CAMP4 Presents Translational Data from SYNGAP1-Related Disorders Program Showcasing Increased Protein in Non-Human Primates and Reviews Preclinical and Detailed Single Ascending Dose Safety Data from Urea Cycle Disorders Program at the 28th American Society of Gene and Cell Therapy Annual Meeting

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Haploinsufficient SYNGAP1 mice treated with CMP-SYNGAP-01 demonstrated an increase in SYNGAP1 protein levels; treatment rescued multiple SYNGAP1-dependent behavioral phenotypes

CMP-SYNGAP-01 administration led to a significant increase in SYNGAP1 protein levels in relevant brain regions in non-human primates (NHPs)

Patient safety and pharmacokinetic data from single ascending dose (SAD) cohorts of the first-in-human Phase 1 clinical trial of CMP-CPS-001 in healthy volunteers highlighted

CAMBRIDGE, Mass., May 16, 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 delivered three oral presentations on its SYNGAP1-related disorders and Urea Cycle Disorders (UCDs) programs and shared favorable safety and pharmacokinetics data from the ongoing Phase 1 trial of CMP-CPS-001 in healthy volunteers at the 28th Annual Meeting of the American Society of Gene and Cell Therapy, taking place in New Orleans, May 13 – 17, 2025.

"Patients living with SYNGAP1-related disorders and UCDs currently face a critical dearth of disease-modifying treatment options to manage their condition," said Dan Tardiff, Ph.D., Senior Vice President, Head of Discovery at CAMP4. "These proof-of-mechanism data indicate CMP-SYNGAP-01 can restore SYNGAP1 protein levels to mitigate disease-relevant phenotypes in haploinsufficient mice and increase SYNGAP1 protein in disease-relevant brain regions in non-human primates when delivered by the clinical route of administration. Additionally, our UCD clinical candidate is well tolerated, and we are preparing to evaluate CMP-CPS-001 in OTC heterozygotes, a population with reduced urea cycle function. We're excited to continue pioneering our novel approach of upregulating gene expression and addressing the unmet needs of many patients living with genetic diseases characterized by haploinsufficiency or recessive loss of function."

Josh Mandel-Brehm, Chief Executive Officer of CAMP4, added, "These compelling data underscore the expansive potential of our RAP Platform to address a wide spectrum of genetic conditions, starting with neurologic and metabolic disorders. By pairing clinically validated antisense technologies with newly discovered regulatory RNA targets to upregulate gene expression, we have an opportunity to rapidly advance therapeutics for disorders characterized by insufficient protein production including SYNGAP1-related disorders, where there is significant unmet need for disease-modifying therapies. We look forward to progressing toward additional clinical trials and exploring strategic partnerships that can accelerate our development plans and deliver long-term value for patients and shareholders."

Key findings for each program are as follows:

SYNGAP1-related disorders program

- In haploinsufficient mice carrying a single copy of the human *SYNGAP1* gene, intracerebroventricular (ICV) injection of CMP-SYNGAP-01, a development candidate targeting a regulatory RNA sequence mapped to a *SYNGAP1* gene regulatory region, resulted in:
  - Restored SYNGAP1 protein levels to near normal range after a single dose
  - Rescue of motor defects and spatial learning defects following two doses
- In NHPs, biweekly intrathecal injections of CMP-SYNGAP-01 resulted in a ~1.5-fold increase in SYNGAP1 protein levels across multiple brain regions clinically relevant to the disease
  - Dose-linear increase in CMP-SYNGAP-01 in disease-relevant brain regions
  - CMP-SYNGAP-01 was well tolerated

UCD program

- Preclinical data
  - In *Otc*-deficient mice, treatment with CMP-CPS-001 resulted in dose-dependent reductions in ammonia levels, which persisted for approximately 4 weeks
    - Increases in mRNA levels of additional enzymes of the urea cycle were observed, suggesting increased metabolic activity
  - In mice with humanized livers, administration of CMP-CPS-001 following an ammonia challenge resulted in increased ureagenesis and decreased ammonia levels
  - Administration of CMP-CPS-001 in NHPs resulted in up to 40% increase in ureagenesis, supporting the MOA to convert ammonia to urea
- Phase 1 clinical data
  - 48 healthy adult participants were enrolled across four SAD cohorts and randomized 3:1 to a single subcutaneous dose of CMP-CPS-001, with 36 participants randomized to CMP-CPS-001
  - CMP-CPS-001 was well tolerated, with no evidence of a maximum tolerated dose and no safety trends of concern
  - All treatment-emergent adverse events (TEAEs) were Grade 1 (mild) or Grade 2 (moderate) with no serious or severe adverse events (AEs) or TEAEs and no participants discontinued study drug due to a TEAE
    - Most common TEAEs were headache (n=6) followed by nausea (n=4)
  - Pharmacokinetics
    - Dose-dependent increase in exposure (Cmax and AUC) with clear separation between dose levels
    - Greater than dose-proportional increase in exposure (Cmax and AUC0-24)
- Study Update
  - Dosing complete in MAD Cohort 1 through Cohort 3
  - Anticipate expansion into OTC heterozygotes to assess safety and CMP-CPS-001 effect on ureagenesis in patients with evidence of reduced urea cycle function

The presentations can be accessed on the CAMP4 website at https://investors.camp4tx.com/news-events/presentations after the presentations.

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.

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