



Pain Therapeutics, Inc.

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FOR IMMEDIATE RELEASE

NIH Awards \$1.7 Million Innovation Grant To Pain Therapeutics

AUSTIN, TX – Sept 21, 2015 – Pain Therapeutics, Inc. (Nasdaq:PTIE) today reported that the National Institutes of Health (NIH) has awarded the Company a \$1.7 million innovation grant. This grant award provides a path forward for the Company to develop a small molecule drug, called PTI-125, that offers a promising new approach to treat Alzheimer’s Disease.

"We are grateful to the National Institutes of Health for its support of our research program," said Remi Barbier, Chairman, President & CEO of Pain Therapeutics. "NIH has long been a supporter of innovative technologies that stand up to rigorous, peer reviewed scientific evaluation and that have the potential to benefit human health in areas of unmet needs, such as Alzheimer’s Disease."

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The NIH's *National Institute on Aging* awarded this innovation grant to Pain Therapeutics following an in-depth evaluation of PTI-125 for scientific and technical merit. This competitive review process relies on academic, clinical and industry experts in Alzheimer's Disease and other neurological disorders. The science that underlies PTI-125 was initially published in *The Journal of Neuroscience* (2012-32:9773-9784), the highest-ranking journal in its discipline.

PTI-125 prevents the formation of both amyloid plaques and neurofibrillary tangles (composed of *tau* protein), the hallmarks of Alzheimer's Disease (AD). Its novel mechanism of action and early data suggest this drug may improve cognition and protect against the toxic effects of amyloid in neurons, raising hopes for a treatment to combat cognitive decline in patients with AD.

With this NIH award, Pain Therapeutics will conduct pre-clinical studies that are expected to enable an Investigational New Drug (IND) regulatory filing for PTI-125. PTI-125 is an oral, small molecule drug candidate that was designed in-house and characterized by outside collaborators. The Company owns exclusive, worldwide commercial rights to PTI-125 and related technologies.

About PTI-125

AD patients suffer from progressive cognitive and memory impairments, widely believed to be caused by a protein in the brain called amyloid. Many drug candidates for AD focus on reducing or clearing amyloid from the brain. In contrast, PTI-125 blocks the toxic signaling of amyloid and reduces aberrant *tau* protein. PTI-125 binds to a novel target critical to amyloid's toxic cascade: the scaffolding protein filamin A (FLNA). By binding FLNA, PTI-125 blocks amyloid's toxicity and *tau* phosphorylation. In animal models and human postmortem AD brain tissue, the beneficial effects included restoring neuronal function and reducing neuro-inflammation. These effects suggest PTI-125 may improve cognition and reduce neurodegeneration. The mechanism also indicates the development of a non-invasive, blood-based biomarker and diagnostic for AD.

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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 approved our drug candidates for commercial sale. For more information, please visit 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 about plans to develop PTI-125, conducting pre-clinical studies that are expected to enable an IND and the potential benefits of PTI-125, including possible improved cognition and protection against the toxic effects of amyloid in neurons. 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 difficulties or delays in developing PTI-125 and the results of our pre-clinical studies of PTI-125 not supporting further development, which could result in termination of NIH funding prior to receipt of the entire \$1.7 million grant. 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. The entire contents of this press release are solely the responsibility of Pain Therapeutics and do not necessarily represent the official views of the NIH or any of its centers.

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