



# Pediatric Open-Label Extension of Voxelotor

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## 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  $\geq 4$  to  $\leq 18$  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**

عامًا المصابين بمرض الخلايا المنجلية و الذين شاركوا 18 و 4 للمشاركين الأطفال الذين تتراوح أعمارهم بين voxelotor دراسة تكميلية على voxelotor في التجارب السريرية السابقة على مستحضر

**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  $\geq 4$  to  $\leq 18$  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**