DFT383 in Pediatric Participants With Nephropathic Cystinosis

Last Update: Jul 04, 2025
An Open-label, Multi-center, Phase I/II Study to Assess Safety, Tolerability and Efficacy of DFT383 in Pediatric Participants With Nephropathic Cystinosis
ClinicalTrials.gov Identifier:
NCT06910813
Novartis Reference Number:CDFT383A12101
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All compounds are either investigational or being studied for (a) new use(s). Efficacy and safety have not been established. There is no guarantee that they will become commercially available for the use(s) under investigation.

Study Description

An open-label, multi-center, phase I/II study to assess the safety, tolerability and efficacy of DFT383 in pediatric participants with nephropathic cystinosis.

The purpose of this clinical study is to assess safety, tolerability, and efficacy of DFT383 in participants aged 2 to ≤ 5 years with nephropathic cystinosis. DFT383 is a cellular gene therapy.

This study includes an active arm (Cohort 1) of participants treated with study treatment DFT383 and a concurrent reference arm (Cohort 0) treated with Standard of care (SoC). The study is not randomized and Cohort 0 aims to collect prospective and concurrent data in this rare disease. This study is an open-label, multi-center, phase I/II study to assess the safety, tolerability, and efficacy of DFT383 in participants aged 2 to 5 years with nephropathic cystinosis. The study consists of participants receiving DFT383 in Cohort 1 and Standard of Care (SoC) in Cohort 0. The two cohorts will be run in parallel. Investigational sites may participate in one or both cohorts.

Cohort 1 Approximately 15 participants will receive treatment with DFT383 in 3 cohorts (1A, 1B and 1C) dosed in a staggered approach. The total study duration for a participant in Cohort 1 will be up to 32 months.

Cohort 0 Approximately 15 participants meeting similar inclusion/exclusion criteria and receiving SoC will be enrolled. The Schedule of Activities will be reduced for this Cohort. This cohort 0 is not a direct control but will provide essential context for interpreting the results observed in the participants receiving DFT383. The total study duration for a participant in Cohort 0 will be up to 24 months.

Condition
Nephropathic Cystinosis
Phase
Phase1, Phase2
Overall Status
Recruiting
Number of Participants

30
Start Date
Jun 02, 2025
Completion Date
Aug 26, 2030
Gender
All
Age(s)
2 Years - 5 Years (Child)

Interventions

Genetic

DFT383

DFT383 is an autologous hematopoietic stem cell (HSC) gene therapy.

Eligibility Criteria

Key Inclusion Criteria:

Participants eligible for inclusion in this study must meet all the following criteria:

1. Informed consent in writing from parent(s) or legal guardian(s) must be provided
2. 2 to 5 years of age (including 5 years and 364 days old) at Screening
3. Weight-for-stature is ≥ the third percentile is ≥ 10 kg
4. Oral cysteamine therapy for at least 6 months
5. Historic clinical diagnosis of nephropathic cystinosis
6. Laboratory evidence of of renal fanconi syndrome (RFS)
7. Preserved kidney function (eGFR ≥ 90mL/min/1.73m2)
8. Received all age-appropriate vaccinations

Key exclusion Criteria for Cohort 1 and 0

1. A history of kidney transplantation
2. A prior or planned bone marrow or stem cell transplantation or prior treatment with gene therapy
3. History of malignancy
4. A severe or uncontrolled medical disorder
5. Major surgery within 90 days

Additional Key exclusion criteria for Cohort 1 - The following exclusion criteria only apply to Cohort 1 only as they are important for procedures related to DFT383 treatment:

1. Indomethacin within 2 weeks prior to Screening

Other protocol-defined inclusion/exclusion criteria may apply.

United States

Emory University School of Medicine / Children's Healthcare of Atlanta(recuiting Cohort 0)

Recruiting

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Worldwide Contacts

If the location of your choosing does not feature any contact detail, please reach out using the information below.

Novartis Pharmaceuticals

Phone: 1-888-669-6682
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Source URL: *https://prod1.novartis.com/clinicaltrials/study/nct06910813*

List of links present in page

1. https://clinicaltrials.gov/ct2/show/NCT06910813
2. #trial-eligibility
3. tel:404-712-6374
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