



ALS Therapy Development Institute and to-BBB Collaborate on Potential Treatments for Motor Neuron Disease

CNS-targeted Liposomal Drug Delivery System Enhances Institute's Formulation Abilities

January 8, 2013 – CAMBRIDGE, USA & LEIDEN, the NETHERLANDS – The [ALS Therapy Development Institute](#) (ALS TDI) announced today that it has entered into a new collaboration with to-BBB to investigate potential treatments for ALS (aka Motor Neuron or Lou Gehrig's disease).

"This partnership brings a new potential tool to safely enhance the delivery of drugs that may slow or stop the progression of ALS," said Steve Perrin, Ph.D., CEO & CSO of ALS TDI. "Being able to dose a drug systemically, but yet have it cross the blood-brain barrier, allows us to try things we weren't able to do before. It enhances the arsenal of weapons we have available in our search for effective treatments for ALS."

The blood-brain barrier (BBB) fortifies the walls of blood vessels to prevent the entry of certain toxic substances into the brain. While maintaining this crucial protection, the barrier also poses challenges to delivering potential therapeutics for neurodegenerative disorders such as ALS.

"We strongly believe that we can facilitate in creating treatment options for devastating brain diseases faster by providing sustained and enhanced delivery of known compounds to the Central Nervous System (CNS)," said Pieter Gaillard, Ph.D., founder and Chief Scientific Officer of to-BBB. "It is a major acknowledgement from ALS TDI to partner with to-BBB in applying such a novel approach to ALS. This collaboration will provide to-BBB with the opportunity to jointly pursue several treatment options for ALS, and we look forward to collaborating with the experts of the Institute."

Under the terms of the agreement, ALS TDI will use to-BBB's CNS-targeted liposomal drug delivery system, [the G-Technology®](#), as a tool to safely enhance the transport of several different compounds to the brain across the blood-brain barrier. The Institute will screen compounds in a preclinical model of ALS to determine if the enhanced formulation of the proposed treatments have an improved effect on disease course. The collaborators have been working together since early in 2012, designing and completing proof-of-concept experiments confirming that to-BBB's G-Technology indeed accomplishes sustained and effective delivery of drugs to the CNS in the SOD1 preclinical model of ALS.

About ALS

[Amyotrophic lateral sclerosis](#) (ALS, Lou Gehrig's disease) is a progressive neurodegenerative disease that leads to paralysis, due to the death of motor neurons in the spinal cord and brain. There is no known cause, cure or effective treatment for the disease. About 5,000 people in the US are diagnosed with ALS each year; the incidence is similar to Multiple Sclerosis. However, with no effective treatment and an aggressive and rapid disease progression, the average patient survives only 2-5 years following their diagnosis. There are about 30,000 US citizens diagnosed with the disease at any given time. The worldwide population of ALS patients is estimated at 400,000.



About the ALS Therapy Development Institute (ALS TDI)

The mission of the [ALS Therapy Development Institute](http://www.als.net) (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 to-BBB

[to-BBB](#) is a clinical stage biotechnology company focusing on enhanced drug delivery across the blood-brain barrier. The Company is developing novel treatments for devastating brain disorders, such as brain cancer, neurodegenerative diseases and lysosomal storage diseases, by combining existing drugs with the G-Technology®, to-BBB's proprietary brain delivery platform. This technology combines the widely used drug delivery approach of pegylated liposomes with the endogenous tripeptide glutathione as targeting ligand in a novel and safe way. Together with several top tier pharma and biotech companies, to-BBB is investigating the versatility of the G-Technology for drugs that are unable to reach the brain at systemically tolerable therapeutic doses. to-BBB is applying the G-Technology for the delivery of doxorubicin for the treatment of brain cancer as its internal lead product 2B3-101.

to-BBB is headquartered in the Netherlands at the Leiden Bio Science Park and established a fully owned subsidiary, to-BBB Taiwan Ltd., in Taipei, Taiwan. Investors in to-BBB include Aescap Venture, Antea Participaties, Jonghoud International and the Industrial Bank of Taiwan Management Corporation (IBTM).

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