



CAMP4 Therapeutics Preprints Pioneering Study on Regulatory RNA Profiling and Gene Upregulation

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Using epigenomic mapping and regulatory RNA (regRNA) Capture-seq, CAMP4 has assembled what it believes to be the largest catalog of regRNAs ever compiled, uncovering thousands of previously undiscovered regRNAs in human cells

New research enables the discovery of antisense oligonucleotides (ASOs) to increase expression of disease-relevant genes for therapeutic potential

CAMBRIDGE, Mass., April 28, 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 submission of a preprint to *bioRxiv*, an open-access server for life sciences research, titled, "[Profiling and Targeting of Regulatory RNAs to Upregulate Gene Expression](#)."

The preprint describes CAMP4's development of regulatory RNA (regRNA) Capture-seq methodology, a highly sensitive method for characterizing regRNAs, which have previously been difficult to detect using conventional sequencing methods due to their short-lived nature and low expression levels. In combination with epigenetic mapping, this technology uniquely enables the first step in CAMP4's novel therapeutic approach to increasing expression of disease-relevant genes by targeting regRNAs with antisense oligonucleotides.

"Submitting this work to bioRxiv is a milestone that reflects years of rigorous science executed by a dedicated team, and we are thrilled to share it with the broader scientific community," said Alla Sigova, Ph.D., Senior Vice President, Platform. "The application of this technology and the vast regRNA catalog we have assembled represent a meaningful advance in our ability to understand and harness this fundamental layer of gene regulation. Translating these insights into a potentially novel strategy for increasing gene expression speaks to the strength and versatility of our platform."

Applicable across all human cell types, epigenomic mapping and regRNA Capture-seq enables high-resolution characterization of regRNAs at scale. Applying this technology to primary human liver cells, CAMP4 has constructed what it believes to be the largest catalog of regRNAs ever assembled in any human cell type, identifying thousands of previously undescribed molecules in the process.

To illustrate the therapeutic potential of mapping and targeting regRNAs, CAMP4 developed ASOs targeting a regRNA produced by an enhancer that controls the expression of the ornithine transcarbamylase (OTC) gene in the liver. Mutations in this gene cause OTC deficiency (OTCD), the most common urea cycle disorder and a life-threatening condition in which the body cannot properly process nitrogen, leading to toxic ammonia buildup in the blood. CAMP4 designed ASOs that increased OTC expression in human hepatocytes, suggesting a potentially novel therapeutic approach for diseases where increased expression of a functional gene copy may improve clinical outcomes. The preprint describes mechanistic studies on the ASOs' ability to reshape regRNA structure, increase its levels within cells, and displace transcriptional repressors from the associated enhancer, providing new insights into how regRNA modulation regulates gene expression.

Building on this platform, CAMP4 has applied advanced regRNA mapping across additional cells of the central nervous system to identify CMP-002, its lead product candidate, which is designed to target a SYNGAP1 regRNA to increase protein levels. CAMP4 expects to advance CMP-002 into a Phase 1/2 clinical trial in individuals with SYNGAP1 as early as the second half of 2026.

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](#).

About bioRxiv

bioRxiv (pronounced "bio-archive") is a free online archive and distribution service for [unpublished preprints](#) in the life sciences. bioRxiv is operated by [openRxiv](#), a non-profit organization dedicated to advancing science communication. It was founded by Cold Spring Harbor Laboratory in 2013. By posting preprints on bioRxiv, authors are able to make their findings immediately available to

the scientific community.

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 timing to advance CMP-002 into a clinical trial and the therapeutic potential of the Company’s platform technology. 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 uncertainty of preclinical and 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; 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; 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 platform technology and product candidates; 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, 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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