HARMONY BIOSCIENCES TO PRESENT RESULTS FROM BEHAVIORAL STUDY IN 22Q11.2 DELETION SYNDROME AT AMERICAN COLLEGE OF NEUROPSYCHOPHARMACOLOGY ANNUAL MEETING

December 5, 2023 1:05 PM EST

Qualitative interview study informs future clinical trial endpoints for evaluating ZYN002

PLYMOUTH MEETING, Pa., Dec. 5, 2023 /PRNewswire/ -- Harmony Biosciences Holdings, Inc. ("Harmony" or the "Company") (Nasdaq: HRMY), a pharmaceutical company dedicated to developing and commercializing innovative therapies for patients with rare neurological diseases, will present caregiver-reported impacts of 22q11.2 deletion syndrome (22q) and data from the Phase 2 INSPIRE trial at the American College of Neuropsychopharmacology Annual Meeting from December 3-6, 2023.

The INSPIRE trial evaluated ZYN002 as a potential treatment for anxiety and other irritable behavioral symptoms associated with 22q. Qualitative behavioral analyses of patients and their caregivers, both from INSPIRE and an independent panel, as well as prospective post-hoc analyses of the INSPIRE trial, informed the creation of a conceptual framework for the selection of patient-centered assessments for future clinical trials. This framework proposes endpoint measures to assess the treatment of behavioral symptoms associated with the condition, which currently lacks FDA-approved treatments and affects approximately 80,000 individuals in the U.S. and 129,000 in the EU and UK.

"Harmony is dedicated to finding effective treatments for children and their families living with 22q and other rare neuropsychiatric conditions that currently lack approved therapies," said Kumar Budur, M.D., M.S., Chief Medical Officer at Harmony Biosciences. "Through our recent acquisition of Zynerba, we are making meaningful strides toward developing new treatments for individuals with the behavioral symptoms associated with 22q and Fragile X syndrome (FXS). The development of endpoint measures for future clinical trials is an important step forward in our work for 22q patients who have high unmet medical needs."

Poster: Anxious and Irritable Behaviors in Children with 22q11.2 Deletion Syndrome: A Qualitative Interview Study and Development of a Conceptual Framework

- Poster Session II: Tuesday, December 5, 5PM – 7PM (ET)

ZYN002 is being evaluated as an investigational treatment for 22q. It is not approved for commercial distribution by government regulatory bodies, including the U.S. Food and Drug Administration (FDA).

About the INSPIRE Trial

The 38-week INSPIRE trial was an open-label, Phase 2 clinical trial designed to evaluate the safety, tolerability and effectiveness of ZYN002 in children and adolescents (ages four through 15) with genetically confirmed 22q11.2 deletion syndrome. Enrolled

patients received weight-based doses of 250 mg or 500 mg daily of ZYN002. Patients were allowed to increase the daily dose after six weeks of treatment to 500 mg and 750 mg if the investigator felt such an increase was appropriate. At the completion of the first 14-week period of treatment, patients who demonstrated an improvement in symptoms of irritability continued ZYN002 for an additional six months, for a total of 38 weeks of treatment.

About ZYN002
ZYN002 is the first-and-only pharmaceutically manufactured synthetic cannabidiol devoid of THC and formulated as a patent-protected permeation-enhanced gel for transdermal delivery through the skin and into the circulatory system. The product is manufactured through a synthetic process in a cGMP facility and is not extracted from the cannabis plant. ZYN002 does not contain THC, the compound that causes the euphoric effect of cannabis, and has the potential to be a nonscheduled product if approved. Cannabidiol, the active ingredient in ZYN002, has been granted orphan drug designation by the United States Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for the treatment of FXS and for the treatment of 22q. Additionally, ZYN002 has received FDA Fast Track designation for the treatment of behavioral symptoms in patients with FXS.

About 22q11.2 Deletion Syndrome
22q11.2 deletion syndrome (22q) is a disorder 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 symptoms, including anxiety, social withdrawal, ADHD, cognitive impairment and autism spectrum disorder. It is estimated that 22q occurs in one in 4,000 live births, suggesting that there are approximately 80,000 people living with 22q in the U.S. and 129,000 in the European Union and the UK. Patients with 22q deletion syndrome are managed by multidisciplinary care providers, and there are currently no FDA approved treatments for this disorder.

About Fragile X Syndrome
Fragile X syndrome (FXS) is a rare genetic disorder that is the leading known cause of both inherited intellectual disability and autism spectrum disorder, affecting 1 in 3,600 to 4,000 males and 1 in 4,000 to 6,000 females. The disorder negatively affects synaptic function, plasticity and neuronal connections, and results in a spectrum of intellectual disabilities and behavioral symptoms, such as social avoidance and irritability. There are approximately 80,000 people in the U.S. and approximately 121,000 people in the European Union and UK living with FXS. There is a significant unmet medical need in patients living with FXS as there are currently no FDA approved treatments for this disorder.

FXS is caused by a mutation in FMR1, a gene which modulates a number of systems, including the endocannabinoid system, and most critically, codes for a protein called FMRP. The FMR1 mutation manifests as multiple repeats of a DNA segment, known as the CGG triplet repeat, resulting in deficiency or lack of FMRP. FMRP helps regulate the production of other proteins and plays a role in the development of synapses, which are critical for relaying nerve impulses, and in regulating synaptic plasticity. In people with full mutation of the FMR1 gene, the CGG segment is repeated more than 200 times, and in most cases causes the gene to not function. Methylation of the FMR1 gene also plays a role in determining functionality of the gene. In approximately 60% of patients with FXS, who have complete methylation of the FMR1 gene, no FMRP is produced, resulting in dysregulation of the systems modulated by FMRP.

About Harmony Biosciences
At Harmony Biosciences, we specialize in developing and delivering treatments for rare neurological diseases that others often overlook. We believe that where empathy and innovation meet, a better life can begin for people living with neurological diseases. Established by Paragon Biosciences, LLC, in 2017 and headquartered in Plymouth Meeting, Pa., our team of experts from a wide variety of disciplines and experiences is driven by our shared conviction that innovative science translates into therapeutic possibilities for our patients, who are at the heart of everything we do. For more information, please visit www.harmonybiosciences.com.

Forward Looking Statement
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. These statements are based on the current beliefs and expectations of Harmony's management and are subject to significant risks and uncertainties. Actual results may differ materially from those described in the forward-looking statements. All statements contained in this press release that do not relate to matters of historical fact, including statements about our beliefs and expectations, should be considered forward-looking statements. Forward-looking statements include information concerning possible or assumed future results of operations, including descriptions of our business plans and strategies. These statements often include words such as "anticipate," "expect," "guidance," "suggest," "plan," "believe," "intend," "estimate," "target," "project," "should," "could," "would," "may," "will," "forecast," "outlook," "potential," "continues," "seeks," "predicts," or the negative of these words and other similar expressions. Factors that could cause actual results to differ materially from those described in the forward-looking statements include: risks related to the distraction of management from ongoing business operations and other opportunities due to recent acquisitions; our ability to acquire businesses, successfully secure financing for our acquisitions and timely consummate such acquisitions; the possibility that we will not successfully integrate the operations of our acquisitions, control the costs of integrating our acquisitions or realize the intended benefits of such acquisitions, including our recent Zynerba acquisition; government regulation and changes in the regulatory environment; litigation or regulatory proceedings; our ability to effectively manage our costs; our ability to retain or renew existing agreements with large or long-term customers; our ability to manage and expand our operations and keep up with rapidly changing technologies; our ability to maintain the security and integrity of our data; losses against which we do not insure; our ability to make timely payments of principal and interest on our indebtedness; our ability to satisfy covenants in the agreements governing our indebtedness; our ability to maintain our liquidity;

our reliance on key management personnel; and other one-time events and other factors that can be found in our Annual Report on Form 10-K for the year ended December 31, 2022, and any subsequent Quarterly Report on Form 10-Q or Current Report on Form 8-K, which are filed with the Securities and Exchange Commission. Many of these factors are beyond our control. The forward-looking statements contained in this earnings release speak only as of the date of this earnings release. We undertake no obligation to publicly release the result of any revisions to these forward-looking statements to reflect the impact of events or circumstances that may arise after the date of this earnings release.

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