Zynerba Pharmaceuticals Announces Achievement of Enrollment Target in Phase 2 Trial of Zygel™ in Autism Spectrum Disorder

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- Topline Results from BRIGHT Study Expected in the Second Quarter of 2020 -

DEVON, Pa., Jan. 13, 2020 (GLOBE NEWSWIRE) -- Zynerba Pharmaceuticals, Inc. (NASDAQ:ZYNE), the leader in innovative pharmaceutically-produced transdermal cannabinoid therapies for rare and near-rare neuropsychiatric disorders, today announced the achievement of its enrollment target for the 14-week Phase 2 BRIGHT (An Open-La**b**el Tole**r**ab**i**lity and Efficacy Study of ZYN002 Administered as a Transdermal **G**el to C**h**ildren and Adolescen**t**s with Autism Spectrum Disorder) trial of Zygel™ for the treatment of pediatric and adolescent patients with autism spectrum disorder (ASD). The Company expects to announce topline results from this study in the second quarter of 2020.

"We are committed to developing new treatment options to improve outcomes in patients suffering from a variety of neuropsychiatric conditions including autism spectrum disorder, and achieving our enrollment target in the BRIGHT trial is an especially meaningful step toward this goal," said Joseph M. Palumbo, MD, FAPA, MACPsych, Chief Medical Officer of Zynerba. "We would like to thank everyone involved in this study to date, particularly the participating patients and their caregivers, as well as the investigators. We look forward to announcing topline results from this study in the second quarter of 2020."

The 14-week exploratory Phase 2 BRIGHT trial has enrolled 36 patients with ASD at a single clinical site in Australia. The trial is designed to evaluate the efficacy and safety of Zygel in children and adolescents (ages four through 17) with ASD as confirmed by DSM-5 diagnostic criteria for ASD. Enrolled patients are receiving weight-based initial doses of 250 mg daily or 500 mg daily of Zygel. The efficacy assessments include the Aberrant Behavior Checklist (ABC), Parent Rated Anxiety Scale – Autism Spectrum Disorder (PRAS-ASD), Autism Impact Measure (AIM), the Children's Sleep Habit Questionnaire (CSHQ), and Clinical Global Impression – Severity and Improvement (CGI-S, CGI-I). After completing dosing in the 14-week period, participants may enroll in a six-month extension trial.

Using the Autism Diagnostic Observation Schedule (ADOS-2) which is administered at baseline by a qualified clinician, 94% of enrolled patients had moderate-to-severe symptoms of ASD at baseline. The mean baseline ABC-C Irritability subscale score of 30.0 further supports the severity of the enrolled patient population. Thirty-three (92%) are male. The mean age of these patients is 9.3 years.

About Autism Spectrum Disorder (ASD)
Autism Spectrum Disorder is a developmental disorder that affects communication and behavior in approximately one million pediatric and adolescent patients between the ages of five and 17 in the U.S. It refers to a range of conditions characterized by anxiety, repetitive patterns of behavior, impairments in social communication including verbal and non-verbal communication, and deficits in developing and maintaining relationships. Although autism can be diagnosed at any age, it is said to be a "developmental disorder" because symptoms generally appear in the first two years of life. Research suggests that genes can act together with influences from the environment to affect development in ways that lead to ASD. Newer studies suggest that ASD is linked to disruption in the endocannabinoid system.

About Zygel™
Zygel (cannabidiol [CBD] gel) is the first and only pharmaceutically-manufactured CBD formulated as a patent-protected permeation-enhanced clear gel, designed to provide controlled drug delivery into the bloodstream transdermally (i.e. through the skin). Recent studies suggest that Fragile X syndrome (FXS) and other neuropsychiatric conditions including ASD may be associated with a disruption in the endocannabinoid (EC) system. Clinical and anecdotal data suggest that CBD may modulate the EC system and improve certain core social and behavioral autism-related symptoms, including social avoidance and anxiety.

Zygel is being studied in clinical trials in a number of rare and near-rare neuropsychiatric conditions. Target enrollment has been achieved in the Phase 2 BRIGHT trial of Zygel in ASD, with topline data anticipated in the second quarter of 2020. Enrollment is ongoing in CONNECT-FX, a multi-national, randomized, double blind, placebo-controlled pivotal clinical trial of Zygel in Fragile X syndrome (https://www.connectfxtrial.com/) and in the Phase 2 INSPIRE trial in 22q11.2 deletion syndrome. Zynerba also expects to meet with the U.S. Food and Drug Administration in the first half of 2020 to discuss the recently announced positive topline safety and efficacy data from its Phase 2 BELIEVE 1 clinical trial in patients with developmental and epileptic encephalopathies, and the clinical path forward.

About Zynerba Pharmaceuticals, Inc.
Zynerba Pharmaceuticals is the leader in pharmaceutically-produced transdermal cannabinoid therapies for rare and near-rare neuropsychiatric disorders. We are committed to improving the lives of patients and their families living with severe, chronic health conditions including Fragile X syndrome, autism spectrum disorder, 22q11.2 deletion syndrome, and a heterogeneous group of rare and ultra-rare epilepsies known as developmental and epileptic encephalopathies. Learn more at www.zynerba.com and follow us 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 Zygel from the U.S. Food and Drug Administration (FDA) or foreign regulatory authorities; even if Zygel is 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 Company's cash and cash equivalents may not be sufficient to support its operating plan for as long as anticipated; the Company's ability to obtain additional funding to support its clinical development programs; the results, cost and timing of the Company's clinical development programs, including any delays to such clinical trials relating to enrollment or site initiation; clinical results for the Company's product candidates may not be replicated or continue to occur in additional trials and may not otherwise support further development in a specified indication or at all; actions or advice of the FDA and foreign regulatory agencies may affect the design, initiation, timing, continuation and/or progress of clinical trials or result in the need for additional clinical trials; the Company's ability to obtain and maintain regulatory approval for its product candidates, and the labeling under any such approval; the Company's reliance on third parties to assist in conducting pre-clinical and clinical trials for its product candidates; delays, interruptions or failures in the manufacture and supply of the Company's product candidates 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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