



## **Zynerba Pharmaceuticals Receives Orphan Drug Designation for Cannabidiol for the Treatment of 22q11.2 Deletion Syndrome**

September 17, 2020

DEVON, Pa., Sept. 17, 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 announced that the U.S. Food and Drug Administration has granted orphan drug designation for cannabidiol (CBD) for use in treating 22q11.2 deletion syndrome (22q). 22q is a rare midline condition featuring physical abnormalities and debilitating neuropsychiatric and behavioral symptoms including anxiety, withdrawn behavior, and social interaction problems.

"Zynerba is committed to developing Zygel™ CBD gel in certain rare and near-rare conditions, including 22q, for which there is an urgent need for new, innovative therapeutics," said Armando Anido, Chairman and Chief Executive Officer of Zynerba. "We are pleased that the FDA shares our sense of urgency regarding the development of effective therapeutics in this important patient population. The receipt of this designation represents another important milestone for us, and we look forward to working closely with the FDA to develop Zygel in pediatric and adolescent patients with 22q as expeditiously as possible."

### **About Orphan Drug Designation**

Under the Orphan Drug Act (ODA), the FDA may grant orphan drug designation to drugs intended to treat rare diseases or conditions that affect fewer than 200,000 individuals in the U.S. The first NDA applicant to receive FDA approval for a particular active moiety to treat a particular disease with FDA orphan drug designation is entitled to various incentives of the ODA, including tax credits for qualified clinical testing, waiver of new drug application (NDA) / biologics license application (BLA) user fees, and eligibility for a seven-year exclusive marketing period for that drug and use upon marketing approval.

### **About 22q11.2 Deletion Syndrome (22q)**

As the second most common chromosomal disorder after Down syndrome, 22q is caused by a small missing piece of the 22nd chromosome. The deletion occurs near the middle of the chromosome at a location designated q11.2. It is considered a mid-line condition, with physical symptoms including characteristic palate abnormalities, heart defects, immune dysfunction, and esophageal / GI issues, as well as debilitating neuropsychiatric and behavioral challenges. Anxiety is among the most common neuropsychiatric symptoms of 22q and researchers have found that for children with 22q, anxiety is linked to poorer adaptive behaviors such as self-care and communication skills that affect daily life. Children with 22q also experience withdrawn behavior, ADHD, cognitive impairment, and autism spectrum disorder that affect communication and social interaction. Later in life, they are at an increased risk of developing mental illnesses such as schizophrenia. It is estimated that 22q occurs in between one in 3,000 and one in 6,000 live births, suggesting that there are approximately 81,000 people living with 22q in the U.S.

### **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](http://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 expectations, projections and estimates regarding expenses, future revenue, capital requirements, incentive and other tax credit eligibility, collectability and timing, and availability of and the need for additional financing; 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](http://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.

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