Zynerba Pharmaceuticals Initiates Open-Label Phase 2 Trial of ZYN002 in Developmental and Epileptic Encephalopathies (DEE)

April 10, 2018

DEVON, Pa., April 10, 2018 (GLOBE NEWSWIRE) -- Zynerba Pharmaceuticals, Inc. (NASDAQ:ZYNE), a clinical-stage specialty neuropsychiatric pharmaceutical company dedicated to developing and commercializing innovative pharmaceutically-produced transdermal cannabinoid treatments for rare and near-rare neurological and psychiatric disorders with high unmet medical needs, today announced that it has initiated the Phase 2 BELIEVE 1 (Open La**b**el Study to Assess the Safety and **E**fficacy of ZYN002 Administered as a Transderma**l** Gel to Ch**i**ldren and Adol**e**scents with De**v**elopmental and **E**pileptic Encephalopathy) clinical trial. The study will assess the safety and efficacy of ZYN002 in DEE, including Lennox-Gastaut and Dravet syndromes. ZYN002 is the first and only pharmaceutically-produced cannabidiol (CBD) formulated as a patent-protected permeation-enhanced transdermal gel.

“DEE is a heterogeneous group of epilepsy syndromes that involve significant developmental impairment or regression of developmental progress, and are highly resistant to treatment,” said Dr. Liza Squires, Zynerba’s Chief Medical Officer. “Little attention is paid to DEE outside of Lennox-Gastaut and Dravet syndromes despite the high unmet need. The BELIEVE 1 study will assess children and adolescents with a variety of epilepsy syndromes that are characterized as DEE. Our goal is to deliver an effective, well tolerated, and easy to administer therapeutic option for individuals living with DEE and their families.”

The six-month BELIEVE 1 clinical trial is an open label multi-dose Phase 2 clinical trial designed to evaluate the efficacy and safety of ZYN002 in children and adolescents (three to <18 years) with DEE as classified by the International League Against Epilepsy (ILAE) (Scheffer et al. 2017). Approximately 50 patients with confirmed DEE will be enrolled in the clinical trial, approximately half of whom may have either Dravet or Lennox-Gastaut syndrome. Enrolled patients will receive weight-based initial doses of 125 mg BID (250 mg daily) or 250 mg BID (500 mg daily) of ZYN002 CBD gel. The primary efficacy assessment is change in seizure frequency. The Company expects to report topline results in 2019.

About Developmental and Epileptic Encephalopathies (DEE)
DEE is a heterogeneous group of epilepsy syndromes that may be associated with severe cognitive impairment and behavioral disturbances. These disorders are often progressive, and are highly resistant to treatment. DEE includes a number of rare and ultra-rare epilepsy syndromes including early myoclonic encephalopathy, epileptic encephalopathy with continuous spike and wave during sleep, and certain syndromes including Ohtahara, West, Landau-Kleffner, Lennox-Gastaut, Doose and Dravet. Improved seizure control may have a positive impact on development and quality of life.

About ZYN002
Zynerba’s ZYN002 CBD gel is the first and only pharmaceutically-produced CBD formulated as a patent-protected permeation-enhanced transdermal gel and is being studied in children and adolescents with Fragile X syndrome and developmental and epileptic encephalopathies, and in adult epilepsy patients with focal seizures. ZYN002 is a clear, permeation-enhanced gel that is designed to provide controlled drug delivery transdermally with once- or twice-daily dosing.

About Zynerba Pharmaceuticals, Inc.
Zynerba Pharmaceuticals (NASDAQ:ZYNE) is a clinical-stage specialty 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 potentially 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 from the U.S. Food and Drug Administration (FDA) or foreign regulatory authorities; even if ZYN002 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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Source: Zynerba Pharmaceuticals, Inc.