Zynerba Pharmaceuticals Provides Update on Recent Milestones

September 14, 2020

- Meeting with the U.S. Food and Drug Administration (FDA) Planned for 4Q2020 to Discuss the Regulatory Path Forward for Zygel™ in Children and Adolescents with Fragile X Syndrome and a Fully Methylated *FMR1* Gene -

- Newly Issued FXS Patent Supplements Intellectual Property Protection –

- FDA Suggests Pursuing Individual Syndromes in Developmental and Epileptic Encephalopathies Program; Evaluation of Initial Targets for Late Stage Clinical Evaluation Ongoing -

DEVON, Pa., Sept. 14, 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 provided an update on its Fragile X syndrome (FXS) and developmental and epileptic encephalopathies (DEE) programs.

Fragile X syndrome (FXS)

Zynerba has been notified that the U.S. Food and Drug Administration (FDA) will meet with the Company via teleconference in the fourth quarter of 2020 to discuss the pivotal CONNECT-FX data and the regulatory path forward in patients with FXS and a fully methylated *FMR1* gene (FMet). The Company also expects to disclose the outcome of the meeting in the fourth quarter of this year.

“The meeting with the FDA will be an important milestone for patients and their families who live with the debilitating behavioral impact of Fragile X,” said Armando Anido, Chairman and Chief Executive Officer of Zynerba. “Our ongoing evaluation of the pivotal CONNECT-FX data continues to clarify the impact that Zygel achieved in the most severely impacted children and adolescents with FXS, as well as the excellent tolerability profile. We look forward to discussing the pivotal data and the regulatory path for potential approval in FMet patients with the FDA in the fourth quarter of this year.”

Zynerba also announced that the U.S. Patent and Trademark Office has issued US Patent No. 10,758,497, titled “Treatment of Fragile X Syndrome with Cannabidiol” which includes claims directed to a method of treating FXS comprising administering 250mg or 500mg of synthetic or purified cannabidiol in a pharmaceutically acceptable carrier to a person in need thereof. This new patent, which expires in 2038, is part of an expanding intellectual property portfolio covering Zygel.

Developmental and epileptic encephalopathies (DEE)

Zynerba has concluded its iterative meetings with the FDA utilizing their ‘Written Response Only’ (WRO) format to discuss the clinical pathway for Zygel in DEE. The FDA supports a development program which would treat focal-impaired awareness and convulsive seizures. However, due to the heterogeneity of patients who fall under the DEE umbrella, the FDA suggests that Zynerba pursue individual syndromes rather than considering DEE patients as a single disorder or condition. The Company is in the process of finalizing its evaluation of which epileptic syndromes it may pursue with Zygel.

“We appreciate our partnership with the FDA, and thank them for their input and support as we seek to advance the development of Zygel in certain rare epilepsy syndromes,” continued Anido. “We look forward to completing our target assessments and communicating our path forward around the end of this year.”

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. 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 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; the Company's expectations regarding its ability to obtain and adequately maintain sufficient intellectual property protection for its product candidates; the timing and outcome of current and future legal proceedings; 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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