Zynerba Pharmaceuticals Presents Longer Term Tolerability and Efficacy Data of Zygel™ in Children and Adolescents with Autism Spectrum Disorder (ASD) at The Society for the Study of Behavioural Phenotypes (SSBP) Conference 2021

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DEVON, Pa., Sept. 09, 2021 (GLOBE NEWSWIRE) -- Zynerba Pharmaceuticals, Inc. (Nasdaq: ZYNE), the leader in innovative pharmaceutically-produced transdermal cannabinoid therapies for rare and near-rare neuropsychiatric disorders, today presented data from the Phase 2 BRIGHT trial describing tolerability and efficacy of Zygel (cannabidiol formulated in a transdermal gel [ZYN002]) in children and adolescents with autism spectrum disorder (ASD) over a longer term, 38-week treatment period. These data were presented as an oral presentation at The Society for the Study of Behavioural Phenotypes (SSBP) Conference 2021 being held virtually from September 9-10, 2021. A copy of the presentation is available on the Zynerba corporate website at http://zynerba.com/publications/.

Helen (Honey) Heussler, MBBS FRACP MRCPCH, DM, Associate Professor, Faculty of Medicine, Child Health Research Centre, University of Queensland, Australia, delivered the oral presentation titled, "*Longer Term Tolerability and Efficacy of ZYN002 Cannabidiol Transdermal Gel in Children and Adolescents with Autism Spectrum Disorder (ASD): An Open-Label Phase 2 Study (BRIGHT [ZYN2-CL-030]).*" The presentation shows that through 38 weeks of treatment, the BRIGHT trial provides initial evidence suggesting a positive benefit-risk profile for Zygel when administered in addition to stable standard of care in children and adolescents with moderate-to-severe ASD. Furthermore, in patients who completed the 38-week treatment period, statistically significant improvements compared to baseline were sustained in all efficacy measures of ASD compared to baseline.

"Current management options for ASD are restricted to behavioral therapies and a limited number of approved pharmacologic treatments, highlighting the substantial unmet need for novel therapies in this difficult-to-treat population," said Joseph M. Palumbo, M.D., LFAPA, MACPsych, Chief Medical Officer of Zynerba. "These data are encouraging, and if confirmed, suggest meaningful therapeutic utility in ASD."

The open-label Phase 2 study evaluated the safety, tolerability and efficacy of Zygel in children and adolescents aged 3-17 years with ASD in addition to stable standard of care. Eighteen of the 27 patients that completed week 14 (Period 1) demonstrated ≥35% improvement in the Aberrant Behavior Checklist-Community (ABC-C) irritability subscale at week 14 and were allowed to continue treatment for an additional 24 weeks (Period 2). In the 18 patients who completed treatment through Period 2, Zygel showed improvement in all the Aberrant Behavior Checklist-Community (ABC-C) subscale scores (51% to 61% across domains; P<0.0001), as well as improvements in the Parent-Rated Anxiety Scale-ASD score (42%; P<0.0001), the Autism Parenting Stress Index (40%; P<0.0001) and the Autism Impact Measure (19% to 36% across domains; P≤0.0008), relative to baseline.

Zygel was generally well tolerated, and the safety profile was consistent with data from previous Zygel clinical trials. Adverse Events (AEs) were mild (80%) or moderate (20%). Treatment-related AEs were reported in 19% of patients; most were mild and transient. One patient discontinued due to application site reaction. No serious or severe AEs or clinically significant changes in vital signs, laboratory tests or ECG parameters were reported during the study.

In addition, Randi J. Hagerman, M.D., Medical Director and Endowed Chair in Fragile X Research at UC Davis MIND Institute and Distinguished Professor at the Department of Pediatrics at UC Davis School of Medicine, will present previously-announced data from the CONNECT-FX trial, a randomized, double-blind, placebo-controlled trial which assessed the efficacy and safety of Zygel as a treatment for the behavioral symptoms of FXS, on September 10, 2021, at 2:15 p.m. at SSBP. A copy of the presentation, titled "*A Pivotal Study of ZYN002 Cannabidiol (CBD) Transdermal Gel in Children and Adolescents With Fragile X Syndrome [CONNECT-FX (ZYN2-CL-016)]*" will be available on the Zynerba corporate website following the presentation at http://zynerba.com/publications/.

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, 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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