REPUBLIC OF LEBANON MINISTRY OF PUBLIC HEALTH Lebanon Clinical Trials Registry

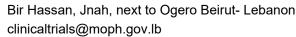
Open-Label Extension of Voxelotor

12/08/2025 01:04:46 Main Information Primary registry identifying number Protocol number LBCTR2020063513 GBT440-038 MOH registration number Study registered at the country of origin Study registered at the country of origin: Specify Yes Type of registration Type of registration: Justify Prospective N/A Date of registration in national regulatory agency 25/06/2020 **Primary sponsor** Primary sponsor: Country of origin United States of America Global Blood Therapeutics Inc. Date of registration in primary registry Date of registration in national regulatory agency 26/10/2022 25/06/2020 **Public title** Acronym Open-Label Extension of Voxelotor Scientific title Acronym An Open-Label Extension Study of Voxelotor Administered Orally to Participants with Sickle Cell Disease Who Have Participated in Voxelotor Clinical Trials Brief summary of the study: English Open-label extension (OLE) study of Voxelotor for participants with Sickle Cell Disease who have participated in Voxelotor clinical trials. Approximately 600 participants with sickle cell disease (SCD), aged ≥ 4 to >18year years will be enrolled at approximately 70 global clinical sites. Participants aged ≥ 12 years will receive a voxelotor dose of 1500 mg QD, regardless of their body weight. Participants aged < 12 years will receive a voxelotor dose based on their body weight, to provide exposure corresponding to the adult dose of 1500 mg QD. The participant's weight at study entry will be used to determine the starting voxelotor dose in this study. The dose should be adjusted if the participant's weight increases or decreases at a scheduled clinic visit. The objective of this OLE is to assess the safety of, and SCD-related complications of, long-term treatment with Voxelotor, in participants who have completed treatment in a Global Blood Therapeutics (GBT)-sponsored Voxelotor clinical study. Brief summary of the study: Arabic

أعوام و المصابين بمرض الخلايا المنجلية و الذين شاركوا في التجارب السريرية لمالمشاركين ابتداءاً من عمر voxelotor دراسة تكميلية على السابقة على مستحضر

Health conditions/problem studied: Specify

Sickle Cell Disease





REPUBLIC OF LEBANON MINISTRY OF PUBLIC HEALTH Lebanon Clinical Trials Registry

Interventions: Specify

Drug: Voxelotor (GBT440)

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

Key inclusion and exclusion criteria: Inclusion criteria

1. Male or female participant with SCD, aged ≥ 4 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.

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age maximum

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)

5. Subjects with abnormal TCD who have not completed Study GBT440-032 can participate in OLE study

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Age minimum

4

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

5. Based on the most recent Oxbryta® US label (December 2021), co-administration with both moderate and strong CYP3A4 inducers should be avoided

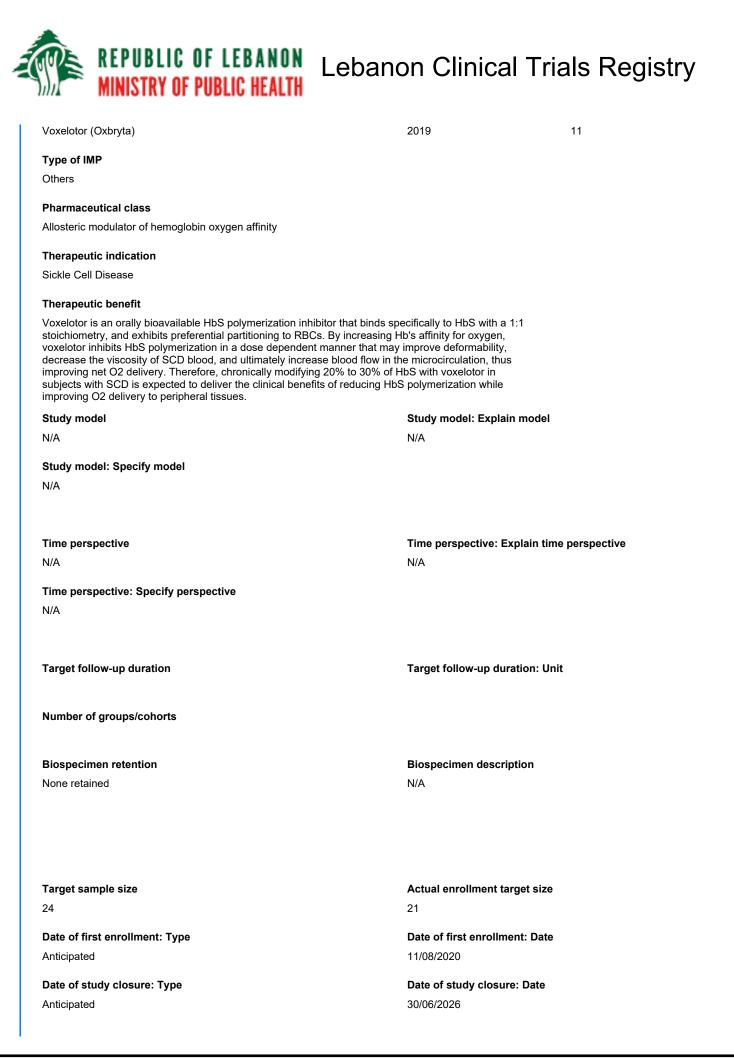
99

Type of study

Interventional

Type of intervention	Type of intervention: Specify type	
Pharmaceutical	N/A	
Trial scope	Trial scope: Specify scope	
Therapy	N/A	
Study design: Allocation	Study design: Masking	
N/A	Open (masking not used)	
Study design: Control	Study phase	
Active	3	
Study design: Purpose	Study design: Specify purpose	
Treatment	N/A	
Study design: Assignment	Study design: Specify assignment	
Single	N/A	
IMP has market authorization	IMP has market authorization: Specify	
Yes, Worldwide	United States of America	
Name of IMP	Year of authorization Month of authorization	





 \sim



Recruitment status	Recruitment status: Specify
Pending	
Date of completion	
IPD sharing statement plan	IPD sharing statement description
No	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	

	E
\sim	6



Contac	Contact for Public/Scientific Queries					
Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Dr. Miguel Abboud	Beirut	Lebanon	961135000 0	ma56@aub.edu.l b	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.co m	Global Blood Therapeuti cs Inc.
Public	Dr. Adlette Inati	Tripoli	Lebanon	961322803 3	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 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
Brazil
Egypt
Oman
Kenya
Nigeria
Ghana

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	