Zynerba Pharmaceuticals Announces Update on RECONNECT, the Pivotal Phase 3 Trial of Zygel™ in Fragile X Syndrome

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RECONNECT initial screening visits are being adversely impacted by environmental factors, such as the "Tripledemic" (high rates of RSV, influenza and COVID-19); topline results now expected first half of 2024

Cash runway extended to mid-year 2024 due to prioritization of resources on the completion of the RECONNECT trial

DEVON, Pa., Dec. 21, 2022 (GLOBE NEWSWIRE) -- Zynerba Pharmaceuticals, Inc. (Nasdaq: ZYNE), the leader in innovative pharmaceutically-produced transdermal cannabinoid therapies for orphan neuropsychiatric disorders, today announced that the Company is revising its target for announcing topline results from the pivotal Phase 3 RECONNECT trial of Zygel in Fragile X syndrome (FXS). The Company now expects topline results in the first half of 2024, rather than the second half of 2023.

The impact of environmental factors including unusually high rates of RSV (respiratory syncytial virus) and influenza and the continued impact of COVID-19 among children, adolescents, family members, and investigational center personnel, has resulted in higher than expected cancellations of initial screening visits at the investigational centers which has led to slower than anticipated enrollment. Despite these issues, clinical investigators at the 27 sites in the U.S., Australia, the UK and Ireland remain committed to completing the trial.

"Though we are still striving to report topline results by the end of 2023, when we look at the current and projected impact of the Tripledemic, we believe that the first half of 2024 is a more reasonable timeframe to have topline results from RECONNECT," said Armando Anido, Chairman and Chief Executive Officer of Zynerba. "We have also prioritized our corporate business plans to focus almost exclusively on completing RECONNECT and now expect we have cash into mid-year 2024."

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 endocannabinoid 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), 22q11.2 deletion syndrome (22q) and autism spectrum disorder (ASD). The Company has received orphan drug designation for cannabidiol, the active ingredient in Zygel, from the FDA and the European Commission in the treatment of FXS and the treatment of 22q. Additionally, Zygel has been designated a Fast Track development program for treatment of behavioral symptoms of FXS.

About RECONNECT

RECONNECT is a randomized, double-blind, placebo-controlled, multiple-center efficacy and safety trial of Zygel administered as a transdermal gel to children and adolescents ages 3 through 17 with Fragile X syndrome. For more information regarding participation visit www.fragilexhelp.com.

About Zynerba Pharmaceuticals, Inc.

Zynerba Pharmaceuticals is the leader in innovative pharmaceutically-produced transdermal cannabinoid therapies for orphan neuropsychiatric disorders. We are committed to improving the lives of patients and their families living with severe, chronic health conditions including Fragile X syndrome, 22q11.2 deletion syndrome and autism spectrum disorder. Learn more at www.zynerba.com and follow us on Twitter at @ZynerbaPharma.

About Fragile X Syndrome (FXS)

Fragile X syndrome is a rare genetic developmental disability that is the leading known cause of both inherited intellectual disability and autism spectrum disorder, affecting 1 in 4,000 males and 1 in 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 about 78,000 people suffering with FXS, approximately 60% of whom have complete methylation of the *FMR1* gene.

FXS is caused by a mutation in *FMR1*, a gene which modulates a number of systems, including important effects on the endocannabinoid system, and most critically, codes for a protein called FMRP. This protein helps regulate the production of other proteins and plays a role in the development of synapses, which are critical for relaying nerve impulses, and in regulating synaptic plasticity. The *FMR1* mutation manifests as multiple repeats of a DNA segment, known as the CGG triplet repeat. In most neurotypical people, the *FMR1* gene correctly codes for the FMRP protein. As a result, FMRP is produced at levels that enable control over behaviors like social avoidance and anxiety. In people with full mutation of the *FMR1* gene, the CGG segment is repeated more than 200 times, and in most cases causes the gene to not function. Methylation of the *FMR1* gene also plays a role in determining functionality of the gene. For patients with complete methylation, no FMRP is produced. With no FMRP, the systems and processes that are modulated by FMRP become dysregulated.

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; 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; and the extent to which inflation or global instability, including political instability, may disrupt our business operations or our financial condition. 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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