A Phase I/II Study of ITU512 in Healthy Participants and Patients With Sickle Cell Disease

Last Update: Mar 11, 2025
A Phase I/II Clinical Study to Investigate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics, and Efficacy of ITU512 in Healthy Participants and Patients With Sickle Cell Disease
ClinicalTrials.gov Identifier:
NCT06546670
Novartis Reference Number:CITU512A12101
See if you Pre-qualify
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

The purpose of this study is to evaluate the safety, tolerability, pharmacokinetics (PK), and preliminary food effect of ITU512 as well as the fetal hemoglobin (HbF)-inducing capacity of ITU512. This will be the first evaluation of the potential therapeutic effect of ITU512 in healthy participants and patients with sickle cell disease (SCD). This is a global, randomized, Phase I/II study to assess the safety, tolerability, pharmacokinetics (PK), pharmacodynamics (PD), and preliminary food effect of single-agent ITU512 in adult healthy participants, and safety, tolerability, PK, PD, and efficacy of ITU512 in adolescent and adult patients with sickle cell disease (SCD). The study consists of a first-in-human Phase I study (Part 1) in healthy participants, and a Phase II study (Part 2) in patients with SCD.

Part 1 will comprise of Part 1A, Part 1B, and Part 1C. Part 2 may include an extension part.

Condition
Sickle Cell Disease
Phase
Phase1, Phase2
Overall Status
Recruiting
Number of Participants
161
Start Date
Aug 15, 2024
Completion Date
Apr 18, 2029
Gender
All
Age(s)
12 Years - 55 Years (Child, Adult)

Interventions

Drug

ITU512

ITU512 is an investigational, oral, low molecular weight (LMW) compound.
Drug

Placebo

An inactive substance that looks like and is given the same way as ITU512. The effect(s) of ITU512 will be evaluated against the placebo. Placebos are designed as a control and to have no real effect.

Eligibility Criteria

Key Inclusion Criteria:

Part 1 (Healthy participants)

* Healthy male participants and female participants of non-childbearing potential between 18-55 years of age
* In good health as determined by the investigator's assessment of medical history, physical examination, vital signs, ECG, and laboratory tests
* Participants must weigh at least 50 kg at screening and first baseline (admission) and must have a body mass index (BMI) within the range of 18.0-32.0 kg/m2 inclusive.

Part 2 (Sickle Cell Disease)

- Male and female participants with a diagnosis of sickle cell disease

Key Exclusion Criteria:

Part 1 (Healthy participants)

* QTcF ≥ 450 msec (as a mean value of triplicates)
* History of arrhythmias
* History of significant illness which has not resolved within two (2) weeks prior to initial dosing
* Women of child-bearing potential (WOCBP)

Part 2 (Sickle Cell Disease)

* Current use of hydroxyurea/hydroxycarbamide (HU/HC)
* QTcF ≥ 450 msec (as a mean value of triplicates)
* History of arrhythmias

Other protocol-defined inclusion/exclusion criteria may apply.

United States

Quotient Sciences Sea View

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.

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