



CAMP4 Reports Third Quarter 2025 Financial Results and Corporate Highlights

November 6, 2025

Strengthened balance sheet with private placement of up to \$100 million to advance CMP-002, a first-in-class treatment for SYNGAP1-related disorders

Initiated Good Laboratory Practice (GLP) toxicology studies for CMP-002 in support of a planned clinical trial application for a Phase 1/2 clinical trial expected to initiate as early as 2H 2026

Completed analysis from multiple ascending dose (MAD) portion of the CMP-001 Phase 1 clinical trial demonstrating favorable safety and pharmacokinetics in line with expectations; Company plans to pursue partnership for further development of CMP-001

CAMBRIDGE, Mass., Nov. 06, 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 third quarter ended September 30, 2025, and provided recent corporate highlights.

"The third quarter marked a critical milestone for CAMP4, as we positioned the company to bring a potential first-in-class treatment for SYNGAP1-related disorders into the clinic. We initiated GLP toxicology studies for CMP-002 in October and continue to expect we could initiate a first-in-human Phase 1/2 clinical trial as early as the second half of 2026," said Josh Mandel-Brehm, President and Chief Executive Officer of CAMP4. "As we prioritize our SYNGAP1 lead program, we have made a strategic decision to pursue partnerships for further development of CMP-001. We continue to believe CMP-001 has potential to be the first disease-modifying therapy for the most prevalent urea cycle disorders and were encouraged by the safety and pharmacokinetics data we observed in our Phase 1 SAD/MAD clinical trial. We also continue to explore new candidates for both in-house development and potential partnerships, as we continue our mission of developing potentially disease modifying medicines for patients with disorders marked by suboptimal gene expression."

Corporate Highlights:

CMP-002 Program for SYNGAP1 related disorders

- Initiated GLP toxicology studies for CMP-002 (formerly known as CMP-SYNGAP-01) in support of a planned clinical trial filing, which could enable the launch of a global Phase 1/2 clinical trial as early as H2 2026.
- Completed initial closing of \$50 million in upfront proceeds in a private placement with potential proceeds of up to \$100 million to fund the preclinical and clinical development of the SYNGAP1 program, extending cash runway into 2027.

CMP-001 Program for Urea Cycle Disorders

- Completed the analysis from multiple ascending dose (MAD) portion of the CMP-001 (formerly known as CMP-CPS-001) Phase 1 clinical trial in healthy volunteers.
- Received Clinical Trial Application (CTA) approval from the Central Committee on Research Involving Human Subjects (CCMO) in the Netherlands to initiate a Phase 1b study of CMP-001 in OTC heterozygotes.
- Made strategic decision to pursuing partnership to support continued development of CMP-001 in urea cycle disorders.

Results from the CMP-001 Phase 1 SAD/MAD Study in Healthy Volunteers

The Phase 1 clinical trial for CMP-001 evaluated safety, pharmacokinetics, and pharmacodynamic biomarkers from four SAD cohorts and three completed MAD cohorts. In total, 86 healthy volunteer participants were observed, including 51 receiving CMP-001. CMP-001 demonstrated a favorable safety profile in both the SAD and MAD portions, with no serious adverse events or discontinuations due to adverse events. Pharmacokinetic data similarly was observed to be consistent with expectations, demonstrating a dose-dependent increase in exposure (C_{max} and AUC) with clear separation between groups and low-to-moderate variability in key pharmacokinetic parameters at all dose levels. There were no conclusive determinations of pharmacodynamic activity or non-activity in this healthy volunteer population, which may have resulted from intrinsic variability in the ureagenesis rates of healthy individuals measured with the investigational ^{13}C -sodium acetate test used in the study.

Third Quarter 2025 Financial Results

Cash, cash equivalents, and marketable securities as of September 30, 2025, were \$75.3 million, compared to \$39.1 million as of

June 30, 2025.

R&D Expenses: Research and development expenses for the quarter ended September 30, 2025, were \$9.4 million, compared to \$9.7 million for the quarter ended September 30, 2024. The expenses were primarily driven by an increase in clinical and preclinical study costs.

G&A Expenses: General and administrative expenses were \$4.6 million for the quarter ended September 30, 2025, compared to \$3.8 million for the quarter ended September 30, 2024. The expenses were primarily driven by an increase in personnel-related and overhead costs.

Net Loss: Net loss for the quarter ended September 30, 2025, was \$15.1 million compared to \$13.5 million for the quarter ended September 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-001, including its plan to pursue partnership opportunities to support the further development of the program; the anticipated timing and results of the Company's future clinical trials, including expectations regarding the timing to advance CMP-002 into a clinical trial; and the Company's plans to explore new candidates for both in-house development and potential partnerships. 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 September 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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenue				
Research and collaboration revenue	\$ 795	\$ —	\$ 3,150	\$ —
Operating Expenses:				
Research and development	9,356	9,702	29,845	28,821
General and administrative	4,596	3,814	12,590	10,233
Impairment of right-of-use asset	494	—	494	—
Total operating expenses	14,446	13,516	42,929	39,054
Loss from operations	(13,651)	(13,516)	(39,779)	(39,054)
Other (expense) income, net:				
Interest income	423	94	1,464	720
Loss on change in fair value of derivative tranche liability	(1,800)	—	(1,800)	—
Other expense	(71)	(62)	(4)	(178)
Total other (expense) income, net	(1,448)	32	(340)	542
Net loss attributable to common stockholders and comprehensive loss	<u>\$ (15,099)</u>	<u>\$ (13,484)</u>	<u>\$ (40,119)</u>	<u>\$ (38,512)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.55)</u>	<u>\$ (24.19)</u>	<u>\$ (1.78)</u>	<u>\$ (76.50)</u>
Weighted-average shares of common stock outstanding, basic and diluted	<u>27,274,721</u>	<u>557,437</u>	<u>22,554,427</u>	<u>503,455</u>

Unaudited Condensed Balance Sheet Data:

(in thousands)

	September 30,	December 31,
	2025	2024
Cash and cash equivalents	\$ 75,255	\$ 64,039
Working capital(1)	69,805	56,785
Total assets	86,395	78,307
Total liabilities	27,728	15,163
Accumulated deficit	(251,872)	(211,753)
Total stockholders' equity	58,667	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 nine months ended September 30, 2025 for further details regarding our current assets and current liabilities.