AcelRx Pharmaceuticals' Moderate-to-Severe Acute Pain Candidate ARX-04 Shows Improved Pain Scores in ER Patients in Interim Phase 3 Analysis

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REDWOOD CITY, Calif., Feb. 25, 2016 /PRNewswire/ -- AcelRx Pharmaceuticals, Inc. (Nasdaq: ACRX), a specialty pharmaceutical company focused on the development and commercialization of innovative therapies for the treatment of moderate-to-severe acute pain, today reported encouraging interim efficacy and safety results of the ongoing single-arm, open-label Phase 3 study (SAP302) of ARX-04 for the treatment of adult patients who present in the emergency room with moderate-to-severe acute pain associated with trauma or injury. The primary endpoint of this initial single-dose phase of the study is the time-weighted summed pain intensity difference to baseline over the first hour, or SPID1. The 40 patients who have been enrolled and treated to date in this study experienced a substantial clinical reduction in pain intensity, resulting in a SPID1 value that is similar to previous studies of sublingual sufentanil in post-operative patients. Patients treated with one dose of ARX-04 experienced a mean decrease from baseline of 2.7 on a 0 – 10 numeric rating scale for pain intensity one hour after dosing. Adverse events were consistent with previous clinical studies, with the most frequent events, nausea and somnolence, each reported in two of the 40 patients. None of the participants to date have terminated the study early due to adverse events.

"The safety and efficacy profile we have observed in this Phase 3 ER study is consistent with previous trial results," commented Dr. Pamela Palmer, co-founder and chief medical officer of AcelRx Pharmaceuticals. "In addition to its analgesic efficacy, we assessed the cognitive impact of ARX-04 on patients in this study. We conducted this analysis at the request of the United States Department of Defense, since drug-induced cognitive impairment on the battlefield is a particular concern. Using a well-known cognitive test, the Six-Item Screener, patients demonstrated no change in mean test scores before and after dosing."

Dr. James Miner, the chief of emergency medicine at Hennepin County Medical Center in Minneapolis, MN and primary investigator of the ARX-04 studies, added, "In my clinical experience in a high-capacity emergency department, I believe a product with the ability to mitigate pain rapidly, without the practical and logistical impediment of starting an intravenous line, holds great potential for use in the ER. My experience with ARX-04 in this trial has been positive from both an efficacy and safety standpoint and I look forward to completing the trial and in the near future possibly having a new treatment option to offer my patients."

The SAP302 study will continue to enroll patients, with a goal of enrolling up to 120 patients in total. The extension arm of the study will allow for multiple doses of ARX-04, given hourly as needed for pain, for up to 4 doses. The company expects to initiate the extension portion of the study by the end of the first quarter of 2016.

About ARX-04
ARX-04 is a non-invasive investigational product candidate consisting of 30 mcg sufentanil tablets delivered sublingually via a disposable, pre-filled, single-dose applicator (SDA). AcelRx is developing ARX-04 for the management of moderate-to-severe acute pain in a variety of medically supervised settings, including the emergency room, outpatient or ambulatory surgery, non-surgical patients experiencing pain in the hospital, and post-operative patients following short-stay surgery, who do not require more long-term patient-controlled analgesia (PCA). ARX-04 is funded in part by the US Army Medical Research and Materiel Command (USAMRMC).

Based on its market research, the Company estimates there are more than 51 million injury-related emergency department visits annually that on average receive 2 doses of opioids for moderate-to-severe acute pain in the United States.

About AcelRx Pharmaceuticals, Inc.
AcelRx Pharmaceuticals, Inc. is a specialty pharmaceutical company focused on the development and commercialization of innovative therapies for the treatment of acute pain. The company's late-stage pipeline includes ARX-04 (sufentanil sublingual tablet, 30 mcg) designed for the treatment of moderate-to-severe acute pain in a medically supervised setting; and Zalviso™ (sufentanil sublingual tablet system) designed for the management of moderate-to-severe acute pain in adult patients in the hospital setting.

ARX-04 delivers 30 mcg sufentanil, a high therapeutic index opioid, sublingually through a disposable, pre-filled, single-dose applicator. AcelRx has reported positive results from the pivotal Phase 3 SAP301 ambulatory surgery study, and has advanced ARX-04 into a study (SAP-302) in emergency room patients. In addition, AcelRx intends to initiate SAP303 in the first quarter of 2016, with a focus on enrolling patients greater than 40 years of age, allowing for administration of ARX-04 for up to 12 hours. Zalviso delivers 15 mcg sufentanil sublingually through a non-invasive delivery route via a pre-programmed, patient-controlled analgesia device. In response to the New Drug Application (NDA) AcelRx submitted to the U.S. Food and Drug Administration (FDA) seeking approval for Zalviso, AcelRx received a Complete Response Letter (CRL) on July 25, 2014. The FDA has requested an additional clinical study (IAP312), which AcelRx is planning to initiate in the first quarter of 2016, to support resubmission of the NDA.

For additional information about AcelRx's clinical programs, please visit www.acelrx.com.

Forward Looking Statements

This press release contains forward-looking statements, including, but not limited to, statements related to the process and timing of anticipated future development of AcelRx's product candidates, including Zalviso and ARX-04; timing and completion of SAP302, timing for initiation of IAP312 for Zalviso and SAP303 studies for ARX-04; AcelRx's plans to seek a pathway forward towards gaining approval of Zalviso in the United States; and anticipated resubmission of the Zalviso NDA to the FDA. These forward-looking statements are based on AcelRx's current expectations and inherently involve significant risks and uncertainties. AcelRx's actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation, risks related to: any delays or inability to obtain and maintain regulatory approval of its product candidates, including Zalviso and ARX-04; its ability to successfully design and complete the additional clinical study requested by the FDA to support resubmission of the Zalviso NDA; its ability to timely resubmit the Zalviso NDA to the FDA and to receive regulatory approval for Zalviso; the fact that the FDA may dispute or interpret differently positive clinical results obtained to date from the pivotal Phase 3 ambulatory surgery study of ARX-04 (SAP301); its ability to complete Phase 3 clinical development of ARX-04; the success, cost and timing of all product development activities and clinical trials, including the SAP302 and SAP303 ARX-04 trials and the IAP312 Zalviso trial; and other risks detailed in the "Risk Factors" and elsewhere in AcelRx's U.S. Securities and Exchange Commission filings and reports, including its Quarterly Report on Form 10-Q filed with the SEC on November 3, 2015. AcelRx undertakes no duty or obligation to update any forward-looking

statements contained in this release as a result of new information, future events or changes in its expectations.

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