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PHARMACEUTICALS

Zynerba Pharmaceuticals Reports Third Quarter 2018 Financial Results and Operational Highlights

November 8, 2018

DEVON, Pa., Nov. 08, 2018 (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, 2018 and provided an overview of recent operational highlights.

"The momentum we established in the first half of 2018 continued through the third quarter," said Armando Anido, Chairman and Chief Executive Officer of Zynerba. "We began enrolling patients into CONNECT-FX, our pivotal study in Fragile X syndrome and we expect to complete enrollment in BELIEVE-1, our Phase 2 study in developmental and epileptic encephalopathies, before year end. With our third quarter 2018 follow-on offering, we are well capitalized and expect our current cash to take us through the presentation of top line data for both of these studies."

Third Quarter 2018 and Recent Highlights

ZYN002 in Fragile X Syndrome (FXS)

Initiated CONNECT-FX, a Pivotal Clinical Trial of ZYN002 in FXS

Enrollment is progressing in CONNECT-FX (**Cl**inical study **o**f Ca**nn**abidiol (CBD) in Childr**e**n and Adoles**c**e**n**ts with **F**ragile **X**), a pivotal, multi-national, randomized, double blind, placebo-controlled study evaluating the efficacy and safety of ZYN002 in three to 17-year old FXS patients with full mutation of the FMR1 gene. The primary endpoint is the change from baseline to the end of the treatment period in the Aberrant Behavior Checklist-Community FXS Specific (ABC-C_{FXS}) Social Avoidance subscale. Top line data are expected in the second half of 2019; there are currently no approved products indicated for FXS.

Presented New Data at the 16th NFXF International Fragile X Conference from the Ongoing Open Label FAB-C Phase 2 Study of ZYN002

Zynerba presented new data demonstrating that treatment with ZYN002 improved core behavioral symptoms of FXS with statistical significance versus baseline across multiple measures of efficacy at week 12, and these improvements were sustained through 38 weeks of treatment. For example, significant improvements vs. baseline in social avoidance as measured by the ABC-C_{FXS} were demonstrated at 12 weeks (58%; p=0.0040) and 38 weeks (75%; p=0.0013) of treatment with ZYN002.

Poster Accepted for Presentation at the 57th Annual Meeting of the American College of Neuropsychopharmacology (ACNP)

The poster presentation is on Wednesday, December 12, 2018 in poster session III from 5:30 PM to 7:30 PM EST. The meeting will be at the Diplomat Beach Resort in Hollywood, Florida from December 9 through 12, 2018.

ZYN002 in Developmental and Epileptic Encephalopathies (DEE)

Enrollment Nearing Completion in the Phase 2 BELIEVE 1 Clinical Trial in DEE; Topline Results Expected in 2019

Enrollment is progressing in BELIEVE 1 (Open L**ab**el Study to Assess the Safety and **E**fficacy of ZYN002 Administered as a Transderm**a**l Gel to Chi**l**dren and Ado**le**scents with De**v**elopmental and **E**pileptic Encephalopathy). This is a six-month multi-dose Phase 2 clinical trial evaluating the efficacy and safety of ZYN002 in approximately 50 children and adolescents with DEE, a category of epilepsy syndromes that involve significant developmental impairment or regression of developmental progress, and are highly resistant to treatment. The Company expects to complete enrollment by year-end 2018.

ZYN002 in Focal Epilepsy

STAR-2 Long-Term Data Accepted for Poster Presentation at the 2018 Annual Meeting of the American Epilepsy Society (AES)

The poster presentation is on Sunday, December 2, 2018 in poster session 2 from 12:00 PM to 2:00 PM EST. The meeting will be at the Ernest N. Morial Convention Center in New Orleans, LA from November 30 to December 4, 2018. The poster will highlight efficacy and safety data from adult patients with refractory focal epilepsy receiving treatment with ZYN002 for up to 18 months. These data contribute to the design of the upcoming double blind, placebo-controlled Phase 2b study, which the Company expects to initiate in the fourth quarter of 2018.

Corporate

Completed Successful $30 Million Follow-on Offering

The Company improved its capital structure through a completed follow-on offering, resulting in net proceeds of $29.9 million after deducting underwriting discounts and commissions and offering expenses.

Third Quarter 2018 Financial Results

As of September 30, 2018, cash and cash equivalents were $66.2 million, compared to $62.5 million as of December 31, 2017. Research and development expenses for the third quarter of 2018 were $4.9 million, including stock-based compensation of $0.7 million. General and administrative expenses for the third quarter of 2018 were $3.1 million, including stock-based compensation expense of $0.8 million. Net loss for the third quarter of 2018 was $7.8 million with basic and diluted net loss per share of $(0.47).

Financial Outlook
The Company's cash and cash equivalent position as of September 30, 2018 was $66.2 million, which management believes is sufficient to fund operations and capital requirements into the first half of 2020.

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 and refractory epilepsies. 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 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; and the Company's expectations regarding its ability to obtain and adequately maintain sufficient intellectual property protection for its product candidates. 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,	
	2018	2017	2018	2017
Operating expenses:				
Research and development	$ 4,859,902	$ 5,753,764	$ 22,368,881	$ 16,978,016
General and administrative	3,125,780	2,795,839	9,982,743	7,640,489
Total operating expenses	7,985,682	8,549,603	32,351,624	24,618,505
Loss from operations	(7,985,682)	(8,549,603)	(32,351,624)	(24,618,505)
Other income (expense):				
Interest income	278,214	161,930	639,702	363,350
Foreign exchange (loss) gain	(99,897)	76,468	(409,010)	361,450
Total other income (expense)	178,317	238,398	230,692	724,800
Net loss	$ (7,807,365)	$ (8,311,205)	$ (32,120,932)	$ (23,893,705)
Net loss per share - basic and diluted	$ (0.47)	$ (0.63)	$ (2.21)	$ (1.87)
Basic and diluted weighted average shares outstanding	16,587,353	13,098,914	14,531,272	12,743,332
Non-cash stock-based compensation included above:				
Research and development	$ 743,153	$ 591,898	$ 2,267,783	$ 1,722,456
General and administrative	841,077	1,130,745	2,759,330	2,544,260
Total	$ 1,584,230	$ 1,722,643	$ 5,027,113	$ 4,266,716

ZYNERBA PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS
(unaudited)

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 66,177,376	$ 62,510,277
Incentive and tax receivables	3,095,195	3,983,604
Prepaid expenses and other current assets	3,413,029	1,733,701
Total current assets	72,685,600	68,227,582
Property and equipment, net	248,741	164,527
Other assets	—	662,200
Total assets	$ 72,934,341	$ 69,054,309
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 3,553,350	$ 3,355,255
Accrued expenses	5,415,742	3,915,491
Deferred grant revenue	171,975	171,975
Total current liabilities	9,141,067	7,442,721
Deferred grant revenue, long-term	—	662,000
Total liabilities	9,141,067	8,104,721
Stockholders' equity:		
Common stock	17,623	13,554
Additional paid-in capital	173,877,449	138,916,900
Accumulated deficit	(110,101,798)	(77,980,866)
Total stockholders' equity	63,793,274	60,949,588
Total liabilities and stockholders' equity	$ 72,934,341	$ 69,054,309

Investor Contacts

Jim Fickenscher, CFO and VP Corporate Development
Zynerba Pharmaceuticals
484.581.7483
fickenscherj@zynerba.com

Will Roberts, VP Investor Relations and Corporate Communications
Zynerba Pharmaceuticals

484.581.7489
robertsw@zynerba.com

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