

New Caregiver-Reported Data Further Validate the Use of the Aberrant Behavior Checklist Community: FXS Specific (ABC-C_{FXS}) Subscales to Assess Core Behavioral Symptoms of Fragile X Syndrome (FXS)

September 5, 2019

- Ninety Percent of Interviewed Caregivers Reported Social Avoidant, Irritability, and Socially Lethargic Behaviors in their Children with FXS -
- New Data Presented Today at the Society for the Study of Behavioural Phenotypes (SSBP) Research Symposium -

DEVON, Pa., Sept. 05, 2019 (GLOBE NEWSWIRE) -- Zynerba Pharmaceuticals, Inc. (NASDAQ:ZYNE), the leader in innovative pharmaceutically-produced transdermal cannabinoid therapies for rare and near-rare neuropsychiatric disorders, is presenting data today evaluating the Social Avoidance, Irritability, and Socially Unresponsive/Lethargic subscales of the Aberrant Behavior Checklist Community: FXS Specific (ABC-C_{FXS}) in relation to the experience of caregivers caring for a child with Fragile X syndrome. The poster describes data collected via web-based journals and in-depth interviews of caregivers of children with Fragile X syndrome (FXS). The data indicate that nine of ten caregivers reported their children had behaviors representative of social avoidance, socially unresponsiveness/lethargic, and irritability and the behaviors described had strong concordance with individual items of the ABC-C_{FXS}.

The poster, entitled *Content Validity of the ABC-C_{FXS} and Subscales in Fragile X Syndrome*, is being presented on September 5th and 6th at the 22nd Society for the Study of Behavioural Phenotypes (SSBP) Research Symposium at Aston University in Birmingham, UK. A copy of the poster is available on the Zynerba corporate website at <http://zynerba.com/publications/>.

Terri Browning Sebree, Zynerba's President, is presenting data providing qualitative evidence of the appropriateness of Social Avoidance, Irritability, and Socially Unresponsive/Lethargic subscales of the ABC-C_{FXS} in assessing core symptoms of FXS. In addition, the data further validate social avoidance, irritability, and socially unresponsive/lethargic behaviors as core FXS behavioral symptoms from the perspective of caregivers of children with the disorder. The Company is utilizing the ABC-C_{FXS} to assess improvements in core behaviors of FXS in the ongoing 14-week pivotal CONNECT-FX study of Zysel™ (CBD gel). Ms. Sebree will present the poster from 12:40 to 1:45 British Summer Time (BST) on September 5th and 6th, 2019.

"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 Ms. Sebree. "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-C_{FXS} as an effective tool for use in clinical studies as a means to measure improvements in these core and common FXS behaviors."

Ten caregivers of children formally diagnosed with FXS participated in this study with assistance from the National Fragile X Foundation (NFXF) via online invitation through the NFXF website. The study found that 90% of caregivers reported at least one behavior that was representative of social avoidance, socially unresponsive/lethargic, and irritability

Social Avoidance

It was common for their children with FXS to prefer the company of single family members to groups of people, even friends, and to seek isolation from others either via physical setting (staying in their room or in the car) or blocking out the world (using headphones). Social avoidance had a negative impact on important activities, such as travel, schooling, or visits to the doctor.

The social-avoidant behaviors reported by caregivers, including 'seeks isolation from others', 'prefers to be alone', and 'prefers solitary activities' corresponded to items on the Social Avoidance subscale, including seeks isolation from others and isolates himself/herself from other children or adults

Irritability

Irritability was associated with a broad spectrum of verbal (talking back, hollering, articulate yelling, non-verbal screams) and physical behaviors (hitting siblings, overturning furniture) at inappropriate times.

Frequently reported aspects of misbehavior and irritability mapped onto items of the Irritability subscale, specifically 'aggressive to others', 'irritability', 'temper tantrums/outbursts', 'screaming/yelling inappropriately', 'harming of others', and 'stubbornness'.

Socially Unresponsive/Lethargic

Caregivers frequently reported of 'lack of interaction' and 'lack of attention' in their children with FXS, which mapped to subscale items such as 'preoccupied', 'stares into space', 'unresponsive to structured activities (does not react)', and 'shows few social reactions to others'. Items on the subscale pertaining to communication were identified as important by caregivers, particularly with respect to how they may exacerbate other syndrome-related behaviors.

The authors conclude that these data further validate social avoidance, irritability, and socially unresponsive/lethargic as core phenotypic behaviors of children with FXS and show that caregiver experiences correspond with ABC-C_{FXS} subscales, further validating the appropriateness of the ABC-C_{FXS}

as an effective assessment tool in clinical trials.

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.

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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.

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