Acadia Pharmaceuticals Initiates Pivotal Phase 3 Study of Carbetocin (ACP-101) for the Treatment of Hyperphagia in Prader-Willi Syndrome

November 30, 2023

SAN DIEGO--(BUSINESS WIRE)--Nov. 30, 2023-- Acadia Pharmaceuticals Inc. (Nasdaq: ACAD) today announced the initiation of the Phase 3 COMPASS PWS study evaluating the efficacy and safety of carbetocin nasal spray (ACP-101) for the treatment of hyperphagia in Prader-Willi syndrome (PWS). PWS is a rare, neurobehavioral genetic disorder that is estimated to affect 8,000 to 10,000 patients in the United States.[1-4] The most common symptom is hyperphagia, which is an unrelenting lack of satiety. Other defining features of PWS may include altered metabolism, developmental delays, behavioral challenges and moderate cognitive deficits.[5]

“Currently there is no FDA-approved treatment for hyperphagia in PWS, which presents serious challenges for those living with this condition and their families,” said Shawn McCandless, M.D., Chair, Department of Genetics and Metabolism, Children’s Hospital Colorado and COMPASS PWS study investigator. “Essentially all experience hyperphagia, feeling a near-constant state of hunger, as though their body is telling them that they are starving, despite being actually well-nourished. As a result, individuals with PWS often exhibit food-seeking behaviors that require constant supervision to prevent life-threatening risks, including gastric rupture, irregular swallowing and choking. I look forward to the outcome of this Phase 3 clinical trial, and the potential of having a treatment option to offer those living with PWS.”

COMPASS PWS is a 12-week, double-blind, randomized, placebo-controlled global Phase 3 trial evaluating the efficacy and safety of carbetocin nasal spray 3.2 mg three times daily (TID) in approximately 170 children and adults aged five to 30 years with PWS. The primary efficacy endpoint of the study is change from baseline to week 12 on the hyperphagia questionnaire for clinical trials (HQ-CT) score, a caregiver assessment for hyperphagia-related behaviors. Participants who complete the Phase 3 study will be eligible to enroll in a long-term, open-label extension study designed to investigate the safety and tolerability of long-term treatment with ACP-101.

“The launch of the Phase 3 COMPASS PWS trial will build on previous Phase 3 clinical trial experience, where carbetocin nasal spray 3.2 mg was observed to reduce hyperphagia-related behaviors,” said Ponni Subbiah, M.D., M.P.H., Senior Vice President, Global Head of Medical Affairs and Chief Medical Officer. “We look forward to working with the Prader-Willi community and clinical research sites as we continue to advance the ACP-101 clinical development program for those living with this debilitating syndrome and their families.”

More information about the COMPASS PWS study is available at www.CompassPWS.com.

About Prader-Willi Syndrome

Prader-Willi syndrome (PWS) is a rare neurobehavioral genetic disorder that affects both males and females.[6] PWS is estimated to affect approximately 8,000 to 10,000 patients in the United States.[3,4] PWS affects the functioning of the hypothalamus and other aspects of the brain with symptoms varying by individual.[5,6] The most common symptom is hyperphagia, which is an unrelenting lack of satiety, to which a deficiency in oxytocin is believed to be contributory.[5,7] Individuals living with PWS have fewer oxytocin-producing neurons in the brain.[7] Other defining features of the syndrome may include altered metabolism, developmental delays, behavioral challenges and moderate cognitive deficits.[3] Patients may also experience high pain tolerance, sleep disturbances, gastrointestinal issues, respiratory and temperature regulation abnormalities.[5,7-9] There is no FDA-approved treatment for the hyperphagia associated with PWS.[5]

About Carbetocin Nasal Spray (ACP-101)

Carbetocin nasal spray is an investigational drug being developed for the treatment of hyperphagia in Prader-Willi syndrome (PWS). Carbetocin has improved drug qualities relative to oxytocin, including an extended half-life and greater specificity for the oxytocin receptor compared to vasopressin receptors which could provide meaningful efficacy with an attractive safety profile in patients with PWS.[10] For the treatment of PWS specifically, a central nervous system disorder, an intranasal formulation of carbetocin was developed, which provides direct delivery of the drug to the brain, greatly reducing systemic exposure and the potential for side effects. Acadia acquired Levo Therapeutics and worldwide rights to carbetocin nasal spray in June 2022. Carbetocin nasal spray has been granted Orphan Drug, Fast Track, and Rare Pediatric Disease designations by the FDA.

About Acadia Pharmaceuticals

Acadia is advancing breakthroughs in neuroscience to elevate life. For 30 years we have been working at the forefront of healthcare to bring vital solutions to people who need them most. We developed and commercialized the first and only approved therapies for hallucinations and delusions associated with Parkinson’s disease psychosis and for the treatment of Rett syndrome. Our clinical-stage development efforts are focused on treating the negative symptoms of schizophrenia, Prader-Willi syndrome, Alzheimer’s disease psychosis and neuropsychiatric symptoms in central nervous system disorders. For more information, visit us at www.acadia.com and follow us on LinkedIn and Twitter.

Forward-Looking Statements

Statements in this press release that are not strictly historical in nature are forward-looking statements. These statements include but are not limited to statements regarding the timing of future events. These statements are only predictions based on current information and expectations and involve a number of risks and uncertainties. Actual events or results may differ materially from those projected in any of such statements due to various factors, including the risks and uncertainties inherent in drug development, approval and commercialization. For a discussion of these and other factors, please refer to Acadia’s annual report on Form 10-K for the year ended December 31, 2022, as well as Acadia’s subsequent filings with the Securities and Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date

hereof. This caution is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. All forward-looking statements are qualified in their entirety by this cautionary statement and Acadia undertakes no obligation to revise or update this press release to reflect events or circumstances after the date hereof, except as required by law.

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Media Contact:
Acadia Pharmaceuticals Inc.
Deb Kazenelson
(818) 395-3043
media@acadia-pharm.com

Investor Contact:
Acadia Pharmaceuticals Inc.
Al Kildani
(858) 261-2872
ir@acadia-pharm.com

Acadia Pharmaceuticals Inc.
Jessica Tieszen
(858) 261-2950
ir@acadia-pharm.com

Source: Acadia Pharmaceuticals Inc.