CAMP4 Therapeutics Announces Dosing of First Participant in Phase 1 Clinical Study of CMP-CPS-001, a Potential First-in-Class Therapeutic for Urea Cycle Disorders

March 21, 2024

Lead program in urea cycle disorders highlights power of CAMP4's RAP Platform ™ to increase gene expression using programmable, targeted medicines, with clinical candidate moving from screen-to-clinic within 3 years

CAMBRIDGE, Mass., March 21, 2024 – CAMP4 Therapeutics Corp., a clinical-stage biotechnology company harnessing the power of regulatory RNA to restore healthy gene expression, today announced that the first participant has been dosed in the company's Phase 1 clinical study of CMP-CPS-001, a potential first-in-class therapeutic for the treatment of urea cycle disorders (UCDs).

"This trial initiation is an exciting milestone, representing CAMP4's first investigational drug candidate to be studied in a clinical trial and one of the first mRNA-amplifying therapeutics in the clinic," said Josh Mandel-Brehm, CEO of CAMP4. "Most importantly, the clinical development of CMP-CPS-001 is a step toward potentially bringing a new, disease-modifying treatment to individuals living with UCDs. Going from initial screen to clinical initiation within three years provides further validation of the power and hyper-efficiency of our RAP Platform to create precisely targeted therapies for genetic diseases by amplifying mRNA to increase healthy gene expression."

UCDs are a group of rare, severe, inherited metabolic diseases impacting protein metabolism. People with urea cycle disorders accumulate excessive ammonia in their blood, which may cause irreversible brain damage, disability, and seizures, and may be fatal. These disorders occur across all age groups, from infants to adults, and mild symptoms may go unnoticed until a stressor — such as illness, protein consumption, or environmental stress — overwhelms compensatory functions, resulting in an acute metabolic crisis. No approved, disease-modifying therapeutics exist for the most prevalent forms of UCD, leaving patients, and their clinicians, with few tools other than nitrogen scavengers, strict diet, lifestyle constraints, and hyper-vigilant monitoring with supportive care during crises.

Mutations in genes encoding urea cycle enzymes result in insufficient levels of these important proteins. CMP-CPS-001 targets carbamoyl phosphate synthetase 1 (CPS1), a key enzyme that catalyzes the first step of the urea cycle. CMP-CPS-001 is designed to amplify CPS1 mRNA to potentially improve or restore urea cycle activity.

Phase 1 Study of CMP-CPS-001

The Phase 1 study is a randomized, double-blind, and placebo-controlled study designed to evaluate the safety, tolerability, and pharmacokinetics of CMP-CPS-001 in healthy volunteers. The study is currently active in Australia and anticipates enrolling a total of 96 participants across single- and multiple-ascending dose cohorts. For more information about the Phase 1 clinical study of CMP-CPS-001, please visit clinicaltrials.gov (NCT06247670).

About CMP-CPS-001

CMP-CPS-001 is an antisense oligonucleotide (ASO) designed to amplify CPS1 mRNA by harnessing fundamental cellular gene expression control mechanisms. CAMP4's proprietary RAP Platform enabled the discovery of CMP-CPS-001, a potential new therapeutic to treat urea cycle disorders.

About CAMP4 Therapeutics

CAMP4 is developing disease-modifying treatments for a broad range of genetic diseases where amplifying healthy protein may offer therapeutic benefits. Our approach amplifies mRNA by harnessing a fundamental mechanism of how genes are controlled. To amplify mRNA, our therapeutic ASO drug candidates target regulatory RNAs (regRNAs), which act locally on transcription factors and are the master regulators of gene expression. CAMP4's proprietary RAP Platform enables the mapping of regRNAs and design of optimal chemistry to generate potent therapeutic candidates to address hundreds of genetic diseases across multiple tissues. Learn more about us at www.CAMP4tx.com and follow us @CAMP4tx.

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