National Institute on Aging Workshop:
Expanding the Therapeutic Modalities for AD/ADRD
Virtual Live Event: September 21-22, 2020

Registration link for Day 1, CLICK HERE
Registration link for DAY 2, CLICK HERE

EXECUTIVE SUMMARY: To improve, diversify and reinvigorate the Alzheimer's disease (AD) and Alzheimer's disease related disorders (ADRD) drug development pipeline the NIA has spearheaded several innovative programs such as Accelerating Medicines Partnership-Alzheimer's Disease (AMP-AD) aimed at identifying the next generation of therapeutic targets. These target discovery programs have identified and made publicly available more than 500 novel candidate targets (to view the list of targets and supporting evidence see the Agora web platform). Detailed assessment of these nascent targets using a standard biopharma target tractability evaluation has revealed that a significant number of them have low small-molecule druggability. Therefore, an expanded tool-kit of therapeutic modalities will be required to integrate many of the next generation targets into drug discovery campaigns. This expanded tool kit includes traditional biotherapeutic modalities such as gene and immunotherapies and other modalities such as genome editing, gene silencing, human derived stem cells and PROTAC.

To explore this opportunity the NIA is convening a workshop on the currently available biologic approaches and their use in drug development for AD/ADRD and other neurodegenerative disorders. The workshop brings together representatives from academia, biotech and pharmaceutical industries, NIH and the FDA to initiate an interactive framework to promote the development of innovative biologic therapies and to accelerate their delivery to AD/ADRD patients.

Workshop Goals: The overarching goals of this workshop are two-fold: (1) to examine how an expanding tool-kit of novel therapeutic modalities, fueled by innovation in molecular engineering and delivery systems, can be used to advance the next generation of AD therapeutic targets into preclinical development campaigns and eventually deliver new therapeutic agents that will enable a precision medicine approach to the treatment of AD/ADRD; (2) to discuss challenges to the use of these innovative therapies such as unexpected technical, safety, rigor/reproducibility and regulatory issues which will require significant efforts to understand and monitor.

WORKSHOP AGENDA

Day 1: 10:00am – 4:15pm EDT

10:00am – 10:15am
Introduction: National Institute on Aging (NIA) AD Translational Research Program
Lorenzo Refolo and Suzana Petanceska, NIA

10:15am – 11:00am
Plenary Talk (30min presentation time and 15min Q&A)

- David Collier, Eli Lilly
  - New Therapeutic Modalities as Tools to Unlock the Potential for Drug Development from Novel Disease Biology
  - Q&A

11:00am – 1:00pm EDT
Session One: Gene Therapy (gene replacement/addition, editing)

11:00am – 11:20am
- Kelly Bales, Voyager Therapeutics
  - Expanding the Therapeutic Modalities for AD/ADRD

11:20am – 11:40am
- Robert Bell, Pfizer
  - Gene Therapy for Rare Neurological Disorders and Potential Insight into the Treatment of More Complex Neurogenerative Diseases

11:40am – 12:00pm
- Mark Tuszynski, University of California San Diego
  - Growth Factor Gene Therapy for Alzheimer's Disease

12:00pm – 12:20pm
- Ronald Crystal, Weill Cornell Medicine
  - APOE2 Gene Therapy for APOE4 Homozygotes

12:20pm – 1:00pm
- Q&A

1:00pm – 1:15pm Break

1:15pm – 2:15pm EDT
Session Two: Antisense Oligonucleotides

1:15pm – 1:35pm

- Timothy Miller, Washington University in St. Louis
    - RNA-Targeted Therapeutics for Neurodegenerative Diseases

1:35pm – 1:55pm

- Holly Kordasiewicz, Ionis Pharmaceuticals
    - Antisense oligonucleotides for Neurodegenerative diseases: Lessons from Bench to Clinic

1:55pm – 2:15pm

- Q&A

2:15pm – 4:15pm EDT
Session Three: Cell-based Therapy, PROTAC and Other New Therapeutic Modalities

2:15pm – 2:35pm

- Eva Lucille Feldman, University of Michigan
    - Neural Stem Cell Transplantation: A Novel Cellular Therapy for Alzheimer's Disease

2:35pm – 2:55pm

- Cheryl Arrowsmith, Structural Genomics Consortium, University of Toronto
    - Open Science Tools for Proximity-Mediated Pharmacology

2:55pm – 3:25pm

- Jorge Palop and Cory Nicholas, University of California, San Francisco
    - Interneuron Transplants to Improve Brain Network Functions in Alzheimer's and Epilepsy

3:25pm – 3:45pm

- Claes Wahlestedt, University of Miami
    - Transcriptome Informed Therapeutics for Alzheimer's Disease

3:45pm – 4:15pm

- Q&A

Day 2: 10:00am – 1:15pm EDT

10:00am – 12:00pm EDT
Session Four: TREAT-AD Centers – from Open Science to Open Drug Discovery

10:00am – 10:45am (30min presentation time plus 15min Q&A)

- Alan Palkowitz, Indiana University
  - IUSM-Purdue TREAT-AD Center
  Perspective on Expanding Therapeutic Diversity for Alzheimer's
  - Q&A

10:45am – 11:30am (30min presentation time plus 15min Q&A)

- Opher Gileadi, Oxford University and Lara Mangravite, Sage Bionetworks
  - Emory-Sage-SGC TREAT-AD Center
  Diversifying the AD Target Landscape: Enabling Tools for Unconventional Targets
  - Q&A

11:30am – 12:00pm (20min presentation time plus 10min Q&A)

- Aled Edwards, Structural Genomics Consortium, University of Toronto
  - Open Drug Discovery – A Path to Generate New, and Affordable, Medicines for AD
  - Q&A

12:00pm – 1:00pm EDT
Session Five: Regulatory Considerations in Preclinical and Clinical Drug Development for New Therapeutic Modalities

12:00pm – 12:15pm

- Theresa Chen, CBER/FDA
  - Preclinical Considerations for Cell and Gene Therapy Products for Alzheimer's and Related Diseases

12:15pm – 12:30pm

- Mike Singer, CBER/FDA
  - Clinical Considerations for Cell and Gene Therapy Products for Alzheimer's and Related Diseases

12:30pm – 1:00pm

- Q&A

1:00pm-1:15pm: Wrap up and Adjourn