Fulcrum Therapeutics

An introduction to Fulcrum and overview of how new treatments are developed

- Fulcrum Therapeutics was founded in 2015 in Cambridge, Massachusetts, US, with the aim of developing disease-modifying treatments that result in meaningful outcomes for patients, caregivers, families, and medical teams
- We listen to and learn from patients, parents, caregivers, and patient communities to develop programs, initiatives, and treatments
- Our goal is to develop a new sickle cell disease (SCD) treatment that improves signs and symptoms of SCD as well as quality of life for people living with SCD

How new treatments are developed

Data must be gathered to prove it is safe and effective for its intended use before it can be approved for sale

Laboratory studies

Study treatments are typically tested on animals before being given to people

This helps find a safe starting dose for Phase 1

Researchers assess potential safety concerns

Phase 1

Small numbers of people take part

Early information is gathered about the study treatment with focus on safety and unwanted effects

Participants can be healthy volunteers or have the condition being studied

Phase 2

More information is gathered about the study treatment in a larger study

How well it works

Unwanted effects it causes

The best amount (dose) to give

Phase 3

Focused on how well the treatment works and unwanted effects

Larger numbers of people take part

The Phase 1b PIONEER Study in SCD is Underway

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What is the PIONEER study?

Fulcrum Therapeutics is conducting the PIONEER study to find out more about how a study treatment may work and the side effects it may cause

What is the study treatment?

- Researchers are working to understand the effect of pociredir (poe sir' e dir) on the production of fetal hemoglobin (HbF) in the body
  - High HbF can lead to reduced symptoms in people living with SCD
- Pociredir is planned to be a pill that is swallowed and taken once every day
- Pociredir is not approved by the US Food and Drug Administration or any other regulatory agency
- It is not yet known if this investigational drug works, or if it will meet safety standards
- If you participate in this study with pociredir, your health may get better, worse, or not change at all

What will participants be asked to do?

- Eligible participants will be asked to come to the study site for treatment, tests, and procedures
- The total length of individual participation in the study will be 15 to 16 weeks
- Please speak with your study doctor for more information about the potential risks and discomforts you may experience by participating in this study

Who can take part?

- Adults 18 to 65 years old who have been diagnosed with SCD, have previous experience with hydroxyurea for at least 6 months at the highest dose possible and it has not been effective, or are unable to take hydroxyurea.

Available support

- Participants will receive the study treatment, tests, and procedures at no cost
- Daily compensation will be provided
- Fulcrum may be able to cover the costs of transportation, gas, meals, and hotels for patients and caregivers

Other criteria also apply for this clinical study
Our research team will help determine if the study is right for you

Where is the PIONEER study taking place?

The study will take place at different hospitals in the United States. You can find your nearest location on the study website or https://clinicaltrials.gov/study/NCT05169580

Scan this QR code to find out more