



Christopher P. Austin, MD
Director
National Center for Advancing Translational Sciences
National Institutes of Health
6701 Democracy Boulevard
Bethesda, Maryland 20892

December 17, 2015

Re: Request for Information: Soliciting Input for the National Center for Advancing Translational Sciences Strategic Planning Process

Dear Dr. Austin,

The Alzheimer's Association appreciates the opportunity to respond to the National Center for Advancing Translational Sciences (NCATS) Strategic Planning Process. As the largest non-profit funder of Alzheimer's research, the Association is committed to accelerating the progress of new treatments, preventions, and, ultimately, a cure for the disease.

Today, there are more than 5 million Americans living with Alzheimer's disease. It remains the sixth leading cause of death in the United States, and the only cause of death among the top 10 without a way to prevent, cure, or even slow its progression.¹ Given the depth and breadth of the disease, the Alzheimer's Association encourages NCATS to consider the following in order to speed the delivery of new treatments to patients and realize its mission.

1. NCATS should pursue work on translatable animal models.

Animal models for Alzheimer's disease that are predictive of clinical efficacy have, so far, eluded researchers because the underlying biology is not well understood. Researchers need a greater understanding of the basic biology behind the causes of human Alzheimer's disease in order to develop predictive models. NCATS should advance this field by exploring new models that expand the current understanding of the disease as whole and not just specific pathologies. NCATS should also add scientific rigor to the evaluation of animal models by improving standards and experimental design in order to improve reproducibility and quality.

2. NCATS should support research on Alzheimer's disease biomarker qualifications.

The standards for qualifying biomarkers are established by the Food and Drug Administration (FDA) and, to date, FDA has not qualified any Alzheimer's disease biomarkers. Just one qualified biomarker will provide a reference standard for new biomarkers, thus facilitating translational research, clinical trials, and the development of accurate diagnostic tools and additional biomarkers. NCATS should contribute to this important research.

3. NCATS should continue to pursue innovative drug discovery strategies beyond high-throughput screening.

As noted above, Alzheimer's disease is a complex disease currently without translatable animal models. Thus, in addition to high-throughput screening approaches, NCATS should utilize and develop additional drug discovery approaches for hit / lead finding, such as bio- and chemoinformatics, to identify new

¹ Alzheimer's Association. (2015). *2015 Alzheimer's Disease Facts and Figures*.

molecular entities for Alzheimer's disease. Such compounds can be used both to help validate new targets and provide leads for new drug candidates.

Thank you for the opportunity to comment. The Alzheimer's Association would welcome the opportunity to serve as a resource to NCATS as it develops its strategic plan and works to deliver new treatments and cures to patients. Please contact Laura Thornhill, Manager of Regulatory Affairs, at 202-638-7042 or lthornhill@alz.org if you have questions or if we can be of additional assistance.

Sincerely,

A handwritten signature in black ink, appearing to read 'R. Egge', with a long horizontal flourish extending to the right.

Robert Egge
Executive Vice President, Government Affairs