Pain Therapeutics, Inc.

Pain Therapeutics Reports 2018 Financial Results and Corporate Update

– 2019 Focus Is on Alzheimer's Disease, Rebranding the Company, Conducting Phase II Studies and Fiscal Discipline -

AUSTIN, Texas – March 25, 2019 – Pain Therapeutics, Inc. (Nasdaq: PTIE) today reported financial results for the year ended December 31, 2018. Net loss in 2018 was $6.6 million, or $0.61 per share, compared to a net loss in 2017 of $11.9 million, or $1.82 per share. Cash used in operations during the year ended December 31, 2018 was $4.8 million. Cash and cash equivalents were $19.8 million as of December 31, 2018, with no debt. We believe net cash utilization in 2019 will be in the range of $5.0 - $6.0 million.

Historically, our focus was on analgesic drug development. In 2019, however, we will rebrand around neurodegenerative diseases, such as Alzheimer's disease. Our rebranding plans include a new company name, logo, ticker symbol and website, as well as a comprehensive strategy to bolster media outreach and an active approach to engage with potential new shareholders.

"There's never been a more exciting time to be in Alzheimer's research," said Remi Barbier, President & CEO. "For many years, the prevailing scientific hypothesis said amyloid must be cleared out of the brain. This hypothesis has been tested in clinical studies using a variety of antibody backbones, epitopes, target conformations, biomarkers and in various stages of disease. These amyloid-clearing studies have one thing in common: they've all failed. It's now prudent to consider more recent scientific breakthroughs in Alzheimer's research. We think these are the innovations that stand a chance of making a difference for patients with Alzheimer's disease."

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Overview of Alzheimer's Program

Over the past ten years, we have developed a new and highly promising scientific approach for the treatment and detection of Alzheimer's disease. Importantly, our science does not seek to clear amyloid from the brain. Our approach is to stabilize a critical protein in the brain.

Starting with basic research, we have identified a structurally altered protein in the brain, also called a 'proteopathy'. This proteopathy plays a critical role in the neurodegeneration observed in Alzheimer's disease. Using scientific insight and advanced tools in biochemistry, bioinformatics and imaging, we have elucidated this protein dysfunction. We engineered a family of high-affinity small molecules to target the structurally altered protein and to restore this protein to its normal shape and function. Our drug candidate, PTI-125, is a small molecule that targets an altered form of a scaffolding protein called filamin A (FLNA). *Study animals treated with PTI-125 showed significant improvements in neuronal function and decreases in neuroinflammation, resulting in cognitive improvement and slowing of disease progression.*

In 2017, we successfully completed a Phase I clinical study with PTI-125. In 2018, we initiated a Phase IIa study with PTI-125 in patients with mild-to-moderate Alzheimer's disease, with scientific and financial support from the National Institutes of Health (NIH). In 2019, we expect to conclude our Phase IIa study and announce clinical results.

We are also developing an experimental biomarker/diagnostic, called PTI-125Dx, to detect Alzheimer's disease with a simple blood test. This program has financial support from the NIH.

The underlying science for our programs in neurodegeneration is published in several prestigious, peer-reviewed technical journals, including *Journal of Neuroscience*, *Neurobiology of Aging*, and *Journal of Biological Chemistry*.

In addition, in 2018 the *National Institute on Aging* of the NIH awarded our scientific programs two research grants. Collectively, these represent up to $6.7 million of non-dilutive financing.

Financial Highlights for 2018

- At December 31, 2018, cash and cash equivalents were $19.8 million, compared to $10.5 million in 2017. We have no debt.

- Net cash used in operations during the year ended December 31, 2018 was $4.8 million.

- Research and development expenses for the year ended December 31, 2018 were $3.0 million compared to $7.6 million for the same period in 2017, or a 61% decrease. This was due primarily to decreases in analgesic drug development related expenses.

- We received reimbursements of $3.0 million in 2018 from research grants from the NIH that we recorded as a reduction of research and development expense compared to $1.4 million in 2017.

- Research and development expenses included non-cash stock related compensation costs of $1.0 million for the year ended December 31, 2018 and $1.2 million for the same period in 2017.

- General and administrative expenses for the year ended December 31, 2018 were $3.7 million compared to $4.3 million for the same period in 2017, or a 15% decrease. This was due primarily to a decrease in non-cash stock-based compensation expenses as well as outside professional fees. General and administrative expenses included non-cash stock-based compensation costs of $1.4 million in the year ended December 31, 2018 and $1.8 million for the same period in 2017.

- On August 17, 2018, we announced the closing of a registered direct offering of 8,860,778 shares of our common stock and issuance of warrants. Total net proceeds from the offering were approximately $10.2 million. In addition, we raised approximately $3.9 million of net proceeds through our At-The-Market common stock offerings during 2018.

- In August and in October 2018, we announced that the NIH had awarded us research grants to support a Phase II program with PTI-125, our drug candidate to treat Alzheimer's disease. Collectively, the NIH grants represent up to $6.7 million of non-dilutive financing.

Operating Highlights for 2018 and Forecast for 2019

- Historically, our lead drug candidate had been REMOXY, which is the trade name for an abuse-deterrent, extended-release form of oxycodone to treat severe chronic pain. The U.S. Food and Drug Administration (FDA) has previously found REMOXY to be an effective analgesic drug for the treatment of severe chronic pain. However, FDA has not approved REMOXY on the basis that additional demonstrations of its abuse deterrent properties are needed, a matter of dispute between us and FDA.

- On March 20, 2019, we provided Durect Corporation with written notice of termination of a Development and License Agreement (DLA). *Termination of the DLA effectively ends our clinical development of REMOXY.*

- In October 2018, we announced a strategic reorganization to align Company resources on advancing our programs in neurodegenerative diseases, such as Alzheimer's disease.

- In December 2018, we announced the initiation of a Phase II study to evaluate PTI-125 in patients with Alzheimer's disease. This clinical study is supported by a research grant award from the National Institute on Aging of the NIH, the primary Federal agency supporting innovative new research in Alzheimer's disease.

- In 2019, we expect to rebrand the Company around neurodegeneration. Our rebranding plans include a new company name, logo, ticker symbol, website, as well as a comprehensive strategy to bolster media outreach and an active approach to engage with potential new shareholders.

About Alzheimer's Disease

Alzheimer's disease is a progressive brain disorder that destroys memory and thinking skills. Eventually, a person with Alzheimer's disease may be unable to carry out even simple tasks. Currently, there are no drug therapies to halt Alzheimer's disease, much less reverse its course. Alzheimer's disease is likely to become one of the world's most serious future health care crisis.

About Pain Therapeutics

Pain Therapeutics is focused on the early detection and treatment of Alzheimer's disease. Since 2010, we have combined state-of-the-art technology with new insights in neurobiology to develop novel solutions for Alzheimer's disease. We own worldwide development and commercial rights to our research programs in Alzheimer's disease, and related technology, without royalty obligations to any third-parties. For more information, please visit www.paintrials.com.

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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 timing of clinical studies and the potential benefits of the Company's programs in Alzheimer's disease including our ongoing Phase II program, plans for rebranding the Company in 2019, and expected cash use in future periods. The Company cautions that forward-looking statements are inherently uncertain. 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 development and testing of our drug candidates; unexpected adverse side effects or inadequate therapeutic efficacy of our drug candidates; the uncertainty of patent protection for our intellectual property or trade secrets; unanticipated additional research and development, litigation and other costs; the need to raise additional funding from time-to-time, and the potential for competing products to be developed by competitors and potential competitors or others. Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. Except as required by law, the Company disclaims any intention or responsibility for updating or revising any forward-looking statements contained in this press release. 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 (SEC), which are available for free on the SEC's website at www.sec.gov.*

– Financial Tables Follow –

PAIN THERAPEUTICS, INC.
CONDENSED STATEMENTS OF OPERATIONS
(unaudited, in thousands, except per share amounts)

	Three Months Ended December 31,		Years Ended December 31,	
	2018	2017	2018	2017
Operating expenses:				
Research and development, net of grant reimbursement	2	1,544	2,969	7,615
General and administrative	748	879	3,693	4,334
Total operating expenses	750	2,423	6,662	11,949
Operating loss	(750)	(2,423)	(6,662)	(11,949)
Interest income	73	5	105	38
Net loss	$ (677)	$ (2,418)	$ (6,557)	$ (11,911)
Net loss per share, basic and diluted	$ (0.04)	$ (0.37)	$ (0.61)	$ (1.82)
Weighted-average shares used in computing net loss per share, basic and diluted	17,162	6,538	10,682	6,537

CONDENSED BALANCE SHEETS
(unaudited, in thousands)

	December 31,	
	2018	2017
Assets		
Current assets		
Cash, cash equivalents	$ 19,807	$ 10,479
Other current assets	233	184
Total current assets	20,040	10,663
Other non-current assets	99	168
Total assets	$ 20,139	$ 10,831
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 294	$ 424
Accrued development expense	156	399
Other accrued liabilities	61	309
Total current liabilities	511	1,132
Total liabilities	511	1,132
Stockholders' equity		
Common stock	17	7
Additional paid-in-capital	183,567	167,091
Accumulated deficit	(163,956)	(157,399)
Total stockholders' equity	19,628	9,699
Total liabilities and stockholders' equity	$ 20,139	$ 10,831

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