Zynerba Pharmaceuticals Reports First Quarter 2020 Financial Results and Operational Highlights

May 11, 2020

Topline Results from Pivotal CONNECT-FX and Phase 2 BRIGHT Trials Expected in the Second Quarter of 2020

DEVON, Pa., May 11, 2020 (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 first quarter ended March 31, 2020 and provided an overview of recent operational highlights.

"The past few months have been historic due to the outbreak of COVID-19, and we applaud everyone on the front line of this global pandemic, including healthcare workers, first responders, humanitarians, and our industry colleagues who are dedicated to the rapid development of treatments and vaccines," said Armando Anido, Chairman and Chief Executive Officer of Zynerba. "In response to the pandemic, Zynerba implemented important initiatives that we believe protect the safety of patients, clinical investigators and their staff, and our Zynerba employees and should allow us to conclude all three of our ongoing clinical trials and report top line results within our stated timelines."

First Quarter 2020 and Recent Highlights

COVID-19 Preparedness

Implemented Plan to Prioritize the Safety of Patients and Caregivers, Minimize Risk of Supply Disruption, and Enable the Company to Achieve its Milestones

Zynerba and its contract research and manufacturing partners began contingency planning for the COVID-19 outbreak in January 2020 in conjunction with its clinical investigator sites. Helping to ensure the safety of clinicians and participating patients and their families is paramount; as such, the Company made early and actionable adjustments including remote site monitoring, patient visits using telemedicine where needed, direct shipment of study drug supplies to caregivers, and use of local clinical laboratories to collect study related blood samples. The approach is consistent with the U.S. Food and Drug Administration's (FDA) Guidance on Conduct of Clinical Trials of Medical Products during COVID-19 Pandemic. At this time, we expect our timelines for delivery of top line results from all of our ongoing trials to not be impacted.

Zygel in Fragile X Syndrome (FXS)

Topline Results Expected from Pivotal CONNECT-FX Trial Late in the Second Quarter of 2020

Two hundred and twelve (212) children and adolescents with genetically-confirmed Fragile X syndrome (FXS) have been randomized into CONNECT-FX, a pivotal, multi-national, randomized, double blind, placebo-controlled trial evaluating the efficacy and safety of Zygel in treating common behavioral symptoms of FXS. Zynerba completed enrollment in February 2020 and expects to report topline results late in the second quarter of 2020. If the results are positive, Zynerba intends to request a meeting with the FDA to determine the acceptability of the data as a basis for a New Drug Application (NDA) and to seek advice on preparation of the marketing authorization. The Company expects to submit its NDA for Zygel in FXS to the FDA in the second half of 2020, with potential approval by mid-year 2021. (Press release)

Robust Enrollment Continues into Open Label Extension Study

During the screening phase of CONNECT-FX, caregivers of patients in the trial were informed that their participating child may have the opportunity to receive Zygel in an open label extension trial following the child's compliant completion of CONNECT-FX, regardless of their child's perceived response or actual blinded drug assignment at randomization in CONNECT-FX. As of May 8, 2020, 96% of the 188 patients who have completed the 14-week blinded portion of the CONNECT-FX trial have enrolled in the open label extension trial.

Zygel in Autism Spectrum Disorder (ASD)

Topline Results from Phase 2 BRIGHT Trial of Zygel in ASD Expected in the Second Quarter of 2020

Thirty-seven (37) children and adolescents with moderate-to-severe ASD have been enrolled in the 14-week open label exploratory Phase 2 BRIGHT trial. The clinical trial is designed to evaluate the safety, tolerability and efficacy of Zygel as an add-on to standard-of-care for the treatment of pediatric and adolescent patients with ASD. The Company is utilizing a number of efficacy assessments, including the Aberrant Behavior Checklist, Parent Rated Anxiety Scale – Autism Spectrum Disorder, and Clinical Global Impression – Severity and Improvement, to identify the appropriate endpoint to use in future placebo-controlled trials. Zynerba completed enrollment in this trial in January 2020 and expects to report topline results in the second quarter of 2020 (Press release)

New U.S. Patent Received for Treatment of ASD with Transdermal Cannabidiol

The U.S. Patent and Trademark Office has issued U.S. Patent No. 10,568,848, titled "Treatment of Autism with Cannabidiol" which includes claims directed to methods of treating ASD by transdermally administering, via a gel or cream, a therapeutically effective amount of purified CBD. The patent expires in 2038. (Press release)

Zygel in 22q11.2 Deletion Syndrome (22q)

Phase 2 Open Label Trial of Zygel in 22q Ongoing; Data Expected in the Third Quarter of 2020

The Company is conducting the 14-week Phase 2 INSPIRE trial to evaluate the safety, tolerability and efficacy of Zygel in approximately 20 children and adolescents (ages six through 17) with genetically-confirmed 22q. Zynerba expects to report topline results from this study in the third quarter of 2020.

Zygel in Developmental and Epileptic Encephalopathies (DEE)

Outcome of Discussions with FDA on Clinical Pathway for Zygel in DEE Expected in the Second Quarter of 2020

Based on the Phase 2 trial design and positive efficacy and safety results, Zynerba anticipates that it will pursue an indication that includes the syndromes and encephalopathies in the DEE category that present with focal impaired-awareness seizures (FIAS; previously known as complex partial seizures) and/or convulsive seizures (CS), the most common and debilitating seizure types representing 75% to 80% of all seizures. Zynerba expects to disclose the outcome of the interactions with the FDA in the second quarter of 2020.

First quarter 2020 Financial Results

As of March 31, 2020, cash and cash equivalents were $60.6 million, compared to $70.1 million as of December 31, 2019. Research and development expenses for the first quarter of 2020 were $6.9 million, including stock-based compensation of $0.5 million. General and administrative expenses for the first quarter of 2020 were $3.9 million, including stock-based compensation expense of $0.8 million. The net loss for the first quarter of 2020 was $12.3 million with basic and diluted net loss per share of $(0.53). Included in the net loss was $1.7M in non-cash foreign currency losses, which are primarily due to the remeasurement of our Australian subsidiary's assets and liabilities, which are denominated in the local currency to the subsidiary's functional currency, which is the U.S. dollar.

Financial Outlook

Management believes that the cash runway is sufficient to fund operations and capital requirements beyond the expected NDA submission and potential approval of Zygel in FXS and into the second half 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; the Company's expectations regarding its ability to obtain and adequately maintain sufficient intellectual property protection for its product candidates; the timing and outcome of current and future legal proceedings; and the extent to which health epidemics and other outbreaks of communicable diseases, including COVID-19, could disrupt our operations or adversely affect our business and financial conditions. 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,	
	2020	**2019**
Operating expenses:		
Research and development	$ 6,882,793	$ 6,306,712
General and administrative	3,916,569	3,159,657
Total operating expenses	10,799,362	9,466,369
Loss from operations	(10,799,362)	(9,466,369)
Other income (expense):		
Interest income	201,684	350,951
Foreign exchange loss	(1,740,151)	(31,599)

Total other income (expense)	(1,538,467)	319,352
Net loss	$ (12,337,829)	$ (9,147,017)
Net loss per share - basic and diluted	$ (0.53)	$ (0.47)
Basic and diluted weighted average shares outstanding	23,399,438	19,452,088
Non-cash stock-based compensation included above:		
Research and development	$ 510,476	$ 666,179
General and administrative	812,876	830,113
Total	$ 1,323,352	$ 1,496,292

ZYNERBA PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS

	(unaudited) March 31, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 60,638,853	$ 70,063,242
Incentive and tax receivables	12,906,735	14,613,969
Prepaid expenses and other current assets	1,474,930	2,378,812
Total current assets	75,020,518	87,056,023
Property and equipment, net	587,267	362,724
Incentive and tax receivables	571,329	—
Right-of-use assets	287,160	345,849
Total assets	$ 76,466,274	$ 87,764,596
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 3,328,339	$ 4,740,981
Accrued expenses	6,676,988	7,073,506
Lease liabilities	252,394	243,677
Total current liabilities	10,257,721	12,058,164
Lease liabilities, long-term	44,237	109,689
Total liabilities	10,301,958	12,167,853
Stockholders' equity:		
Common stock	23,572	23,211
Additional paid-in capital	229,314,197	226,409,156
Accumulated deficit	(163,173,453)	(150,835,624)
Total stockholders' equity	66,164,316	75,596,743
Total liabilities and stockholders' equity	$ 76,466,274	$ 87,764,596

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