

Community Update: First Patient Treated in Phase I/II CYStem Clinical Trial in Nephropathic Cystinosis

July 24, 2026

Dear Cystinosis Community,

We are pleased to share an important update on CYStem, the Phase I/II clinical trial evaluating the safety and efficacy of the investigational therapy DFT383 in pediatric participants with cystinosis. The first participant has now been treated. As specified in the study protocol, the Data Monitoring Committee (an independent group of experts that reviews study data to protect participants' safety) has reviewed available study information and recommended that the study may proceed as planned. We would like to thank everyone involved for all their efforts and dedication in reaching this important milestone.

About the CYStem Study

CYStem is an open-label, multi-center, Phase I/II study to assess safety, tolerability and efficacy of DFT383 in pediatric participants with nephropathic cystinosis [Study Details | DFT383 in Pediatric Participants With Nephropathic Cystinosis | ClinicalTrials.gov](#)

The purpose of the study is to investigate if DFT383 is safe and effective in children aged 2-5 years who have nephropathic cystinosis. DFT383 is a hematopoietic stem cell-based gene therapy. This study includes an active treatment group (Cohort 1), where participants will receive DFT383, and a concurrent reference group (Cohort 0). Participants in Cohort 0 will not receive study treatment and will continue receiving standard of care (i.e. cysteamine). The study is not randomized. Data from the reference group will help better understand the disease and the results observed in the children receiving DFT383. Investigational sites may enroll participants in one or both cohorts.

We understand the significant impact that cystinosis has on individuals and families. Research studies like CYStem are only possible because of the commitment of the cystinosis community. We are deeply grateful to everyone who contributes to advancing research, whether through participation, advocacy, or support.

Novartis remains committed to transparency, collaboration with the cystinosis community, and sharing updates as development continues. We encourage anyone who is interested to review the study information posted on ClinicalTrials.gov and to speak to their healthcare provider.

Kind regards,

The Novartis CYStem Cystinosis Study Team

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