FDA Authorizes Sorrento Phase 2 Trial of Epidural Resiniferatoxin for the Orphan Indication of Control of Intractable Cancer Pain

October 13, 2021

- Phase 2 trial of resiniferatoxin (RTX) to proceed following clearance from FDA.
- The decision is supported by the Phase 1 data confirming RTX safety for epidural administration without dose limiting toxicity at any doses tested up to 30 ug.
- Safety and initial pain relief observed in patients with advanced cancer support RTX as a good candidate for long-term control of refractory cancer pain in a broad spectrum of patients.
- RTX is a drug that very specifically targets cells carrying the TRPV1 “heat and pain” receptors (the discovery of which led to the 2021 Nobel Prize in Medicine). By modulating pain signaling, RTX has the potential to reduce or completely eliminate pain and the need for high dose opioids in advanced cancer patients.

SAN DIEGO, Oct. 13, 2021 (GLOBE NEWSWIRE) -- Sorrento Therapeutics, Inc. (NASDAQ: SRNE, "Sorrento") announced that the company has received FDA clearance to proceed with a global Phase 2 clinical study of resiniferatoxin (RTX), entitled “A Multicenter, Phase 2 Study to Assess the Safety and Efficacy of Epidural Resiniferatoxin for the Treatment of Intractable Pain Associated with Advanced Cancer”. The Phase 2 trial, a multicenter, double blind, controlled study will assess the “efficacy and safety of several RTX doses vs. placebo controls to manage intractable pain in up to 120 patients with advanced cancer” (NCT05067257). Three RTX dose groups (15, 20 and 25 mcg) will be evaluated against both a vehicle control group and a concurrent control group over a year of follow up. The primary objective of the study is to identify the recommended Phase 3 dose for later studies.

The decisions related to this indication follow the analysis of the significant observations from the Phase 1b trial results (NCT03226574). The Phase 1 was an ascending dose safety study in 17 patients to assess the safety and preliminary efficacy of epidural administration of resiniferatoxin for the treatment of intractable pain due to cancer. RTX was generally well tolerated after epidural administration at doses up to 30 mcg with the most common adverse event of “procedural pain” experienced by just over half of subjects (52.9%) and all of these events were considered at most moderate severity and lasted only a few hours. Pharmacokinetic sampling showed no measurable systemic RTX levels in nearly all subjects. Preliminary efficacy showed promising long-standing benefit in pain reduction.

In the Phase 1 study, clinical efficacy was defined as a 30% decrease in average pain scores (CE30), calculated for both average pain for three consecutive days from original baseline score of ≥ 6 on a scale of 1 to 10 (NRS rating scale) and worst average pain, compared to baseline using the NRS during the 3 months post injection. Eleven of 17 subjects achieved this efficacy endpoint with subjects receiving 15 or 25 mcg showing the best results. For CE50 and CE70, 6 and 4 subjects, respectively, achieved these endpoints again with those receiving 15 or 25 mcg showing best results. These results are promising in view of the challenging, intractable pain conditions due to advanced cancer, however the somewhat small sample size enrolled requires confirmation in larger follow-on studies.

About Resiniferatoxin (RTX)

Resiniferatoxin is a small-molecule derivative (diterpene ester), purified from a cactus-like plant (Euphorbia sp.). It is a highly potent agonist of the transient receptor potential vanilloid-1 (TRPV1) receptor which are specifically upregulated with chronic severe noxious pain. A thousand times “hotter” than pure capsaicin (16 billion Scoville units versus 16 million), and with a high affinity for afferent sensory pain nerves, resiniferatoxin binds to TRPV1 receptors and selectively ablates the neurons responsible for perpetuating chronic severe pain signals experienced by patients.

More information on this trial can be found at www.clinicaltrials.gov (NCT03542838).

About Sorrento Therapeutics, Inc.

Sorrento is a clinical and commercial stage biopharmaceutical company developing new therapies to treat cancer, pain (non-opioid treatments), autoimmunity disease and COVID-19. Sorrento’s multimodal, multipronged approach to fighting cancer is made possible by its extensive immuno-oncology platforms, including key assets such as fully human antibodies (“G-MAB™ library”), immuno-cellular therapies (“DAR-T™”), antibody-drug conjugates (“ADCs”), and oncolytic virus (“Seprehvec™”). Sorrento is also developing potential antiviral therapies and vaccines against coronaviruses, including Abibertinib, COVIGUARD™, COVI-AMG™, COVISHIELD™, COVI-MSC™ and COVIDROPS™; and diagnostic test solutions, including COVITRACK™, COVISTIX™ and COVITRACE™.

Sorrento’s commitment to life-enhancing therapies for patients is also demonstrated by our effort to advance a first-in-class (TRPV1 agonist) non-opioid pain management small molecule, resiniferatoxin (“RTX”), and SP-102 (10 mg, dexamethasone sodium phosphate viscous gel) (SEMDEXA™), a novel, viscous gel formulation of a widely used corticosteroid for epidural injections to treat lumbosacral radicular pain, or sciatica, and to commercialize ZTlido® (lidocaine topical system) 1.8% for the treatment of post-herpetic neuralgia. RTX has completed a Phase 1b trial for intractable pain associated with cancer and a Phase 1b trial in osteoarthritis patients. SEMDEXA is in a pivotal Phase 3 trial for the treatment of lumbosacral radicular pain, or sciatica. ZTlido® was approved by the FDA on February 28, 2018.
Forward-Looking Statements

This press release and any statements made for and during any presentation or meeting contain forward-looking statements related to Sorrento Therapeutics, Inc., under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995 and subject to risks and uncertainties that could cause actual results to differ materially from those projected. Forward-looking statements include statements regarding the expectations for Sorrento's and its subsidiaries' technologies and product candidates, including, but not limited to, resiniferatoxin (RTX), the clinical potential of RTX, including its potential efficacy and the potential for RTX to address long-term control refractory cancer pain in a broad spectrum of patients. RTX's potential to reduce or completely eliminate pain and/or the need for high dose opioids in advanced cancer patients, timing for commencing additional Phase 2 and/or 3 clinical trials for RTX, and the possibility of proceeding to a Phase 3 trial. Risks and uncertainties that could cause our actual results to differ materially and adversely from those expressed in our forward-looking statements, include, but are not limited to: risks related to Sorrento's technologies and prospects, including, but not limited to risks related to seeking regulatory approval for RTX; clinical development risks, including risks in the progress, timing, cost, and results of clinical trials and product development programs; risk of difficulties or delays in obtaining regulatory approvals; risks that clinical study results may not meet any or all endpoints of a clinical study and that any data generated from such studies may not support a regulatory submission or approval; risks that prior test, study and trial results may not be replicated in future studies and trials; risks of manufacturing and supplying drug product; risks related to leveraging the expertise of its employees, subsidiaries, affiliates and partners to assist Sorrento in the execution of its therapeutic antibody product candidate strategies; risks related to the global impact of COVID-19; and other risks that are described in Sorrento's most recent periodic reports filed with the Securities and Exchange Commission, including Sorrento's Annual Report on Form 10-K for the year ended December 31, 2020, and subsequent Quarterly Reports on Form 10-Q filed with the Securities and Exchange Commission, including the risk factors set forth in those filings. Investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release and we undertake no obligation to update any forward-looking statement in this press release except as required by law.

Media and Investor Relations Contact
Alexis Nahama, DVM (SVP Corporate Development)
Email: mediarelations@sorrentotherapeutics.com

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