CAMP4 Reports Second Quarter 2025 Financial Results and Corporate Highlights

August 14, 2025

- *Presented positive translational data from SYNGAP1-related disorders program showcasing efficacy in a humanized SYNGAP mouse model and increased protein in non-human primates at the 28th American Society of Gene and Cell Therapy (ASGCT) Annual Meeting*

- *Initiating GLP toxicology studies evaluating CMP-SYNGAP-01 in Q3 2025*

- *Dosing completed in multiple ascending dose (MAD) cohort 3 of CMP-CPS-001 and data from single ascending dose (SAD) & MAD cohorts expected in Q4 2025*

CAMBRIDGE, Mass., Aug. 14, 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 second quarter ended June 30, 2025, and provided a corporate update.

"This past quarter, we presented compelling translational data from our SYNGAP1 program, reinforcing our confidence in CMP-SYGNAP-01's potential to transform the lives of patients living with this devastating neurological disorder, which currently has no approved treatments addressing the root cause," said Josh Mandel-Brehm, President and Chief Executive Officer of CAMP4. "With strong translational results and a clear unmet need, we remain committed to advancing CMP-SYNGAP-01 with urgency and are on track to initiate GLP toxicology studies in the third quarter, which could support initiation of a global Phase 1/2 clinical trial in SYNGAP patients in the second half of 2026."

Mr. Mandel-Brehm continued, "We continue to see strong potential in CMP-CPS-001 as a first-in-class, disease-modifying therapy for the most common UCDs and plan to announce safety and biomarker data from the SAD and MAD portions of the ongoing Phase 1 trial in healthy volunteers in Q4. These data could position CMP-CPS-001 as a valuable asset with an established safety profile that is ready for evaluation in symptomatic individuals, making it an ideal candidate for potential partnerships or further in-house development."

Corporate Highlights:

- Presented new translational data from SYNGAP1-related disorders program and highlighted interim safety results from the ongoing Phase 1 UCD trial at the 28th American Society of Gene and Cell Therapy Annual Meeting.
  - In a humanized disease-relevant mouse model, a single intracerebroventricular dose of CMP-SYNGAP-01 restored SYNGAP1 protein to near-normal levels, and two doses rescued motor and spatial learning deficits, reflective of the disease state in patients.
  - In non-human primate studies using the clinical route of administration, intrathecal biweekly administration of CMP-SYNGAP-01 led to a significant increase in SYNGAP1 protein at levels believed to be therapeutically relevant across multiple disease-relevant brain regions.
  - Presented positive safety and pharmacokinetics data from the ongoing Phase 1 trial of CMP-CPS-001.
- Expect to initiate GLP toxicology studies evaluating CMP-SYNGAP-01 in Q3 2025; potential to initiate a global Phase 1/2 trial as early as H2 2026.
- Completed dosing in the third MAD cohort in the ongoing Phase 1 clinical trial of CMP-CPS-001; expect to report data from all four cohorts of the SAD portion and the first three cohorts of the MAD portion of the trial, including safety, pharmacokinetic and pharmacodynamic biomarker results, in Q4 2025.

Second Quarter 2025 Financial Results
Cash, cash equivalents, and marketable securities as of June 30, 2025, were $39.1 million, compared to $49.3 million as of March 31, 2025.

R&D Expenses: Research and development expenses for the quarter ended June 30, 2025, were $10.3 million, compared to $9.4 million for the quarter ended June 30, 2024. The expenses were primarily driven by an increase in clinical and preclinical study costs.

G&A Expenses: General and administrative expenses were $4.2 million for the quarter ended June 30, 2025, compared to $3.3 million for the quarter ended June 30, 2024. The expenses were primarily driven by an increase in personnel-related and overhead costs.

Net Loss: Net loss for the quarter ended June 30, 2025, was $12.6 million compared to $12.6 million for the quarter ended June 30, 2024.

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 the Company's clinical development plans and expectations regarding CMP-CPS-001; the anticipated timing and results of the Company's ongoing and future clinical trials, including expectations regarding the timing to advance the Company's SYNGAP1 program into a clinical trial and to report data from the CMP-CPS-001 clinical trial; the expected timing for the Company's initiation of GLP toxicology studies relating to its SYNGAP1 program; and the therapeutic potential of the Company's product candidates. 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.

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

	Three months ended June 30,		Six months ended June 30,	
	2025	2024	2025	2024
Revenue				
Research and collaboration revenue	$ 1,497	$ -	$ 2,355	$ -
Operating expenses				
Research and development	10,343	9,389	20,489	19,129
General and administrative	4,182	3,273	7,994	6,408
Total operating expenses	14,525	12,662	28,483	25,537
Loss from operations	(13,028)	(12,662)	(26,128)	(25,537)
Other income, net:				
Interest income	453	231	1,041	626
Other income (expense)	(12)	(145)	67	(117)
Total other income, net	441	86	1,108	509
Net loss attributable to common stockholders and comprehensive loss	(12,587)	(12,576)	(25,020)	(25,028)
Net loss per share attributable to common stockholders, basic and diluted	(0.62)	(26.00)	(1.24)	(52.56)
Weighted average shares of common stock outstanding, basic and diluted	20,159,666	483,640	20,155,161	476,167

Unaudited Condensed Balance Sheet Data:	June 30,	December 31,
(in thousands)	2025	2024
Cash and cash equivalents	$ 39,052	$ 64,039
Working capital(1)	33,751	56,785
Total assets	51,275	78,307
Total liabilities	11,288	15,163
Accumulated deficit	(236,773)	(211,753)
Total stockholders' equity	39,987	63,144

(1) Working capital is defined as total current assets less total current liabilities. See our unaudited condensed consolidated financial statements and the related notes thereto included in our Quarterly Report on Form 10-Q for the six months ended June 30, 2025 for further details regarding our current assets and current liabilities.