Zynerba Pharmaceuticals Reports Second Quarter 2017 Financial Results and Operational Highlights

August 1, 2017

Top-line Phase 2 results for STAR 1 trial in epilepsy and STOP trial in osteoarthritis remain on track for reporting in August 2017

DEVON, Pa., Aug. 01, 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 second quarter ended June 30, 2017 and provided an overview of recent operational highlights.

“We expect to announce top-line results from the STAR 1 trial soon, followed by top-line data from the STOP trial later this month; and we remain on track to report top-line results from the FAB-C Fragile X study in September,” said Armando Anido, Chairman and Chief Executive Officer. “We also met a significant milestone during the quarter in initiating the Phase 1 program for ZYN001, a pro-drug of THC delivered via patch, and expect to initiate our Phase 2 program by the end of this year. With two clinical stage assets, Zynerba is well-positioned to address a number of serious unmet medical needs.”

Second Quarter 2017 and Recent Highlights

Completed Dosing in Phase 2 STAR 1 Clinical Trial for ZYN002 Cannabidiol (CBD) Gel in Adult Epilepsy Patients; Top Line Data Expected Soon

A total of 188 patients were 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 were 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.

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

Ninety-eight percent of the patients who completed the STAR 1 trial elected 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 daily, respectively) for up to 52 weeks.

Completed Dosing of Phase 2 STOP Clinical Trial for ZYN002 CBD Gel in Adult Osteoarthritis Patients; Top Line Data Expected this Month

Dosing is complete in the randomized, double-blind, placebo-controlled Phase 2 STOP trial in osteoarthritis of the knee. We exceeded the initial target enrollment of 300 patients with 320 patients randomized into one of three dosing groups. Patients received 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.

Completed Enrollment in FAB-C Exploratory Phase 2 Clinical Trial of ZYN002 CBD Gel in Pediatric Fragile X Syndrome Patients; Top Line Data Expected in September 2017

Enrollment is complete and dosing is ongoing in the Phase 2 exploratory FAB-C clinical trial designed to evaluate the safety and efficacy of ZYN002 CBD gel administered over a 12-week period in pediatric patients with Fragile X syndrome (FXS). We exceeded the initial target enrollment of 16 patients with 20 patients enrolled. The study is evaluating 50 mg of CBD in ZYN002 4.2% gel once daily, which may be titrated up to 125 mg two times per day during the six-week titration period. Between weeks six and twelve, patients receive a maintenance dose of 50 mg, 100 mg or 250 mg daily of CBD in ZYN002 4.2% gel. The primary objective is to assess intra-patient changes in anxiety, depression and mood (as measured by the ADAMS scale) versus baseline. The U.S. Food and Drug Association has granted orphan-drug designation to ZYN002 CBD gel for the treatment of Fragile X syndrome.

Initiated Phase 1 Program for ZYN001 Pro-drug of Tetrahydrocannabinol (THC) Delivered via Transdermal Patch

The company has initiated a Phase 1 program to assess ZYN001, a patent-protected, pro-drug of THC delivered via a patch. This first in man study is a randomized, double-blind, placebo-controlled Phase 1 trial. First, the safety, tolerability and pharmacokinetic profile of a single dose of ZYN001 versus placebo will be evaluated. Several formulations and patch wear times ranging from 24 hours to 7 days will be assessed in up to 48 healthy subjects. Based on results from the single dose portion of this trial, two formulations will be evaluated in multiple patch applications for 14 days in up to 32 healthy subjects who will be randomized 3:1 to ZYN001 or placebo. Results from this study will inform the planned Phase 2 program for ZYN001 in fibromyalgia and neuropathic pain, expected to initiate in the fourth quarter of 2017.

Second Quarter 2017 Financial Results

As of June 30, 2017, cash and cash equivalents were $70.2 million, compared to $77.5 million as of March 31, 2017. Research and development expenses for the second quarter of 2017 were $5.7 million, including stock-based compensation of $0.6 million. General and administrative expenses for the second quarter of 2017 were $2.6 million, including stock-based compensation expense of $0.8 million. Net loss for the second quarter of 2017 was $8.3 million with basic and diluted net loss per share of $0.64.

Financial Outlook

The Company believes that the current cash and cash equivalent position of $70.2 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 June 2017, the Company completed enrollment in its exploratory Phase 2 FAB-C (Treatment of Fragile X Syndrome Anxiety and Behavioral Challenges with CBD) clinical trial in children with Fragile X syndrome. Zynerba is also developing ZYN001, which utilizes a synthetically manufactured pro-drug of THC. A Phase 1 clinical program for ZYN001 was initiated in the second quarter 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		Six months ended	
	June 30, 2017	**June 30, 2016**	**June 30, 2017**	**June 30, 2016**
Revenue	$ —	$ —	$ —	$ 7,250
Operating expenses:				
Research and development	5,732,797	4,807,177	11,224,252	7,376,167
General and administrative	2,632,857	1,476,357	4,844,650	3,156,487
Total operating expenses	8,365,654	6,283,534	16,068,902	10,532,654
Loss from operations	(8,365,654)	(6,283,534)	(16,068,902)	(10,525,404)
Other income (expense):				
Interest income	124,535	18,118	201,420	30,496
Foreign exchange (loss) gain	(82,360)	(20,250)	284,982	(43,398)
Total other income (expense)	42,175	(2,132)	486,402	(12,902)
Loss before income taxes	(8,323,479)	(6,285,666)	(15,582,500)	(10,538,306)
Income tax expense	—	(56,277)	—	(27,543)
Net loss	$ (8,323,479)	$ (6,229,389)	$ (15,582,500)	$ (10,510,763)
Net loss per share - basic and diluted	$ (0.64)	$ (0.70)	$ (1.24)	$ (1.19)
Basic and diluted weighted average shares outstanding	13,052,294	8,860,592	12,562,594	8,842,271
Non-cash stock-based compensation included above:				
Research and development	$ 588,713	$ 374,919	$ 1,130,558	$ 623,651
General and administrative	766,661	437,820	1,413,515	952,887
Total	$ 1,355,374	$ 812,739	$ 2,544,073	$ 1,576,538

ZYNERBA PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS
(Unaudited)

	June 30, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 70,179,199	$ 30,965,791
Incentive and tax receivables	3,971,828	3,613,943
Prepaid expenses and other current assets	2,307,466	1,830,958
Total current assets	76,458,493	36,410,692
Property and equipment, net	177,994	143,382
Incentive and tax receivables	2,456,286	—
Other assets	200	200
Total assets	$ 79,092,973	$ 36,554,274
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,024,991	$ 1,848,084
Accrued expenses	5,004,856	4,284,907
Deferred grant revenue	833,975	833,975
Total current liabilities	7,863,822	6,966,966
Stockholders' equity:		
Common stock	13,257	9,995
Additional paid-in capital	132,766,956	75,545,875
Accumulated deficit	(61,551,062)	(45,968,562)
Total stockholders' equity	71,229,151	29,587,308
Total liabilities and stockholders' equity	$ 79,092,973	$ 36,554,274

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