



Zynerba Pharmaceuticals Reports First Quarter 2017 Financial Results and Operational Highlights

May 9, 2017

DEVON, Pa., May 09, 2017 (GLOBE NEWSWIRE) -- [Zynerba Pharmaceuticals](#), Inc. (NASDAQ:ZYNE), a clinical-stage specialty pharmaceutical company dedicated to developing and commercializing innovative transdermal synthetic cannabinoid treatments, today reported financial results for the first quarter ended March 31, 2017 and provided an overview of recent operational highlights.

"This quarter, we were very pleased to have surpassed target enrollment in both the STAR 1 and STOP trials for ZYN002 in adult epilepsy and adult osteoarthritis patients, respectively, which we believe indicates strong patient and physician interest in these indications for ZYN002," said Armando Anido, Chairman and Chief Executive Officer. "We are swiftly advancing our Phase 2 programs for ZYN002 and expect to announce top-line results for the STAR 1 trial first and then the STOP trial in the July/August timeframe, followed by top-line results from the FAB-C study in pediatric Fragile X syndrome patients later in the third quarter. We have also made important progress toward initiating our Phase 1 program for ZYN001, a pro-drug THC patch, which we expect to start by the end of the second quarter of 2017. Momentum across our pipeline continues to build and we remain poised for a transformational 2017."

First Quarter 2017 and Recent Highlights

Exceeded Target Enrollment of Phase 2 STAR 1 Clinical Trial for ZYN002 CBD Gel in Adult Epilepsy Patients

A total of 188 patients have been randomized in the Phase 2 STAR 1 double-blind, placebo-controlled clinical trial in adult patients with refractory epilepsy, exceeding the 180-patient enrollment target. Following randomization, patients are dosed with either 195 mg or 390 mg of CBD in ZYN002 4.2% gel or placebo daily for 12 weeks. The primary endpoint of the trial is the median reduction in seizure frequency per 28-day period compared to baseline. The company expects to report top-line data from this trial in July/August 2017.

Enrollment Continues in Phase 2 STAR 2 Open-Label Extension Clinical Trial for ZYN002 CBD Gel in Adult Epilepsy Patients

Patients who complete the STAR 1 trial may elect to enroll into the STAR 2 trial, designed to evaluate long-term safety and tolerability of ZYN002 CBD gel across a range of doses. In the open-label extension study, patients receive a high or low-dose of ZYN002 (390 mg or 195 mg of CBD in ZYN002 4.2% gel, respectively) for up to 52 weeks. Of the 151 patients who have completed the STAR 1 trial through May 8, 2017, 147 have enrolled into STAR 2.

Exceeded Target Enrollment of Phase 2 STOP Clinical Trial for ZYN002 CBD Gel in Adult Osteoarthritis Patients

Dosing is ongoing in the randomized, double-blind, placebo-controlled Phase 2 STOP trial in osteoarthritis of the knee. We have exceeded the initial target enrollment of 300 patients with 320 patients randomized into one of three dosing groups. Patients are receiving either 250 mg or 500 mg of CBD in ZYN002 4.2% gel or placebo daily for 12 weeks. The primary endpoint of the trial is the change from baseline in the weekly mean of the 24-hour average worst pain score at week 12. Top-line results are expected to be released after the STAR 1 Trial results in July/August 2017.

FAB-C Exploratory Phase 2 Clinical Trial of ZYN002 CBD Gel in Pediatric Fragile X Syndrome Patients Remains on Track for Top-line Data in 3Q 2017
The Phase 2 exploratory FAB-C clinical trial is designed to evaluate the safety and efficacy of ZYN002 CBD gel in pediatric patients with Fragile X syndrome (FXS). The primary objective is to assess intra-patient changes in anxiety, depression and mood (as measured by the ADAMS scale) versus baseline. The company is targeting enrollment of 16 patients and expects to announce top-line data from the trial in the third quarter of 2017.

Initiation of Phase 1 Programs for ZYN001 On Track for 1H 2017

By the end of the first half of this year, the company expects to initiate Phase 1 trials of ZYN001, a patent-protected, pro-drug of THC that enables transdermal delivery via a patch. The Phase 1 program will evaluate the safety and tolerability of ZYN001 through single and multiple rising dose trials in normal subjects and patients with fibromyalgia. Pending successful Phase 1 results, a Phase 2 program for ZYN001 in fibromyalgia and neuropathic pain is planned to start in the second half of 2017.

Strengthened Senior Management Team

In March 2017, Ken Jones, CPA was named Corporate Controller of Zynerba. Mr. Jones brings over 30 years of experience in financial reporting, accounting and tax functions across multiple industries, including a combined 16 years serving as Corporate Controller at Vitae Pharmaceuticals and UbiquiTel, Inc.

In April 2017, Ray Mannion joined Zynerba as Vice President, Manufacturing. Mr. Mannion has spent over 35 years in international manufacturing, operations and engineering in the pharmaceutical, medical devices and electrical connection systems industries with Teva Pharmaceuticals, NuPathe, Puricore, Kensey Nash Corporation, AMP Incorporated and others.

In May 2017, Will Roberts was appointed Vice President, Investor Relations and Corporate Communications at Zynerba. Mr. Roberts is an experienced pharmaceutical executive with 25 years of broad communications and scientific research experience with companies including Adaptimmune Therapeutics, ViroPharma Incorporated and MedImmune, Inc.

Strengthened Balance Sheet with Successful Follow-On Offering Raising \$58 Million in Gross Proceeds

In the first quarter of 2017, the company completed a follow-on offering, selling 3,220,000 shares of our common stock at an offering price of \$18.00 per share, resulting in gross proceeds of \$58.0 million. Net proceeds received after deducting underwriting and commissions and offering expenses were \$54.2 million, which Zynerba intends to use for the clinical development of ZYN002 and ZYN001, general research and development, and

general corporate purposes.

First Quarter 2017 Financial Results

As of March 31, 2017, cash and cash equivalents were \$77.5 million, compared to \$31.0 million as of December 31, 2016. Research and development expenses for the first quarter of 2017 were \$5.5 million, including stock-based compensation of \$0.5 million. General and administrative expenses for the first quarter of 2017 were \$2.2 million, including stock-based compensation expense of \$0.6 million. Net loss for the first quarter of 2017 was \$7.3 million with basic and diluted net loss per share of \$0.60.

Financial Outlook

The company believes that the current cash position of \$77.5 million is sufficient to develop five Phase 3-ready programs and, assuming support from the FDA to move forward, initiate at least one Phase 3 program and fund operations and capital requirements into 2019.

About Zynerba Pharmaceuticals, Inc.

Zynerba Pharmaceuticals (NASDAQ:ZYNE) is a clinical-stage specialty pharmaceutical company focused on developing and commercializing proprietary next-generation synthetic cannabinoid therapeutics formulated for transdermal delivery. Zynerba is developing therapeutic candidates based on proprietary transdermal technologies that, if successfully developed, may allow sustained, consistent and controlled delivery of therapeutic levels of two cannabinoids: cannabidiol (CBD), a non-psychoactive cannabinoid, and tetrahydrocannabinol (THC). Transdermal delivery has the potential to reduce adverse effects associated with oral dosing. ZYN002, the Company's CBD gel, is the first and only synthetic CBD formulated as a patent-protected permeation-enhanced gel. In March 2017, the Company completed enrollment in the Phase 2 STAR 1 (Synthetic Transdermal Cannabidiol for the Treatment of Epilepsy) clinical trial of ZYN002 CBD gel in refractory epilepsy patients with focal seizures, the most common form of epilepsy in adults. Also in March 2017, the Phase 2 STOP (Synthetic Transdermal Cannabidiol for the Treatment of Knee Pain due to Osteoarthritis) clinical trial in patients with knee pain due to osteoarthritis was fully enrolled. In December 2016, the Company initiated the exploratory Phase 2 FAB-C (Treatment of Fragile X Syndrome Anxiety and Behavioral Challenges with CBD) clinical trial in children with Fragile X syndrome (FXS). Zynerba is also developing ZYN001, which utilizes a synthetically manufactured pro-drug of THC. A Phase 1 clinical study for ZYN001 is planned to begin by the end of the first half of 2017. Learn more at www.zynerba.com and follow the Company 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. For example, there can be no guarantee that the Company will obtain approval for ZYN002 or ZYN001 from the U.S. Food and Drug Administration (FDA) or foreign regulatory authorities; even if ZYN002 or ZYN001 are approved, the Company may not be able to obtain the label claims that it is seeking from the FDA. In addition, the Company's cash and cash equivalents may not be sufficient to support its operating plan for as long as anticipated. 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 success, cost and timing of the Company's product development activities, studies and clinical trials; the success of competing products that are or become available; 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	
	March 31, 2017	March 31, 2016
Revenue	\$ —	\$ 7,250
Operating expenses:		
Research and development	5,491,455	2,568,989
General and administrative	2,211,793	1,680,130
Total operating expenses	7,703,248	4,249,119
Loss from operations	(7,703,248)	(4,241,869)
Other income (expense):		
Interest income	76,885	12,377
Foreign exchange gain (loss)	367,342	(23,148)
Total other income (expense)	444,227	(10,771)
Loss before income taxes	(7,259,021)	(4,252,640)

Income tax expense	—	28,734
Net loss	\$ (7,259,021)	\$ (4,281,374)
Net loss per share - basic and diluted	\$ (0.60)	\$ (0.49)
Basic and diluted weighted average shares outstanding	12,067,453	8,823,951
Non-cash stock-based compensation included above:		
Research and development	\$ 541,845	\$ 248,732
General and administrative	646,854	515,067
Total	\$ 1,188,699	\$ 763,799

ZYNERBA PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS
(Unaudited)

	March 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	\$ 77,493,189	\$ 30,965,791
Incentive and tax receivables	3,867,811	3,613,943
Prepaid expenses and other current assets	2,002,966	1,830,958
Total current assets	83,363,966	36,410,692
Property and equipment, net	198,197	143,382
Incentive and tax receivables	1,141,533	—
Other assets	200	200
Total assets	\$ 84,703,896	\$ 36,554,274
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 996,866	\$ 1,848,084
Accrued expenses	5,110,490	4,284,907
Deferred grant revenue	833,975	833,975
Total current liabilities	6,941,331	6,966,966
Stockholders' equity:		
Common stock	13,215	9,995
Additional paid-in capital	130,976,933	75,545,875
Accumulated deficit	(53,227,583)	(45,968,562)
Total stockholders' equity	77,762,565	29,587,308
Total liabilities and stockholders' equity	\$ 84,703,896	\$ 36,554,274

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