



CAMP4 Presents First Preclinical Data Demonstrating the Therapeutic Potential of its RNA Actuating Platform at the 17th Annual Meeting of the Oligonucleotide Therapeutics Society

September 27, 2021

- *Novel approach to upregulating gene expression by targeting regulatory RNAs*
- *Company to advance development candidate for urea cycle disorders; expected to enter clinical trials in 2023*

CAMBRIDGE, Mass., September 27, 2021 – CAMP4 Therapeutics, a biotechnology company harnessing the power of RNA to restore healthy gene expression, today announced the presentation of compelling preclinical data demonstrating the utility of its RNA Actuating Platform (RAP) and the therapeutic potential of oligonucleotide-mediated gene upregulation targeting a new class of RNA known as regulatory RNAs ("regRNAs"). The data, which describe this novel treatment approach for two genetically defined diseases, were presented at the 17th Annual Meeting of the Oligonucleotide Therapeutics Society (OTS) held virtually from September 26-29, 2021.

"These data demonstrate that by using oligonucleotides to target regRNA, we can directly upregulate endogenous gene expression at the transcriptional level and achieve a therapeutically relevant result," said David Bumcrot, Ph.D., Chief Scientific Officer of CAMP4. "With the results of these studies, we have the early proof points that our novel approach to harness the power of regRNA for gene upregulation works across multiple genes, fueling our conviction as we advance the development of precise, potent, programmable and durable therapeutics for genetic diseases that lack disease-modifying therapies."

RegRNAs act as gene-specific rheostats that can finely modulate gene expression upstream of mRNA transcription, but not to excessive levels that might generate toxicity. CAMP4 leverages its extensive set of proprietary genomic data to efficiently and systematically identify gene-specific regRNA targets and generate precise oligonucleotide drug candidates, or RNA Actuators™, to drug them. This approach is applicable to any genetic disease whereby a small increase in gene output can lead to meaningful therapeutic outcomes.

The data at OTS show that CAMP4's RNA Actuators resulted in dose-dependent upregulation of the OTC gene, one of several genes that can play a role in a set of debilitating and life-threatening liver diseases called urea cycle disorders (UCD). UCD results in excessive build-up of ammonia in the body, leading to cognitive delay, coma and seizures. CAMP4 used its platform to identify the novel regRNAs that control OTC gene expression in primary human and mouse hepatocytes and subsequently designed and screened RNA Actuators to modulate the target. The data demonstrate that RNA Actuators can lead to dose-dependent increases in OTC mRNA in hepatocytes from multiple donors, reaching up to a 5-fold increase. When tested in human OTC patient cells, the RNA Actuators similarly increased OTC mRNA and led to a corresponding ~2-fold increase in ureagenesis. Analysis of individual OTC-deficient patient data show that increasing OTC enzyme activity to at least 5-10% of normal can have a tangible therapeutic benefit. Based on these findings, CAMP4 will pursue clinical development for UCD with the expectation of entering the clinic in 2023.

In a second presentation, CAMP4 described similar applications of its platform and results in upregulating the Serping1 gene for the treatment of hereditary angioedema (HAE), a life-threatening genetic condition involving painful swelling across different body parts. In primary mouse hepatocytes, RNA Actuators increased Serping1 expression by 2-to-3-fold with minimal impact on neighboring genes. The RNA Actuators also normalized the levels of Serping1 in a HAE mimic in vitro model and led to an increase in Serping1 protein in mice.

The presentations can be viewed on the [Publications](#) section of the CAMP4 website.

About CAMP4 Therapeutics

At CAMP4, we are pioneering a novel approach to programmable therapeutics. We combine a deep understanding of regulatory RNA and gene expression with a complementary and customizable oligonucleotide modality. Our RNA Actuating Platform's proprietary insights enable us to harness the power of RNA to upregulate the expression of genes and unlock the potential to create treatments for hundreds of diseases affecting millions of patients. Learn more about us at www.CAMP4tx.com and follow us @CAMP4tx.

Contact:

Lindy Devereux

Scient PR

lindy@scientpr.com

646-515-5730