



# Study to Evaluate the Effect of Voxelotor Administered Orally to Patients With Sickle Cell Disease (GBT\_HOPE)

04/04/2025 15:12:43

## Main Information

**Primary registry identifying number**

LBCTR2019080217

**Protocol number**

GBT440-031

**MOH registration number**

2017/2/19436

**Study registered at the country of origin**

Yes

**Study registered at the country of origin: Specify**

**Type of registration**

Retrospective

**Type of registration: Justify**

Sponsor's request and registry was not available when study started

**Date of registration in national regulatory agency**

24/05/2017

**Primary sponsor**

Global Blood Therapeutics, Inc.

**Primary sponsor: Country of origin**

United States of America

**Date of registration in primary registry**

29/06/2020

**Date of registration in national regulatory agency**

24/05/2017

**Public title**

Study to Evaluate the Effect of Voxelotor Administered Orally to Patients With Sickle Cell Disease (GBT\_HOPE)

**Acronym**

**Scientific title**

A Phase 3, Double-blind, Randomized, Placebo-controlled, Multicenter Study of Voxelotor Administered Orally to Patients With Sickle Cell Disease

**Acronym**

**Brief summary of the study: English**

Study to Evaluate the Effect of Voxelotor Administered Orally to Patients With Sickle Cell Disease

**Brief summary of the study: Arabic**

المأخوذ عن طريق الفم عند المرضى الذين يعانون من مرض الخلايا المنجلية Voxelotor-دراسة لتقييم تأثير ال

**Health conditions/problem studied: Specify**

Sickle Cell Disease

**Interventions: Specify**

GBT440 (Voxelotor) tablets orally administered

**Key inclusion and exclusion criteria: Inclusion criteria**

- Male or female study participants with sickle cell disease
- Participant has had at least 1 episode of vaso-occlusive crisis (VOC) in the past 12 months.
- Age 12 to 65 years





- Hemoglobin (Hb)  $\geq 5.5$  and  $\leq 10.5$  g/dL during screening
- For participants taking hydroxyurea (HU), the dose of HU (mg/kg) must be stable for at least 3 months prior to signing the ICF

**Key inclusion and exclusion criteria: Gender**

Both

**Key inclusion and exclusion criteria: Specify gender**

**Key inclusion and exclusion criteria: Age minimum**

12

**Key inclusion and exclusion criteria: Age maximum**

65

**Key inclusion and exclusion criteria: Exclusion criteria**

- More than 10 VOCs within the past 12 months that required a hospital, emergency room or clinic visit
- Patients who are receiving regularly scheduled blood (RBC) transfusion therapy (also termed chronic, prophylactic, or preventive transfusion) or have received a RBC transfusion for any reason within 60 days of signing the ICF
- Hospitalized for sickle cell crisis or other vaso-occlusive event within 14 days of signing the ICF (i.e., a vaso-occlusive event cannot be within 14 days prior to signing the ICF)
- Hepatic dysfunction characterized by alanine aminotransferase (ALT)  $> 4 \times$  ULN
- Severe renal dysfunction (estimated glomerular filtration rate at the Screening visit; calculated by the central laboratory)  $< 30$  mL/min/1.73m<sup>2</sup> or on chronic dialysis

**Type of study**

Interventional

**Type of intervention**

Pharmaceutical

**Type of intervention: Specify type**

N/A

**Trial scope**

Therapy

**Trial scope: Specify scope**

N/A

**Study design: Allocation**

Randomized controlled trial

**Study design: Masking**

Blinded (masking used)

**Study design: Control**

Placebo

**Study phase**

3

**Study design: Purpose**

Treatment

**Study design: Specify purpose**

N/A

**Study design: Assignment**

Parallel

**Study design: Specify assignment**

N/A

**IMP has market authorization**

No

**IMP has market authorization: Specify**

**Name of IMP**

Voxelotor (previously GBT440)

**Year of authorization**

**Month of authorization**

**Type of IMP**

Others

**Pharmaceutical class**

Allosteric modulator of hemoglobin oxygen affinity

**Therapeutic indication**

Treatment of sickle cell disease

**Therapeutic benefit**



Voxelotor is an orally bioavailable HbS polymerization inhibitor that binds specifically to HbS with a 1:1 stoichiometry, and exhibits preferential partitioning to RBCs. By increasing Hb's affinity for oxygen, voxelotor inhibits HbS polymerization in a dose dependent manner that may improve deformability, decrease the viscosity of SCD blood, and ultimately increase blood flow in the microcirculation, thus improving net O2 delivery. Therefore, chronically modifying 20% to 30% of HbS with voxelotor in subjects with SCD is expected to deliver the clinical benefits of reducing HbS polymerization while improving O2 delivery to peripheral tissues.

**Study model**

N/A

**Study model: Explain model**

N/A

**Study model: Specify model**

N/A

**Time perspective**

N/A

**Time perspective: Explain time perspective**

N/A

**Time perspective: Specify perspective**

N/A

**Target follow-up duration****Target follow-up duration: Unit****Number of groups/cohorts****Biospecimen retention**

Samples without DNA

**Biospecimen description**

Samples not including DNA

**Target sample size**

13

**Actual enrollment target size**

7

**Date of first enrollment: Type**

Actual

**Date of first enrollment: Date**

14/09/2017

**Date of study closure: Type**

Actual

**Date of study closure: Date**

16/06/2020

**Recruitment status**

Complete

**Recruitment status: Specify**

Active, not recruiting

**Date of completion****IPD sharing statement plan**

No

**IPD sharing statement description**



No Plan of data sharing

**Additional data URL**

<https://clinicaltrials.gov/ct2/show/record/NCT03036813>

**Admin comments**

**Trial status**

Approved

## Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
International Clinical Trials Registry Platform WHO (EUCTR)	EUCTR2016-003370-40
Clinicaltrials.gov	NCT03036813

## Sources of Monetary or Material Support

Name
Global Blood Therapeutics Inc. USA

## Secondary Sponsors

No Sponsors



## Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Dan Rudin	171 Oyster Point Boulevard Suite 300 South San Francisco, CA 94080	United States of America	+1 650 534 2574	drudin@gbt.com	Global Blood Therapeutics Inc.
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Public	Dr. Miguel Abboud	American University of Beirut Medical Center	Lebanon	+961 1 350 000	ma56@aub.edu.lb	American University of Beirut Medical Center

## Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
American University of Beirut Medical Center	Dr. Miguel Abboud	Pediatric Hematology and Oncology	Approved
Rafik Hariri University Hospital	Dr. Adlette Inati	Pediatric Hematology and Oncology	Approved

## Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
American University of Beirut Medical Center	21/02/2019	Dr. Fuad Ziyadeh	irb@aub.edu.lb	+961 1 350000 ext 5445
Rafic Hariri University Hospital	31/01/2018	Dr.lyad Issa	NA	+961 1 830000



## Countries of Recruitment

Name
Lebanon
United States of America
United Kingdom
Netherlands
Canada
France
Egypt
Italy
Turkey
Jamaica
Oman
Kenya

## Health Conditions or Problems Studied

Condition	Code	Keyword
Sickle cell disease	Sickle-cell disorders (D57)	Anemia, Sickle Cell, Hemolytic, Congenital, Hemoglobinopathies, Genetic Diseases, Inborn

## Interventions

Intervention	Description	Keyword
Drug	Voxelotor	Oral tablet

## Primary Outcomes

Name	Time Points	Measure
Change in hemoglobin (Hb)>1g/dl	Baseline to Week 24	Proportion of participants with increase in Hb >1 g/dL





## Key Secondary Outcomes

Name	Time Points	Measure
Change from baseline in hemolysis measures	Baseline to Week 24	Analyze hemoglobin, unconjugated bilirubin, absolute reticulocyte, reticulocytes %, and LDH
Annualized VOC incidence rate	Baseline to Week 72	Number of VOC events

## Trial Results

Summary results

Study results globally

Date of posting of results summaries

Date of first journal publication of results

Results URL link

Baseline characteristics

Participant flow

Adverse events

Outcome measures

URL to protocol files