AcelRx Pharmaceuticals Advances ARX-04 into Extension Phase of Open-Label Phase 3 Emergency Room Study

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REDWOOD CITY, Calif., March 14, 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, announced the initiation of the extension phase of the SAP302 study 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. This ongoing open-label Phase 3 study has completed the initial phase, enrolling 40 patients who each received a single dose of ARX-04. In this extension phase, up to an additional 100 patients may be enrolled, each of whom may receive multiple doses of ARX-04, given hourly as needed for pain, for up to 4 doses. The primary endpoint of SAP302 is the time-weighted summed pain intensity difference to baseline over one hour, or SPID-1.

Howie Rosen, interim chief executive officer of AcelRx, noted, "Our discussions with the U.S. Food and Drug Administration regarding the regulatory path for ARX-04 have been very productive. As part of our pre-NDA meeting, we agreed to expand enrollment in SAP302 in order to gain experience in patients who require multiple doses of ARX-04 in the emergency department. Pending completion of SAP303 and all other necessary activities, the initiation of this portion of the study keeps us on track for an anticipated submission of an NDA for ARX-04 for the treatment of moderate-to-severe acute pain in a medically supervised setting in the fourth quarter of 2016."

Top-line interim results from the initial phase of SAP302, which were reported in February 2016, showed a clinically meaningful reduction in pain intensity with a single ARX-04 dose and an adverse event profile that was consistent with previous clinical studies. Nausea and somnolence were the most common adverse events and were each reported in two of the 40 patients. There have been no early terminations among participants due to adverse events.

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 U.S. 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 studies in emergency room patients (SAP302) and post-operative patients 40 years and older (SAP303). 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 later this month, 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 for initiation and completion along with anticipated results of IAP312 for Zalviso; anticipated results and timing of the completion of the SAP302 and SAP303 studies for ARX-04; AcelRx's planned pathway forward towards gaining approval of Zalviso in the United States; and anticipated resubmission of the Zalviso NDA to the FDA, including the scope and timing of resubmission. 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, timely enroll 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 SAP301 ambulatory surgery study of ARX-04; 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 Annual Report on Form 10-K filed with the SEC on March 7, 2016. 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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