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CONTACT:  
Erin Collier, Board of Trustees  
[erin@creatineinfo.org](mailto:erin@creatineinfo.org)

## FOR IMMEDIATE RELEASE

### **ACD ENTERS AGREEMENT WITH ULTRAGENYX TO INVEST IN CLINICAL DEVELOPMENT OF THERAPY FOR CREATINE TRANSPORTER DEFICIENCY**

*ACD commits \$4 million investment with Ultragenyx to accelerate path to treatment for CTD patients*

Carlsbad, CA, July 2, 2026 — The [Association for Creatine Deficiencies](#) (ACD) today announced it has entered into a venture philanthropy agreement with Ultragenyx Pharmaceutical Inc. (Ultragenyx) in which ACD will invest in the clinical development of an investigational therapy for Creatine Transporter Deficiency (CTD) with a total commitment of \$4 million as the program progresses.

"Our partnership with ACD represents a shared commitment toward a new future for CTD and addresses the urgency that treatments are needed now," said Emil Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx, who delivered the keynote address at ACD's 2026 CCDS Scientific & Patient Symposium on June 26 in Utah.

[Ultragenyx](#), a biopharmaceutical company focused on developing first-ever approved treatments for rare and ultra-rare diseases, and ACD will work collaboratively through a joint steering committee to review progress and advance the program. ACD is investing in the clinical development of UX068, a new investigational small molecule therapy for CTD.

CTD is an ultra-rare, devastating genetic disorder that deprives the brain and body of creatine — an essential molecule for cellular energy. The condition produces a range of serious neurological and physical symptoms, including epilepsy, intellectual disabilities, speech and language delays, gastrointestinal complications, and low muscle tone. There are currently no approved treatments.

ACD's commitment of \$4 million to fund UX068 clinical trials is the largest research investment in ACD's history and a milestone achievement of ACD's [Race for a Cure](#), launched in 2025 to accelerate high-impact research toward treatments for Cerebral Creatine Deficiency Syndromes.

"This agreement reflects what our community has been working toward since ACD was founded," said Heidi Wallis, executive director of ACD. "CTD families have waited long enough. This is how we close that gap — by stepping in, taking on risk alongside committed partners,



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and making sure therapies have the resources needed to reach clinical trials. ACD remains committed to funding multiple avenues of research to treat and cure all creatine deficiency disorders and will continue funding a variety of efforts in addition to this incredibly important project."

The Ultragenyx agreement follows ACD's announcement last week of over [\\$1.1 million invested in Race for a Cure research awards](#) to [Johns Hopkins University](#), the [University of California Berkeley](#), and [Stanford University](#) – representing a broad, coordinated push to move multiple promising CTD and GAMT therapies toward the clinic.

### How You Can Help

As part of ACD's Race for a Cure, the patient advocacy organization's next milestone goal is to raise \$2 million by Dec. 31, 2026, to support this investment and others. The community's support will be crucial in meeting this goal.

Any financial return ACD receives from philanthropic investments such as this will be directed back into the organization's mission: funding research, supporting families, and advancing newborn screening for all three creatine deficiency syndromes.

To make a gift, visit [creatineinfo.org/race-for-a-cure](https://creatineinfo.org/race-for-a-cure).

**About ACD:** The Association for Creatine Deficiencies' mission is to eliminate the challenges of 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 Cerebral Creatine Deficiency Syndromes. 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 interventions needed to improve life quality and longevity for the CCDS patient. For more information regarding ACD, please visit [creatineinfo.org](https://creatineinfo.org).

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