CAMP4 Enters Strategic Research Collaboration to Advance Novel Regulatory RNA-Targeting Medicines for Rare Genetic Conditions

October 1, 2024

– Collaboration combines BioMarin's expertise in drug development with CAMP4's platform-based target discovery capabilities
– CAMP4 to receive upfront payment, with eligibility for additional milestones and tiered royalties

CAMBRIDGE, Mass., October 1, 2024 — CAMP4 Therapeutics Corporation, 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 a new research collaboration with BioMarin Pharmaceutical Inc. aimed at advancing novel therapeutics that increase protein levels by targeting regulatory RNA (regRNA) sequences, which are key elements controlling gene expression.

Josh Mandel-Brehm, Chief Executive Officer of CAMP4, said, "BioMarin is a leader in genetic medicines with a successful track record of advancing disease-modifying therapies for patients with rare genetic conditions. We believe this collaboration will expand the reach of our approach and enable us to leverage our RAP Platform to identify and target additional regRNAs associated with disease. We look forward to partnering with the BioMarin team with the aim of bringing forward novel therapeutic targets."

CAMP4's RAP Platform™ is designed to rapidly and systematically identify and characterize the active RNA regulatory elements controlling every expressed gene and tens of thousands of druggable enhancer and promoter regRNA sequences that control protein-coding genes. Once a disease-associated target gene is identified, CAMP4 applies its RAP Platform to identify the controlling regRNA and rapidly generate novel antisense oligonucleotide candidates, called RNA Actuators™, designed to bind to the identified regRNA and amplify the expression of the target gene in a specific and controllable way, with the goal of restoring healthy protein levels.

"We are excited to work with CAMP4 and their RAP Platform to further understand how regulatory RNAs could unlock novel ways to address diseases characterized by suboptimal protein expression," said Stuart Bunting, Group Vice President, Head of Research at BioMarin.

Under the terms of the agreement, BioMarin has the right to select two targets identified by CAMP4's RAP Platform to advance into clinical development.

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 to 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**.

Contacts

Media:
Jason Braco, Ph.D.
LifeSci Communications
jbraco@lifescicomms.com
(646) 876-4932