



Zynerba Pharmaceuticals Initiates Phase 2 Trial of Zygel™ in Autism Spectrum Disorder

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- Topline Data from BRIGHT Study Expected in the First Half 2020 -

- Company Selects Zygel™ as Brand Name for ZYN002 CBD Transdermal Gel -

DEVON, Pa., March 07, 2019 (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 that it has initiated the Phase 2 BRIGHT (An Open-Label Tolerability and Efficacy Study of ZYN002 Administered as a Transdermal Gel to Children and Adolescents with Autism Spectrum Disorder) trial. The trial will assess the safety, tolerability and efficacy of Zygel (previously referred to as ZYN002) for the treatment of child and adolescent patients with Autism Spectrum Disorder (ASD). The Company expects to present topline data from this study in the first half of 2020.

"Autism spectrum disorder can have a devastating impact on a child and their family," said Armando Anido, Chairman and Chief Executive Officer of Zynerba. "The medical need is significant and unmet despite high awareness and advocacy efforts. Though there has been an accelerating rate of diagnosis, to date there are only two FDA approved products indicated for the treatment of ASD symptoms. Both have significant side effect profiles, and neither have been approved to address the key symptoms of social impairment and anxiety. We are excited to initiate the BRIGHT trial evaluating the role of Zygel in ASD and are hopeful that Zygel may improve some of the core social and behavioral symptoms of ASD. We look forward to presenting topline data in the first half of 2020."

The 14-week BRIGHT trial is an open-label multi-dose Phase 2 clinical trial designed to evaluate the efficacy and safety of Zygel in approximately 36 children and adolescents (ages four through 17) with ASD as confirmed by DSM-5 diagnostic criteria for ASD. Enrolled patients will receive weight-based initial doses of 250 mg daily or 500 mg daily of Zygel. The efficacy assessments include the Aberrant Behavior Checklist, Parent Rated Anxiety Scale – Autism Spectrum Disorder, Autism Impact Measure, and Clinical Global Impression – Severity and Improvement.

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.

About Zygel™

Zygel (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 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.

Enrollment is ongoing in a multi-national, randomized, double blind placebo controlled Clinical study of Cannabidiol (CBD) in Children and Adolescents with Fragile X (CONNECT-FX), a pivotal clinical trial of ZYN002 in FXS (<https://www.connectfxtrial.com/>); topline data from CONNECT-FX are expected in the second half of 2019. Additionally, Zynerba expects topline data from its Phase 2 Open Label Study to Assess the Safety and Efficacy of ZYN002 Administered as a Transdermal Gel to Children and Adolescents with Developmental and Epileptic Encephalopathy (BELIEVE 1) clinical trial in the third quarter of 2019.

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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