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CONTACT:
Erin Coller, ACD Board of Trustees
erin@creatineinfo.org

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ASSOCIATION FOR CREATINE DEFICIENCIES ANNOUNCES OVER $1 MILLION INVESTED IN RACE FOR A CURE INITIATIVES

Carlsbad, CA (June 22, 2026)— The Association for Creatine Deficiencies (ACD) announced the investment of over $1.1 million in Race for a Cure initiatives with a focus on Creatine Transporter Deficiency (CTD) and Guanidinoacetate Methyltransferase (GAMT) Deficiency treatment-focused research.

Johns Hopkins University - Dr. Kannan Rangaramanujam and Dr. Sujatha Kannan of Johns Hopkins University have been selected by ACD for a two-year Race for a Cure research investment to accelerate their work to deliver creatine to the brain, including to neurons, using dendrimer nanoparticles, without the need of a creatine transporter, to help patients with CTD. Published research has already demonstrated that dendrimer nanoparticles carrying creatine cross the blood-brain barrier, and release creatine sustainably inside cells. ACD's investment will pave the way to clinical translation.

"To successfully treat an orphan disorder like CTD, it takes the entire community of patients, caretakers, doctors, and researchers," said Dr. Rangaramanujam. "We are fortunate to be able to work with ACD, a dynamic unit that has been pushing hard against this challenge, and catalyzing new knowledge and bringing it to patients," said Dr. Rangaramanujam.

Dr. Rangaramanujam is the Arnall Patz Distinguished Professor of Ophthalmology and Co-Director of the Center for Nanomedicine at the Wilmer Eye Institute, Johns Hopkins School of Medicine. His research focuses on translational nanomedicine, where his team pioneered the hydroxyl dendrimer platform to develop targeted therapies for ocular, neurological and other disorders. Dr. Rangaramanujam is an author of more than 190 patents and 160 peer-reviewed publications.

Dr. Kannan is the Richard J. Traystman Endowed Professor of Anesthesiology and Critical Care Medicine & Pediatrics at Johns Hopkins University and a research scientist at the Hugo Moser Research Institute, Kennedy Krieger Institute. Her translational research focuses on nanotechnology and nanomedicine to understand and manipulate glial and neuronal responses

in disorders such as cerebral palsy, autism, Rett syndrome, and other neuroinflammatory and neurodegenerative conditions. Dr. Kannan holds more than 110 patents, has authored more than 90 peer-reviewed publications, and is co-founder of Ashvattha Therapeutics, Samata Therapeutics, and Avatar Precision Therapeutics.

University of California, Berkeley - Dr. Alanna Schepartz of The University of California, Berkeley, (UC Berkeley), has been selected for a Race for a Cure research award to accelerate the development of a novel method to deliver a key enzyme directly into cells to restore creatine production in individuals with GAMT deficiency. Her team is exploring a novel strategy to improve GAMT's ability to escape the cell's lysosomal degradation pathway and reach the cytoplasm, where it can restore normal creatine production. If successful, this approach could offer an entirely new therapeutic strategy for individuals with GAMT deficiency by bypassing the genetic defect and replenishing creatine production directly at the cellular level.

“GAMT is exceptionally well-suited for delivery using the mini-proteins we discovered,” said Dr. Schepartz. “The team is incredibly excited to get started on this important project."

Dr. Alanna Schepartz is the C.Z. and Irmgard Chu Distinguished Professor of Chemistry and Molecular and Cell Biology at UC Berkeley, where her research focuses on the chemistry and biology of complex cellular machines to design and discover molecules with unique or useful properties. A former Sterling Professor at Yale — the university's highest faculty honor — she is a member of both the American Academy of Arts and Sciences and the National Academy of Sciences, and her work has been recognized with numerous prestigious awards including the ACS Chemical Biology Prize, the Frank H. Westheimer Prize, and the Ronald Breslow Award for Achievement in Biomimetic Chemistry.

Stanford University - ACD fellowship awardee Alex Edwin, alongside Al Garofalo and Dr. Thomas Montine at the Montine Lab at Stanford University, have been selected for a Race for a Cure research award to build upon their work developing new ways to deliver creatine directly into cells without relying on the faulty transporter protein that causes CTD. Because no approved therapy currently exists for CTD, the team is pursuing two complementary strategies — cell-penetrating peptides and nanoparticle-based molecular transporters — each exploiting a different mechanism to bypass the broken transporter and restore creatine where it is needed most.

"The Montine Lab is developing innovative therapeutics capable of restoring creatine in cells lacking the creatine transporter, the hallmark of CTD,” stated Edwin. “Our approach seeks to reestablish cellular energy homeostasis in the brain, muscle, and other metabolically active tissues, with the goal of improving both neurological and systemic outcomes for people with CTD."

Edwin graduated with a BS in Neuroscience from Santa Clara University in 2023. Since 2021, he has worked as a Life Science Research Professional in the Montine Lab at Stanford School of Medicine, where he focuses on developing a small molecule therapeutic for CTD.

Launched in 2025, ACD's Race for a Cure aims to accelerate high-impact research projects that are poised to make meaningful advancements toward clinical trials. These three sponsored research awards represent ACD's commitment to funding the most promising, translatable science in the field and over $1 million invested in the urgent pursuit of effective treatments for CCDS.

"ACD exists to improve the lives of those impacted by cerebral creatine deficiency syndromes, and effective treatments are the most urgent need we have," said Heidi Wallis, ACD executive director. "We launched Race for a Cure to expedite the research behind them, and with over $1 million committed, we're funding promising, translatable science that's poised to reach patients. The race is on!" To support this work and help fund the next round of research, visit creatineinfo.org/race-for-a-cure.

About ACD: ACD's mission is to eliminate the challenges of Cerebral Creatine Deficiency Syndromes (CCDS). ACD is committed to providing patient, family, and public education to advocate for early intervention through newborn screening, and to promote and fund medical research for treatments and cures for CCDS. For more information regarding ACD, please visit creatineinfo.org.

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