



CAMP4 Therapeutics Receives Authorization in the United Kingdom for Phase 1/2 Human Clinical Trial of CMP-002 in Patients with SYNGAP1-Related Disorder

September 10, 2026

Authorization from the UK's Medicines and Healthcare products Regulatory Agency (MHRA) builds on CAMP4's recent clearances to initiate first-in-human studies for CMP-002 in Australia and Argentina

Company anticipates initiation of the clinical trial in 4Q 2026

WATERTOWN, Mass., Sept. 10, 2026 (GLOBE NEWSWIRE) -- [CAMP4 Therapeutics Corporation](#) ("CAMP4" or "the Company") (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 the company has received authorization from the United Kingdom's Medicines and Healthcare Products Regulatory Agency (MHRA) to include UK sites in the Company's global Phase 1/2 clinical trial of CMP-002, a potential first-in-class, disease-modifying therapeutic candidate for patients with SYNGAP1-related disorder.

"This authorization by the MHRA expands the reach of our Phase 1/2 clinical trial and reinforces our commitment to rapidly advancing CMP-002 for patients and their families who have been without an intervention that tackles the underlying drivers of this disease," said Josh Mandel-Brehm, President and Chief Executive Officer of CAMP4. "This milestone builds on the regulatory momentum we established with recent clearances in Australia and Argentina, and we remain on track to initiate the study during the fourth quarter of 2026."

In July 2026, the company received clearance to initiate the Phase 1/2 clinical trial from Australia's Therapeutic Goods Administration (TGA) and local Human Research Ethics Committee (HREC), as well as Argentina's Administración Nacional de Medicamentos, Alimentos y Tecnología Médica (ANMAT), supporting initiation of a Phase 1/2 clinical trial of CMP-002 in Q4 2026. The company has also submitted regulatory filings in the European Union, which remain under review.

About SYNGAP1-Related Disorder

SYNGAP1-related disorder (also referred to as SYNGAP1) is a rare, haploinsufficient CNS disorder caused by mutations in the SYNGAP1 gene, resulting in approximately 50% of normal SYNGAP protein levels. The condition affects over 10,000 individuals in the United States and is characterized by intellectual disability in 100% of patients, epilepsy in approximately 85%, severe behavioral problems in approximately 70%, sleep problems in approximately 60%, and limited communication, with approximately 30% of patients being non-verbal. There are currently no approved disease-modifying therapies for patients living with SYNGAP1.

About CMP-002

CMP-002 is CAMP4's lead investigational antisense oligonucleotide (ASO) therapeutic candidate designed to bind to a SYNGAP1-specific regRNA to increase SYNGAP1 gene expression and restore SYNGAP protein toward near wild-type levels. Administered intrathecally, CMP-002 has demonstrated dose-dependent increases in SYNGAP protein expression in patient-derived neurons, reversal of disease-relevant behavioral phenotypes in a humanized haploinsufficient mouse model, statistically significant improvement of seizure phenotypes and parameters in a chemically induced seizure mouse model, and broad brain distribution with significant SYNGAP protein upregulation in non-human primates.

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 anticipated timeline for initiation of the Company’s planned Phase 1/2 clinical trial of CMP-002 in patients with SYNGAP1-related disorder; the therapeutic potential and intended mechanism of action of CMP-002; the potential of the Company’s RAP Platform technology; the expected outcome of the Company’s pending regulatory submissions in other jurisdictions; the Company’s ability to satisfy the conditions of regulatory authorizations received to date; and the Company’s strategy, goals, business plans and focus. 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 inception and anticipated incurrence of substantial and increasing losses for the foreseeable future; the Company’s need for substantial additional financing to achieve its goals; the uncertainty of clinical development and risks related to additional costs or delays in the development and commercialization of the Company’s 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 manage the Company’s growth and expansion of the Company’s operations; risks related to the manufacturing of the Company’s product candidates; the Company’s ability to obtain and maintain sufficient intellectual property protection for its product candidates; 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, 2025 and Quarterly Report on Form 10-Q for the quarter ended June 30, 2026, 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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