



Zynerba Pharmaceuticals Reports Fourth Quarter and Year End 2016 Financial Results and Operational Highlights

March 27, 2017

Conference call to be held today at 8:30 am ET

DEVON, Pa., March 27, 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 fourth quarter and year ended December 31, 2016 and provided an overview of recent operational highlights.

"2016 was a year marked by tremendous progress for our lead development candidate, ZYN002 CBD gel, as we initiated Phase 2 trials for epilepsy, osteoarthritis and Fragile X syndrome. I am pleased that we have completed enrollment in the epilepsy and osteoarthritis trials," said Armando Anido, Chairman and CEO. "We enter 2017 with great momentum and expect it to be a transformational year with several key milestones expected in the coming months, including top-line results from the three ZYN002 clinical trials and the commencement of our clinical program for ZYN001, our patent-protected pro-drug of THC in development for the treatment of fibromyalgia and peripheral neuropathic pain."

Fourth Quarter 2016 and Recent Highlights

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

Zynerba has completed enrollment in the Phase 2 STAR 1 randomized, double-blind, placebo-controlled clinical trial in adult patients with refractory epilepsy. Of the 224 patients that have been screened, 170 patients have been randomized into the trial and there are 19 patients still in the eight-week baseline period. The Company expects to meet or exceed the 180 patient target for randomization in this trial once all patients have completed the baseline period. Patients are receiving 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. Top-line results are expected in July/August 2017.

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

In November 2016, Zynerba initiated an open-label clinical trial in adult patients with refractory epilepsy who complete the STAR 1 trial. Patients who elect to enroll into the STAR 2 trial receive treatment with ZYN002 for up to 52 weeks. The open-label clinical trial is designed to support long-term safety and tolerability of ZYN002 CBD gel, and is intended to evaluate how ZYN002 CBD gel is tolerated across a range of doses over long-term use. Of the 110 patients who have completed the STAR 1 trial to date, 106 have enrolled into STAR 2.

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

Zynerba has completed enrollment in the Phase 2 STOP randomized, double-blind, placebo-controlled clinical trial in osteoarthritis of the knee. A total of 320 patients have been randomized which exceeds the 300 patient randomization target for the trial. 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 in July/August 2017.

Top-line results for the FAB-C Exploratory Phase 2 Clinical Trial of ZYN002 CBD Gel in Pediatric Fragile X Syndrome Patients Now Expected in Q3 2017

The Phase 2 exploratory clinical trial called FAB-C is designed to evaluate the safety and efficacy of ZYN002 CBD gel in patients between the ages of 8-17 years with Fragile X syndrome (FXS) and is targeting to enroll 16 patients. The complex healthcare needs of children with FXS and the significant impact on families and caregivers have caused delays in patients enrolling into the study. Top-line data are now expected to be available in the third quarter of 2017 rather than the end of the first half of 2017.

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 \$54.3 million, which Zynerba intends to use for the clinical development of ZYN002 and ZYN001, general research and development, and general corporate purposes.

Presented Phase 1 Data on ZYN002 CBD Gel at the 70th Annual Meeting of the American Epilepsy Society

At the 70th Annual Meeting of the American Epilepsy Society held in December, results from a Phase 1 double-blind, placebo-controlled single ascending dose study involving 32 healthy adults were presented in two posters entitled, "Neuropsychological Effects of ZYN002 (Synthetic Cannabidiol) Transdermal Gel in Healthy Subjects and Patients With Epilepsy: Phase 1, Randomized, Double-Blind, Placebo-Controlled Studies" and "Safety and Tolerability of ZYN002 (Synthetic Cannabidiol) Transdermal Permeation-Enhanced Gel in Healthy Subjects and Patients with Epilepsy: Three Phase 1, Randomized, Double-Blind, Placebo-Controlled Studies", which illustrated that ZYN002 CBD gel was safe and well-tolerated at all dose levels ranging from 50 mg to 504 mg and did not produce impairment in critical areas of cognitive function often impacted by medications used to treat central nervous system conditions.

Strengthened Senior Management Team

In December, Marcel Bonn-Miller, PhD, joined Zynerba as Director of Cannabinoid Research. Dr. Bonn-Miller has spent over a decade investigating the interrelations between cannabis and various diseases and disorders. Dr. Bonn-Miller is a world-renowned expert and has published well over 100 peer-reviewed empirical publications, and he serves on the editorial boards of six scientific journals.

In January 2017, Brian Rosenberger was appointed Vice President, Commercial. Mr. Rosenberger is an experienced pharmaceutical executive who has held leadership roles in marketing, sales, business development, analytics and alliance management at Cipher Pharmaceuticals, Auxilium Pharmaceuticals, Neurocrine Biosciences and GlaxoSmithKline.

Anticipated 2017 Milestones

ZYN002, a patent-protected, synthetic CBD formulated as a permeation-enhanced gel for transdermal delivery

• ZYN002 is currently being evaluated in three Phase 2 clinical trials in epilepsy patients with focal seizures, in osteoarthritis and in pediatric patients with FXS which has been designated as an Orphan drug by the US FDA. The Company expects to report top-line data for all three trials in 2017.

- Top-line results from the Phase 2 STAR1 clinical trial in adult epilepsy patients with focal seizures and from the Phase 2 STOP clinical trials in patients with knee pain due to osteoarthritis are anticipated in July/August 2017;
- Top-line results from the FAB-C exploratory Phase 2 clinical trial in pediatric patients with Fragile X syndrome are expected in the third quarter of 2017;

ZYN001, a patent-protected, pro-drug of THC that enables transdermal delivery via a patch

- In the first half of 2017, Zynerba expects to initiate Phase 1 studies to evaluate the safety and pharmacokinetic (PK) profile and tolerability of ZYN001 in healthy volunteers
- Zynerba expects to begin two Phase 2 clinical trials for ZYN001 in patients with fibromyalgia and peripheral neuropathic pain in the second half of the year.

Fourth Quarter and Year End 2016 Financial Results

As of December 31, 2016, cash and cash equivalents totaled \$31.0 million, compared to \$41.5 million as of December 31, 2015. During the year ended December 31, 2016, the Company entered into an Open Market Sales Agreement with Jefferies LLC pursuant to which the Company sold and issued 794,906 shares of common stock in the open market at a weighted average selling price of \$13.39 per share, for net proceeds of \$10.0 million, \$5.3 million of which were received and included as cash and cash equivalents as of September 30, 2016. The remaining \$4.7 million in net proceeds were received and included as cash and cash equivalents as of December 31, 2016.

Research and development expenses for the fourth quarter of 2016 were \$4.9 million, including stock-based compensation of \$0.4 million. General and administrative expenses for the fourth quarter of 2016 were \$1.8 million, including stock-based compensation expense of \$0.5 million. Net loss for the fourth quarter of 2016 was \$6.9 million with basic and diluted net loss per share of \$(0.71).

2017 Financial Outlook

In the first quarter of 2017, the Company completed a follow-on public 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 \$54.3 million. Based on Zynerba's cash position of \$31.0 million in cash and cash equivalents at year-end 2016, and including proceeds from the Company's follow-on public offering in the first quarter of 2017, the Company estimates that this balance is sufficient to develop five Phase 3 ready programs and, assuming feedback from the FDA supports a decision to move forward, initiate at least one Phase 3 program and fund operations and capital requirements into 2019.

Conference Call & Webcast Information

Zynerba management will host a live conference call and webcast today at 8:30 am Eastern Time to discuss the fourth quarter and 2016 financial results as well as operational highlights. The call can be accessed by dialing (844) 815-4960 (U.S. and Canada) or (210) 229-8835 (international) and referencing conference ID 84591304. 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 (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 June 2016, the company initiated 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. In August 2016, 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 initiated. 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 in the first half of 2017. Learn more at www.zynerba.com and follow the Company on Twitter at [@ZynerbaPharma](https://twitter.com/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		Year ended	
	December 31, 2016	December 31, 2015	December 31, 2016	December 31, 2015
Revenue	\$ -	\$ 49,275	\$ 7,250	\$ 278,900
Operating expenses:				
Research and development	4,904,363	3,309,008	16,784,626	7,445,669
General and administrative	1,780,304	2,156,388	6,430,252	5,364,390
Total operating expenses	6,684,667	5,465,396	23,214,878	12,810,059
Loss from operations	(6,684,667)	(5,416,121)	(23,207,628)	(12,531,159)
Other income (expense):				
Interest income	26,980	4,403	80,222	7,352
Foreign exchange loss	(139,829)	-	(189,497)	-
Loss on disposal of equipment	(99,147)	-	(99,147)	-
Total other income (expense)	(211,996)	4,403	(208,422)	7,352
Loss before income taxes	(6,896,663)	(5,411,718)	(23,416,050)	(12,523,807)
Income tax expense (benefit)	-	27,543	(27,543)	27,543
Net loss	\$ (6,896,663)	\$ (5,439,261)	\$ (23,388,507)	\$ (12,551,350)
Net loss per share - basic and diluted	\$ (0.71)	\$ (0.62)	\$ (2.58)	\$ (2.82)
Basic and diluted weighted average shares outstanding	9,678,924	8,787,855	9,070,232	4,457,719
Non-cash stock-based compensation included above:				
Research and development	\$ 365,072	\$ 248,732	\$ 1,281,108	\$ 545,901
General and administrative	522,352	515,069	1,988,258	1,054,724
Total	\$ 887,424	\$ 763,801	\$ 3,269,366	\$ 1,600,625

ZYNERBA PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS
(Unaudited)

December 31, 2016 December 31, 2015

Assets

Current assets:

Cash and cash equivalents	\$ 30,965,791	\$ 41,513,060
Incentive and tax receivables	3,613,943	356,718
Prepaid expenses and other current assets	1,830,958	1,545,917
Total current assets	36,410,692	43,415,695
Property and equipment, net	143,382	227,646
Other assets	200	200
Total assets	\$ 36,554,274	\$ 43,643,541
Liabilities and Stockholders' Equity		
Current Liabilities:		
Accounts payable	\$ 1,848,084	\$ 823,401
Accrued expenses	4,284,907	2,272,991
Deferred grant revenue	833,975	841,225
Total current liabilities	6,966,966	3,937,617
Stockholders' equity:		
Common stock	9,995	9,200
Additional paid-in capital	75,545,875	62,276,779
Accumulated deficit	(45,968,562)	(22,580,055)
Total stockholders' equity	29,587,308	39,705,924
Total liabilities and stockholders' equity	\$ 36,554,274	\$ 43,643,541

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