



Zynerba Pharmaceuticals Reports Fourth Quarter and Year End 2018 Financial Results and Operational Highlights

March 11, 2019

DEVON, Pa., March 11, 2019 (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 fourth quarter and year ended December 31, 2018 and provided an overview of recent operational highlights. The Company also announced that its current cash and cash equivalent position is expected to fund operations and capital requirements into the first quarter of 2021.

"The fourth quarter of 2018 was a period of strong clinical and corporate momentum, as we achieved our remaining 2018 milestones and positioned ourselves well for a watershed year in 2019," said Armando Anido, Chairman and Chief Executive Officer of Zynerba. "We continued enrollment into CONNECT-FX, our pivotal trial of Zygel™ in Fragile X Syndrome, and completed enrollment in BELIEVE 1, our Phase 2 trial in Developmental and Epileptic Encephalopathies. We also initiated the BRIGHT trial, which will evaluate Zygel in Autism Spectrum Disorder and are on track to initiate a Phase 2 trial in 22q11.2 Deletion Syndrome in the second quarter of 2019. Finally, we have a cash position that we expect to take us through our expected NDA submission and potential approval for Zygel in Fragile X Syndrome."

Fourth Quarter 2018 and Recent Highlights

Zygel in Fragile X Syndrome (FXS)

Fragile X Syndrome Pivotal Data Expected in the Second Half of 2019

Enrollment is progressing in CONNECT-FX, a pivotal, multi-national, randomized, double blind, placebo-controlled trial evaluating the efficacy and safety of Zygel (formerly referred to as ZYN002) in three through 17-year old patients with FXS. 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. Clinical investigative sites are enrolling patients in the United States, Australia, and New Zealand. Patients who have completed the double-blind phase are now enrolling into the 12-month open label extension phase. The Company expects to report top line data in the second half of 2019. If the data are positive, the Company expects to submit its New Drug Application (NDA) for Zygel in FXS to the U.S. Food and Drug Administration in the first half of 2020, with potential approval by year-end 2020. There are currently no approved products indicated for FXS.

Presented New Data at the 57th Annual Meeting of the American College of Neuropsychopharmacology (ACNP) from the Ongoing Open Label FAB-C Phase 2 Trial of Zygel

Zynerba presented new data demonstrating that treatment with Zygel improved core emotional and behavioral symptoms of FXS with statistical significance versus baseline across multiple measures of efficacy at month three, and that these improvements were sustained through 12 months of treatment. For example, significant improvements vs. baseline in social avoidance as measured by the ABC-C_{FXS} were demonstrated at three months (58% improvement; $p=0.0040$) and 12 months (77% improvement; $p=0.0013$) of treatment with Zygel.

Announced Receipt of New U.S. Patent for Treatment of Fragile X Syndrome with Cannabidiol (CBD)

The U.S. Patent and Trademark Office issued U.S. Patent No. 10,213,390 titled "Treatment of Fragile X Syndrome with Cannabidiol" which includes claims directed to methods of treating Fragile X Syndrome by administering a therapeutically effective amount of synthetic or purified CBD. This new patent expires in 2038 and is part of an expanding intellectual property portfolio covering Zygel.

FAB-C Data Accepted for Presentation at the Annual Meeting of the American Psychiatric Association (APA), May 18–22, 2019 in San Francisco, CA

The presentation will describe data from the FAB-C (Treatment of Fragile X Syndrome Anxiety and Behavioral Challenges with CBD) trial that highlight the short- and long-term positive impact of ZYN002 on children and adolescents with FXS. The poster entitled "Transdermal Cannabidiol (CBD) Gel for the Treatment of Fragile X Syndrome" (poster #P5-092) will be presented on Monday, May 20, 2019 from 10:00AM to 12:00PM PDT during Poster Session 5.

Zygel in Developmental and Epileptic Encephalopathies (DEE)

Enrollment Complete in Phase 2 BELIEVE 1 Trial; Topline Results Expected in the Third Quarter of 2019

The Company has completed enrollment in BELIEVE 1, an open label multi-dose Phase 2 clinical trial evaluating the efficacy and safety of Zygel in children and adolescents (three through 17 years) with DEE. The primary efficacy assessment is reduction in seizure frequency at week 26 compared to baseline. Patients successfully completing the 26 weeks of the trial may elect to enter a 6-month extension of the trial. The Company expects to announce topline data through week 26 in the third quarter of 2019.

Zygel in Autism Spectrum Disorder (ASD) and 22q11.2 Deletion Syndrome (22q)

Expanded Clinical Pipeline into Two New Neuropsychiatric Indications

The Company completed an extensive review of the neuropsychiatric disorder landscape for additional priority indications for clinical development, and announced that it would evaluate Zygel in ASD and 22q.

- Zynerba recently initiated the Phase 2 BRIGHT (An Open-Label Tolerability and Efficacy Trial of ZYN002 Administered as a Transdermal Gel to Children and Adolescents with Autism Spectrum Disorder) clinical trial. The trial will assess the safety, tolerability and efficacy of Zygel for the treatment of child and adolescent patients with ASD. The Company expects to release top line data from this trial in the first half of 2020.
- 22q is the most common gene deletion syndrome affecting as many as 81,000 patients in the U.S. 22q is associated with increased anxiety, withdrawn behavior and social interaction problems. The Company expects to initiate an open label Phase 2 trial of Zygel in 22q in the second quarter of 2019, and to present top line data in the first half of 2020.

Corporate

Announced Addition of Pamela Stephenson to Board of Directors

Pamela Stephenson brings 24 years of commercialization expertise including health economics, market access, and commercial planning to Zynerba's Board of Directors. Ms. Stephenson's experience includes nearly 11 years at Vertex, including in her tenure as Vice President for Global Market Access and Value, and 10 years with Pfizer in various positions of increasing strategic importance.

Fourth Quarter 2018 Financial Results

As of December 31, 2018, cash and cash equivalents were \$59.8 million, compared to \$62.5 million as of December 31, 2017. Research and development expenses for the fourth quarter of 2018 were \$4.9 million, including stock-based compensation of \$0.8 million. General and administrative expenses for the fourth quarter of 2018 were \$3.3 million, including stock-based compensation expense of \$0.8 million. Net loss for the fourth quarter of 2018 was \$7.8 million with basic and diluted net loss per share of \$(0.44).

On June 9, 2017, we entered into an Open Market Sales Agreement, or "at-the-market" (ATM) offering program, with Jefferies LLC, pursuant to which we may sell, from time to time, up to \$50 million of our common stock. From January 29, 2019 through March 6, 2019, the Company has sold and issued 3,439,523 shares under its ATM program, at a weighted average selling price of \$5.44 per share, for gross proceeds of \$18.7 million. Net proceeds after deducting commissions and offering expenses were \$18.1 million, which will be recorded in the first quarter of 2019 and included in the March 31, 2019 cash and cash equivalents position.

Financial Outlook

The Company's cash and cash equivalent position as of December 31, 2018 was \$59.8 million. Including the \$18.1 million in net proceeds from the shares sold and issued under its ATM program in the first quarter of 2019, management believes that the cash and cash equivalent position is sufficient to fund operations and capital requirements beyond the expected NDA submission and potential approval in FXS and into the first quarter of 2021.

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, Autism Spectrum Disorder, 22q11.2 Deletion Syndrome, and a heterogeneous group of rare and ultra-rare epilepsies known as developmental and epileptic encephalopathies. 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.

	(unaudited)		Year ended December 31,	
	2018	2017	2018	2017
Revenue	\$ 86,000	\$ —	\$ 86,000	\$ —
Operating expenses:				
Research and development	4,876,162	5,828,091	27,245,043	22,806,107
General and administrative	3,256,044	2,376,413	13,238,787	10,016,902
Total operating expenses	8,132,206	8,204,504	40,483,830	32,823,009
Loss from operations	(8,046,206)	(8,204,504)	(40,397,830)	(32,823,009)
Other income (expense):				
Interest income	321,621	156,204	961,323	519,554
Foreign exchange (loss) gain	(65,658)	(70,299)	(474,668)	291,151
Total other income (expense)	255,963	85,905	486,655	810,705
Net loss	\$ (7,790,243)	\$ (8,118,599)	\$ (39,911,175)	\$ (32,012,304)
Net loss per share - basic and diluted	\$ (0.44)	\$ (0.60)	\$ (2.61)	\$ (2.48)
Basic and diluted weighted average shares outstanding	17,616,373	13,423,669	15,308,886	12,914,814
Non-cash stock-based compensation included above:				
Research and development	\$ 819,715	\$ 562,410	\$ 3,087,498	\$ 2,284,866
General and administrative	778,915	817,726	3,538,245	3,361,986
Total	\$ 1,598,630	\$ 1,380,136	\$ 6,625,743	\$ 5,646,852

ZYNERBA PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS

	December 31, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 59,763,773	\$ 62,510,277
Incentive and tax receivables	3,444,620	3,983,604
Prepaid expenses and other current assets	3,747,087	1,733,701
Total current assets	66,955,480	68,227,582
Property and equipment, net	371,963	164,527
Other assets	—	662,200
Total assets	\$ 67,327,443	\$ 69,054,309
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 4,461,567	\$ 3,355,255
Accrued expenses	5,264,215	3,915,491
Deferred grant revenue	—	171,975
Total current liabilities	9,725,782	7,442,721
Deferred grant revenue, long-term	—	662,000
Total liabilities	9,725,782	8,104,721
Stockholders' equity:		
Common stock	17,627	13,554
Additional paid-in capital	175,476,075	138,916,900
Accumulated deficit	(117,892,041)	(77,980,866)
Total stockholders' equity	57,601,661	60,949,588
Total liabilities and stockholders' equity	\$ 67,327,443	\$ 69,054,309

Zynerba Contacts

Jim Fickenscher, CFO and VP Corporate Development
Zynerba Pharmaceuticals
484.581.7483
fickenscherj@zynerba.com

Will Roberts, VP Investor Relations and Corporate Communications
Zynerba Pharmaceuticals
484.581.7489
robertsw@zynerba.com



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