates with significant novel clinical potential continue to emerge from translational programs in universities as well as the continuous emergence of small biotech companies. In both settings, there are key important lessons to learn in terms of reaching value inflection points, avoiding common pitfalls and obtaining funding. We will review the rationale, challenges and alternatives for academic-based faculty spinning a biotechnology company out of an academic program. Key areas of focus will include the following: approaches for creating technology elements that pharma partners seek (including quality of IP, target rationale/validation/engagement, quality and translational value of preclinical work, availability of relevant biomarkers, clinical trial plans and/or data); the basics of starting a company; following university and conflict-of-interest policies; options for funding; elements of the virtual company model; working with CROs; large pharma partnership models and goals; and exit strategy options.
SESSION V: Commercialization Strategies: Developing Science into Products

Laurie McNamara, JD, PhD, Wilson Sonsini Goodrich & Rosati

Dr. Laurie McNamara is a registered patent attorney, and is an associate in the patents and innovations counseling group at Wilson Sonsini Goodrich & Rosati. She focuses her practice on various aspects of intellectual property law for clients in the pharmaceutical and biotechnology industries. She has provided strategic counseling for clients related to patent prosecution, licensing, diligence, freedom to operate, patentability, invalidity and non-infringement analyses. She has managed global patent dockets and has knowledge of United States and international patent laws.

Dr. McNamara holds a PhD in biochemistry and drug discovery from Northwestern University in which her doctoral research focused on the structural biology of protein kinases involved in neurodegenerative diseases. During graduate school, she gained research experience in protein purification, enzymology, x-ray crystallography, structure-assisted inhibitor design and medicinal chemistry. Before graduate school, she worked at Merck Research Laboratories in Rahway, NJ, in the drug metabolism and basic chemistry departments.

Maya Skubatch, JD, Wilson Sonsini Goodrich & Rosati

Dr. Maya Skubatch is a partner at Wilson Sonsini Goodrich & Rosati, where she focuses on patents and innovations counseling. Her practice covers patent prosecution, strategic patent counseling, investor- and company-side due diligence, and license agreements for clients in the life sciences and clean technology industries.

Prior to joining Wilson Sonsini Goodrich & Rosati, Maya was an associate in the intellectual property litigation practice at Gray Cary Ware & Freidenrich LLP (now DLA Piper) in Palo Alto. While at UC Berkeley, Dr. Skubatch participated in research in the laboratory of Nobel Laureate Professor Randy Scheckman in the Department of Molecular and Cell Biology, where she studied Golgi protein transport in yeast cells. She also participated in research in the laboratory of Professor Sung-Hou Kim in the Department of Chemistry, where she studied various zinc-finger domains. After completing her BA, Dr. Skubatch was a researcher at Genentech, where she helped clone and characterize APO2 ligand, a player in apoptosis signaling.

Intellectual Property Considerations for Early Stage Life Science Companies

Laurie McNamara & Maya Skubatch

Wilson Sonsini Goodrich & Rosati, Palo Alto, CA, USA

This presentation will cover best practices for starting a life sciences company and for working with technology transfer offices at universities. The presentation will also cover a discussion of working with venture capitalists, and tips for developing an intellectual property portfolio that is attractive to investors. A review of financing rounds from seed/early stage financing to Series B and beyond will be discussed. Intellectual property risk analysis at each round of financing will be covered, including such topics as IP ownership, freedom to operate, scope of protection, and defensibility of patents.
Melissa Krauth, MBA, Angel Investor

Melissa Krauth is an experienced biotech investor, entrepreneur, and executive. Over the past several years, she has served as the head of life science investments at 2M Companies, a family office and investment company, and as Interim CEO of Odin Biotech, a 2M portfolio company. Prior to that, she spent eight years as a senior executive with Reata Pharmaceuticals, where she oversaw company and product-level strategies and operations. During her tenure at Reata, the company grew from a start-up employing a highly virtual drug development model to a mature, 200-employee organization with a multi-billion dollar valuation.

Ms. Krauth also spent a number of years as a strategy consultant to executives at top pharmaceutical and biotech companies. Currently, Ms. Krauth serves as the Chair of the Board of the non-profit trade association bionorthTX and is a member of the board of the non-profit incubator TECH Fort Worth, and she has previously served on the boards of several private biotech companies. She is an honors graduate from Rice University with a BA in biochemistry, and received an MBA in health care management and strategic management from the Wharton School of the University of Pennsylvania.

Strategies for Securing Private Investment

Melissa Krauth

The past few years have been good ones for financing of life science inventions, with traditional life science venture capital funds raising large amounts of capital and delivering exceptional returns to their investors due to the hot IPO and M&A markets. Immuno-oncology remains the most visible area of investment, with disproportionate share or mind and capital devoted to this exciting but increasingly crowded area. In contrast, funding for neurodegenerative diseases has lagged behind in investment despite the enormous and well-understood unmet medical need. Investors and potential partners typically cite the historically low probability of success, difficulty in translating preclinical to clinical data, and long time and great expense of clinical testing in these diseases as reasons for lower investment in the sector. Despite these challenges, there are positive signs for the future of private investment in new approaches to treat devastating neurodegenerative conditions. Several early stage companies in the neurodegenerative space have closed crucial financings in the past year. Additionally, the initial market reaction to positive emerging data from Biogen’s Phase 1 trial of aducanumab was a reminder of the huge value associated with a successful program in this space. Companies seeking funding for novel neurodegenerative programs will have the greatest success by finding ways to reduce risks and/or cost along the path to achieving meaningful human proof of concept data. For example, selecting a lead indication where the hypothesis can be tested at a reasonable cost and time can provide a more investable path than tackling Alzheimer’s as the starting point. Companies should also cast a broad net in their financing strategies by aggressively seeking non-dilutive grant money and reaching out to family offices and other non-traditional capital sources as well as traditional venture investors.
National Institute on Aging
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Taub Institute for Research on Alzheimer’s Disease and the Aging Brain is the nucleus of a dynamic, multidisciplinary endeavor. The Institute brings together Columbia University researchers and clinicians to uncover the causes of Alzheimer’s, Parkinson’s and other age-related brain diseases and to discover ways to prevent and cure these diseases.

The Harrington Project’s ambitious scope and mission are designed to address the gaps and failures that have arisen as economic and regulatory environments have changed. Despite breathtaking advances in basic science, the rate of clinical introduction of new therapeutics has declined. The Harrington Project’s three components offer financial and experiential resources at critical points in the drug development pathway and thus offer physician-scientists, a critical resource to realize their goals and mission.