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

04/04/2025 01:40:23

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

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	96113500000	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