CAMP4 Reports First Quarter 2025 Financial Results and Corporate Highlights

May 13, 2025

- Phase 1 clinical trial of CMP-CPS-001 in Urea Cycle Disorders (UCDs) ongoing, with dosing completed in multiple ascending dose (MAD) cohort 3; safety, pharmacokinetic, and pharmacodynamic data expected Q4 2025
  - CTA successfully submitted in Europe for Phase 1b clinical trial in female OTC heterozygotes
- Nominated development candidate, CMP-SYNGAP-01, to address SYNGAP1-related disorders; GLP toxicology studies expected to be initiated in 2025
- American Society of Cell and Gene Therapy (ASGCT) oral presentations to highlight meaningful increase in SYNGAP1 protein, driven by lead ASO candidate, CMP-SYNGAP-01, in non-human primates (NHP) and review interim SAD data from the Phase 1 trial of CMP-CPS-001 in healthy volunteers
- Expect to receive milestone payment from Fulcrum Therapeutics under the license agreement signed July 2023 for our Diamond-Blackfan Anemia (DBA) program

CAMBRIDGE, Mass., May 13, 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 first quarter ended March 31, 2025, and provided a corporate update.

“We are very pleased with the strong execution during the first quarter of 2025,” said Josh Mandel-Brehm, President and Chief Executive Officer of CAMP4. “We made important advances in our clinical study of CMP-CPS-001 for the treatment of UCDs, submitting a CTA in Europe for a Phase 1b expansion to enroll female participants who are heterozygous for a mutation of the OTC gene, pending regulatory clearance. We remain on track to release safety, pharmacokinetic, and pharmacodynamic data from the MAD portion of this Phase 1 study in Q4 2025. In addition, we learned that we are eligible to receive a milestone payment from Fulcrum Therapeutics under the license agreement signed in July 2023, under which Fulcrum was granted a worldwide exclusive license to rights under our DBA program. This achievement further validates our ability to create meaningful value.”

Mr. Mandel-Brehm continued, “We look forward to presenting at ASGCT, showing that our lead SYNGAP1-related disorders program ASO candidate, CMP-SYNGAP-01, identified through our proprietary RAP Platform, drove a meaningful increase in SYNGAP1 protein levels following intrathecal administration in non-human primates, demonstrating strong translational potential. We will also share evidence of increased SYNGAP1 transcription and protein levels across multiple cell types, including patient-derived neurons, as well as review safety and pharmacokinetic data from the SAD portion of our ongoing Phase 1 study of CMP-CPS-001. We believe these milestones further validate the breadth of our RAP Platform and position CAMP4 for continued momentum throughout 2025.”

Corporate Highlights:

- Completed dosing in the first three multiple ascending dose (MAD) cohorts in our ongoing clinical trial of CMP-CPS-001. Submitted CTA in Europe for Phase 1b clinical trial in female OTC heterozygotes.
- Oral presentations of new preclinical data from the SYNGAP1-related disorders program and review of interim safety results from the SAD portion of the ongoing Phase 1 study of CMP-CPS-001 in 48 healthy volunteers to be showcased at the 28th American Society of Gene and Cell Therapy Annual Meeting.
  - New preclinical data demonstrate a meaningful increase in SYNGAP1 protein levels following intrathecal administration of lead ASO candidate, CMP-SYNGAP-01, in NHPs and the potential of ASOs to target regulatory RNAs to address UCDs.
  - Review of favorable safety and pharmacokinetics data from the ongoing Phase 1 trial of CMP-CPS-001 to provide insights into the safety profile and pharmacokinetic characteristics of the therapeutic candidate.
- In April 2025, the first milestone event under our July 2023 agreement with Fulcrum Therapeutics was achieved, making us eligible to receive a $0.6 million payment from Fulcrum. Under the terms of this agreement, if Fulcrum successfully develops and commercializes a licensed product, CAMP4 will be eligible to receive (i) up to $35.0 million in development and regulatory milestone payments, and (ii) up to $35.0 million in sales milestone payments. CAMP4 is also eligible to receive royalties on worldwide net sales of licensed products ranging from mid-single digits to low-double digits.
- Nominated a new development candidate, CMP-SYNGAP-01, for the treatment of SYNGAP-1 related disorders based on

robust preclinical data, including recent results from NHP studies.

First Quarter 2025 Financial Results

Cash, cash equivalents, and marketable securities at March 31, 2025, were $49.3 million, compared to $64.0 million as of December 31, 2024.

R&D Expenses: Research and development expenses for the quarter ended March 31, 2025, were $10.1 million, compared to $9.7 million for the quarter ended March 31, 2024. The increase in R&D expenses was primarily driven by an increase in clinical and preclinical study costs.

G&A Expenses: General and administrative expenses were $3.8 million for the quarter ended March 31, 2025, compared to $3.1 million for the quarter ended March 31, 2024. The increase in G&A expenses was primarily due to increases in personnel-related and overhead expenses.

Net Loss: Net loss for the quarter ended March 31, 2025, was $ 12.4 million compared to $12.5 million for the quarter ended March 31, 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 CAMP4's plans and expectations regarding its ongoing Phase 1 clinical trial of CMP-CPS-001 and its 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; and the therapeutic potential of CAMP4'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 March 31, 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:
Dan Ferry (US)
LifeSci Advisors
daniel@lifesciadvisors.com

Sandya von der Weid (Europe)
LifeSci Advisors
svonderweid@lifesciadvisors.com

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

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

	Three months ended March 31,	
	2025	**2024**
Revenue		
Research and collaboration revenue	$ 858	$ -
Operating expenses		
Research and development	10,146	9,740
General and administrative	3,812	3,135
Total operating expenses	13,958	12,875
Loss from operations	(13,100)	(12,875)
Other income, net:		
Interest income	588	395
Other income (expense)	79	28
Total other income, net	667	423
Net loss attributable to common stockholders and comprehensive loss	$ (12,433)	$ (12,452)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.62)	$ (26.57)
Weighted average shares of common stock outstanding, basic and diluted	20,152,872	468,695

Unaudited Condensed Balance Sheet Data:	**March 31,**	**December 31,**
(in thousands)	**2025**	**2024**
Cash and cash equivalents	$ 49,323	$ 64,039
Working capital(1)	45,234	56,785
Total assets	62,773	78,307
Total liabilities	11,201	15,163
Accumulated deficit	(224,186)	(211,753)
Total stockholders' equity	51,572	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 three months ended March 31, 2025 for further details regarding our current assets and current liabilities.