



## Top-line Data Show AcelRx Pharmaceuticals' ARX-04 Achieves Primary Endpoint in Dose-Finding Phase 2 Clinical Trial

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- Patients in the 30 mcg sufentanil NanoTab treatment group experienced significantly greater reduction in pain as measured by SPID-12 vs. placebo -
- ARX-04 is a rapid-onset, non-invasive investigational pain medication being evaluated for the short-term treatment of acute moderate-to-severe pain in a range of ambulatory care environments -

REDWOOD CITY, Calif., April 24, 2013 /PRNewswire/ --- AcelRx Pharmaceuticals, Inc. (Nasdaq: ACRX), a specialty pharmaceutical company focused on the development and commercialization of innovative therapies for the treatment of acute and breakthrough pain, today announced top-line results demonstrating that a placebo-controlled, dose-finding, Phase 2 study of its investigational single-dose sublingual sufentanil NanoTab for acute pain, ARX-04, successfully met its primary endpoint. Results demonstrated that patients receiving 30 mcg sufentanil NanoTab doses, administered by a healthcare professional, no more frequently than once per hour, had significantly greater pain reduction as measured by Summed Pain Intensity Difference to baseline during the 12-hour study period (SPID-12) than placebo-treated patients ( $p=0.003$ ). Adverse events reported in the study were generally mild-to-moderate in nature, with two serious adverse events of post-surgical infection reported, both of which were determined by the investigator to be unrelated to study drug. Two patients dropped out of the study due to adverse events, one patient's discontinuation considered unrelated to study drug, and the other considered probably related to study drug, both in the 30 mcg-treated group.

"The goal of this program, to find an effective sublingual sufentanil dose that could be administered less frequently by a healthcare professional than our patient-administered ARX-01 product candidate, has been met", said Dr. Pamela Palmer, Chief Medical Officer, AcelRx Pharmaceuticals. "There is a need for a non-invasive, rapid-onset strong analgesic to provide short-term treatment of pain for the wounded soldier on the battlefield, the trauma victim at the site of a road-traffic accident, the migraine patient in the emergency room, or in general for patients in moderate-to-severe acute pain where intravenous access is not readily available."

This study randomized 101 patients following bunionectomy surgery in a 2:2:1 ratio to 30 mcg sufentanil, 20 mcg sufentanil or placebo treatment arms. The intent-to-treat (ITT) population in this study averaged 42.5 years of age with an average Body Mass Index of 28.2, and was evenly balanced for males and females (51%:49%) and ethnicity (54% Caucasian:46% non-Caucasian). Ninety-one percent of patients entering the study completed the full 12-hour study period. SPID-12 scores were +6.53 for 30 mcg sufentanil-treated patients and -7.12 for placebo-treated patients, the difference between the two groups being highly statistically significant ( $p=0.003$ ). The 20 mcg sufentanil-treated patients did not achieve SPID-12 scores that differentiated from placebo.

"The results from this study, funded by the U.S. Army Medical Research and Materiel Command, provide further validation of our sufentanil NanoTab product platform as an effective treatment for moderate-to-severe acute pain," said Richard King, president and CEO of AcelRx. "AcelRx will present these positive top-line data to USAMRMC, and intends to hold an End of Phase 2 meeting with the FDA to define the Phase 3 clinical program for ARX-04."

### About ARX-04

ARX-04 is a product candidate in development for the treatment of moderate-to-severe acute pain, consisting of sufentanil, a high therapeutic index opioid, in AcelRx's proprietary NanoTab technology that enables rapid sublingual absorption when the NanoTab is placed under the tongue. As a result, sufentanil NanoTabs are designed to provide rapid onset of analgesia in a non-invasive method of administration and display a consistent pharmacokinetic profile due to a high percentage of drug being absorbed sublingually instead of through the gastrointestinal tract. We believe ARX-04 may ultimately be proven beneficial in a variety of medically supervised settings, including use in battlefield casualty treatment, by paramedics during patient transport, in the emergency room, for non-surgical patients experiencing pain in the hospital, or for post-operative patients, following either short-stay or ambulatory surgery, who do not require more long-term patient-controlled analgesia (PCA). According to the Centers for Disease Control and Prevention data, there are more than 45 million injury-related emergency department visits and 43 million ambulatory surgery procedures annually in the United States.

### About Acute Pain

In situations of trauma or injury, it is advantageous to have a rapid-acting, non-invasive method of treating acute pain. In the battlefield, in the emergency room and in ambulatory care environments, patients often do not have immediate intravenous, or IV, access available. Intramuscular injections are the current standard of care on the battlefield, but they are invasive, painful, and present an increased risk of infection to both patient and healthcare professional. In addition, in cases of severe trauma where the patient is often in hypovolemic shock and muscles are not well perfused, pain medication given by intramuscular injection may not readily reach the blood stream to provide pain relief, rendering this route of delivery suboptimal. Oral pills and liquids generally have slow and erratic onset of analgesia. Even patients with IV access may have undesirable side effects with the commonly used IV opioids morphine and hydromorphone, such as sedation or oxygen desaturation. Moreover, IV dosing results in high peak plasma levels, thereby limiting the opioid dose and requiring frequent redosing intervals to titrate to satisfactory analgesia. Additional treatment options are needed which can safely and rapidly treat acute pain, in both civilian and military settings.

### 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 and breakthrough pain. AcelRx's lead product candidate, the Sufentanil NanoTab PCA System (ARX-01 or the NanoTab System), is designed to solve the problems associated with post-operative intravenous patient-controlled analgesia, which has been shown to cause harm to patients following surgery because of the side effects of morphine, the invasive IV route of delivery and the complexity of infusion pumps. AcelRx has announced results from two Phase 3 clinical trials for the NanoTab System, and a third Phase 3 study has completed enrollment, with data expected in the second quarter of 2013. A New Drug Application submission is planned for the third quarter of 2013. AcelRx has two additional product candidates, ARX-02 and ARX-03, which have completed Phase 2 clinical development. For additional information about AcelRx's clinical programs please visit [www.acelrx.com](http://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 clinical development of AcelRx Pharmaceuticals' product candidates, including the advancement of ARX-04 beyond Phase 2 clinical trials and the potential submission of an NDA for the NanoTab System and the timing thereof, therapeutic and commercial potential of the NanoTab System, and the anticipated timing of the Phase 3 results and therapeutic and commercial potential of other AcelRx Pharmaceuticals' product candidates. These forward-looking statements are based on AcelRx Pharmaceuticals' current expectations and inherently involve significant risks and uncertainties. AcelRx Pharmaceuticals' 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: the ability of AcelRx Pharmaceuticals to successfully complete the clinical trials for the sufentanil NanoTab System; that subsequent analyses of data by regulatory agencies may be subject to differing interpretations; the success, cost and timing of all product development; the uncertain clinical development process, including the risk that clinical trials have an effective design or deliver data on schedule; AcelRx Pharmaceutical's ability to attract funding partners or collaborators with development, regulatory and commercialization expertise; its ability to obtain sufficient financing to complete registration of its product candidates in the United States and Europe; its ability to obtain and maintain regulatory approvals of its product candidates in the United States and Europe; the market potential for its product candidates; the accuracy of AcelRx Pharmaceuticals' estimates regarding expenses, capital requirements and needs for financing; and other risks detailed in the "Risk Factors" and elsewhere in AcelRx Pharmaceuticals' U.S. Securities and Exchange Commission filings and reports, including its Annual Report on Form 10-K filed with the SEC on March 12, 2013. AcelRx Pharmaceuticals 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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