CAMP4 Therapeutics Initiates GLP Toxicology Studies for CMP-SYNGAP-01

October 1, 2025

CAMBRIDGE, Mass., Oct. 01, 2025 (GLOBE NEWSWIRE) -- **CAMP4 Therapeutics Corporation** (“CAMP4” or “the Company”) (Nasdaq: CAMP), a clinical-stage biopharmaceutical company developing a pipeline of regulatory RNA-targeting therapeutics designed to upregulate gene expression with the goal of restoring healthy protein levels to treat a broad range of genetic diseases, today announced the initiation of toxicology studies conducted under Good Laboratory Practice (GLP) standards for its lead product candidate, CMP-SYNGAP-01. These studies will support the Company’s planned submission of a clinical trial application, which could enable the initiation of a first-in-human Phase 1/2 clinical trial in SYNGAP1-related disorders as early as the second half of 2026.

“CMP-SYNGAP-01 has demonstrated robust preclinical activity, both restoring SYNGAP1 protein levels to improve phenotypes in a mouse model and increasing SYNGAP1 protein in brain regions of non-human primates that are affected in human disease,” said Daniel Tardiff, Ph.D., Chief Scientific Officer of CAMP4. “Our preclinical data gives us confidence in the potential of CMP-SYNGAP-01 to translate into meaningful clinical benefit for patients living with SYNGAP1-related disorders, and advancing our candidate through GLP toxicology studies marks a critical step towards the clinic.”

SYNGAP1-related disorders are a group of neurodevelopmental conditions caused by pathogenic variants in the SYNGAP1 gene that result in a haploinsufficient state, reducing SYNGAP protein levels by up to 50%. SYNGAP plays a critical role in cognitive development and synaptic function. SYNGAP-related disorders are reported to represent 0.5% to 1.0% of all intellectual disability cases, making them among the most common causes of intellectual disability in patients with epilepsy, and indicating that the patient population may be significantly larger than incidence estimates suggest.

Today, there are no FDA-approved, disease-modifying therapies for SYNGAP1-related disorders, with treatment often limited to supportive physical, occupational, and speech therapy. A combination of non-specific anti-seizure drugs and other medications may be prescribed, though SYNGAP1-related disorders have proven difficult to control with available therapeutics.

About CMP-SYNGAP-01
CMP-SYNGAP-01 is an investigational, novel approach that targets the SYNGAP1 gene at the transcriptional level to restore SYNGAP function and improve symptoms by utilizing an intrathecally delivered antisense oligonucleotide. Upregulation of SYNGAP1 gene expression may increase SYNGAP protein levels in amounts sufficient to yield therapeutic benefit. Preclinical studies have demonstrated a dose-dependent increase in SYNGAP mRNA levels accompanied by an increase in SYNGAP protein expression.

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 associated with genes underlying 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.

Forward-Looking Statements
This press release contains forward-looking statements which involve risks, uncertainties and contingencies, many of which are beyond the control of the Company, which may cause actual results, performance, or achievements to differ materially from anticipated results, performance, or achievements. All statements other than statements of historical facts contained in this press release are forward-looking statements. In some cases, you can identify forward-looking statements by terms such as “may,” “will,” “should,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “contemplate,” “believe,” “estimate,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions, although not all forward-looking statements contain these words. Forward-looking statements include, but are not limited to, statements concerning the potential for GLP toxicology studies to generate regulatory-compliant data to support a clinical trial in SYNGAP1-related disorders; expectations regarding the timing to advance the Company’s SYNGAP1 program into a Phase 1/2 clinical trial; and the therapeutic potential of CMP-SYNGAP-01; and the incidence and prevalence of SYNGAP1-related disorders. The forward-looking statements in this press release speak only as of the date of this press release and are subject to a number of known and unknown risks, uncertainties and assumptions that could cause the Company’s actual results to differ materially from those anticipated in the forward-looking statements, including, but not limited to: the Company’s limited operating history, incurrence of substantial losses since the Company’s inception and anticipation of incurring substantial and increasing losses for the foreseeable future; the Company’s need for substantial additional financing to achieve the Company’s goals; the uncertainty of clinical development, which is lengthy and expensive, and

characterized by uncertain outcomes, and risks related to additional costs or delays in completing, or failing to complete, the development and commercialization of the Company's current product candidates or any future product candidates; delays or difficulties in the enrollment and dosing of patients in clinical trials; the impact of any significant adverse events or undesirable side effects caused by the Company's product candidates; potential competition, including from large and specialty pharmaceutical and biotechnology companies; the Company's ability to realize the benefits of the Company's current or future collaborations or licensing arrangements and ability to successfully consummate future partnerships; the Company's ability to obtain regulatory approval to commercialize any product candidate in the United States or any other jurisdiction, and the risk that any such approval may be for a more narrow indication than the Company seeks; the Company's dependence on the services of the Company's senior management and other clinical and scientific personnel, and the Company's ability to retain these individuals or recruit additional management or clinical and scientific personnel; the Company's ability to grow the Company's organization, and manage the Company's growth and expansion of the Company's operations; risks related to the manufacturing of the Company's product candidates, which is complex, and the risk that the Company's third-party manufacturers may encounter difficulties in production; the Company's ability to obtain and maintain sufficient intellectual property protection for the Company's product candidates or any future product candidates the Company may develop; the Company's reliance on third parties to conduct the Company's preclinical studies and clinical trials; the Company's compliance with the Company's obligations under the licenses granted to the Company by others, for the rights to develop and commercialize the Company's product candidates; risks related to the operations of the Company's suppliers; and other risks and uncertainties described in the section "Risk Factors" in the Company's Annual Report on Form 10-K for the year ended December 31, 2024 and Quarterly Report on Form 10-Q for the quarter ended June 30, 2025, as well as other information the Company files with the Securities and Exchange Commission. The forward-looking statements in this press release are inherently uncertain and are not guarantees of future events. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond the Company's control, you should not unduly rely on these forward-looking statements. The events and circumstances reflected in the forward-looking statements may not be achieved or occur and actual future results, levels of activity, performance and events and circumstances could differ materially from those projected in the forward-looking statements. Moreover, the Company operates in an evolving environment. New risks and uncertainties may emerge from time to time, and management cannot predict all risks and uncertainties. Investors, potential investors, and others should give careful consideration to these risks and uncertainties. Except as required by applicable law, the Company does not undertake to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

Contacts

Investor Relations:
Sara Michelmore
Milestone Advisors
sara@milestone-advisorsllc.com

Media:
Jason Braco, Ph.D.
LifeSci Communications
jbraco@lifescicomms.com