Zynerba Pharmaceuticals Announces European Commission Has Granted Orphan Drug Designation for Zygel™ in Fragile X Syndrome

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Provides 10-year market exclusivity in EU upon regulatory approval

DEVON, Pa., Feb. 28, 2022 (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 the European Commission (EC) has granted orphan drug designation to cannabidiol, the active ingredient in its transdermal gel, Zygel™, for the treatment of Fragile X syndrome (FXS).

“Orphan drug designation by the EC is another important milestone for Zygel,” said Armando Anido, Chairman and Chief Executive Officer of Zynerba. “It underscores the urgent, unmet medical need for patients diagnosed with Fragile X syndrome, and will benefit us as we continue to advance our clinical programs and prepare to bring Zygel to market.”

Zynerba is currently enrolling patients in RECONNECT, a confirmatory pivotal Phase 3 trial of Zygel in children and adolescents with FXS, and continues to expect topline results from this trial in the second half of 2023. As previously announced, the Company believes, based on the EMA’s scientific advice, that the successful completion of the current development program for Zygel in FXS will satisfy the requirements of a marketing authorization application in the European Union (EU).

Orphan drug designation is granted to medicines that treat, prevent or diagnose a life-threatening or chronically debilitating rare disease, with a prevalence in the EU of not more than 5 in 10,000, and with either no currently approved method of diagnosis, prevention or treatment or with significant benefit to those affected by the disease. The designation potentially provides certain benefits to Zynerba, including 10-year EU market exclusivity upon regulatory approval, if received, reductions in EMA application fees, and access to protocol assistance.

The Company believes that there are approximately 105,000 patients with FXS in the EU and approximately 121,000 in Europe if the United Kingdom (UK) is included. Approximately 60% of all patients are believed to be completely methylated, which indicates that there are approximately 73,000 patients with FXS who are completely methylated in the EU and UK.

As previously disclosed, Zygel has also been granted orphan drug designation by the U.S. Food and Drug Administration (FDA) for the treatment of FXS and 22q11.2 deletion syndrome. Additionally, the FDA has granted Fast Track designation for Zygel for the treatment of behavioral symptoms associated with FXS. Based on latest census data available, we believe there are approximately 78,000 patients with FXS in the U.S., and approximately 47,000 of those patients are completely methylated.

About Zygel™

Zygel is the first and only pharmaceutically-manufactured cannabidiol 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 cannabidiol may modulate the endocannabinois system and improve certain behavioral symptoms associated with neuropsychiatric conditions. Zygel is an investigational drug product in development for the potential treatment of behavioral symptoms associated with Fragile X syndrome (FXS), autism spectrum disorder (ASD), and 22q11.2 deletion syndrome (22q). Zygel has been granted orphan drug designation by the FDA and the EC in the treatment of FXS and by the FDA for the treatment of 22q. Additionally, Zygel has been designated a Fast Track development program for treatment of behavioral symptoms of FXS.

About Fragile X Syndrome (FXS)

FXS is a rare genetic developmental disability that is the leading known cause of both inherited intellectual disability and autism spectrum disorder, affecting 1 in 3,600 to 4,000 males and 1 in 4,000 to 6,000 females. It is the most common inherited intellectual disability in males and a significant cause of intellectual disability in females, and the leading genetic cause of autism spectrum disorder (ASD). The disorder negatively affects synaptic function, plasticity and neuronal connections, and results in a spectrum of intellectual disabilities and behavioral symptoms, such as social avoidance and irritability. In the U.S., there are approximately 78,000 people suffering with FXS, and approximately 121,000 in the EU and UK. We believe that approximately 60% of all patients with FXS have complete methylation of their *FMR1* gene.

About Zynerba Pharmaceuticals, Inc.

Zynerba Pharmaceuticals is the leader in innovative 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, and 22q11.2 deletion syndrome. 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. 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 expectations, projections and estimates regarding expenses, future revenue, capital requirements, incentive and other tax credit eligibility, collectability and timing, and availability of and the need for additional financing; 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 U.S. Food and Drug Administration, the European Medicines Agency and other 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; the Company's expectations regarding its ability to obtain and adequately maintain sufficient intellectual property protection for its product candidates; and the extent to which health epidemics and other outbreaks of communicable diseases, including COVID-19, could disrupt our operations or adversely affect our business and financial conditions. 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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