



Chaperone Therapeutics and ALS Therapy Development Institute Enter into Research Partnership *Protein Folding Modulators Evaluated for Efficacy as Treatment for ALS*

August 10, 2015 – CAMBRIDGE, MA & RESEARCH TRIANGLE PARK, NC – The [ALS Therapy Development Institute](#) and [Chaperone Therapeutics, Inc.](#), announced today that they have entered into a research partnership to explore potential treatments for [amyotrophic lateral sclerosis](#) (ALS). Chaperone has developed several promising small molecules targeting the activation of HSF1, a key modulator of pathways involved in protein folding and neuronal stress protection. The objective of the partnership is to complete preclinical optimization of lead candidates and develop a clinical development plan in ALS.

“Like many neurodegenerative disease indications, ALS appears to be a protein misfolding disorder,” said Steve Perrin, Ph.D., CEO and CSO of the ALS Therapy Development Institute. “The accumulation of these errant proteins results in cellular stress in motor neurons, loss of innervation of skeletal muscle, and paralysis in ALS patients. We are excited about the partnership with Chaperone. These lead molecules could be first in class treatments for neurodegenerative diseases such as ALS.”

Preclinical experiments with Chaperone Therapeutics’ small molecules are taking place at the ALS Therapy Development Institute’s facility in Cambridge, Massachusetts. The partners have designed cell-based assays to test the potential treatment’s ability to correct protein misfolding, protect neurons from stress-induced cell death as well as in vivo studies to assess the treatment’s effect on the progression of ALS.

“Protein misfolding is the cause of many neurodegenerative diseases, and data from a number of disease models demonstrate efficacy through enhancing the cellular protein folding capacity,” said Dennis J. Thiele, Ph.D., CSO at Chaperone Therapeutics. “We are delighted to work closely with the outstanding team at the ALS Therapy Development Institute to test our molecules that increase protein chaperone activity, as a potential disease modifying therapy for ALS.”

The research partners are working collaboratively to determine the potential application of Chaperone’s technology in ALS and will provide more information as it becomes available or published through a peer-reviewed journal.

The ALS Therapy Development Institute and Chaperone Therapeutics will hold a webinar about the research partnership on **August 18 at 2:00 PM ET**. To register, please visit <http://www.alstdi.org/als-events/als-webinars/register/?id=08182015>.

About the ALS Therapy Development Institute

The ALS Therapy Development Institute and its scientists actively discover and develop treatments for ALS. The Institute is the world’s first and largest nonprofit biotech focused 100 percent on ALS research. Led by ALS patients and their families, the charity understands the urgent need to slow and stop this horrible disease. Based in Cambridge, MA, the Institute has served as one of the leaders in sharing data and information with academic and ALS research organizations, patients and their families. For more information, visit www.alstdi.org.

About Chaperone Therapeutics, Inc.

Chaperone Therapeutics is developing frontline therapies to address protein misfolding and neuronal cell loss, the underlying causes of neurodegenerative disease. Chaperone’s therapeutic approaches elevate the



natural cellular protein folding and stress protection mechanisms to prevent protein misfolding and the ensuing neuronal cell death. Chaperone's unique drug discovery platforms, broad knowledge in small molecule optimization and expertise in animal models of neurodegenerative disease and clinical evaluation propel our therapeutic discovery programs forward. For more information, visit www.chaperontherapeutics.com.

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