Pain Therapeutics, Inc.

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FOR IMMEDIATE RELEASE

Pain Therapeutics Reports Q2 2017 Financial Results And Mid-year Corporate Update

AUSTIN, Texas – August 9, 2017 – Pain Therapeutics, Inc. (Nasdaq: PTIE) today reported financial results for the second quarter ended June 30, 2017 and provided an update on mid-year progress and upcoming milestones.

"We made steady progress in the first half of 2017 toward building the business for the long-term," said Remi Barbier, President & CEO of Pain Therapeutics. "This includes meeting with FDA to better understand the regulatory requirements for approval of REMOXY ER; completing patient enrollment for a clinical abuse potential study and initiating a non-clinical abuse potential study, both with REMOXY; publishing ground-breaking data for PTI-125 in a peer-reviewed scientific journal; being awarded a $1.7 million grant from NIH following a highly competitive, peer-reviewed evaluation of PTI-125; receiving FDA clearance of an IND application for PTI-125; and preparing for a Phase I 'first-in-human' study with PTI-125 in the near future, with funding to be provided by NIH. We believe these activities demonstrate our commitment to develop pioneering new drug assets, while maintaining a careful watch on expenses and our balance sheet".

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Net loss for the three months ended June 30, 2017 was $4.2 million, or $0.64 per share, compared to a net loss of $3.0 million, or $0.46 per share, for the three months ended June 30, 2016. Net cash used during the three months ended June 30, 2017 was $3.2 million. Cash and investments were $14.1 million as of June 30, 2017, with no debt. The Company continues to expect net cash usage in 2017 may be approximately $10 million.

Update on REMOXY ER™ (extended-release oxycodone capsules CII)

We are still targeting resubmission of the New Drug Application (NDA) for REMOXY ER for Q1 2018. In Q1 2017, we met with the U.S. Food and Drug Administration (FDA) to better understand the requirements for the regulatory approval of REMOXY ER. These discussions confirmed two key requirements are needed for the resubmission and potential approval of the REMOXY NDA:

- A clinical abuse potential study via the nasal route of abuse; and
- A non-clinical (*in vitro*) abuse potential study using household solvents.

In Q2 2017, we initiated an abuse potential study via the nasal route of abuse with REMOXY ER. We recently completed patient enrollment for this study. We expect to announce top-line results by year-end 2017. In Q2 2017, we also finalized testing methodologies for a non-clinical (*in vitro*) abuse potential study that evaluates the REMOXY formulation in a variety of household solvents.

Update on PTI-125, a novel drug candidate to treat Alzheimer's Disease

In Q2 2017, we announced a peer-reviewed publication in a scientific journal that further details our novel therapeutic approach for Alzheimer's Disease (AD). This ground-breaking publication showed that PTI-125's novel mechanism of action significantly improved working memory and spatial ability in a transgenic mouse model of AD, and reversed pathology in post-mortem brain tissue from patients. Building on this science, we are also developing and evaluating a blood-based diagnostic for AD.

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In Q2 2017, we announced that the National Institutes of Health (NIH) awarded us a $1.7 million research grant to evaluate PTI-125 in Alzheimer's Disease. We also announced that the FDA had cleared our investigational new drug application (IND) for PTI-125. As a result, we plan to conduct a Phase I 'first-in-human' study with PTI-125 in the near future, with funding provided by NIH. We expect to announce top-line results of this Phase I study by year-end 2017.

Financial Highlights for Q2 2017

- At June 30, 2017, cash and investments were $14.1 million, compared to $17.3 million at March 31, 2017. The Company has no debt.
- Net cash used during the three months ended June 30, 2017 was $3.2 million.
- Research and development expenses for the three months ended June 30, 2017 were $3.1 million compared to $1.6 million in the prior year period. The increase was primarily due to increased activities related to REMOXY NDA resubmission. Research and development expenses included non-cash stock-related compensation of $0.3 million in the three months ended June 30, 2017 compared to $0.4 million in the prior year period.
- General and administrative expenses for the three months ended June 30, 2017 were $1.1 million compared to $1.5 million in the prior year period. The decrease was primarily due to a decrease in compensation expenses. General and administrative expenses included non-cash stock-related compensation of $0.4 million in the three months ended June 30, 2017 compared to $0.6 million in the prior year period.
- During Q2 2017, we completed a 7-for-1 Reverse Split of our outstanding shares of common stock. This reduced the number of shares of common stock outstanding from about 46.1 million shares pre-Reverse Split to about 6.6 million shares post-Reverse Split. The Reverse Split did not affect any stockholder's percentage equity ownership in Pain Therapeutics.

About REMOXY ER (extended-release oxycodone capsules CII)

REMOXY ER is a proprietary, abuse-deterrent, extended-release oral formulation of oxycodone. The proposed indication for this drug candidate is for "the management of pain severe enough to require daily, around-the-clock, long-term opioid treatment and for which alternative treatment options are inadequate." We developed REMOXY ER to make oxycodone difficult to abuse yet provide 12 hours of steady pain relief when used appropriately by patients. REMOXY's sticky, high-viscosity gel formulation may deter unapproved routes of drug administration.

Pain Therapeutics owns exclusive, worldwide commercial rights to REMOXY ER.

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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. Building on this science, we also have under development a blood-based diagnostic for AD.

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

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.

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 our projected net cash usage in 2017; statements regarding regulatory plans and strategies to resubmit the REMOXY NDA; statements regarding the timing or estimated costs of studies and actions needed to resubmit the REMOXY NDA to the FDA; and our plans to evaluate PTI-125 in a first-in-human study. 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; and the potential for abuse-deterrent pain medications or other competing products to be developed by competitors and potential competitors or others. 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.*

– Financial Tables Follow –

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

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Operating expenses				
Research and development	$ 3,063	$ 1,589	$ 4,452	$ 5,184
General and administrative	1,103	1,455	2,478	3,689
Total operating expenses	4,166	3,044	6,930	8,873
Operating loss	(4,166)	(3,044)	(6,930)	(8,873)
Interest income	6	29	27	63
Net loss	$ (4,160)	$ (3,015)	$ (6,903)	$ (8,810)
Net loss per share, basic and diluted	$ (0.64)	$ (0.46)	$ (1.06)	$ (1.35)
Weighted-average shares used in computing net loss per share, basic and diluted	6,537	6,530	6,536	6,505

CONDENSED BALANCE SHEETS
(in thousands)
(Unaudited)

	June 30, 2017	December 31, 2016
Assets		
Current assets		
Cash, cash equivalents and marketable securities	$ 14,067	$ 18,714
Other current assets	12	356
Total current assets	14,079	19,070
Other assets	190	232
Total assets	$ 14,269	$ 19,302
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable and accrued development expenses	$ 690	$ 330
Other accrued liabilities	309	335
Total current liabilities	999	665
Non-current liabilities	—	—
Total liabilities	999	665
Stockholders' equity		
Common Stock and additional paid-in-capital	165,661	164,125
Accumulated other comprehensive income	—	—
Accumulated deficit	(152,391)	(145,488)
Total stockholders' equity	13,270	18,637
Total liabilities and stockholders' equity	$ 14,269	$ 19,302

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