# ALS Therapy Development Institute and Gladstone Institutes Collaborate to Discover Potential ALS Treatments

*Institutes to Share Resources to Identify Potential Compounds Targeting TDP-43 Form of ALS*

CAMBRIDGE, MA and SAN FRANCISCO, CA—December 4, 2012— The ALS Therapy Development Institute (ALS TDI) and the Gladstone Institutes today announced the formation of a research collaboration to speed the discovery of potential treatments for ALS through the preclinical drug development process.

"We are thrilled about the potential this collaboration holds to accelerate ALS therapeutic development," said Steve Perrin, PhD, CEO and Chief Scientific Officer at ALS TDI. "Both our organizations have unique infrastructures, and by linking them this way, we may be able to advance potential treatments faster than before."

Amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease, is a neurodegenerative disease that leads to paralysis—and eventually death—due to the loss of motor neurons in the spinal cord and brain. About 30,000 people in the United States live with the disease at any given time, and the global population of ALS patients is approximately 400,000. Approximately 5,000 new cases of ALS are diagnosed in the United States each year, and there is no known cause, cure or treatment to halt or reverse the disease. The average patient survives only two to five years following their diagnosis.

Under this new agreement, which starts immediately, Gladstone will evaluate potential pharmaceutical compounds using a human model of ALS. Gladstone generated the model by transforming skin cells from ALS patients into stem cells, known as induced pluripotent stem cells (iPS cells), and then programming them into neurons. The technique builds on a discovery for which Shinya Yamanaka, MD, PhD, a Gladstone senior investigator, won the 2012 Nobel Prize in Physiology or Medicine.

This particular iPS-based ALS model includes a gene mutation that produces TDP-43, a protein commonly found in most forms of ALS. Promising drug compounds that pass the initial evaluation process at Gladstone will be fast-tracked for pre-clinical testing at ALS TDI, which will assess the compounds for activity and efficacy in various mouse models of human neurodegeneration.

"We hope our human model of ALS will help us to move quickly and effectively to identify promising therapeutic candidates for ALS," said Gladstone Senior Investigator Steve Finkbeiner, MD, PhD, who is also a professor of neurology and physiology at the University of California, San Francisco, with which Gladstone is affiliated. "The strong evidence that abnormal TDP-43 protein is involved in the development of ALS, coupled with models that may replicate ALS more faithfully

than other tools, may speed development of therapies for the thousands of individuals diagnosed with this devastating disease."

The collaborators will conduct a joint webinar to discuss the project plan in depth in January 2013. To register, visit www.als.net/webinars.

**About the ALS Therapy Development Institute (ALS TDI)**

The mission of the ALS Therapy Development Institute (ALS TDI) is to develop effective therapeutics that slow or stop amyotrophic lateral sclerosis (ALS, Lou Gehrig's disease) as soon as possible for patients today. Focused on meeting this urgent unmet medical need, ALS TDI executes a robust target discovery program, while simultaneously operating the world's largest efforts to preclinically validate potential therapeutics; including a pipeline of dozens of small molecules, protein biologics, gene therapies and cell-based constructs. The world's first nonprofit biotech institute, ALS TDI has developed an industrial-scale platform, employs 30 professional scientists and evaluates dozens of potential therapeutics each year. ALS TDI collaborates with leaders in both academia and industry to accelerate ALS therapeutic development, including Biogen Idec, UCB, Aestus Therapeutics, MDA and RGK Foundation. ALS TDI is a 501(c)3 registered charitable nonprofit organization. For more information, please visit us online at www.als.net.

**About the Gladstone Institutes**

Gladstone is an independent and nonprofit biomedical-research organization dedicated to accelerating the pace of scientific discovery and innovation to prevent, treat and cure cardiovascular, viral and neurological diseases. Gladstone is affiliated with the University of California, San Francisco. For more information, visit us online at www.gladstoneinstitutes.org.

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