CAMP4 Therapeutics Secures Orphan Drug Designation for CMP-CPS-001 for the Treatment of Urea Cycle Disorders

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CAMBRIDGE, Mass., September 17, 2024 — CAMP4 Therapeutics, a clinical-stage biotechnology company developing a pipeline of regulatory RNA-targeting therapeutics designed to upregulate gene expression with the goal of restoring healthy protein levels across a range of genetic diseases, today announced the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to CMP-CPS-001 for the treatment of urea cycle disorders (UCDs).

"Today, there are no disease-modifying therapies available for patients with UCDs, and this community is in desperate need of innovative approaches to help mitigate potentially devastating health outcomes, including neurologic disability, seizures, and death," said Josh Mandel-Brehm, Chief Executive Officer of CAMP4. "Based on our preclinical studies, we believe CMP-CPS-001 has the potential to shift the paradigm in how patients with UCDs are treated. Our preclinical studies have demonstrated that by modulating the activity of the target regRNA, CMP-CPS-001 increases the levels of a key enzyme in the urea cycle, which allows for more ammonia to be converted into urea, thereby lowering ammonia levels to normal, healthy ranges."

About Orphan Drug Designation

Orphan Drug Designation is granted by the FDA and is designed to support the development of new therapeutics for rare diseases or conditions that affect fewer than 200,000 people in the United States. Orphan Drug Designation qualifies companies for certain incentives, which may include tax credits for qualified clinical trials, exemption from FDA user fees, and potential eligibility for seven years of market exclusivity in the United States upon FDA approval. Drugs for rare diseases are held to the same rigorous scientific review processes as any other drug for approval or licensing.

About CMP-CPS-001

CMP-CPS-001 is an antisense oligonucleotide (ASO) therapeutic candidate for the treatment of UCDs targeting carbamoyl phosphate synthetase 1 (CPS1), an enzyme that catalyzes the first step of the urea cycle, which converts ammonia to urea. CMP-CPS-001 is designed to upregulate CPS1 gene expression by binding to a CPS1-specific regulatory RNA sequence to ultimately increase CPS1 protein levels. CMP-CPS-001 is currently under evaluation in a Phase 1 clinical trial in healthy volunteers (NCT06247670). CMP-CPS-001 has been granted Rare Pediatric Disease Designation by the FDA.

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 generation of therapeutic candidates designed target the regRNAs of haploinsufficient and recessive partial loss-of-function disorders, of which there are more than 1,200, in which a modest increase in protein expression may have the potential to be clinically meaningful. Learn more about us at **www.CAMP4tx.com** and follow us on **LinkedIn** and **X**.

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