

**FOR IMMEDIATE RELEASE**

## **Four Non-Profits Covering the Spectrum of Neurodegenerative Disease Collaborate on Key Disease Modeling Project**

*ADDF, MDA, AFTD and ALS TDI Jointly Fund Characterization of TDP-43 Mouse*

CAMBRIDGE, MASS. – March 22, 2011 – The ALS Therapy Development Institute (ALS TDI) announced on March 22, 2011 that it has entered into collaboration with the Muscular Dystrophy Association (MDA), the Alzheimer’s Drug Discovery Foundation (ADDF) and The Association for Frontotemporal Degeneration (AFTD) to fund the characterization of a new model of neurodegeneration called the TDP-43 mouse.

“ALSTDI has already made an important contribution in advancing the science of preclinical animal trials for new drugs for neurodegenerative disease,” said Howard Fillit, M.D., executive director of the Alzheimer’s Drug Discovery Foundation. “In this program, they will carefully characterize an animal model for a novel target (TDP-43) in neurodegenerative disease, including Alzheimer’s and frontotemporal degeneration, improving the quality of preclinical testing of new drugs targeting TDP-43 in animals. As a result, the predictive value of success for new drugs targeting TDP-43 in humans should be improved.”



TAR-P DNA Binding Protein 43 (TDP-43) has been implicated as a potential molecular marker or cause of ALS in a subset of people living with the progressive neurodegenerative disease. The exact role that TDP-43 activity plays in the onset or progression of ALS is unknown. However, there is vast literature which suggests that TDP-43 activity is linked to several neurodegenerative diseases, including ALS (Lou Gehrig’s disease), frontotemporal degeneration, Alzheimer’s Disease, and traumatic brain injury, among others.



The funding received from these organizations will culminate in the characterization of the TDP-43 mouse model. The characterization experiment was designed by ALS TDI in consultation with the creator of the mouse model, Robert Baloh, M.D., Ph.D., and his MDA-funded team, at Washington University in St. Louis. One of the goals of this collaboration is to determine the animal’s phenotype (how it displays disease) in order to propose potential efficacy screening protocols. At the same time, ALS TDI will collect disease associated tissue samples and identify key changes of gene expression related to disease onset or progression in the TDP-43 model. The Institute will then conduct comparative analysis by relating this new information to that it has created from similar characterization efforts on the SOD1 mouse, other models of neurodegeneration and from people living with ALS today.



“Ours is one of the only labs in the world with the experience to conduct this type of characterization experiments. We are grateful to be able to bring together so many key stakeholders to get this project done for the entire neuroscience community,” said Steve Perrin, Ph.D., CEO & CSO of ALS TDI.

### **About the Alzheimer's Drug Discovery Foundation (ADDF)**

The ADDF ([www.AlzDiscovery.org](http://www.AlzDiscovery.org)) is the only public charity whose sole mission is to accelerate the discovery and development of drugs to prevent, treat and cure Alzheimer's disease, related dementias and cognitive aging. Since 1998, the ADDF has granted more than \$45 million to fund over 325 Alzheimer's drug discovery programs in academic centers and biotechnology companies in 17 countries.

### **About MDA**

MDA ([www.mda.org](http://www.mda.org)) is the world's largest nonprofit provider of ALS services and funder of ALS research. Over the years, MDA has invested more than \$290 million specifically fighting ALS. MDA operates more than 200 clinics at hospitals across the county, 38 of which are ALS-specific research and care centers. The Association's unparalleled health care services, research, advocacy and education programs provide help and hope to more than 1 million Americans affected by ALS and 42 other neuromuscular diseases.

MDA has been funding Baloh's development of the TDP-43 mouse model at Washington University in St. Louis since 2009, and that project is continuing through 2012. Also, MDA-funded investigators identified the SOD1 gene mutation in 1993 (Drs. Robert Brown and Teepu Siddique), and MDA-grantee Mark Gurney created the popular SOD1 mouse model in 1994.

### **About 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. Since its founding in 1999 as the world's first "nonprofit biotech", ALS TDI has developed an internationally recognized, industrial-scale platform that allows for the development and testing of dozens of potential therapeutics each year. The Cambridge, Massachusetts based research Institute collaborates with worldwide leaders from both academia and industry to accelerate ALS therapeutic development. For more information about the Institute's current research pipeline and to take a virtual tour of its laboratory, please visit us online at [www.als.net](http://www.als.net)

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