Zynerba Pharmaceuticals Announces ZYN002 Granted Orphan Drug Designation for Fragile X Syndrome

February 25, 2016

DEVON, Pa., Feb. 25, 2016 (GLOBE NEWSWIRE) -- In a release issued under the same headline earlier today by Zynerba Pharmaceuticals, Inc. (NASDAQ:ZYNE), please note that in the second paragraph of the release, the quote should end with "in the second half of this year," instead of "by the end of this year."

The corrected release follows:

Zynerba Pharmaceuticals, Inc. (NASDAQ:ZYNE), a specialty pharmaceutical company dedicated to the development of innovative transdermal synthetic cannabinoid treatments, today announced that the U.S. Food and Drug Association has granted orphan-drug designation to ZYN002 cannabidiol (CBD) gel, for the treatment of Fragile X syndrome (FXS). Fragile X syndrome is a genetic condition that causes intellectual disability, anxiety disorders, behavioral and learning challenges and various physical characteristics.

"Fragile X syndrome is a rare but debilitating genetic condition for which there are no effective treatment options," said Armando Anido, Chairman and CEO of Zynerba Pharmaceuticals. "By granting orphan drug designation to this promising synthetic CBD compound, the FDA recognizes the significant need for new therapies, and we are working with key opinion leaders to advance ZYN002 CBD Gel into a Phase 2 clinical study in the second half of this year."

Orphan-drug designation by the FDA is granted to novel drugs that treat a rare disease or condition affecting fewer than 200,000 patients in the U.S. The designation provides the drug developer with a seven year period of U.S. marketing exclusivity upon marketing approval for the designated indication, as well as with tax credits for clinical research costs, the ability to apply for annual grant funding, clinical research trial design assistance and the waiver of prescription drug user fees.

CBD has been shown to reduce the metabolism of two endocannabinoids (2-AG and anandamide) in laboratory studies. The reduction in metabolism increases 2-AG and anandamide concentrations, which modulates neurotransmitter release and restores endogenous stimulation of endocannabinoid receptors. In mouse knockout models for FXS, symptoms are improved when endocannabinoid levels are increased. There is also anecdotal evidence that high levels of CBD oil from plants have proven effective at improving socialization and language skills in children.

ZYN002 is currently being evaluated in a Phase 1 multiple rising dose trial in healthy volunteers and patients with epilepsy. Zynerba expects to report results from this Phase 1 study in the first half of 2016.

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 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 *in vitro* study.

About Fragile X Syndrome
Fragile X syndrome is a genetic condition that causes intellectual disability, anxiety disorders, behavioral and learning challenges and various physical characteristics. The impairment can range from learning disabilities to more severe cognitive or intellectual disabilities. Patients with Fragile X syndrome exhibit autism-like symptoms including cognitive impairment, anxiety and mood swings, attention deficit and heightened stimuli. Approximately 71,000 patients in the US battle Fragile X syndrome.

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, Fragile X syndrome 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 under the heading "Risk Factors" in the registration statement on Form S-1 (commission file number 333-205355), which was declared effective by the Securities and Exchange Commission on August 4, 2015. 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
Angeli Kolhatkar Argot Partners 212.600.1902 angeli@argotpartners.com

Media Contact
Eliza Schleifstein Argot Partners 973.361.1546 eliza@argotpartners.com


Primary Logo

Zynerba Pharmaceuticals, Inc.