



## **Pain Therapeutics, Inc.**

**For More Information Contact:**

Ruth Araya  
Pain Therapeutics, Inc.  
IR@paintrials.com  
(512) 501-2485

### **Pain Therapeutics Announces Research Publication on Alzheimer's Disease**

**- PTI-125, an Experimental Drug, Restored Receptor Function and Significantly Improved Working Memory and Spatial Ability in a Transgenic Mouse Model of Alzheimer's Disease -**

**AUSTIN, Texas – June 1, 2017** – Pain Therapeutics, Inc. (Nasdaq: PTIE), a biopharmaceutical company, today announced a technical publication authored by its scientists and academic collaborators that further demonstrates a potentially promising new therapeutic approach to treat Alzheimer's Disease with PTI-125, a new drug candidate with a novel mechanism of action.

Transgenic mice with genetic mutations for Alzheimer's disease were treated with PTI-125. After two months of treatment, the mice showed significant improvements in working memory, spatial ability and social behavior. Importantly, their brains also showed significant improvements in multiple Alzheimer's neuropathologies, such as improved function of receptors key to cognition; improved synaptic function; decreased neuroinflammation; decreased insulin resistance; reduced amyloid plaques; and reduced tau hyperphosphorylation. This study is published in the current issue of *Neurobiology of Aging*, one of the highest-ranking journals in its field, and was funded in part by a research grant from the National Institutes of Health (NIH).

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“These data suggest PTI-125 improves cognition and slows the course of Alzheimer’s Disease,” said Remi Barbier, President & CEO. “These are exciting potential health benefits. Through innovative science and our desire to work collaboratively, our vision for PTI-125 is to advance the treatment of Alzheimer’s Disease.”

An Investigational New Drug (IND) application to test PTI-125 in humans is on-track for submission to the U.S. Food and Drug Administration (FDA) in the third quarter of 2017.

The *Neurobiology of Aging* (Vol 55, July 2017, Pages 99–114) publication is titled “*PTI-125 binds and reverses an altered conformation of filamin A to reduce Alzheimer’s disease pathogenesis*”.

It may be found at: [http://www.neurobiologyofaging.org/article/S0197-4580\(17\)30087-8/](http://www.neurobiologyofaging.org/article/S0197-4580(17)30087-8/)

### **About Alzheimer's Disease and PTI-125**

Alzheimer’s Disease (AD) is a progressive brain disorder that slowly destroys memory and thinking skills, and eventually the ability to carry out the simplest tasks. There is no approved drug therapy to reverse, or even halt, the course of AD. PTI-125 is an oral, small molecule drug candidate that was designed in-house and characterized by outside collaborators. PTI-125 has been shown to significantly improve AD neuropathologies in mouse models of the disease and in post-mortem brain tissue from AD patients, including receptor dysfunctions, neuroinflammation, tau hyperphosphorylation, insulin resistance and plaques and tangles that are hallmarks of AD.

To date, the underlying science for PTI-125 has been published in *Journal of Neuroscience*, *Neurobiology of Aging*, *Journal of Biological Chemistry*, *PLOS-One* and other peer-reviewed scientific journals.

Pain Therapeutics owns worldwide commercial rights to PTI-125 and related technology.

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**About Pain Therapeutics, Inc.**

Pain Therapeutics, Inc. is a clinical-stage biopharmaceutical company that develops novel drugs. The FDA has not yet established the safety or efficacy of any of our drug candidates. For more information, please visit [www.paintrials.com](http://www.paintrials.com).

***Note Regarding Forward-Looking Statements:** This press release contains forward-looking statements for purposes of the Private Securities Litigation Reform Act of 1995 (the "Act"). Pain Therapeutics disclaims any intent or obligation to update these forward-looking statements, and claims the protection of the Safe Harbor for forward-looking statements contained in the Act. Examples of such statements include, but are not limited to, statements regarding the safety or effectiveness of PTI-125 and the Company's plan to file an IND with the FDA for PTI-125 in 2017. Such statements are based on management's current expectations, but actual results may differ materially due to various factors. Such statements involve risks and uncertainties, including, but not limited to, those risks and uncertainties relating to the ability to demonstrate the safety, efficacy or potential health benefits of PTI-125 in humans and to determine which patient, or subpopulation of patients, may benefit from treatment. For further information regarding these and other risks related to our business, investors should consult our filings with the U.S. Securities and Exchange Commission.*

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