New Data Describing Statistically Significant Results from the Phase 2 BRIGHT Trial in Patients with Autism Spectrum Disorder (ASD) Presented at the Virtual Joint 16th International Child Neurology Congress (ICNC) & 49th Annual Child Neurology Society (CN

October 15, 2020

DEVON, Pa., Oct. 15, 2020 (GLOBE NEWSWIRE) -- Zynerba Pharmaceuticals, Inc. (NASDAQ:ZYNE), the leader in innovative pharmaceutically-produced transdermal cannabinoid therapies for rare and near-rare neuropsychiatric disorders, is presenting a poster describing data from the Phase 2 BRIGHT (An Open-La**b**el Tole**r**ab**i**lity and Efficacy Study of ZYN002 Administered as a Transdermal **G**el to C**h**ildren and Adolescen**t**s with Autism Spectrum Disorder) trial further demonstrating the potential for Zygel™ (ZYN002) to improve the core behavioral symptoms of autism when administered in addition to stable standard of care in children and adolescents with moderate-to-severe ASD. These open label data are being presented at the virtual Joint 16th International Child Neurology Congress (ICNC) & 49th Annual Child Neurology Society (CNS) Meeting. These data will also be presented as an oral presentation during the “Research Pipeline: New Findings on Diagnostic and Therapeutics” session of the virtual American Academy of Child and Adolescent Psychiatry (AACAP) 2020 Annual Meeting on Friday, October 23rd, 2020.

A copy of the poster entitled, “*Tolerability and Efficacy of ZYN002 Cannabidiol (CBD) Transdermal Gel in Children and Adolescents With Autism Spectrum Disorder: An Open-Label Phase 2 Study [BRIGHT (ZYN2-CL-030)]*” is available on the Zynerba corporate website at http://zynerba.com/publications/.

“The Phase 2 BRIGHT trial provides the first clinical evidence of the potential for Zygel to improve behavioral symptomology in a group of highly symptomatic pediatric and adolescent patients with ASD,” said Helen Heussler, FRACP, Associate Professor at Children’s Health Queensland, Medical Director Child Development and principal investigator in the BRIGHT trial. “In these children receiving Zygel, the observed changes from baseline are promising. In particular, the improvements seen in core symptoms of autism, as specifically assessed by the Autism Impact Measure, are of special interest. Though open label, these results are compelling and we look forward to continuing the evaluation of Zygel in ASD in future placebo-controlled clinical trials.”

The BRIGHT Phase 2 trial is an exploratory, single-center, open-label Phase 2 study evaluating the safety and tolerability and efficacy of Zygel in children and adolescents with ASD who are 3 to <18 years old. The study enrolled patients with Clinical Global Impression (CGI)-Severity score ≥4 (moderate or greater) and Aberrant Behavior Checklist-Community (ABC-C) Irritability score ≥18. The primary objective of the trial was to evaluate the safety and tolerability of Zygel for up to 38 weeks (14-week treatment period and a 6 month extension period). Secondary objectives comprised evaluation of the efficacy of Zygel in the treatment of symptoms of ASD, including measuring parental/caregiver stress, Autism Impact Measure (AIM), and caregiver reported behavioral problems. The results provided in the poster are after 14 weeks of treatment with Zygel.

New data presented today include that patients receiving Zygel in this study achieved statistically significant caregiver-reported improvements compared to baseline across all subscales of the AIM, which was designed to track incremental change in frequency and impact of core ASD symptoms: Atypical behavior (p<0.001), Communication (p<0.001), Peer Interaction (p<0.001), Repetitive Behavior (p<0.001), and Social Reciprocity (p=0.0053).

Figure 1. Statistically Significant Improvements in Autism Impact Measure Scores

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In addition, statistically significant improvements compared to baseline were observed at week 14 of treatment with Zygel in the Autism Parenting Stress Index (p<0.0001).

Figure 2. Statistically Significant Improvements in PRAS-ASD, APSI, and CGI-I

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Zynerba also measured notable improvements in behaviors utilizing the Qualitative Caregiver Behavioral Problems Survey after 14 weeks of study drug. Clinically meaningful improvements were observed by a majority of surveyed caregivers in behavioral, social and emotional behavioral

Figure 1. Statistically Significant Improvements in Autism Impact Measure Scores

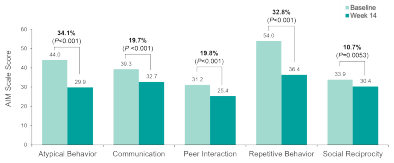

Figure 1. Statistically Significant Improvements in Autism Impact Measure Scores

Figure 2. Statistically Significant Improvements in PRAS-ASD, APSI, and CGI-I

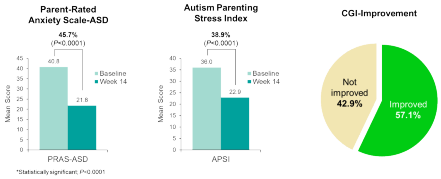

Figure 2. Statistically Significant Improvements in PRAS-ASD, APSI, and CGI-I

Figure 3. Notable Improvements in the Qualitative Caregiver Behavioral Problems Survey at Week 14

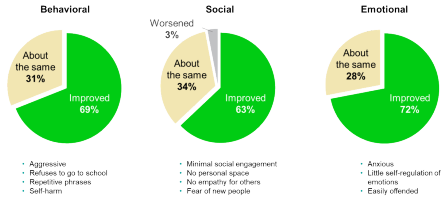

Figure 3. Notable Improvements in the Qualitative Caregiver Behavioral Problems Survey at Week 14

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The authors of the poster concluded that:

- 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;
- Zygel showed improvement in all ASD measures (ABC-C, AIM, PRAS-ASD, CGI and Qualitative Caregiver Assessments);
- Further controlled studies are warranted in this difficult-to-treat population.

These results supplement the topline data initially disclosed by the Company in May 2020 (Press release), including that:

- All five subscales of the ABC-C showed both statistically significant ($p<0.0002$) and clinically meaningful improvements at 14 weeks of treatment from baseline.
- The results of other efficacy assessments reinforce the results demonstrated in the ABC-C, including a mean improvement of 46% at week 14 from baseline as measured by the Parent Reported Anxiety Scale for ASD (PRAS-ASD; $p<0.0001$) and 57% of patients were assessed as “very much improved” or “much improved” at week 14 as measured by the Clinical Global Impression - Improvement scale (CGI-I).
- Zygel was very well tolerated in this trial and the safety profile was consistent with previously released data from other Zygel clinical trials. No serious or severe adverse events were reported.

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.

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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 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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