Aardvark Therapeutics Announces Receipt of FDA Rare Pediatric Disease Designation for Prader-Willi Syndrome and Expands the Ongoing Phase 2 Clinical Trial

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- The FDA granted Aardvark Therapeutics a Rare Pediatric Designation for the use of ARD-101 in Prader-Willi Syndrome (PWS), a rare genetic disease characterized by extreme and unabating hunger.
- Aardvark's Phase 2 trial of oral ARD-101 in young adults with PWS is now open for enrollment of additional subjects.

SAN DIEGO, Aug. 3, 2023 /PRNewswire/ -- Aardvark Therapeutics, Inc., a clinical stage biopharmaceutical company, today reported receipt of a Rare Pediatric Disease Designation in Prader-Willi Syndrome (PWS) from the FDA for its lead program ARD-101. This designation means Aardvark is eligible for a Rare Pediatric Disease Priority Review Voucher when ARD-101 receives approval in PWS. The early clinical study results suggest a promising future for a new class of pharmaceuticals that could benefit people with insatiable hunger (hyperphagia) and aggressive food-seeking behaviors, as well as for obesity and metabolic conditions, which are prominent features of the rare genetic condition PWS. Thus far, no drug has been approved by the FDA to treat the hyperphagia associated with PWS.

Prader-Willi Syndrome (PWS)

PWS is a severe neurodevelopmental disorder with a prevalence of about 1 in 15,000-20,000 births. The disorder is caused by the loss of function of several genes located on chromosome 15. PWS impacts multiple organ systems and is characterized by metabolic, endocrine, and neurological dysfunction. One of the hallmark characteristics of PWS is morbid obesity in early childhood, accompanied by developmental delays and musculoskeletal malformations. Obesity in PWS is driven by hyperphagia and mitigated by strict control of food intake. Strict food control, if anything, exacerbates hyperphagia, anxiety and aggressive food-seeking behavior, which is most stressful for those afflicted as well as their caregivers.

About PWS Clinical Study

Previously, Aardvark had enrolled 12 subjects in a Phase 2 study of oral ARD-101 in PWS (ClinicalTrials.gov ID NCT05153434) led by Dr. Diane Stafford at Stanford Children's Health and Dr. Shawn McCandless at Colorado Children's Hospital. Based on promising results, Aardvark is enrolling additional subjects and exploring higher drug doses.

About ARD-101

Aardvark's lead product, ARD-101, is a first-in-class oral composition that has shown promising activity in reducing hunger cravings in clinical studies and promoting weight loss in pre-clinical studies. ARD-101 is substantially gut-restricted and has minimal systemic exposure. It is postulated that ARD-101 conveys its systemic effects by activating secretion of several gut peptide hormones, including glucagon-like peptides-1 and -2(GLP-1, GLP-2), and cholecystokinin (CCK). Gut CCK is considered a "satiety signal" that acts via the gut-brain axis to control hunger. PWS patients have a normal CCK receptor but their CCK release from gut enteroendocrine I-cells in response to food is impaired, leaving PWS patients with a continuous sense of extreme hunger. Phase I studies demonstrated safety and tolerability in healthy human volunteers. Three Phase II studies have demonstrated an impact on hunger suppression.

About Aardvark Therapeutics, Inc.

Aardvark Therapeutics is a clinical stage biopharmaceutical company focused on developing novel small molecule therapeutics to activate innate homeostatic pathways for the treatment of metabolic diseases, inflammation, and other indications. Founded in 2017, the company has now advanced ARD-101 to Phase II clinical trials. Aardvark has multiple other programs in its pipeline.

For more information visit www.aardvarktherapeutics.com.

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