



Pilot human study shows novel treatment suppresses mutant ALS gene *The Angel Fund for ALS Research provides major funding*

(Wakefield, MA) – A clinical trial funded by The Angel Fund for ALS Research has shown significant progress in suppressing expression of the most commonly mutated ALS gene, C9ORF72 (C9). The C9 gene mutation, a lengthy expansion of a repeated segment of six molecules of DNA, causes both familial ALS and frontotemporal dementia. The results of the human trial, initiated and led by Drs. Robert H. Brown and Jonathan Watts at UMass Chan Medical School, were published in Nature Medicine.

This is a major milestone and an exciting breakthrough in the efforts to find a treatment for this neurodegenerative disease, according to Dr. Brown. The study was conducted on one patient after being sanctioned by the FDA. Dr. Brown and the study team hope to expand the study to as many as 10 patients in the coming months with further FDA approval.

To silence the C9 gene, the research team developed antisense oligonucleotides (ASOs) that target the two RNA transcripts of the gene that contain the toxic, expanded segment of nucleic acids. When the ASO was delivered into the spinal fluid, the activity of the gene was substantially suppressed in the participant. The suppression was maintained by repeated doses of the ASO, which were well tolerated without safety concerns in this pilot study. According to Dr. Brown, while ASOs against this target region have previously been shown by investigators to

attenuate expression of the C9 gene in neurons in cell culture and mouse models, the UMass-led trial was the first to demonstrate this in a human. A trial of a comparable ASO is now also being conducted by Biogen, Inc, in Cambridge; results from that study have not yet been reported.

“The Angel Fund for ALS Research has been committed to finding a treatment and a cure for ALS for nearly three decades,” The Angel Fund said. “This is a giant leap forward on the road to such a discovery. We are proud to fund this research and are excited with the promising results of this clinical trial.”

Dr. Jonathan Watts commented, “The research team is excited and encouraged by these results and we look forward to expanding our trial to include more individuals with C9 ALS and frontotemporal dementia. We are grateful to The Angel Fund for ALS Research for their funding.”

In addition to the lead role taken by Drs. Brown and Watts, key participants included Drs. Helene Tran and Michael Moazami, as well as an extensive clinical trials team. Beyond the major funding from the Angel Fund, support was also provided by the National Institutes of Health and other ALS organizations.

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The Angel Fund for ALS Research (Angel Fund, Inc.) is an independent 501(c)3 nonprofit charity dedicated to supporting ALS (Lou Gehrig’s Disease) investigations at the Cecil B. Day Laboratory for Neuromuscular Research at UMass Chan Medical School in Worcester, MA.

The Angel Fund mission is to support ALS research and scientific investigations at the Cecil B. Day Laboratory to find a cause, treatment and cure for ALS. Ninety-two percent of funds expended goes to research, which is accomplished by raising funds through events, campaigns, foundation grants and numerous other community outreach activities. The Angel Fund has donated millions of dollars to fund the research of Dr. Robert H. Brown and his team.