Zynerba Pharmaceuticals Announces Positive Top Line Results from ZYN002 CBD Gel Phase 1 Multiple Rising Dose Trial and Initiation of Phase 2 Study in Adult Epilepsy Patients with Refractory Focal Seizures

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Two additional Phase 2 clinical trials are expected to begin in 2H16 in patients with Osteoarthritis and Fragile X Syndrome

DEVON, Pa., June 27, 2016 (GLOBE NEWSWIRE) -- Zynerba Pharmaceuticals, Inc. (NASDAQ:ZYNE), a specialty pharmaceutical company dedicated to the development of innovative transdermal synthetic cannabinoid treatments, today announced positive top line results from a Phase 1 multiple rising dose clinical trial of ZYN002 cannabidiol (CBD) gel in development for the treatment of epilepsy, osteoarthritis and Fragile X Syndrome (FXS). The Phase 1 study was a randomized, double-blind, placebo-controlled trial to evaluate the pharmacokinetic (PK) profile and tolerability of several dose levels of ZYN002 (200, 250 and 500 mg) in 24 healthy volunteers and 12 patients with epilepsy. The healthy volunteers and patients were dosed for seven days with either ZYN002 CBD gel or placebo gel.

Top line results from the 24 healthy volunteers ranging from 25 to 53 years old and 12 epilepsy patients from 19 to 65 years old demonstrated that ZYN002 CBD gel was safe and well-tolerated at all dose levels. The PK findings are being used to establish the high and low doses for the Phase 2 studies. The twice daily dosing provided a more favorable PK profile with comparable results between healthy volunteers and epilepsy patients.

Transdermal application of ZYN002 was very well tolerated with minimal skin erythema. Skin dryness at the application site was common for both ZYN002 and placebo gel. Overall, the incidence of adverse events associated with ZYN002 CBD gel was similar to placebo in both healthy volunteers and adult epilepsy patients. There were no reports of somnolence or fatigue and a very low incidence of gastrointestinal events was observed. There were no serious adverse events or discontinuations for healthy volunteers and epilepsy patients receiving ZYN002 CBD gel. One healthy volunteer receiving placebo gel developed a serious adverse event suspected to be a catheter infection and was discontinued from the study.

In addition, healthy volunteers and epilepsy patients had no drug related changes in performance on the Trail Making Test, a test of visual attention, psychomotor ability, and task switching; a divided attention task; and the Paced Auditory Serial Addition Task (PASAT), a test that measures working memory and focused attention. These results indicate that ZYN002 did not produce impairment in critical areas of cognitive functioning often impacted by CNS drugs. No changes in mood symptoms as accessed by the Inventory of Depression and Anxiety Symptoms (IDAS) and the Positive and Negative Affect Schedule (PANAS) were observed for ZYN002 indicating that ZYN002 is not associated with declines in psychological health.

Zynerba also announced the results in epilepsy patients in the single rising dose trial. The results demonstrated that ZYN002 is safe and well tolerated in patients with epilepsy. The incidence of adverse events associated with ZYN002 was similar to placebo and similar to the healthy volunteers.

“We are extremely encouraged by these results from our Phase 1 multiple rising dose trial, which are consistent with the results of the single rising dose trial, and reinforce that ZYN002 CBD gel is well-tolerated across a range of doses,” said Armando Anido, Chairman and CEO of Zynerba. “Importantly, the results from these Phase 1 trials will inform the doses to be evaluated in the Phase 2 clinical trial of ZYN002 CBD gel in adult patients with refractory epilepsy. We have now initiated the baseline phase of the trial, which we have named the STAR 1 study, and we expect to randomize patients and begin dosing in the third quarter of 2016.”

Mr. Anido continued, “In addition to STAR 1 in refractory epilepsy, we also expect to initiate two Phase 2 trials for ZYN002 CBD gel in osteoarthritis and Fragile X Syndrome during the second half of 2016. We expect to report top line results from all three studies in the first half of 2017.”

More About the STAR 1 Study

The STAR 1 (**S**ynthetic **T**ransdermal C**a**nnabidiol for the T**r**eatment of Epilepsy) clinical study is a Phase 2 multi-center, double-blind, placebo-controlled, multi-dose clinical trial designed to evaluate the efficacy and safety of ZYN002 in patients with refractory focal seizures. Approximately 180 patients will be enrolled in the trial and will be followed for 8 weeks during the baseline phase. After the baseline phase, patients will be randomized (1:1:1) to receive one of two doses of ZYN002 or placebo for 12 weeks. The primary endpoint of the study is median percentage change in seizure frequency at 12 weeks. The Company expects to commence dosing in STAR1 during the third quarter of 2016, with preliminary results expected in the first half of 2017.

About ZYN002 CBD Gel

Zynerba’s ZYN002 CBD gel is the first and only synthetic CBD formulated as a patent-protected permeation-enhanced gel and is being studied in refractory epilepsy, Fragile X Syndrome and osteoarthritis. ZYN002 is a clear, permeation-enhanced gel that is designed to provide consistent, controlled drug delivery transdermally with convenient twice-daily dosing. Transdermal therapeutics are absorbed through the skin directly into the systemic circulation, avoiding first-pass liver metabolism and potentially enabling lower dosage levels of active pharmaceutical ingredients and rapid and reliable absorption with high bioavailability. In addition, transdermal delivery avoids the gastrointestinal tract and potential stomach acid degradation of CBD into THC (associated with psychoactive effects), as demonstrated in a Zynerba in vitro study.

About Epilepsy

Epilepsy is a disease characterized by an enduring predisposition to generate epileptic seizures (transient symptoms due to abnormal neuronal activity in the brain) and by the neurobiological, cognitive, psychological and social consequences of the condition. Focal seizures usually start in a small area of the temporal lobe or frontal lobe of the brain and quickly involve other areas of the brain that affect alertness and awareness. Approximately 2.2 million patients in the United States and 3.1 in Europe and Japan battle epilepsy. Focal seizures are the most common type of seizure, representing 35% of all epilepsies.

About Zynerba Pharmaceuticals, Inc.
Zynerba Pharmaceuticals (NASDAQ:ZYNE) is a specialty pharmaceutical company focused on developing and commercializing proprietary next-generation synthetic cannabinoid therapeutics formulated for transdermal delivery. Zynerba is developing therapeutic candidates based on proprietary transdermal technologies that, if successfully developed, may allow sustained, consistent and controlled delivery of therapeutic levels of two cannabinoids: cannabidiol (CBD), a non-psychoactive cannabinoid, and THC. Transdermal delivery has the potential to reduce adverse effects associated with oral dosing. ZYN002, the Company's CBD gel, is the first and only synthetic CBD formulated as a patent protected permeation-enhanced gel and is being studied in refractory epilepsy, FXS and osteoarthritis. Zynerba is also developing ZYN001, which utilizes a synthetically manufactured pro-drug of THC in a transdermal patch to deliver THC through the skin and into the bloodstream. ZYN001 will be studied in fibromyalgia and peripheral neuropathic pain. Learn more at www.zynerba.com and follow the Company on Twitter at @ZynerbaPharma.

Cautionary Note on Forward-Looking Statements
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. We may, in some cases, use terms such as "predicts," "believes," "potential," "proposed," "continue," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should" or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from the Company's current expectations. For example, there can be no guarantee that the Company will obtain approval for ZYN002 or ZYN001 from the U.S. Food and Drug Administration (FDA) or foreign regulatory authorities; even if ZYN002 or ZYN001 are approved, the Company may not be able to obtain the label claims that it is seeking from the FDA. Management's expectations and, therefore, any forward-looking statements in this press release could also be affected by risks and uncertainties relating to a number of other factors, including the following: the success, cost and timing of the Company's product development activities, studies and clinical trials; the success of competing products that are or become available; the Company's ability to commercialize its product candidates; the size and growth potential of the markets for the Company's product candidates, and the Company's ability to service those markets; the Company's ability to develop sales and marketing capabilities, whether alone or with potential future collaborators; the rate and degree of market acceptance of the Company's product candidates; and the Company's expectations regarding its ability to obtain and adequately maintain sufficient intellectual property protection for its product candidates. These and other risks are described in the Company's periodic reports, including the annual report on Form 10K, quarterly reports on Form 10Q and current reports on Form 8-K, filed with or furnished to the Securities and Exchange Commission and available at www.sec.gov. Any forward-looking statements that the Company makes in this press release speak only as of the date of this press release. The Company assumes no obligation to update forward-looking statements whether as a result of new information, future events or otherwise, after the date of this press release.

Investor Contacts
Richard Baron, CFO
Zynerba Pharmaceuticals
484.581.7505
baronr@zynerba.com

Kimberly Minarovich
Argot Partners
212.600.1902
kimberly@argotpartners.com

Media Contact
Eliza Schleifstein
Argot Partners
917-763-8106
eliza@argotpartners.com


Primary Logo

Zynerba Pharmaceuticals, Inc.