Zynerba Pharmaceuticals Reports Fourth Quarter and Year End 2015 Financial Results and Operational Highlights

March 14, 2016

Positive Initial Phase 1 ZYN002 Single Rising Dose Results Reported; Phase 1 Multiple Rising Dose Trial Underway

FDA Grants ZYN002 Orphan-Drug Designation for Fragile X Syndrome

Three ZYN002 Phase 2a Trials Planned for Initiation in Second Half 2016

DEVON, Pa., March 14, 2016 (GLOBE NEWSWIRE) -- Zynerba Pharmaceuticals, Inc. (NASDAQ:ZYNE), a specialty pharmaceutical company dedicated to the development of innovative transdermal synthetic cannabinoid treatments, today reported financial results for the quarter and year ended December 31, 2015, and provided an overview of recent operational highlights.

"We are making significant progress in advancing our pipeline of first-in-class transdermal cannabinoid treatments. Most notably, the initial results of the ZYN002 cannabidiol (CBD) gel single rising dose study demonstrated excellent safety and tolerability, and provide compelling rationale for continued development," said Armando Anido, Chairman and CEO of Zynerba Pharmaceuticals. "In 2016, we are expecting a productive year with several upcoming milestones, including the final results of the ZYN002 single and multiple rising dose studies, the initiation of Phase 2 ZYN002 studies in three indications, and the start of the Phase 1 clinical program for ZYN001, our THC pro-drug transdermal patch. We look forward to keeping you updated on our progress."

Fourth Quarter 2015 and Recent Highlights

Reported Positive Initial Results from a Phase 1 Single Rising Dose Trial of ZYN002 CBD Gel: In January 2016, Zynerba reported positive initial safety results from its Phase 1 single rising dose clinical trial of its ZYN002 CBD gel in 32 healthy volunteers. Initial results from the 32 healthy volunteers demonstrated that ZYN002 was safe and well tolerated at all four dose levels. Final results from this Phase 1 trial, including results from patients with epilepsy, are expected in the first half of 2016.

Initiated a Phase 1 Multiple Rising Dose Clinical Trial of ZYN002 CBD Gel: In January 2016, the Company announced the initiation of a Phase 1 multiple rising dose clinical trial to evaluate the pharmacokinetic (PK) profile and tolerability of ZYN002 in 24 healthy volunteers, followed by 12 patients with epilepsy. Results from this second Phase 1 trial are expected in the first half of 2016.

ZYN002 Granted Orphan Drug Designation for Fragile X Syndrome: In February 2016, the U.S. Food and Drug Administration granted orphan-drug designation to ZYN002 CBD gel, for the treatment of Fragile X syndrome (FXS). FXS is a genetic condition that causes intellectual disability, anxiety disorders, behavioral and learning challenges and various physical characteristics. In laboratory studies, CBD has been shown to reduce the metabolism of two endocannabinoids (2-AG and anandamide), which increases 2-AG and anandamide concentrations. In the mouse knockout model for FXS, symptoms are improved when endocannabinoid levels are increased. There is also anecdotal evidence to suggest that CBD oil from plants is effective at improving socialization and language skills in children with FXS.

Appointed Warren Cooper Lead Independent Director: In November 2015, Zynerba announced that its Board of Directors had appointed Dr. Warren Cooper as Lead Independent Director. Dr. Cooper has served on Zynerba's board of directors since August of 2015. He is a UK-trained physician with over 35 years of experience in the global pharmaceutical industry, most recently as CEO of Prism Pharmaceuticals for seven years leading up to the sale of that company to Baxter International in 2011. Dr. Cooper previously served on the Boards of Nutrition 21 Inc., Nuron Biotech Inc., Cardiorentis AG and the World Affairs Council of Philadelphia.

Anticipated 2016 Milestones

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

- In the first half of 2016, Zynerba expects to report final results from its ongoing Phase 1 single rising dose clinical trial, including results from 12 patients with epilepsy.
- In the first half of 2016, Zynerba expects to report results from its second ongoing Phase 1 multiple rising dose clinical trial.
- Pending the results of the Phase 1 trials, Zynerba plans to initiate Phase 2 trials in refractory epilepsy, osteoarthritis (OA), and FXS in the second half of 2016.

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

- In the second half of 2016 Zynerba expects to initiate Phase 1 studies to evaluate the PK profile and tolerability of ZYN001 in healthy volunteers and patients with fibromyalgia.

Fourth Quarter and Year End 2015 Financial Results

In August 2015, Zynerba completed an initial public offering, raising net proceeds of $42.1 million. As of December 31, 2015, cash and cash equivalents were $41.5 million, compared to $9.3 million as of December 31, 2014.

Research and development expenses for the fourth quarter of 2015 were $3.3 million, including stock-based compensation of $0.3 million. General and administrative expenses for the fourth quarter of 2015 were $2.2 million, including stock-based compensation expense of $0.5 million. Net loss for the fourth quarter of 2015 was $5.4 million with basic and diluted net loss per share of $0.62. Adjusted EBITDA for the period, which excludes stock-based compensation, was $4.7 million, or $0.53 per basic and diluted net loss per share.

For the year ended December 31, 2015, research and development expenses were $7.4 million, including stock-based compensation of $0.5 million. General and administrative expenses for the year ended December 31, 2015 were $5.4 million, including stock-based compensation of $1.1 million. Net loss for the year ended December 31, 2015 was $12.6 million with basic and diluted net loss per share of $2.82. Adjusted EBITDA for the period, which excludes stock-based compensation and a transaction related expense, was $10.5 million, or $2.35 per basic and diluted net loss per share. For the year ended December 31, 2015, the Company had 4,457,719 basic and diluted weighted average shares outstanding.

2016 Financial Outlook

The Company ended 2015 with $41.5 million in cash and cash equivalents and believes its current cash position will support its operating plan through Phase 2 data readout for the five indications of ZYN002 and ZYN001.

About Zynerba Pharmaceuticals, Inc.

Zynerba Pharmaceuticals (NASDAQ:ZYNE) is a 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 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 and is being studied in refractory epilepsy, FXS and osteoarthritis. Zynerba is also developing ZYN001, which utilizes a synthetically manufactured pro-drug of THC in a transdermal patch to deliver THC through the skin and into the bloodstream. ZYN001 will be studied in fibromyalgia and peripheral neuropathic pain. Learn more at www.zynerba.com and follow the Company on Twitter at @ZynerbaPharma.

Non-GAAP Financial Measures

The non-GAAP financial information contained herein is a supplement to the corresponding financial measures prepared in accordance with accounting principles generally accepted in the United States of America (GAAP). Non-GAAP measures should be considered in addition to, but not as a substitute for, reported GAAP results. Further, non-GAAP financial measures, even if similarly titled, may not be calculated in the same manner by all companies, and therefore should not be compared.

Management uses adjusted EBITDA (defined as earnings before interest, income taxes, depreciation, amortization, stock-based compensation and transaction related expense) in its evaluation of the Company's core results of operations and trends between fiscal periods and believes that these measures are important components of its internal performance measurement process. Management believes that this non-GAAP financial information reflects an additional way of viewing aspects of our business that, when viewed with our GAAP results, provide a more complete understanding of factors and trends affecting our business. Please see the section of this press release titled "Reconciliation of Adjusted EBITDA."

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. 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. These and other risks are described in the Company's periodic reports, including the annual report on Form 10K, quarterly reports on Form 10Q 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, 2015	**December 31, 2014**	**December 31, 2015**	**December 31, 2014**
Revenues	$ 49,275	$ 74,157	$ 278,900	$ 810,012

Operating expenses:				
Research and development	3,309,008	815,547	7,445,669	2,401,406
General and administrative	2,156,388	662,927	5,364,390	4,076,339
Total operating expenses	5,465,396	1,478,474	12,810,059	6,477,745
Loss from operations	(5,416,121)	(1,404,317)	(12,531,159)	(5,667,733)
Other income (expense):				
Interest income (expense), net	4,403	-	7,352	(1,844)
Loss before income taxes	(5,411,718)	(1,404,317)	(12,523,807)	(5,669,577)
Income tax expense	(27,543)	-	(27,543)	-
Net loss	(5,439,261)	(1,404,317)	(12,551,350)	(5,669,577)
Accretion of redeemable convertible preferred stock	-	-	-	(87,954)
Net loss applicable to common stockholders	$ (5,439,261)	$ (1,404,317)	$ (12,551,350)	$ (5,757,531)
Net loss per share - basic and diluted	$ (0.62)	$ (0.96)	$ (2.82)	$ (6.44)
Basic and diluted weighted average shares outstanding	8,787,855	1,462,101	4,457,719	894,575

ZYNERBA PHARMACEUTICALS, INC.

CONSOLIDATED BALANCE SHEETS

(Unaudited)

	December 31, 2015	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	$ 41,513,060	$ 9,330,681
Deferred offering costs	—	1,080,199
Prepaid expenses and other current assets	1,902,635	1,183,949
Total current assets	43,415,695	11,594,829
Property and equipment, net	227,646	19,642
Other assets	200	2,200
Total assets	$ 43,643,541	$ 11,616,671
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current Liabilities:		
Accounts payable	$ 823,401	$ 313,937
Accrued expenses	2,272,991	1,711,473
Deferred grant revenue	841,225	1,120,125
Total current liabilities	3,937,617	3,145,535
Convertible Preferred Stock:		
Series 1 convertible preferred stock	—	16,522,811
Stockholders' equity (deficit):		
Common stock	9,200	2,030
Additional paid-in capital	62,276,779	1,975,000
Accumulated deficit	(22,580,055)	(10,028,705)
Total stockholders' equity (deficit)	39,705,924	(8,051,675)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 43,643,541	$ 11,616,671

ZYNERBA PHARMACEUTICALS, INC.

RECONCILIATION OF ADJUSTED EBITDA AND ADJUSTED EBITDA PER SHARE

(Unaudited)

	Three months ended		Year ended	
	December 31, 2015	**December 31, 2014**	**December 31, 2015**	**December 31, 2014**
GAAP Net loss	$ (5,439,261)	$ (1,404,317)	$ (12,551,350)	$ (5,757,531)
Add back:				
Depreciation	13,255	1,451	26,027	27,063
Interest (income) expense	(4,403)	-	(7,352)	1,844
Income tax expense	(27,543)	-	(27,543)	-
EBITDA	(5,457,952)	(1,402,866)	(12,560,218)	(5,728,624)
Add back:				
Stock-based compensation	763,801	-	1,600,625	-
Transaction related expense	-	-	500,000	-
Adjusted EBITDA	$ (4,694,151)	$ (1,402,866)	$ (10,459,593)	$ (5,728,624)
GAAP Net loss per share	$ (0.62)	$ (0.96)	$ (2.82)	$ (6.44)
Add back:				
Depreciation	0.00	0.00	0.01	0.03
Interest (income) expense	(0.00)	-	(0.00)	0.00
Income tax expense	(0.00)	-	(0.01)	-
EBITDA per share	(0.62)	(0.96)	(2.82)	(6.41)
Add back:				
Stock-based compensation	0.09	-	0.36	-
Transaction related expense	-	-	0.11	-
Adjusted EBITDA per share	$ (0.53)	$ (0.96)	$ (2.35)	$ (6.41)
Shares used in computation of GAAP and adjusted EBITDA per share - basic and diluted	8,787,855	1,462,101	4,457,719	894,575

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