



# Study to Assess the Effect of Long-term Treatment With GBT440 in Participants Who Have Completed Treatment in Study GBT440-031

22/01/2025 15:09:13

## Main Information

**Primary registry identifying number**

LBCTR2019080216

**Protocol number**

GBT440-034

**MOH registration number**

2018/2/30053

**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**

08/08/2018

**Primary sponsor**

Global Blood Therapeutics, Inc

**Primary sponsor: Country of origin**

United States of America

**Date of registration in primary registry**

13/10/2021

**Date of registration in national regulatory agency**

08/08/2018

**Public title**

Study to Assess the Effect of Long-term Treatment With GBT440 in Participants Who Have Completed Treatment in Study GBT440-031

**Acronym****Scientific title**

An Open Label Extension Study of GBT440 Administered Orally to Patients With Sickle Cell Disease Who Have Participated in GBT440 Clinical Trials

**Acronym****Brief summary of the study: English**

Study to Assess the Effect of Long-term Treatment With GBT440 in Participants Who Have Completed Treatment in Study GBT440-031

**Brief summary of the study: Arabic**

و أتموا العلاج GBT440-031 عند المرضى الذين شاركوا في الدراسة GBT440 دراسة لتقييم تأثير العلاج طويل الأمد مع

**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 who participated and received study treatment in Study GBT440-031. (Note: Participants in GBT440-031 who discontinued study drug due to an AE, but who remained on study may be eligible for treatment in this study provided the AE does not pose a risk for treatment with GBT440.)
- Females of child-bearing potential are required to have a negative urine pregnancy test prior to dosing on Day 1.
- Female participants of child-bearing potential must use highly effective methods of contraception to 30 days after the last dose of study drug.





Male participants must continue to use barrier methods of contraception to 30 days after the last dose of study drug.  
- Participant has provided written informed consent or assent.

**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**

99

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

- Female who is breast-feeding or pregnant.
- Participant withdrew consent from Study GBT440-031.
- Participant was lost to follow-up from Study GBT440-031.
- Participant requiring chronic dialysis.
- Any medical, psychological, safety, or behavioral conditions, which, in the opinion of the Investigator, may confound safety interpretation, interfere with compliance, or preclude informed consent.

**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**

Open (masking not used)

**Study design: Control**

Active

**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**

None retained

**Biospecimen description**

N/A

**Target sample size**

6

**Actual enrollment target size**

6

**Date of first enrollment: Type**

Actual

**Date of first enrollment: Date**

13/02/2019

**Date of study closure: Type**

Actual

**Date of study closure: Date**

31/12/2024

**Recruitment status**

Other

**Recruitment status: Specify**

Enrolling by invitation

**Date of completion**

**IPD sharing statement plan**

No

**IPD sharing statement description**



N/A

**Additional data URL**

<https://clinicaltrials.gov/ct2/show/record/NCT03573882>

**Admin comments**

**Trial status**

Approved

## Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
ClinicalTrials.gov	NCT03573882
EU Clinical Trials Register	EudraCT: 2017-004045-25

## 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	Dr. Adlette Inati	Tripoli	Lebanon	9613228033	adlette.inati@lau.edu.lb	Nini Hospital
Scientific	Margaret Tonda	171 Oyster Point Boulevard, Suite 300 South San Francisco, CA 94080	United States of America	650-741-7761	mtonda@gbt.com	Global Blood Therapeutics Inc.
Public	Dr. Miguel Abboud	Beirut	Lebanon	9611350000	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
Nini 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	07/02/2019	Dr. Fuad Ziyadeh	irb@aub.edu.lb	9611350000 ext 5445
Nini Hospital	29/10/2018	Dr. Nabil Kabbara	n/a	9616431400 ext 1061

## Countries of Recruitment

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



## 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	Volexotor (GBT440) 300 mg Oral tablet	Open Label Extension, Anemia, Sickle Cell , Hemolytic, Congenital

## Primary Outcomes

Name	Time Points	Measure
Hemolysis Markers	5 years	total bilirubin, LDH and reticulocyte lab values
Frequency of sickle cell-related complications	5 years	Frequency of SCD-related complications with long-term dosing with GBT440

## Key Secondary Outcomes

Name	Time Points	Measure
Number of participants with treatment-related adverse events as assessed by CTCAE v4.0	5 years	Safety based on adverse event assessed by CTCAE (Common Terminology Criteria for Adverse 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**