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

14/12/2025 05:55:07

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
17/07/2020

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
دراسة لتقييم تأثير الVoxelotor المأخوذ عن طريق الفم عند المرضى الذين يعانون من مرض الخلايا المنجلية

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) ≥5.5 and ≤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 x ULN
- Severe renal dysfunction (estimated glomerular filtration rate at the Screening visit; calculated by the central laboratory) <30mL/min/1.73m2 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.
Scientific	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.
Public	Dr. Adlette Inati	Rafik Hariri University Hospital	Lebanon	+ 961 1 830000	adlette.inati@lau.edu.lb	Rafik Hariri University Hospital
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.Iyad 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