AcelRx Receives IDE Approval for Niyad and Advances to a Single Registration Study

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Investigational Device Exemption (IDE) approval by the U.S. Food and Drug Administration (FDA) allows AcelRx to begin pivotal study of Niyad™

Single registration study with pre-agreed upon endpoints planned to initiate in Q4 2023 with topline data expected mid-2024; clinical site readiness is currently underway

Niyad would be the first and only approved regional anticoagulant in the U.S. for the extracorporeal circuit, if approved by the FDA

SAN MATEO, Calif., Oct. 3, 2023 /PRNewswire/ -- AcelRx Pharmaceuticals, Inc. (Nasdaq: ACRX), (AcelRx), a specialty pharmaceutical company focused on the development and commercialization of innovative therapies for use in medically supervised settings, today announced that it is advancing Niyad™ (a lyophilized formulation of nafamostat) into a registrational study following the recent approval of an Investigational Device Exemption (IDE) submission to the United States Food and Drug Administration (FDA). This clinical trial will evaluate the safety and efficacy of Niyad to support a premarket approval application (PMA) expected to be submitted in the second half of 2024. The single study will consist of 166 adult patients undergoing renal replacement therapy (RRT) who cannot tolerate heparin or are at risk for bleeding. If approved, Niyad would be the first-ever FDA-approved regional anticoagulant for the extracorporeal circuit in the U.S.

"We are thankful that the FDA has reviewed and approved our IDE application so we can now proceed with our registrational study to evaluate our lead product candidate, Niyad, which we believe to be a paradigm-shifting product candidate for the medically supervised setting," said Vince Angotti, Chief Executive Officer of AcelRx. "We are on track to start this study later this year, with top-line data expected by mid-2024 and submission of a PMA planned in the second half of 2024."

The registration study is designed as a prospective, double-blinded trial to be conducted at approximately 10 U.S. hospital intensive care units that will enroll and evaluate 166 patients that will be randomized into either active or placebo arm. The primary endpoint of the study is mean post-filter activated clotting time (ACT) over the first 24 hours versus placebo. Key secondary endpoints include filter lifespan, number of filter changes over 72 hours, number of transfusions over 72 hours and dialysis efficacy (based on urea concentration) over the first 24 hours.

Pamela P. Palmer, M.D., Ph.D., Chief Medical Officer and Co-Founder of AcelRx, commented, "Importantly, we are encouraged by our interactions with the FDA - not only by being granted Breakthrough Device Designation status for Niyad, but also in terms of the Agency's acceptance of a straightforward registrational trial design to evaluate Niyad. Based on the feedback from the Emergency Use Authorization request, the FDA stated that AcelRx should proceed with the clinical study. We're looking forward to our upcoming Investigator's Meeting just prior to starting enrollment. We remain steadfast in our commitment to advance Niyad for use as a regional anticoagulant during renal replacement therapy, as current options are limited and patients often receive care below the recommended international standards. We also look forward to publishing our independent qualitative market research related to these findings later this year."

About AcelRx Pharmaceuticals, Inc.

AcelRx Pharmaceuticals, Inc. is a specialty pharmaceutical company focused on the development and commercialization of innovative therapies for use in medically supervised settings. AcelRx's lead product candidate, Niyad™ is a lyophilized formulation of nafamostat and is currently being studied under an investigational device exemption, or IDE, as an anticoagulant for the extracorporeal circuit, and has received Breakthrough Device Designation status from the FDA. AcelRx is also developing two pre-filled syringes in-licensed from its partner Aguettant: Fedsyra™, a pre-filled ephedrine syringe, with an expected NDA filing in 2023, and PFS-02, a pre-filled phenylephrine syringe with an expected NDA filing in 2024. This release is intended for investors only. For additional information about AcelRx, please visit www.acelrx.com.

About Nafamostat

Nafamostat is a broad spectrum, synthetic serine protease inhibitor with anticoagulant, anti-inflammatory and potential anti-viral activities. Niyad™ is a lyophilized formulation of nafamostat and is currently being studied under an investigational device exemption, or IDE, as an anticoagulant for the extracorporeal circuit, and has received Breakthrough Device Designation Status from the FDA. LTX-608 is a proprietary nafamostat formulation for direct IV infusion that will be investigated and developed as a potential anti-viral for the treatment of COVID, acute respiratory distress syndrome (ARDS), disseminated intravascular coagulation (DIC) and acute pancreatitis.

Forward-looking statements

This press release contains forward-looking statements based upon AcelRx's current expectations. These and any other forward-looking statements are made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. These statements may be identified by the use of forward-looking terminology such as "potential," "believe," "expect," "expects," "expected," "anticipate," "may," "will," "enable," "should," "seek," "approximately," "intends," "intended," "plans," "planned," "planning," "estimates," "benefits," or the negative of these words or other comparable terminology. The discussion of strategy, plans or intentions may also include forward-looking statements, which are predictions, projections and other statements about future events that are based on current expectations and assumptions. These forward-looking statements involve risks and uncertainties that could cause actual results to differ materially from those projected, anticipated or implied by such statements, including: (i) risks relating to AcelRx's product development activities and ongoing commercial business operations; (ii) risks related to the ability of AcelRx and its business partners to implement development plans, launch plans, forecasts and other business expectations; (iii) risks related to unexpected variations in market growth and demand for AcelRx's commercial and developmental products and technologies; (iv) risks related to AcelRx's liquidity and its ability to maintain capital resources sufficient to conduct the required clinical studies; (v) AcelRx's ability to retain its listing on the Nasdaq exchange; and (vi) risks relating to AcelRx's ability to obtain regulatory approvals for its developmental product candidates. Although it is not possible to predict or identify all such risks and uncertainties, they may include, but are not limited to, those described under the caption "Risk Factors" and elsewhere in AcelRx's annual, quarterly and current reports (i.e., Form 10-K, Form 10-Q and Form 8-K) as filed or furnished with the Securities and Exchange Commission (SEC) and any subsequent public filings. You are cautioned not to place undue reliance on any such forward-looking statements, which speak only as of the date such statements were first made. To the degree financial information is included in this press release, it is in summary form only and must be considered in the context of the full details provided in AcelRx's most recent annual, quarterly or current report as filed or furnished with the SEC. AcelRx's SEC reports are available at www.acelrx.com under the "Investors" tab. Except to the extent required by law,

AcelRx undertakes no obligation to publicly release the result of any revisions to these forward-looking statements to reflect new information, events or circumstances after the date hereof, or to reflect the occurrence of unanticipated events.

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