CAMP4 Therapeutics Secures Rare Pediatric Disease Designation for CMP-CPS-001 for the Treatment of Urea Cycle Disorders

August 27, 2024

CAMBRIDGE, Mass., Aug. 27, 2024 (GLOBE NEWSWIRE) -- CAMP4 Therapeutics, a clinical-stage biotechnology company developing a pipeline of RNA-based 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 Rare Pediatric Disease Designation (RPDD) to the Company's lead investigational drug candidate CMP-CPS-001 for the treatment of urea cycle disorders (UCDs). The safety, tolerability, and pharmacokinetics of CMP-CPS-001 are currently being evaluated in a randomized, placebo-controlled Phase 1 trial in Australia.

"This designation from the FDA underscores the seriousness of this rare disease and the critical need for new therapeutic options for patients living with UCDs who currently rely on supportive treatments that fail to address the underlying driver of the disease," said Josh Mandel-Brehm, Chief Executive Officer of CAMP4. "By upregulating the expression of the gene encoding a key enzyme in the urea pathway to restore healthy protein levels, CMP-CPS-001 has the potential to be the first disease-modifying treatment for UCDs. With our Phase 1 clinical study underway, we look forward to evaluating the potential of this candidate for patients living with serious disorders of urea metabolism."

UCDs are a group of rare, severe, inherited metabolic diseases impacting protein metabolism that affect patients of all ages. People with UCDs accumulate excessive ammonia in their blood, which may cause irreversible brain damage, disability, and seizures, and can be fatal. There are currently no approved disease-modifying therapeutics for the most prevalent forms of UCDs.

About Rare Pediatric Disease Designation
The FDA's Rare Pediatric Disease Designation program incentivizes drug development for rare pediatric conditions. A rare pediatric disease is defined as a serious or life-threatening disease in which the manifestations primarily impact individuals from birth to 18 years of age. A sponsor who receives FDA approval for a therapeutic for a rare pediatric disease may qualify for a voucher that can be used to obtain priority review for a subsequent marketing application for a different product.

About CMP-CPS-001
CMP-CPS-001 is an antisense oligonucleotide (ASO) therapeutic candidate for the treatment of urea cycle disorders targeting carbamoyl phosphate synthetase 1 (CPS1), the first enzyme in the metabolic cycle that 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 study (NCT06247670).

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 more than 1,200 genetic diseases across multiple tissues. Learn more about us at **www.CAMP4tx.com** and follow us on **LinkedIn** and **X**.

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