NDec* clinical development program

First Human Dose phase 1 SCD patients (n=25)

Objectives: Study to evaluate safety, PD, and efficacy parameters of Oral Decitabine and Tetrahydrouridine or Placebo in High Risk SCD EPI01-CP.001²
phase 1
Healthy male subjects
(n=16)

Objectives: Single-dose, Crossover study on the bioavailability and safety of three formulations of NDec EPI01-CP.002³
phase 1
Healthy subjects
(n=48)

Objectives: Study to evaluate the food and gender effects, and safety of three formulations of NDec Completed trial in partnership with EpiDestiny

Ongoing trial

ASCENT14

phase 2

SCD patients (n = 84)

Objectives: Multicenter, randomized, parallel-group, multiple-dose study to assess the safety and efficacy of NDec in adult patients with SCD

*NDec is an investigational, oral formulation of a combination of decitabine and tetrahydrouridine in development for the treatment of sickle cell disease

SCD, Sickle Cell Disease

¹NCT01685515, FHD: First Human Dose, Molokie R et al. PLoS Med 2017 Sep 7;14(9):e1002382. doi: 10.1371/journal.pmed.1002382. ²NCT05405114 ³NCT03828084, Saunthararajah Y. *et al*: Poster #29; presented at ASCAT (Annual SC and Thalassemia meeting), London Oct 20-22, 2022. ⁴NCT04086238, Andemarium B. *et al*: Poster # 2379; accepted/to be presented at ASH, New Orleans, LA Dec 10-13, 2022.



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Sickle Cell Disease

Etavopivat* clinical development program

FT-4202 SAD/MAD¹

phase 1 unteers &

Healthy Volunteers & SCD patients (n=130)

Objectives: Study to evaluate safety, tolerability & PK/PD of FT-4202 (Etavopivat)

HIBISCUS² phase 2/3

SCD patients (n = 344)

Objectives: Study to evaluate safety & efficacy of etavopivat v. placebo in adults and adolescents with SCD

Completed trial

Ongoing trial

GLADIOLUS³ phase 2

TD SCD, TD Thal, or non-TD Thal (n = 60)

Objectives: Single arm study in adults and adolescents to investigate transfusion requirements in TD cohorts and improvements in hemoglobin in non-TD Thal

SCD, Sickle Cell Disease; TD SCD, transfusion dependent sickle cell disease; TD Thal, transfusion dependent thalassemia; non-TD Thal, non-transfusion dependent thalassemia; TD, transfusion dependent

¹NCT03815695, Forsyth S, et al. Clin Pharmacol Drug Dev. 2022 May;11(5):654-665. doi: 10.1002/cpdd.1058. Epub 2022 Jan 12.. ²NCT04624659 ³NCT04987489



^{*}Etavopivat (FT-4202) is an investigational, oral, small molecule activator of erythrocyte pyruvate kinase (PKR) in development for the treatment of sickle cell disease (SCD) and other hemoglobinopathies