

# Core Outcome Set



# Delphi Survey

CTD & GAMT caregivers, patients, and clinicians:  
**WE WANT TO HEAR FROM YOU!**



Researchers across the globe are studying treatments to improve care for individuals with Creatine Transporter Deficiency (CTD) and GAMT Deficiency (GAMT). Those research projects need to measure appropriate outcomes for these disorders.

## WHAT ARE OUTCOMES?

- Outcomes may include how someone feels, what they can do, observable symptoms, or results of lab tests.
- Outcomes are used to determine if a treatment is effective or not.

## WHAT IS A CORE OUTCOME SET (COS) AND WHY IS A COS USED?

- A 'Core Outcome Set' is a small set of outcomes that are established as important, and should be collected, in every research study of the same disease.
- An established COS prevents studies from measuring too few outcomes or inappropriate outcomes for the disorder. It is expected that all outcomes in the COS are reported.

## WHY IS A COS IMPORTANT?

- If researchers measure different outcomes, it is hard to compare and contrast the effectiveness of interventions or treatments.
- A COS will help us compare CTD and GAMT deficiency studies to identify which treatments work best.



Researchers are developing a core outcome set for CTD and GAMT deficiency and we need your input! We want to know which outcomes are important to you!



# Delphi Survey FAQs

## HOW CAN I CONTRIBUTE?

In multiple rounds of an online survey, we will show you a list of outcomes related to CTD and GAMT deficiency and ask you to rate how important each outcome is to you. The survey will be available in English, French, and Spanish. As a rare disease community, we need as many patients, caregivers, and clinicians as possible to participate!

## WHO IS ELIGIBLE?

- An adult (18 years or older) diagnosed with CTD or GAMT deficiency
- A parent or other adult caregiver of a person diagnosed with CTD or GAMT deficiency
- A healthcare professional or additional support (e.g., geneticist, dietician, teacher, physical therapist, social worker) who cares for patients with CTD or GAMT deficiency
- Health policy advisor (e.g., FDA)
- Laboratory and/or research scientist

## WHAT IS THE TIME COMMITMENT?

- You will be asked to complete 2-3 surveys within the next 3-5 months.
- Each survey will take about 1 hour to complete.
- Participation in rounds two and three requires prior completion of the preceding round(s).
- After each survey you complete, you will be entered into a drawing to win:
  - One of five Amazon gift cards valued at \$50 USD for the first survey round
  - One of five Amazon gift cards valued at \$75 USD for the second survey round
  - One of five Amazon gift cards valued at \$75 USD for the third survey round, if required

## WHERE CAN I LEARN MORE? WHEN CAN I ACCESS THE SURVEY?

The first Delphi survey will be released in February 2023. Learn more at: [creatineinfo.org/cos](https://creatineinfo.org/cos).

## QUESTIONS?

Email Emily at [registry@creatineinfo.org](mailto:registry@creatineinfo.org)

