

Zynerba Pharmaceuticals

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Zynerba shares evidence that CBD may be effective in managing constellation of sociobehavioral symptoms

Zynerba Pharmaceuticals Inc (NASDAQ:ZYNE) shared data Thursday on how CBD may be effective in managing sociobehavioral symptoms associated with three different neuropsychiatric disorders at a symposium organized by the Society for the Study of Behavioural Phenotypes (SSBP).

In a statement, the Devon, Pennsylvania-based company said: "Data indicates that patients with autism spectrum disorder, Fragile X Syndrome and 22q11.2 Deletion Syndrome, share a constellation of sociobehavioral symptoms and that the pharmacology of CBD is broad, continues to be defined, and may prove to be beneficial in addressing important behavioral symptoms of these conditions."

Investors responded well, sending shares nearly 4.6% higher to \$11.22 in morning trade.

Dr Honey Heussler, associate professor, at the University of Queensland, Australia is presenting a poster titled "Common Behavioral Features of Autism, Fragile X Syndrome, and 22q11.2 Deletion Syndrome," at the two-day SSBP symposium at Aston University, in Birmingham, UK, starting on Thursday.

READ: Zynerba boosts cash position in 2Q, gears up for top-line data from key trials with Zygel CBD gel

The company said new data shows that patients with the three neuropsychiatric disorders share sociobehavioral symptoms that include anxiety, which may lead to "social avoidant behavior, aggression, irritability, low attention and poor communication."

"Those of us who care for patients and families contending with certain neuropsychiatric dysfunction understand that there are significant shared sociobehavioral symptoms between such disorders, though until now no review has been conducted to examine or clarify the overlap," Dr Heussler said in today's statement.

"These data on the shared behaviors between ASD, FXS and 22qDS are important to understanding disease impact, patient care, and the development of potential treatments. One such potential treatment being studied in well-controlled clinical trials is a proprietary gel formulation of CBD, which has diverse pharmacologic effects and may prove to be important in these neuropsychiatric disorders," she added.

A CBD gel treatment for children with Fragile X

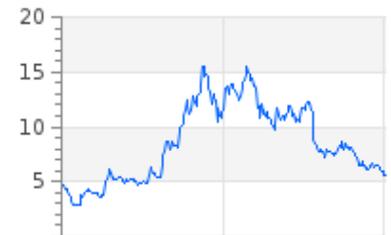
Zynerba Pharmaceuticals is on track to report top-line results from tests of Zygel, a CBD gel treatment for children and adolescents with Fragile X syndrome, the most common form of inherited learning disability and autism spectrum disorder.

The company maintains that pivotal data will be available in the first half of 2020 from the CONNECT-FX study.

Price: 5.62

Market Cap: \$130.37 m

1 Year Share Price Graph



December 2018 June 2019 December 20

Share Information

Code: ZYNE

Listing: NASDAQ

52 week	High	Low
	16.46	2.82

Sector: Pharma & Biotech

Website: zynerba.com

Company Synopsis:

At Zynerba Pharmaceuticals, our team is dedicated to developing next-generation transdermally-delivered cannabinoid therapeutics for patients affected by rare and near-rare neuropsychiatric conditions. Often, these diseases have few, if any, treatment options and can leave patients and their families feeling helpless and alone.

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Zygel was earlier referred to as ZYN002 before Zynerba selected the new brand name for the patent-protected CBD skin gel.

Meanwhile, Zynerba is also presenting data at the symposium showing that 90% of interviewed caregivers of kids with Fragile X Syndrome reported seeing "social avoidant, irritability, and socially lethargic" behavior.

A poster, titled "Content Validity of the ABC-CFXS and Subscales in Fragile X Syndrome," is being presented on September 5 and 6.

Terri Browning Sebree, president at Zynerba, is presenting evidence on the "appropriateness of social avoidance, irritability, and socially unresponsive/lethargic subscales of the ABC-CFXS in assessing core symptoms of Fragile X Syndrome."

The company said it is utilizing the ABC-CFXS to assess improvements in core behaviors of Fragile X Syndrome in its ongoing 14-week pivotal CONNECT-FX study of Zygel.

"Fragile X Syndrome is a complex diagnosis for a child and his/her family, marked by a myriad of specific behavioral and emotional symptoms often manifesting as anxiety and social avoidant behaviors," said Sebree in the company's statement.

"Through studies like this one, we are able to not only elucidate the most common core behaviors of FXS, but also further validate the appropriateness of the ABC-CFXS as an effective tool for use in clinical studies as a means to measure improvements in these core and common Fragile X Syndrome behaviors," she added.

Ten caregivers of children formally diagnosed with Fragile X Syndrome participated in the study with assistance from the National Fragile X Foundation. The study found that 90% of caregivers reported at least one behavior that was representative of social avoidance, socially unresponsive/lethargic, and irritability.

Other Phase 2 clinical developments

In addition to Fragile X syndrome, Zygel is also in Phase 2 clinical development in patients with refractory epilepsy, Autism Spectrum Disorder and 22q11.2 Deletion Syndrome, a disorder caused by a small missing piece of the 22nd chromosome. This tiny missing portion of chromosome 22 can wreck every system in the body.

The US Patent and Trademark Office recently granted Zynerba a patent for treating autism spectrum disorder, characterized by challenges with social skills and speech, with CBD.

Zygel is also being tested for a heterogeneous group of rare and ultra-rare epilepsies known as developmental and epileptic encephalopathies (DEE).

The company expects to share topline data from BELIEVE 1, an open label multidose Phase 2 clinical trial evaluating the efficacy and safety of Zygel in children and adolescents with various DEEs, in September.

The primary efficacy assessment is of a reduction in "seizure frequency at week 26" compared to baseline scores, said the company in a statement.

ROTH Capital repeats Buy rating

In a note on Thursday, analysts at ROTH Capital highlighted the "DEE opportunity" and drew comparisons with GW Pharmaceuticals PLC's (NASDAQ:GWPH) cannabis-derived Epidiolex treatment for seizures associated with Lennox-Gastaut Syndrome in patients two years of age or older. The FDA's approval of that was based on three studies and an open-label extension study, showing Epidiolex, when added to other drugs, significantly reduced the frequency of seizures in patients.

ROTH senior analyst Jerry Isaacson said: "GW Pharma's Epidiolex, is currently approved for a subset of DEE, Dravet syndrome and Lennox-Gastaut syndrome. GW Pharma reported \$68.4 million in revenue for Epidiolex in Q2."

"Zynerba's trial includes a broader group of DEE patients. Potential advantages of Zynerba's product include the need for a much smaller dose of CBD, which may help with safety and ease of administration. We are looking for a reduction in seizures of at least 35% from baseline in this open label trial," he added.

The analyst assumes approval for Fragile X Syndrome in 2021 and DEE in 2023, with pricing starting at \$20,000 annually per patient.

"This pricing is justified by the \$32,500 annual price tag for Epidiolex. Using a 12% discount rate, 60% chance of approval in FXS and 40% chance of approval in DEE, we estimate \$560 million in total sales in 2029," Isaacson said in the note.

"Potential approval in ASD provides upside to current estimates," he added.

ROTH has a "Buy" rating on Zynerba Pharmaceuticals and a 12-month price target of \$36.

-- Adds broker comment, updates share price --

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