ACD Approved for $249,650 Award for Project to Build Upon Prior Work and Empower CCDS Parent Engagement in Research

ACD has been approved for a $249,650 funding award through the Eugene Washington PCORI Engagement Award Program to fund a two year project.

CTD & GAMT Core Outcome Set Consensus Workshop

CARLSBAD, CALIFORNIA, USA, June 21, 2024 /EINPresswire.com/ -- The Association for Creatine Deficiencies (ACD) has been approved for a $249,650 funding award through the Eugene Washington PCORI Engagement Award Program, an initiative of the Patient-Centered Outcomes Research Institute (PCORI). The funds will support a project titled “Parents Advancing REsearch NeTworkS 2.0: Expand Community, Tools, & Engagement (PAReNts 2.0: ExCiTE).” The two-year project will launch on Sept. 1 and through multi-stakeholder collaboration, will establish “Considerations for the Selection of Outcome Measurement Tools for GAMT & CTD” as a companion to the prior project’s “CTD & GAMT Core Outcome Set.”

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>
> *Dr. Audrey Thurm*

The PAReNts 2.0: EXCiTe project will engage a diverse stakeholder group including Creatine Transporter Deficiency (CTD) and Guanidinoacetate Methyltransferase (GAMT) Deficiency patient caregivers, related health professionals, industry representatives, and policy makers. Stakeholders will explore potential tools to measure the outcomes identified in the core outcome set project, the clinically meaningful differences caregivers consider relevant and attainable by the patient within these outcomes, and considerations for selecting tools that would best capture those clinically meaningful differences when assessing the core outcome set in a clinical trial.

The overarching goals of the PARENTS 2.0: ExCiTE project are to:

Engage and train a core group of CTD & GAMT caregivers in the two-year project, increasing caregiver engagement in research;
Expand partnerships to include input from health professionals, industry, and policymakers in the process to increase successful adoption of the outcomes of this project;
Identify important considerations for selecting outcome measurement tools appropriate to the patients enrolled in future trials, ensuring patient-centered trial design.


Consensus Workshop Voting

“Our first PCORI-funded project established the core outcomes caregivers and health professionals agree need to be measured across all clinical trials,” said Heidi Wallis, ACD executive director, GAMT caregiver, and project lead. “This second project will provide researchers with considerations for selecting appropriate tools to measure those outcomes in a patient-centered manner. We want to see a good treatment succeed in a clinical trial. If the wrong tool is used, that might not happen.”

Dr. Audrey Thurm, director of the Neurodevelopmental and Behavioral Phenotyping Service in the Office of the Clinical Director at the National Institute of Mental Health (NIMH) will serve as co-lead on the PAReNts 2.0: ExCiTE project, providing scientific expertise to the project as part of her official duties at NIMH. “We are doing something here that hasn’t been done before. An inappropriate outcome measurement tool is a real concern in trials for patients with neurodevelopmental disorders. Some children have improvements that are meaningful to the patient and family but don’t show up on the assessments that are most often used. For the CTD and GAMT patient community to step up and preemptively define what trials need to consider in selecting measurement tools for their population is really exciting,” said Thurm.

Dr. Sylvia Gerda Stockler-Ipsiroglu, program director in biochemical diseases at BC Children's Hospital, will serve on the project as research director. Dr. Beth Potter, professor at University of Ottawa and holder of the university research chair in health services for children with rare diseases, will be the team’s research advisor. Dr. William Bennett, associate professor of pediatrics, pediatric gastroenterologist, clinical informatician, and clinical researcher at Indiana University joins the team as scientific advisor. Emily Reinhardt, registry coordinator at ACD, will serve as administrative official.

The Indiana Clinical and Translational Sciences Institutes’ Patient Engagement Core will collaborate on the project, facilitating three Research Jam sessions during the project to foster multi-stakeholder collaboration.

PCORI is an independent, nonprofit organization authorized by Congress with a mission to fund patient-centered comparative clinical effectiveness research that provides patients, their caregivers, and clinicians with the evidence-based information they need to make better informed health and healthcare decisions.

About ACD: The Association for Creatine Deficiencies was established in 2012 with the mission to eliminate the challenges of living with cerebral creatine deficiency syndrome. ACD is committed to providing patient, family, and public education to advocate for early intervention through newborn screening, and to support and drive medical research for treatments and cures for CCDS. Because CCDS mimic symptoms of other medical conditions, patients are often first diagnosed with autism, cerebral palsy, epilepsy, and other disorders. Proper diagnosis and early intervention are critical to establishing screening and treatments needed to improve life quality and longevity for the CCDS patient. For more information regarding ACD, please visit creatineinfo.org.

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