Zynerba Pharmaceuticals Announces Dosing of First Patients in Phase 2 STAR 1 Clinical Study of ZYN002 CBD Gel in Adult Epilepsy Patients with Refractory Focal Seizures

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DEVON, Pa., Aug. 01, 2016 (GLOBE NEWSWIRE) -- Zynerba Pharmaceuticals, Inc. (NASDAQ:ZYNE), a clinical-stage specialty pharmaceutical company dedicated to the development of innovative transdermal synthetic cannabinoid treatments, today announced that the first patients have been dosed in the Company's Phase 2 STAR 1 (**S**ynthetic **T**ransdermal C**a**nnabidiol for the T**r**eatment of Epilepsy) clinical trial, a randomized, multi-center, multi-dose study to evaluate ZYN002 cannabidiol (CBD) gel in adult epilepsy patients with refractory focal seizures. ZYN002 CBD gel is the first and only synthetic CBD formulated as a permeation-enhanced gel for transdermal delivery and is in development for the treatment of epilepsy, osteoarthritis (OA) and Fragile X syndrome (FXS).

The STAR 1 clinical trial will evaluate ZYN002 CBD gel in 180 refractory epilepsy patients. The study includes an 8-week baseline period to assess seizure frequency and type. As patients complete the baseline period, they will be randomized 1:1:1 to receive (i)195 mg of ZYN002 4.2% CBD gel every 12 hours, (ii) 97.5 mg of ZYN002 4.2% CBD gel every 12 hours or (iii) placebo gel every 12 hours for 12 weeks. The primary endpoint will assess the median percentage change in seizure frequency over the 12-week treatment period. Safety and tolerability will also be evaluated. The study is being conducted at 14 sites in Australia and New Zealand. Top line results are expected to be reported in the first half of 2017.

"The dosing of the first patients in the STAR 1 clinical trial in adults with refractory epilepsy is a significant milestone for the Company," said Armando Anido, Chairman and CEO of Zynerba Pharmaceuticals. "We are pleased by the pace of the clinical program for ZYN002 and remain on track to initiate additional Phase 2 studies in OA and FXS in the second half of this year. We expect to report top line results from all three studies in the first half of 2017."

The Phase 2 STAR 1 clinical trial is supported by three Phase 1 trials: 1, 7, and 14-day trials with CBD gel doses ranging from 50 mg to 504 mg daily. In the 14-day trial, Zynerba compared a 2.5% CBD concentration to a 4.2% concentration for pharmacokinetics, tolerability and ease of use. The Company found the 4.2% concentration demonstrated a comparable pharmacokinetic and tolerability profile to the 2.5% concentration with an easier to use, lower volume formulation.

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 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-sponsored 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 clinical-stage 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. In June 2016, the company initiated the Phase 2 STAR 1 clinical study of ZYN002 CBD gel in refractory epilepsy patients with focal seizures. Phase 2 studies in osteoarthritis and Fragile X syndrome (FXS) are expected to initiate in the second half of 2016. Zynerba is also developing ZYN001, which utilizes a synthetically manufactured pro-drug of THC. A Phase 1 clinical study for ZYN001 is planned to begin in the second half of 2016. 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.

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