PRESS RELEASE / JANUARY 23, 2019

CAMP4 Therapeutics Announces Rare Disease Research Collaboration with Alnylam

Collaboration will leverage CAMP4's Gene Circuitry Platform™ to rapidly identify novel signaling pathway targets for potential RNAi intervention.

CAMBRIDGE, Mass.– (BUSINESS WIRE)–CAMP4 Therapeutics today announced a research collaboration with Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY) focused on identifying new druggable targets to address an undisclosed rare disease of the liver with a significant unmet need. The collaboration brings together CAMP4's expertise in rapidly pinpointing signaling targets that control the expression of genes with Alnylam's expertise in RNAi therapeutics development to advance novel rare disease drug candidates.

"We are excited to partner with Alnylam given its leadership in the RNAi and liver disease space and our companies' shared commitment to advancing new medicines to address critical unmet patient needs," said Josh Mandel-Brehm, Chief Executive Officer (CEO) of CAMP4. "Our Gene Circuitry Platform is yielding the first-ever, comprehensive and dynamic understanding of the Cellular Operating System™ used by human liver cells to regulate the activation of genes and is applicable to any disease-associated genes in the liver. We look forward to applying this knowledge to elucidate the optimal targets Alnylam can explore for potential RNAi intervention for a disease where there are currently no available treatment options."

Under the terms of the collaboration, CAMP4 will lead all activities to predict and validate disease-modifying targets in signaling pathways. Alnylam will lead in vivo proof-of-concept activities and may elect a target against which to develop RNAi therapies at the completion of the research term. CAMP4 will receive an undisclosed upfront payment from Alnylam as well as milestone payments upon successful achievement of in vitro proof of concept and target election. CAMP4 is further eligible to receive development, approval and sales milestones plus royalties on products developed against the elected target.

"CAMP4's Gene Circuitry Platform provides an innovative approach to target identification and is in line with Alnylam's strategy to fuel its R&D engine ensuring sustainable growth," said Kevin Fitzgerald, Ph.D., Senior Vice President, Research at Alnylam. "We welcome this collaboration and are excited for its potential to yield targets implicated in disease settings for which RNAi can be of therapeutic value."

CAMP4 is combining experimental and computational techniques to discover how every gene responds to its environment through signaling. The company is deriving algorithms to explain how signaling pathways control the transcriptional machinery used by each cell to regulate the output of every disease-associated gene (i.e., the Cellular Operating System). Starting in the liver, CAMP4 is using its proprietary Gene Circuitry Platform to rapidly predict and validate the most effective targets to control gene expression across hundreds of diseases where hepatocytes are core to the pathology, including a large number of rare diseases. The company is aggressively mapping the Cellular Operating Systems of other primary human cell types, with the goal of mapping all cell types central to major disease pathology. CAMP4 is executing on targets to develop a robust internal pipeline of drugs while forging strategic collaborations in parallel to maximize the broad discovery and development opportunities presented by its platform.

"Our conviction is that there is a better way to approach drug development that starts with understanding the mechanisms behind the Cellular Operating System for each primary cell type, which will enable us to ultimately reshape the odds of successfully developing new treatments," said Iris Grossman, Ph.D., Chief Scientific Officer at CAMP4. "By elucidating which pathways are most significant in controlling gene expression, for any gene of interest across the human body, we intend to open up the possibility for industry to develop effective treatment options for many hard-to-treat diseases, with the ambitious goal of leaving no patient behind."

About CAMP4 Therapeutics

CAMP4 Therapeutics was founded based on the seminal discoveries made by founders Dr. Richard Young and Dr. Leonard Zon. We are establishing the first-ever comprehensive and dynamic view of how signaling pathways control our transcriptional machinery, called the Cellular Operating System™, to regulate gene expression. We have started by deciphering the Cellular Operating System of hepatocytes and are in the process of systematically mapping the Cellular Operating Systems for all primary human cell types central to disease. Using our Gene Circuitry Platform™ we are then developing algorithms that enable us to rapidly predict the optimal, druggable targets within signaling pathways. Pursuing targets with a clearly defined mechanism in the underlying biology enables a modality-agnostic approach, in which we can discover and develop novel therapies across hundreds of diseases and unmet needs. To that end, we plan to build a robust pipeline of internal drug candidates across numerous therapeutic areas, complemented by the formation of broad strategic collaborations. Find out more at www.camp4tx.com.

Media Contact:

Liz Melone
617-256-6622
liz@scientpr.com