Zynerba Pharmaceuticals Reports Third Quarter 2020 Financial Results and Operational Highlights

November 9, 2020

DEVON, Pa., Nov. 09, 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 reported financial results for the third quarter ended September 30, 2020, and provided an overview of recent operational highlights.

“We made good clinical, operational and regulatory progress during the third quarter of 2020 including presenting new data from the pivotal CONNECT-FX and Phase 2 BRIGHT trials, and completing our discussions with the FDA to clarify our clinical path forward to late stage clinical trials in patients with certain developmental and epileptic encephalopathies,” said Armando Anido, Chairman and Chief Executive Officer of Zynerba. “The fourth quarter of this year is another important period for Zynerba. In particular, we look forward to announcing the results of our fourth quarter meeting with the FDA to discuss our pivotal CONNECT-FX results in patients with a fully methylated *FMR1* gene and to understand the regulatory path forward.”

Third quarter 2020 and Recent Highlights

Zygel in Fragile X Syndrome (FXS)

Zynerba Expects to Announce the Outcome of its Fourth Quarter Meeting with the U.S. Food and Drug Administration (FDA) to Discuss the CONNECT-FX Trial and the Regulatory Path Forward for Zygel™ in Pediatric and Adolescent Patients with a Fully Methylated FMR1 Gene (FMet) in the Fourth Quarter of 2020 (Press release)

Presented New CONNECT-FX Data Supporting FMR1 Methylation Status as a Correlate to Fragile X Syndrome Severity at the Virtual Joint 16th International Child Neurology Congress (ICNC) & 49th Annual Child Neurology Society (CNS) Meeting

Zynerba utilized psychometric analyses to determine what constitutes a clinically meaningful change from baseline as measured by subscales of the ABC-C_{FXS}. The results of these analyses defined a clinically meaningful treatment response over 12 weeks of treatment as an improvement of three points or greater for the Social Avoidance subscale, nine points or greater for the Irritability subscale, and five points or greater for the Socially Unresponsive / Lethargic subscale. Through this analysis, the Company determined that 58.2% of FMet patients receiving Zygel achieved a clinically meaningful change in their socially avoidant behavior compared to 40.6% of patients receiving placebo (p=0.031; statistically significant), and 40.3% of patients receiving Zygel achieved a clinically meaningful change in Irritability compared to 23.8% of patients receiving placebo (p=0.036; statistically significant). (Press release)

Presented CONNECT-FX Data Showing Statistically Significant Caregiver-Reported Improvements in Most Impactful FXS Behaviors at the 17th NFXF International Conference Research Roundup; Results Support Statistically Significant Results of Pre-planned Ad Hoc Analysis in FMet Patients

Consistent with guidance from the FDA on capturing the voice of the patient in drug development, the Company collected qualitative data on the clinical relevance of various FXS behaviors to caregivers during CONNECT-FX. The results of the Qualitative Caregiver Reported Behavioral Survey indicate that caregivers found anxiety, socially avoidant behaviors, and disruptive behaviors to be the most challenging. The results of the Caregiver Global Impression – Change survey show a broad shift toward global improvement from baseline to week 12 in FMet patients, with three of the four behavioral domains (social avoidance and isolation, irritable and disruptive behaviors, and social interactions) showing statistically significant improvements in favor of patients on Zygel compared to placebo and the fourth domain (overall behavior) trending toward significance. (Press release)

Received New U.S. Patent for Treatment of Fragile X Syndrome with Cannabidiol

The U.S. Patent and Trademark Office 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. (Press release)

Zygel in Autism Spectrum Disorder (ASD)

Zynerba Intends to Discuss the Results of the Phase 2 BRIGHT Trial in Children and Adolescents with Moderate to Severe ASD and the Path Forward with the FDA in the First Half of 2021

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

Patients receiving Zygel in this study achieved statistically significant caregiver-reported improvements compared to baseline across all subscales of the Autism Impact Measure, which was designed to measure 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). 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). 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 problems. (Press release)

Zygel in 22q11.2 Deletion Syndrome (22q)

Received Orphan Drug Designation for Cannabidiol for the Treatment of 22q11.2 Deletion Syndrome

The FDA granted orphan drug designation for cannabidiol for use in treating 22q, a rare midline condition featuring physical abnormalities and debilitating neuropsychiatric and behavioral symptoms including anxiety, withdrawn behavior, and social interaction problems. Companies receiving orphan drug designation may be entitled to various incentives, including tax credits for qualified clinical studies, waiver of new drug application (NDA) / biologics license application (BLA) user fees, and eligibility for a seven-year exclusive marketing period for that drug and use upon marketing approval. (Press release)

Zygel in Developmental and Epileptic Encephalopathies (DEE)

Concluded Successful Discussions with FDA Regarding the DEE Program and Path Forward; Evaluation of Initial Targets for Late Stage Clinical Evaluation Progressing

Zynerba concluded its iterative discussions with the FDA utilizing their 'Written Response Only' (WRO) meeting format regarding the clinical pathway for Zygel in DEE during which the FDA expressed support for a development program which would evaluate the treatment of focal-impaired awareness and convulsive seizures. Due to the heterogeneity of patients who fall under the DEE umbrella, Zynerba will pursue individual syndromes rather than considering DEE 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, and expects to disclose its first clinical target around year end 2020. (Press release)

Third quarter 2020 Financial Results

As of September 30, 2020, cash and cash equivalents were $64.3 million, compared to $70.1 million as of December 31, 2019. Research and development expenses for the third quarter of 2020 were $5.8 million, including stock-based compensation of $0.5 million. General and administrative expenses for the third quarter of 2020 were $3.4 million, including stock-based compensation expense of $0.7 million. The net loss for the third quarter of 2020 was $9.0 million with basic and diluted net loss per share of $(0.31).

Financial Outlook

Management believes that the current cash and cash equivalents is sufficient to fund operations and capital requirements until late in the fourth quarter of 2021.

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.

ZYNERBA PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2020	**2019**	**2020**	**2019**
Operating expenses:				
Research and development	$ 5,805,948	$ (1,604,399)	$ 30,038,582	$ 12,926,096

General and administrative	3,425,831	3,530,617	11,834,434	9,977,550
Total operating expenses	9,231,779	1,926,218	41,873,016	22,903,646
Loss from operations	(9,231,779)	(1,926,218)	(41,873,016)	(22,903,646)
Other income (expense):				
Interest income	10,781	436,846	239,066	1,226,998
Foreign exchange gain (loss)	172,467	(457,018)	(85,171)	(551,944)
Total other income (expense)	183,248	(20,172)	153,895	675,054
Net loss	$ (9,048,531)	$ (1,946,390)	$ (41,719,121)	$ (22,228,592)
Net loss per share - basic and diluted	$ (0.31)	$ (0.08)	$ (1.59)	$ (1.03)
Basic and diluted weighted average shares outstanding	29,243,375	23,186,410	26,258,626	21,598,764
Non-cash stock-based compensation included above:				
Research and development	$ 544,909	$ 573,446	$ 1,590,285	$ 1,915,578
General and administrative	717,716	802,779	2,343,125	2,438,644
Total	$ 1,262,625	$ 1,376,225	$ 3,933,410	$ 4,354,222

ZYNERBA PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS

	(unaudited) September 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 64,311,205	$ 70,063,242
Incentive and tax receivables	6,533,079	14,613,969
Prepaid expenses and other current assets	3,913,112	2,378,812
Total current assets	74,757,396	87,056,023
Property and equipment, net	596,512	362,724
Incentive and tax receivables	1,684,616	—
Right-of-use assets	166,889	345,849
Total assets	$ 77,205,413	$ 87,764,596
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 3,404,129	$ 4,740,981
Accrued expenses	6,987,653	7,073,506
Lease liabilities	174,073	243,677
Total current liabilities	10,565,855	12,058,164
Lease liabilities, long-term	—	109,689
Total liabilities	10,565,855	12,167,853
Stockholders' equity:		
Common stock	29,449	23,211
Additional paid-in capital	259,164,854	226,409,156
Accumulated deficit	(192,554,745)	(150,835,624)
Total stockholders' equity	66,639,558	75,596,743
Total liabilities and stockholders' equity	$ 77,205,413	$ 87,764,596

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