CAMP4 Provides Corporate Updates and Highlights Key Upcoming Milestones

January 7, 2025

- Data from Single Ascending Dose (SAD) portion of Phase 1 study of CMP-CPS-001 for the treatment of urea cycle disorders (UCDs) demonstrates favorable safety results
- Completed dosing in the first two Multiple Ascending Dose (MAD) cohorts, and initiated dosing in Cohort 3
- MAD safety and key study biomarker data expected in 2H 2025
- CAMP4 added to Russell 2000® Index
- Initiation of new discovery program targeting a GBA1 regRNA for the treatment of Parkinson's disease (PD)
- Company to present at the 43rd Annual J.P. Morgan Healthcare Conference on Wednesday, January 15 at 3:45 p.m. PST

CAMBRIDGE, Mass., Jan. 07, 2025 (GLOBE NEWSWIRE) -- CAMP4 Therapeutics Corporation ("CAMP4") (Nasdaq: CAMP), a clinical-stage biotechnology company developing regRNA-targeting antisense oligonucleotide (ASO) therapies to upregulate gene expression to restore healthy protein levels, today provided corporate updates and key objectives for 2025.

"2024 was a transformative year for CAMP4, highlighted by our successful IPO, and we are entering 2025 with tremendous momentum and a clear path towards delivering on our goals," said Josh Mandel-Brehm, Chief Executive Officer of CAMP4. "We are very pleased with the clinical progress of our lead program CMP-CPS-001 for urea cycle disorders, which was granted Rare Pediatric Disease Designation and Orphan Drug Designation, validating the urgency of our novel regRNA-targeting ASOs. Additionally, our strategic partnership with BioMarin highlights confidence in our RAP platform. These achievements, combined with the proceeds from the IPO, position us to continue to progress our pipeline development and execute on key milestones this year."

"We are pleased to report the safety data from the SAD portion of our Phase 1 study of CMP-CPS-001 for UCDs, showing that the drug has been well-tolerated," said Dr. Yuri Maricich, Chief Medical Officer of CAMP4. "We expect 2025 to be a pivotal year as we anticipate reporting MAD safety and key biomarker data in the second half of 2025. Results from these studies could enable us to advance the CMP-CPS-001 program into a registrational Phase 2/3 trial in 2026."

2024 Key Highlights

- Completed IPO of 6,820,000 shares of common stock at an initial public offering price of $11.00 per share. Aggregate gross proceeds to CAMP4 were approximately $75.0 million. The underwriters also partially exercised their option to purchase an additional 643,762 shares of common stock for total offering gross proceeds of $82.1 million.
- Advanced Phase 1 clinical trial of CMP-CPS-001 for UCDs. The Phase 1 study is a randomized, double-blind, and placebo-controlled study designed to evaluate the safety, tolerability, and pharmacokinetics of CMP-CPS-001 in 96 healthy volunteers.
- Completed planned interim analysis of all four SAD cohorts of the Phase 1 clinical trial of CMP-CPS-001 in 48 healthy volunteer participants. Safety results were favorable and consistent with the safety profile of approved liver-targeted ASOs, with all treatment emergent adverse events (TEAEs) being Grade 1 (mild) or Grade 2 (moderate). The two most common TEAEs across all cohorts were headache (six participants) and nausea (four participants). No safety trends of concern have been observed, and CMP-CPS-001 appears to be well-tolerated. Dosing is completed in the first two MAD cohorts, and the Safety Review Committee (SRC) has approved dose escalation to MAD Cohort 3, in which dosing has been initiated.
- The FDA granted Rare Pediatric Disease Designation and Orphan Drug Designation to CMP-CPS-001 for the treatment of UCDs.
- Entered strategic research collaboration agreement with BioMarin Pharmaceutical Inc., under which BioMarin has the right to select two targets identified by CAMP4's RAP Platform to advance into clinical development.
- Company added to the Russell 2000® Index as part of the fourth quarter IPO additions.
- Appointed John Maraganore, Ph.D., and Rachel Meyers, Ph.D., as strategic advisors.

Expected Milestones in 2025

- MAD safety, pharmacokinetic and key pharmacodynamic biomarker data in healthy volunteers in the second half of 2025.
- GLP toxicity studies of lead ASO candidate for neurodevelopmental disorders caused by SYNGAP1 mutations to be initiated this year.
- Advance a new discovery program targeting a GBA1 regRNA to increase gene expression for the treatment of Parkinson's disease (PD) caused by mutations in GBA1, with potential for application in sporadic PD.
- Company to focus on expanding its strategic partnerships to continue maximizing the value of its RAP platform.

About CAMP4 Therapeutics

CAMP4 is developing disease-modifying treatments for a broad range of rare and prevalent genetic diseases where increasing healthy protein levels may offer meaningful therapeutic benefits. Our approach allows for targeted gene upregulation by harnessing a fundamental mechanism of how genes are controlled. To increase gene expression, our therapeutic ASO drug candidates target 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. Learn more about us at www.CAMP4tx.com and follow us on LinkedIn and X.

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 CAMP4's plans, objectives, expectations and intentions; the timing and results of ongoing and future clinical trials, including expectations on the timing of reporting MAD data from the CMP-CPS-001 trial and advancing the CMP-CPS-001 program into a registration-enabling trial; the timing to initiate GLP toxicity studies relating to CAMP4's SYNGAP1 program; the timing to advance new discovery programs; its growth strategy; and cash runway guidance. 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, as well as other information we file 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:
Sandya von der Weid
LifeSci Advisors **svonderweid@lifesciadvisors.com**

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