CAMP4 Reports Full Year 2024 Financial Results and Provides Corporate Update

March 27, 2025

– Phase 1 clinical trial of CMP-CPS-001 in Urea Cycle Disorders (UCDs) ongoing, with dosing completed in two of four multiple ascending dose (MAD) cohorts; safety, pharmacokinetic, and pharmacodynamic data anticipated in Q4 2025

– Initiation of expansion into Phase 1b clinical trial in female OTC heterozygotes expected in Q2 2025

– Nomination of development candidate CMP-SYNGAP-01 to address SYNGAP1-related disorders; GLP toxicology studies expected to be initiated in 2025

CAMBRIDGE, Mass., March 27, 2025 (GLOBE NEWSWIRE) -- CAMP4 Therapeutics Corporation ("CAMP4") (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 financial results for the full year ended December 31, 2024, and provided a corporate update.

"We are off to a strong start in 2025, building on the momentum of a successful 2024, which included advancing our Phase 1 clinical program in UCDs, establishing key research collaborations, securing important regulatory designations, and completing our initial public offering," said Josh Mandel-Brehm, Chief Executive Officer of CAMP4. "We remain focused on the ongoing healthy volunteer Phase 1 clinical trial of CMP-CPS-001 in UCDs, as well as our planned expansion into a Phase 1b clinical trial in Australia in female OTC heterozygotes - a potential additional addressable patient population of UCDs that has previously been underserved. In addition, we intend to submit a Clinical Trial Application (CTA) in Europe and to open an additional clinical trial site, pending regulatory clearance. This expansion reflects both the growing body of evidence supporting our approach and our commitment to addressing critical gaps in genetic disease care."

Mr. Mandel-Brehm continued, "We are also pleased to announce the selection of a development candidate, CMP-SYNGAP-01, for our SYNGAP1-related disorders program based on compelling data across our preclinical studies, including recent non-human primate studies. The discovery and selection of a novel regRNA-targeting antisense oligonucleotide (ASO) that increases SYNGAP1 protein levels underscores the transformative potential of our platform to identify and advance this novel class of therapeutic candidates for SYNGAP1-related disorders, a group of neurodevelopmental conditions with no approved therapies that we believe impacts approximately 10,000 individuals in the U.S."

Ornithine transcarbamylase (OTC) deficiency is a rare genetic disorder that leads to the buildup of ammonia in the blood, which can cause brain damage, coma, or death if left untreated. It is estimated to affect approximately 1 in 56,500 births in the United States and is the only genetic subtype of Urea Cycle Disorders that is X-linked. While males with OTC deficiency almost always experience symptoms, the severity and presence of symptoms in female heterozygotes can vary. CAMP4 estimates that over 1,200 heterozygous female carriers of a mutation in the OTC gene in the United States experience potentially treatable UCD symptoms.

"OTC deficiency has long been seen as a disorder that primarily affects males, with females assumed to be less affected by the disease, and hence this important potential patient population has been overlooked and underserved. Recent research shows that many of these women face chronic, underrecognized symptoms and serious health risks, including the danger of hyperammonemic crises triggered by stress, illness, pregnancy or surgery," said Dr. Yuri Maricich, Chief Medical Officer of CAMP4. "CAMP4's Phase1b clinical trial represents a crucial step forward in redefining care for female OTC heterozygotes - moving from passive observation to proactive monitoring and treatment. We are excited to expand our study of CMP-CPS-001, which appeared to be well-tolerated in a Single-Ascending Dose (SAD) study in normal healthy volunteers, as a potential therapy to help manage ammonia levels and improve patient outcomes."

Recent Corporate Highlights:

- 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 were observed, and CMP-CPS-001 appeared to be well-tolerated.
- Completed dosing in the first two MAD cohorts and dosing has been initiated in MAD Cohort 3.
- Nominated a development candidate, CMP-SYNGAP-01, for the treatment of SYNGAP-1 related disorders based on preclinical data, including recent results from non-human primate studies.

- Initiated a discovery program for the treatment of GBA1-related Parkinson's Disease. Using CAMP4's RAP Platform™, ASOs have been identified that enhance the expression of GBA1 *in vitro*. Preclinical studies are ongoing to enable the company to select a development candidate.
- Appointed Doug E. Williams, Ph.D., and Murray Stewart, DM FRCP, to the Board of Directors, bringing decades of experience in pharmaceutical development and extensive expertise in genetic medicine.

Expected Milestones in 2025

- Initiation of expansion into Phase 1b clinical trial of CMP-CPS-001 in female OTC heterozygotes in Australia expected in Q2 2025, with Europe to follow pending CTA clearance.
- Data from the SAD and MAD portions of the trial evaluating CMP-CPS-001 in 96 healthy volunteer participants, including safety, pharmacokinetic, and pharmacodynamic biomarker data, are expected in Q4 2025.
- GLP toxicity studies of CMP-SYNGAP-01 for the treatment of 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 continues to focus on expanding its strategic partnerships to continue maximizing the value of its RAP Platform.

Full Year 2024 Financial Results

R&D Expenses: Research and development expenses for the year ended December 31, 2024 were $38.8 million, compared to $40.6 million for the year ended December 31, 2023. The decrease was primarily due to decreases in clinical and preclinical expenses and personnel-related expenses.

G&A Expenses: General and administrative expenses for the year ended December 31, 2024, were $14.9 million, as compared to $11.6 million for the year ended December 31, 2023. The increase was primarily due to an increase in personnel-related expenses.

Net Loss: The net loss was $51.8 million for the year ended December 31, 2024, compared to $49.3 million for the year ended December 31, 2023.

Cash Position: As of December 31, 2024, cash and cash equivalents totaled approximately $64.0 million. The Company believes that its current cash and cash equivalents will be sufficient to fund its planned activities into Q2 2026.

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. For more information, visit camp4tx.com.

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 and expectations regarding its ongoing Phase 1 clinical trial of CMP-CPS-001 and its intention to initiate the expansion into a Phase 1b clinical trial of CMP-CPS-001; the anticipated timing and results of the company's ongoing and future clinical trials, including expectations regarding the timing of reporting data from the CMP-CPS-001 clinical trials; the expected timing for the company's initiation of GLP toxicity studies relating to CAMP4's SYNGAP1 program; the therapeutic potential of CAMP4's product candidates; estimates regarding the size of patient populations; 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 Annual Report on Form 10-K for the year ended December 31, 2024, 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.

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CAMP4 Therapeutics Corporation
Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except for share and per share data)

	Year ended December 31,	
	2024	**2023**
Revenue		
Research and collaboration revenue	$ 652	$ 350
Operating expenses		
Research and development	38,817	40,616
General and administrative	14,923	11,613
Total operating expenses	53,740	52,229
Loss from operations	(53,088)	(51,879)
Other income, net:		
Interest income	1,330	2,808
Other expense	(33)	(220)
Total other income, net	1,297	2,588
Net loss attributable to common stockholders and comprehensive loss	$ (51,791)	$ (49,291)
Net loss per share attributable to common stockholders, basic and diluted	$ (11.04)	$ (124.80)
Weighted average shares of common stock outstanding, basic and diluted	4,690,094	394,976

Unaudited Condensed Balance Sheet Data:	**December 31,**	**December 31,**
(in thousands)	**2024**	**2023**
Cash and cash equivalents	$ 64,039	$ 38,380
Working capital(1)	56,785	32,206
Total assets	78,307	54,946
Total liabilities	15,163	16,529
Convertible preferred stock	-	162,147
Accumulated deficit	(211,753)	(159,962)
Total stockholders' equity (deficit)	63,144	(123,730)

(1) Working capital is defined as total current assets less total current liabilities. See our consolidated financial statements and the related notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2024, for further details regarding our current assets and current liabilities.