Aptinyx Reports Top-line Results from Phase 2 Clinical Study of NYX-2925 in Painful Diabetic Peripheral Neuropathy

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NYX-2925 did not achieve statistically significant separation from placebo on primary endpoint

Meaningful improvements on pain and other endpoints observed in subjects receiving 50 mg dose and in subjects not taking a concomitant analgesic medication

NYX-2925 was well-tolerated with no significant adverse events

Further analysis of full dataset to guide potential next steps in development for chronic pain

EVANSTON, Ill., Jan. 16, 2019 (GLOBE NEWSWIRE) -- Aptinyx Inc. (NASDAQ: APTX), a clinical-stage biopharmaceutical company developing transformative therapies for the treatment of brain and nervous system disorders, today announced top-line results from a Phase 2 clinical study of NYX-2925 in subjects with painful diabetic peripheral neuropathy (DPN). In the study, NYX-2925 did not demonstrate statistically significant separation from placebo on the primary endpoint, change in subjects' average daily pain scores on the Numerical Rating Scale (NRS) during the final treatment week compared to baseline. Of the three dose levels evaluated, 50 mg showed the most meaningful improvements across multiple measures. NYX-2925 was well tolerated in the study with no serious adverse events.

In the randomized, double-blind, placebo-controlled study, 300 subjects with painful DPN received daily oral doses of placebo or NYX-2925 at 10 mg, 50 mg, or 200 mg over the course of four weeks. Subjects were randomized in a 1:1:1:1 ratio. Baseline values for each endpoint were measured over the seven-day period prior to randomization. All subjects in the study had moderate to severe pain at baseline. The primary endpoint of the study was the mean change in average daily pain, as measured using the NRS (on which 0 represents no pain and 10 represents worst pain imaginable), at week four of treatment compared to baseline. Key secondary endpoints in the study included worst daily pain, pain on walking, and sleep interference.

In the study, the 50 mg and 200 mg dose levels of NYX-2925 showed the greatest improvements from baseline, with the 50 mg dose showing numerical superiority. The group treated with 50 mg of NYX-2925 showed a 1.61-point reduction from baseline in average daily pain on the NRS, the largest such reduction among the dose levels evaluated. This improvement did not separate statistically (p=0.1586) from the 1.23-point reduction observed in the placebo group. No plateau in the effect of NYX-2925 at 50 mg was observed by the end of this four-week study, suggesting a longer treatment duration may result in a stronger analgesic effect. Subjects receiving NYX-2925 at 50 mg also had clinically meaningful trends of improvement on key secondary endpoints, including sleep and pain on walking.

In a pre-specified subset of subjects who were not taking a concomitant medication, those treated with NYX-2925 across all dose levels showed improvements relative to placebo on pain and other endpoints that were greater than those observed in the overall study population.

"This four-week proof-of-concept study of our novel NMDA receptor modulator, NYX-2925, in painful DPN was designed to evaluate a 20-fold dose range, determine whether efficacy and dose response could be observed on primary and secondary endpoints, and assess safety in a patient population," said Norbert Riedel, Ph.D., president and CEO of Aptinyx. "While the study did not meet its primary endpoint, we observed improvements on multiple measures, differential activity across dose levels, and a very favorable safety profile. Coupled with the positive evidence of biological activity relevant to central pain processing from our recently announced interim analysis of a fibromyalgia study, we believe the total body of clinical data indicates the potential of NYX-2925 to treat chronic pain. We will continue to interrogate the full dataset to determine the most appropriate path forward for NYX-2925 in development for chronic pain. Our talented team also remains steadfastly focused on continued execution across our other pipeline programs, including NYX-783 for the treatment of post-traumatic stress disorder and NYX-458 for the treatment of Parkinson's disease cognitive impairment. Our strong balance sheet will fund our execution and achievements on these programs in 2019 and 2020."

"We are in the midst of a major societal and healthcare crisis in the treatment of pain and many patients currently lack therapeutic options that do not have significant side effects or abuse potential," said John T. Farrar, M.D., Ph.D., Associate Professor of Epidemiology at the University of Pennsylvania. "The intriguing efficacy signals and favorable safety profile observed in this study merit further investigation and analysis in order to fully understand the potential of this drug."

Aptinyx plans to present detailed results from this study at an upcoming medical meeting.

NYX-2925 is also currently in an exploratory Phase 2 study in subjects with fibromyalgia. An interim analysis of this study yielded promising evidence of activity on both objective and subjective measures. Aptinyx expects to announce data from the full analysis of that Phase 2 study in the first half of 2019.

About Neuropathic Pain
Neuropathic pain associated with various conditions affects an estimated 7% to 9% of the U.S. population. Individuals suffering from this condition, across the underlying disorders, are currently treated with a variety of therapies including antidepressants, anticonvulsants, and opioids. These medications offer inadequate efficacy for a large proportion of patients, are often poorly tolerated due to side effects, and in some cases are associated with abuse.

About NYX-2925
NYX-2925 is a novel NMDA receptor modulator currently in Phase 2 clinical development and also under evaluation in an exploratory Phase 2 study in

fibromyalgia. NYX-2925 has demonstrated robust activity in preclinical models of numerous neuropathic pain conditions with a favorable tolerability profile. In a Phase 1 clinical study in healthy human subjects, NYX-2925 was well tolerated across a wide dose range, including dose levels well in excess of the expected therapeutic levels. The U.S. Food and Drug Administration has granted Fast Track designation to Aptinyx's development of NYX-2925 for the treatment of neuropathic pain associated with DPN.

About Aptinyx
Aptinyx Inc. is a clinical-stage biopharmaceutical company focused on the discovery, development, and commercialization of proprietary synthetic small molecules for the treatment of brain and nervous system disorders. Aptinyx has a platform for discovery of novel compounds that work through a unique mechanism to modulate – rather than block or over-activate – NMDA receptors and enhance synaptic plasticity, the foundation of neural cell communication. The company has three product candidates in clinical development in central nervous system indications, including chronic pain, post-traumatic stress disorder, and cognitive impairment associated with Parkinson's disease. Aptinyx is also advancing additional compounds from its proprietary discovery platform, which continues to generate a rich and diverse pipeline of small-molecule NMDA receptor modulators with the potential to treat an array of neurologic disorders. For more information, visit www.aptinyx.com**.**

Forward-Looking Statements
Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Such statements include, but are not limited to, statements regarding the company's business plans and objectives, including future plans or expectations for NYX-2925, therapeutic effects of the company's product candidates, expectations regarding the design, implementation, timing, and success of its current and planned clinical trials, expectations regarding its preclinical development activities, and expectations regarding its uses and sufficiency of capital. Risks that contribute to the uncertain nature of the forward-looking statements include: the success, cost, and timing of the company's product candidate development activities and planned clinical trials; the company's ability to execute on its strategy; positive results from a clinical study may not necessarily be predictive of the results of future or ongoing clinical studies; regulatory developments in the United States and foreign countries; the company's estimates regarding expenses, future revenue, and capital requirements, and other 2018 financial results; the company's ability to fund operations through 2020; as well as those risks and uncertainties set forth in the company's most recent quarterly report on Form 10-Q and in its other filings and reports with the United States Securities and Exchange Commission. All forward-looking statements contained in this press release speak only as of the date on which they were made. Aptinyx undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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