



## **Zynerba Pharmaceuticals Presents Positive Data from Phase 2 INSPIRE Trial in 22q11.2 Deletion Syndrome at the National Organization for Rare Disorders (NORD) Rare Diseases and Orphan Products Breakthrough Summit**

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DEVON, Pa., Oct. 17, 2022 (GLOBE NEWSWIRE) -- [Zynerba Pharmaceuticals](https://www.zynerba.com), Inc. (Nasdaq: ZYNE), the leader in innovative pharmaceutically-produced transdermal cannabinoid therapies for orphan neuropsychiatric disorders, presented a poster at the 2022 National Organization for Rare Disorders (NORD) Rare Diseases and Orphan Products Breakthrough Summit being held October 17-18, 2022, in Washington, D.C. A copy of the poster is available on the Zynerba corporate website at [www.zynerba.com/publications](https://www.zynerba.com/publications).

The poster, titled, "*An Open-Label Tolerability and Efficacy Study of ZYN002 (Cannabidiol) Administered as a Transdermal Gel to Children and Adolescents with 22q11.2 Deletion Syndrome (INSPIRE)*," reports data from the first 14-week treatment period that suggests a positive risk-benefit profile for Zygel™ in improving anxiety-related and behavioral symptoms in children and adolescents with 22q11.2 deletion syndrome (22q). This study enrolled 20 patients in the U.S. and Australia. Statistically significant improvements from baseline were seen in the Pediatric Anxiety Rating Scale (PARS-R), the total score and all five subscales of the Anxiety, Depression and Mood Scale (ADAMS) and all five subscales of the Aberrant Behavior Checklist – Community (ABC-C). In addition, the majority of patients showed clinically meaningful improvements as demonstrated by the Clinical Global Impression – Improvement (CGI-I). Zygel was shown to be well tolerated, and the safety profile was consistent with previously released data from other Zygel clinical trials. Three patients reported treatment related adverse events which were all mild application site adverse events. One patient discontinued treatment due to adverse events not related to Zygel.

"We are encouraged by the results from our Phase 2 INSPIRE study, where we saw statistically significant and clinically meaningful improvements from baseline in multiple efficacy assessments, including significant improvement in anxiety as measured by the clinician-rated PARS-R scale," said Armando Anido, Chairman and Chief Executive Officer of Zynerba. "We believe the Phase 2 data suggests the potential of Zygel for the treatment of anxiety and behavioral symptoms in children and adolescents with 22q, and we look forward to discussing the regulatory path forward with the FDA later this half."

### **About Zygel**

Zygel is the first and only pharmaceutically-manufactured cannabidiol formulated as a patent-protected permeation-enhanced clear gel, designed to provide controlled drug delivery into the bloodstream transdermally (i.e. through the skin). Recent studies suggest that cannabidiol may modulate the endocannabinoid system and improve certain behavioral symptoms associated with neuropsychiatric conditions. Zygel is an investigational drug product in development for the potential treatment of behavioral symptoms associated with Fragile X syndrome (FXS), 22q11.2 deletion syndrome (22q) and autism spectrum disorder (ASD). The Company has received orphan drug designation for cannabidiol, the active ingredient in Zygel, from the FDA and the European Commission in the treatment of FXS and by the FDA for the treatment of 22q. Additionally, Zygel has been designated a Fast Track development program for treatment of behavioral symptoms of FXS.

### **About Zynerba Pharmaceuticals, Inc.**

Zynerba Pharmaceuticals is the leader in innovative pharmaceutically-produced transdermal cannabinoid therapies for orphan neuropsychiatric disorders. We are committed to improving the lives of patients and their families living with severe, chronic health conditions including Fragile X syndrome, 22q11.2 deletion syndrome and autism spectrum disorder. Learn more at [www.zynerba.com](https://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, the European Medicines Agency and other 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 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; and the extent to which inflation or global instability, including political instability, may disrupt our business operations or our financial condition. 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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