Zynerba Pharmaceuticals Achieves Target Enrollment in Exploratory Phase 2 Trial of ZYN002 in Fragile X Syndrome

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- Top-line data from this study remains on track to report in September 2017 -

DEVON, Pa., June 08, 2017 (GLOBE NEWSWIRE) -- Zynerba Pharmaceuticals, Inc. (NASDAQ:ZYNE), a clinical-stage specialty pharmaceutical company dedicated to developing and commercializing innovative transdermal synthetic cannabinoid treatments, today announced that it has met its enrollment target of 16 patients in its Phase 2 FAB-C (Treatment of **F**ragile X Syndrome **A**nxiety and **B**ehavioral Challenges with **C**BD) clinical trial evaluating ZYN002 cannabidiol (CBD) gel in children with Fragile X syndrome (FXS). ZYN002 CBD gel is the first and only patent-protected, synthetic CBD that is formulated as a permeation-enhanced gel for transdermal delivery, and was awarded orphan drug designation by the U.S. Food and Drug Administration for the treatment for FXS.

Patients between the ages of 6 and 17 years with Fragile X syndrome have been enrolled in the exploratory FAB-C trial, which is designed to evaluate the safety and efficacy of ZYN002 CBD gel administered over a 12-week period in FXS. The study is evaluating 50 mg of CBD in ZYN002 4.2% gel once daily, which may be titrated up to 125 mg two times per day during the six-week titration period. Between weeks six and twelve, patients will receive a maintenance dose of 50 mg, 100 mg or 250 mg daily of CBD in ZYN002 4.2% gel. The primary outcome measures include changes in anxiety, depression and mood as measured by the ADAMS scale, a patient reported outcomes questionnaire that has been validated in FXS. Other measurements that are being observed include the Aberrant Behavior Checklist and visual analog scale (VAS) to assess for hyperactivity/impulsivity.

“This achievement marks a key milestone for us and for families facing the challenges of Fragile X syndrome,” said Armando Anido, Chairman and Chief Executive Officer of Zynerba. “Fragile X is a devastating genetic disorder for patients and their caregivers. We believe that synthetic CBD holds great therapeutic promise in this setting, as it may compensate for the dysregulation of the endocannabinoid pathway, and in doing so may treat many of the associated neurological symptoms. We look forward to reporting data from this trial in September of this year.”

Fragile X syndrome is an autism spectrum disorder and the most common inherited intellectual disability in males and a significant cause of intellectual disability in females. It is caused by a mutation in the Fragile X Mental Retardation gene located on the X chromosome and leads to dysregulation of the endocannabinoid pathway including the reduction in endogenous cannabinoids (2-AG and anandamide). The disorder negatively affects synaptic function, plasticity and neuronal connections, and results in a spectrum of intellectual disabilities, social anxiety and memory problems. In the US, there are about 71,000 patients suffering with FXS.

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 adult epilepsy patients with focal seizures, in osteoarthritis and in children with FXS. ZYN002 is a clear, permeation-enhanced gel that is designed to provide consistent, controlled drug delivery transdermally with once- or 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 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 tetrahydrocannabinol (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 March 2017, the Company completed enrollment in the Phase 2 STAR 1 (Synthetic Transdermal Cannabidiol for the Treatment of Epilepsy) clinical trial of ZYN002 CBD gel in refractory epilepsy patients with focal seizures, the most common form of epilepsy in adults. Also in March 2017, the Phase 2 STOP (Synthetic Transdermal Cannabidiol for the Treatment of Knee Pain due to Osteoarthritis) clinical trial in patients with knee pain due to osteoarthritis was fully enrolled. In June 2017, the Company completed target enrollment in its exploratory Phase 2 FAB-C (Treatment of Fragile X Syndrome Anxiety and Behavioral Challenges with CBD) clinical trial in children with Fragile X syndrome. 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 by the end of the first half of 2017. 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. In addition, the Company’s cash and cash equivalents may not be sufficient to support its operating plan for as long as anticipated. 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. This list is not exhaustive and these and other risks are described in the Company's periodic reports, including the annual report on Form 10-K, quarterly reports on Form 10-Q 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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