Zynerba Pharmaceuticals Reports Second Quarter 2018 Financial Results and Operational Highlights

August 2, 2018

Zynerba to host conference call and webcast today at 8:30 am

DEVON, Pa., Aug. 02, 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 second quarter ended June 30, 2018 and provided an overview of recent operational highlights.

“Our strong clinical and corporate momentum continued over the past few months, including initiating CONNECT-FX, a pivotal study in Fragile X syndrome, and the BELIEVE 1 study in developmental and epileptic encephalopathies,” said Armando Anido, Chairman and Chief Executive Officer of Zynerba. “We presented exciting new data from the ongoing FAB-C study highlighting significant and sustained improvements in behavioral symptoms of FXS in children and adolescents treated with ZYN002 out to 38 weeks. Importantly, we closed a follow-on offering on July 24th, which added $30 million to our cash position. We believe this extends our cash runway into the first half of 2020, past the top-line results from the CONNECT-FX and BELIEVE-1 studies. Assuming positive results in CONNECT-FX and supportive regulatory interactions, we believe the additional capital will fund our NDA filing for ZYN002 for the treatment of behavioral symptoms of Fragile X.”

Second Quarter 2018 and Recent Highlights

ZYN002 in Fragile X Syndrome (FXS)

Initiated CONNECT-FX, a Pivotal Clinical Trial of ZYN002 in FXS; Enrollment is Ongoing with Topline Results Expected in the Second Half of 2019

The Company initiated and is currently enrolling patients in CONNECT-FX (**C**linical study **o**f Ca**nn**abidiol (CBD) in Childr**e**n and Adoles**c**en**t**s 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 study will enroll approximately 200 male and female patients from approximately 20 clinical sites in the U.S., Australia, and New Zealand. 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.

Presented New Data at the 16th NFXF International Fragile X Conference from the Ongoing Open Label FAB-C Phase 2 Study of ZYN002 Describing Sustained Improvements in Behavioral Symptoms of FXS

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, after 12 weeks of treatment with ZYN002, patients achieved a statistically significant mean improvement of 58% (n=12; p=0.0040) vs. baseline in the social avoidance subscale of the ABC-C_{FXS} assessment. After 38 weeks of treatment with ZYN002, patients achieved a statistically significant mean improvement of 75% (n=9; p=0.0013) vs. baseline as measured by the same assessment. ZYN002 was well tolerated and no clinically meaningful negative trends in vital signs, ECG, or clinical safety laboratories, including liver function tests (LFTs), were observed.

ZYN002 in Developmental and Epileptic Encephalopathies (DEE)

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

Zynerba initiated BELIEVE 1 (Open La**b**el Study to Assess the Safety and **E**fficacy of ZYN002 Administered as a Transderma**l** Gel to Ch**i**ldren and Adol**e**scents with De**v**elopmental and **E**pileptic Encephalopathy), a six-month multi-dose Phase 2 clinical trial evaluating the efficacy and safety of ZYN002 in approximately 50 children and adolescents with DEE. The primary efficacy assessment is change in seizure frequency. DEE is a group of epilepsy syndromes that involve significant developmental impairment or regression of developmental progress, and are highly resistant to treatment. The category includes a number of syndromes, including Doose, Dravet, Lennox-Gastaut, and West, among others.

ZYN002 in Focal Epilepsy

Presented Clinical Data from the STAR 2 Open Label Study of ZYN002 in Patients with Refractory Focal Seizures at the 2018 American Academy of Neurology (AAN) Meeting

Adult refractory focal seizure patients administering transdermal ZYN002 in STAR 1 and STAR 2 showed continued reductions in focal seizures compared to baseline through 12 months of treatment. ZYN002 was well tolerated. These data continue to suggest the potential for ZYN002 in the treatment of epilepsy, and provide insight into the design of the upcoming double blind, placebo controlled Phase 2b study, which the Company expects to initiate in the second half of 2018.

Corporate

Strengthened Balance Sheet with Successful Follow-On Offering Raising $30.0 Million in Net Proceeds; Expected to Extend Cash Runway into the First Half of 2020

The Company closed a follow-on offering on July 24, 2018, as expected, selling 4,062,500 shares of our common stock at an offering price of $8.00 per share, resulting in net proceeds of $30.0 million after deducting underwriting discounts and commissions and offering expenses. Zynerba has also granted the underwriters a 30-day option to purchase up to 609,375 additional shares of common stock at the public offering price, less underwriting discounts and commissions, which expires on August 19, 2018. Zynerba intends to use the net proceeds of the proposed offering to support the clinical development of ZYN002, for additional research and development, and for general corporate purposes.

Second Quarter 2018 Financial Results

As of June 30, 2018, cash and cash equivalents were $43.1 million, compared to $52.1 million as of March 31, 2018. Research and development expenses for the second quarter of 2018 were $8.5 million, including stock-based compensation of $0.8 million. General and administrative expenses for the second quarter of 2018 were $3.4 million, including stock-based compensation expense of $1.0 million. Net loss for the second quarter of 2018 was $12.0 million with basic and diluted net loss per share of $(0.89).

Financial Outlook

The Company’s cash and cash equivalent position as of June 30, 2018 was $43.1 million. The net proceeds of the follow-on offering that closed in July 2018 were $30.0 million. Management believes that the combined current cash and cash equivalents position, including the net proceeds of the follow-on offering, are sufficient to fund operations and capital requirements into the first half of 2020.

Conference call information
Zynerba management will host a live conference call and webcast today at 8:30 am Eastern Time to provide a corporate update. The call can be accessed by dialing (866) 573-0180 (U.S. and Canada) or (430) 775-1345 (international) and referencing conference ID 9565578. To access the live webcast or the replay, visit the investor page of the Company’s website at http://ir.zynerba.com/. The webcast will be recorded and available on the Company’s website for 30 days.

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 June 30,		Six months ended June 30,	
	2018	**2017**	**2018**	**2017**
Operating expenses:				
Research and development	$ 8,533,466	$ 5,732,797	$ 17,508,979	$ 11,224,252
General and administrative	3,436,340	2,632,857	6,856,963	4,844,650
Total operating expenses	11,969,806	8,365,654	24,365,942	16,068,902
Loss from operations	(11,969,806)	(8,365,654)	(24,365,942)	(16,068,902)
Other income (expense):				
Interest income	186,304	124,535	361,488	201,420
Foreign exchange (loss) gain	(223,731)	(82,360)	(309,113)	284,982
Total other income (expense)	(37,427)	42,175	52,375	486,402
Net loss	$ (12,007,233)	$ (8,323,479)	$ (24,313,567)	$ (15,582,500)
Net loss per share - basic and diluted	$ (0.89)	$ (0.64)	$ (1.80)	$ (1.24)
Basic and diluted weighted average shares outstanding	13,504,485	13,052,294	13,486,191	12,562,594
Non-cash stock-based compensation included above:				
Research and development	$ 776,386	$ 588,713	$ 1,524,630	$ 1,130,558
General and administrative	979,473	766,661	1,918,253	1,413,515
Total	$ 1,755,859	$ 1,355,374	$ 3,442,883	$ 2,544,073

ZYNERBA PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS
(unaudited)

	June 30, 2018	**December 31, 2017**
Assets		
Current assets:		
Cash and cash equivalents	$ 43,115,051	$ 62,510,277
Incentive and tax receivables	3,961,748	3,983,604
Prepaid expenses and other current assets	1,962,291	1,733,701
Total current assets	49,039,090	68,227,582
Property and equipment, net	273,784	164,527
Incentive and tax receivables	2,056,498	—
Other assets	834,174	662,200
Total assets	$ 52,203,546	$ 69,054,309
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 6,379,750	$ 3,355,255
Accrued expenses	4,910,918	3,915,491
Deferred grant revenue	—	171,975
Total current liabilities	11,290,668	7,442,721
Deferred grant revenue, long-term	833,974	662,000
Total liabilities	12,124,642	8,104,721
Stockholders' equity:		
Common stock	13,561	13,554
Additional paid-in capital	142,359,776	138,916,900
Accumulated deficit	(102,294,433)	(77,980,866)
Total stockholders' equity	40,078,904	60,949,588
Total liabilities and stockholders' equity	$ 52,203,546	$ 69,054,309

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