Zynerba Pharmaceuticals Provides 2018 Clinical and Corporate Update

January 3, 2018

- Focusing Strategy on Rare and Near-Rare Neurological and Psychiatric Disorders -

- Pediatric Neurologist Liza Squires, M.D. Joins as Chief Medical Officer -

- Conference Call to be Held at 8:30 AM Tomorrow, January 4, 2018 -

DEVON, Pa., Jan. 03, 2018 (GLOBE NEWSWIRE) -- Zynerba Pharmaceuticals, Inc. (NASDAQ:ZYNE), a clinical-stage specialty pharmaceutical company dedicated to developing and commercializing innovative pharmaceutically-produced transdermal cannabinoid treatments, today announced that it will concentrate its focus on rare (meeting the US FDA designation of an orphan disease, affecting fewer than 200,000 people in the U.S.) and near-rare (affecting fewer than one million people in the U.S.) neurological and psychiatric ("neuropsychiatric") disorders with high unmet medical needs. In 2018, the Company intends to develop ZYN002 in a pivotal Phase 2/3 program in Fragile X syndrome (FXS) and in Phase 2 programs in refractory epilepsies, including adult refractory focal epilepsy and developmental and epileptic encephalopathies (DEE) in pediatric and adolescent patients. Additionally, the Company plans to initiate Phase 2 development of ZYN001 in Tourette Syndrome by year end 2018.

"The decision to concentrate on rare and near-rare neurological and psychiatric disorders is driven by a number of important factors, including the compelling ZYN002 clinical data seen to date in Fragile X syndrome and refractory focal epilepsy and by the opportunities for a more efficient and rapid development and regulatory approval process," said Armando Anido, Zynerba's Chairman and Chief Executive Officer. "We believe that the strategy also allows us to maximize the potential for ZYN002 and ZYN001 with an efficient commercial strategy, and opportunities for consistent pricing across indications. All told, we believe we are well positioned to execute on this strategy, including having sufficient capital to fund operations well into 2019."

2018 Anticipated Milestones

"As we head into 2018, we look forward to delivering on a number of significant corporate and clinical milestones that are important to our investors and the patients we seek to treat," said Anido. "We view 2018 as a year of corporate evolution for Zynerba. We plan to initiate a pivotal study for ZYN002 in FXS that should read out in 2019, initiate two new Phase 2 studies in refractory epilepsies, and initiate a Phase 2 program with ZYN001 in Tourette Syndrome. We also will continue to collect a significant amount of important data from our ongoing open-label extension studies in Fragile X syndrome and adult refractory focal epilepsy that we expect to present and/or publish during the year."

ZYN002 Development Plans

Zynerba is currently developing ZYN002, the first and only patent-protected, pharmaceutically-produced CBD that is formulated as a permeation-enhanced gel for transdermal delivery.

- The Company has a meeting scheduled with the U.S. Food and Drug Administration during the first quarter of 2018 to discuss development strategy including a pivotal clinical program for ZYN002 in FXS, an inherited autism spectrum disorder affecting approximately 71,000 patients in the U.S. The Company expects to initiate the pivotal, double-blind placebo controlled clinical trial mid-year 2018. Zynerba has received U.S. Orphan Drug designation for the use of CBD as treatment of FXS;
- The Company expects to initiate an open-label study of ZYN002 in developmental and epileptic encephalopathies (DEE), a category of rare and ultra-rare, severe brain disorders manifesting with seizures or EEG abnormalities that can directly worsen cognition or behavior. The study will initiate in the first half of 2018 and will enroll approximately 48 pediatric and adolescent patients. It is designed to identify new indications to take into blinded, placebo-controlled studies;
- Zynerba intends to initiate a Phase 2B double-blind placebo controlled clinical trial of ZYN002 in adult refractory focal epilepsy, a subcategory of epilepsy impacting approximately 500,000 patients who are refractory to other anti-epileptic drugs (AED), in the second half of 2018. The design of this study will be modified from the STAR 1 trial design based on the data presented at the 2017 American Epilepsy Society meeting and additional analysis of the STAR 1 and STAR 2 data. Changes to the trial design based on these data are expected to include an increase in the number of patients enrolled, changes in randomization methodologies utilized, an increase in baseline seizure frequency and an increase in the length of the study;
- The Company will continue to generate important open label data in the ongoing 12-month FAB-C extension study in pediatric and adolescent patients with FXS and the ongoing STAR 2 18-month extension study in adult refractory focal epilepsy and plans on presenting/publishing updates in 2018;
- Zynerba is assessing the potential for ZYN002 in other rare and near-rare neuropsychiatric indications;
- Zynerba is discontinuing its investment into the capital-intensive pain space, due to inconsistency with its rare and near-rare neuropsychiatric focus. As a result, although the initial Phase 2 STOP data were encouraging, the Company has discontinued investment into the ZYN002 osteoarthritis program.

ZYN001 Development Plans

Zynerba is currently in Phase 1 development of ZYN001, the Company's patent-protected, pro-drug of tetrahydrocannabinol (THC) delivered via a transdermal patch.

- The Company expects to complete its Phase 1 evaluation of multiple formulations of ZYN001 in the first half of 2018;
- The Company expects to take ZYN001 into a Phase 2 clinical trial in Tourette Syndrome (TS), a neurodevelopmental disorder characterized by motor and vocal tics, late in the second half of 2018. The National Institute of Neurological Disorders and Stroke estimate that 200,000 Americans have the most severe form of TS, and as many as one in 100 exhibit milder and less complex symptoms such as chronic motor or vocal tics. Two third-party controlled Phase 2 trials of THC in Tourette Syndrome have shown significant reduction in tics compared to placebo;
- Zynerba is assessing the potential for ZYN001 in other rare and near-rare neuropsychiatric indications;
- The Company is discontinuing its investment into the capital-intensive pain space, including its pursuit of fibromyalgia and general peripheral neuropathic pain (PNP) indications for ZYN001, due to inconsistency with its rare and near-rare neuropsychiatric focus.

Appointment of new Chief Medical Officer

To complement this concentration in rare and near-rare neuropsychiatric disorders, Liza A. Squires, MD, a pediatric neurologist, has been appointed to the newly created role of Chief Medical Officer reporting to Terri Browning Sebree. Dr. Squires will have oversight of the Company's clinical strategy and activities, including clinical research, medical affairs, regulatory affairs, and drug safety and surveillance. Dr. Squires brings over 25 years of experience in rare and neuropsychiatric disorders to Zynerba, and was most recently the Vice President and Therapeutic Area Head of Neuroscience for Aevi Genomic Medicine, where she led neuroscience product strategy and development. She served as Chief Medical Officer for Lumos Pharma, where she developed an accelerated development strategy for an ultra-orphan population. She spent nearly 10 years at Shire Pharmaceuticals, culminating in her tenure as Vice President of R&D and Product General Manager of CNS Early Pipeline. Dr. Squires served as the Director of Pediatric Neurology for DeVos Children's Hospital. She is a Diplomate of the American Board of Pediatrics and the American Board of Neurology and Psychiatry, with special competence in Child Neurology, and received her Medical Doctorate from the Michigan State University College of Human Medicine.

"I'm pleased to announce the addition of Dr. Liza Squires as our new Chief Medical Officer; she brings a wealth of clinical, medical, and biotechnology industry experience in pediatric neurology and rare neuropsychiatric disorders," said Terri Browning Sebree, Zynerba's President. "We have an excellent team at Zynerba, with a proven track record in orphan drugs, CNS disorders and transdermal delivery. Liza's pediatric neurology background and insights will complement our team well. We welcome her to the Company and look forward to her contributions to the development of our pipeline."

Financial Status

Zynerba believes it can fund operations and capital requirements well into 2019. As of September 30, 2017, the Company had a cash and cash equivalent position of $66.3 million. An additional $3.0 million in net proceeds from shares sold in September and October 2017 under its ATM program was recorded in the fourth quarter of 2017.

Conference call information
Zynerba management will host a live conference call and multimedia webcast tomorrow, January 4, 2018 at 8:30 am Eastern Time to provide a corporate update. The call can be accessed by dialing (866) 573-0180 (U.S. and Canada) or (430) 775-1345 (international) and referencing conference ID 2395619. To access the live webcast or the replay, visit the investor page of the Company's website at http://ir.zynerba.com/. The webcast will be recorded and available on the Company's website for 30 days. A PDF of the slides will also be available in the News, Events and Webcasts section of the Company's investor page.

About ZYN002
Zynerba's ZYN002 CBD gel is the first and only pharmaceutically-produced CBD formulated as a patent-protected permeation-enhanced gel and is being studied in children with Fragile X syndrome and in refractory epilepsies. ZYN002 is a clear, permeation-enhanced gel that is designed to provide controlled drug delivery transdermally with once- or twice-daily dosing.

About ZYN001
ZYN001 is a synthetic pro-drug of THC in a state-of-the-art drug-adhesive matrix transdermal patch, currently in Phase 1 clinical development. THC is a CB_1 agonist which acts at many sites along the central cannabinoid receptor system, and has been shown to be effective in the treatment of motor and vocal tics. A pro-drug is a drug administered in an inactive or less active form and designed to enable more effective delivery, which is then converted into an active form through a normal metabolic process.

About Our Technology
Cannabinoids are a class of chemical compounds found in the *Cannabis* plant. The two primary cannabinoids contained in *Cannabis* are cannabidiol, or CBD, and ∆9-tetrahydrocannabinol, or THC. Clinical and preclinical data support the potential for CBD in treating epilepsy, Fragile X Syndrome and other neuropsychiatric disorders, and THC has positive effects on treating tics. Zynerba is developing therapeutic medicines that utilize innovative transdermal technologies that, if successful, may allow for sustained and controlled delivery of therapeutic levels of CBD and THC. Transdermal delivery of cannabinoids may have benefits over oral dosing because it allows the drug to be absorbed through the skin directly into the bloodstream. This avoids first-pass liver metabolism, potentially enabling lower dosage levels of active pharmaceutical ingredients with a higher bioavailability and improved safety profile. Transdermal delivery also avoids the gastrointestinal tract, lessening the opportunity for GI related adverse events and the potential degradation of CBD by gastric acid into THC, which may be associated with unwanted psychoactive effects. Using an established chemical pharmaceutical process for manufacturing, Zynerba replicates the CBD and THC found in the *Cannabis* plant. We believe that this will allow us to meet stringent global regulatory agencies' standards while ensuring that we can efficiently supply the amount of product required to meet the demand of the markets that we are targeting.

About Zynerba Pharmaceuticals, Inc.
Zynerba Pharmaceuticals (NASDAQ:ZYNE) is a clinical-stage specialty neuropsychiatric pharmaceutical company dedicated to developing and commercializing innovative pharmaceutically-produced transdermal cannabinoid treatments for rare or near-rare neuropsychiatric diseases with high unmet medical needs. We are dedicated to improving the lives of people with severe health conditions by developing cannabinoid medicines designed to meet the rigorous efficacy and safety standards established by global regulatory agencies. Through the discovery and development of these life-changing medicines, Zynerba seeks to improve the lives of patients battling severe, chronic health conditions including Fragile X syndrome, refractory epilepsies, Tourette Syndrome, and other neuropsychiatric disorders. 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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