Pediatric Open-Label Extension of Voxelotor

20/08/2025 12:58:01

Main Information

Primary registry identifying number
LBCTR2020063513

Protocol number
GBT440-038

MOH registration number

Study registered at the country of origin
Yes

Study registered at the country of origin: Specify

Type of registration
Prospective

Type of registration: Justify
N/A

Date of registration in national regulatory agency

Primary sponsor
Global Blood Therapeutics Inc.

Primary sponsor: Country of origin
United States of America

Date of registration in primary registry
14/08/2020

Date of registration in national regulatory agency

Public title
Pediatric Open-Label Extension of Voxelotor

Acronym

Scientific title
An Open-Label Extension Study of Voxelotor Administered Orally to Pediatric Participants With Sickle Cell Disease Who Have Participated in Voxelotor Clinical Trials

Acronym

Brief summary of the study: English
Open-label extension study of voxelotor for pediatric participants ages 4 to 18 years old with Sickle Cell Disease who have participated in voxelotor clinical trials.
Open-label extension (OLE) study of voxelotor for pediatric participants with Sickle Cell Disease who have participated in voxelotor clinical trials. Approximately 50 participants with sickle cell disease (SCD), aged ≥ 4 to ≤ 18 years will be enrolled at approximately 19 global clinical sites. All participants will receive voxelotor once daily, administered orally as tablets, dispersible tablets, or powder for oral suspension formulation. The objective of this OLE is to assess the safety of, and SCD-related complications of, long-term treatment with voxelotor, in pediatric participants who have completed treatment in a Global Blood Therapeutics (GBT)-sponsored voxelotor pediatric clinical study.

Brief summary of the study: Arabic
دراسة تكميلية على voxelotor للمشاركين الأطفال الذين تتراوح أعمارهم بين 4 و 18 عامًا المصابين بمرض الخلايا المنجلية و الذين شاركوا في التجارب السريرية السابقة على مستحضر voxelotor

Health conditions/problem studied: Specify
Sickle Cell Disease

Interventions: Specify
Drug: Voxelotor (GBT440)

All participants will receive voxelotor once daily (QD), administered orally as tablets, dispersible tablets, or a powder for oral suspension formulation

Key inclusion and exclusion criteria: Inclusion criteria

1. Male or female participant with SCD, aged ≥ 4 to ≤ 18 years, who participated and received study drug in a GBT-sponsored voxelotor pediatric clinical study
Note: Participants 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 voxelotor.
2. Female participants of childbearing potential are required to have a negative urine pregnancy test before dosing on Day 1.
Note: Female participants who become childbearing during the study must be willing to have a negative urine pregnancy test to remain in the study.
3. If sexually active, female participants of childbearing potential must use highly effective methods of contraception until 30 days after the last dose of study drug. If sexually active, male participants must use barrier methods of contraception until 30 days after the last dose of study drug.
4. Participant has provided written assent (both the consent of the participant's legal representative or legal guardian and the participant's assent
[where applicable] must be obtained)

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

4

Key inclusion and exclusion criteria: Age maximum

18

Key inclusion and exclusion criteria: Exclusion criteria

1. Female participant who is breastfeeding or pregnant
2. Participant withdrew consent from a GBT-sponsored voxelotor pediatric clinical study
3. Participant was lost to follow-up from a GBT-sponsored voxelotor pediatric clinical study
4. Participant has any medical, psychological, safety, or behavioral conditions that, 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

N/A

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

Single

Study design: Specify assignment

N/A

IMP has market authorization

Yes, Worldwide

IMP has market authorization: Specify

United States of America

Name of IMP

Voxelotor (Oxbryta)

Year of authorization

2019

Month of authorization

11

Type of IMP

Others

Pharmaceutical class

Allosteric modulator of hemoglobin oxygen affinity

Therapeutic indication

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

24

Actual enrollment target size

Date of first enrollment: Type

Anticipated

Date of first enrollment: Date

11/08/2020

Date of study closure: Type

Anticipated

Date of study closure: Date

30/06/2026

Recruitment status

Pending

Recruitment status: Specify

Date of completion

IPD sharing statement plan
No

IPD sharing statement description
N/A

Additional data URL

Admin comments

Trial status
Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
Clinicaltrials.gov	NCT04188509
WHO International Clinical Trials Registry Platform	EUCTR2019-003144-76-GB

Sources of Monetary or Material Support

Name
Global Blood Therapeutics Inc. USA

Secondary Sponsors

Name
N/A

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Dr. Miguel Abboud	Beirut	Lebanon	9611350000	ma56@aub.edu.lb	American University of Beirut Medical Center
Scientific	Margaret Tonda	181 Oyster Point Blvd. South San Francisco, CA 94080	United States of America	650 741 7761	mtonda@gbt.com	Global Blood Therapeutics Inc.
Public	Dr. Adlette Inati	Tripoli	Lebanon	9613228033	adlette.inati@lau.edu.lb	Nini Hospital

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	Hematology	Not approved
Nini Hospital	Dr. Adlette Inati	Hematology	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Nini Hospital	15/06/2020	Dr. Nabil Kabbara	N/A	9616431400

Countries of Recruitment

Name
United States of America
United Kingdom
Lebanon

Health Conditions or Problems Studied

Condition	Code	Keyword
Sickle-Cell Disorder	Sickle-cell disorders (D57)	Hematology, Sickle Cell, Disorder

Interventions

Intervention	Description	Keyword
Drug	Voxelotor	GBT440

Primary Outcomes

Name	Time Points	Measure
Treatment Emergent Adverse Events and Serious Adverse Events	Throughout entire study	N/A
Sickle Cell Disease-Related Complications	Throughout entire study	Frequency of SCD-related complications

Key Secondary Outcomes

Name	Time Points	Measure
N/A	N/A	N/A

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