Study to Evaluate the Effect of GBT440 in Pediatrics With Sickle Cell Disease

12/08/2025 23:51:29

Main Information

Primary registry identifying number
LBCTR2019090195

Protocol number
GBT440-007

MOH registration number
1288/ص

Study registered at the country of origin
Yes

Study registered at the country of origin: Specify

Type of registration
Retrospective

Type of registration: Justify
Requested by Sponsor- Registry not in place upon study initiation

Date of registration in national regulatory agency
11/06/2014

Primary sponsor
Global Blood Therapeutics Inc.

Primary sponsor: Country of origin
United States of America

Date of registration in primary registry
30/09/2019

Date of registration in national regulatory agency
11/06/2014

Public title
Study to Evaluate the Effect of GBT440 in Pediatrics With Sickle Cell Disease

Acronym

Scientific title
A Phase 2a, Open-label, Single and Multiple Dose Study to Evaluate the Pharmacokinetics, Safety, Tolerability, and Exploratory Treatment Effect of GBT440 in Pediatric Participants With Sickle Cell Disease

Acronym

Brief summary of the study: English
This study consists of three parts, Parts A, B, and C.
- Part A is a single dose PK study in pediatric participants with Sickle Cell Disease. (Closed on 07 Aug 2017 (LPLV))
- Part B is a multiple dose, safety, exploratory, efficacy, and PK study in adolescent Sickle Cell Disease participants who were 12-17 years of age (Closed on 04 Jan 2019 (LPLV))
- Part C is a multiple dose, safety, tolerability, and PK study, which includes the assessment of hematological effects and the effect on TCD flow velocity of GBT440 is pediatric participants with Sickle Cell Disease who are 4 to 17 years of age.

Brief summary of the study: Arabic
دراسة لتقييم تأثير GBT440 عند الأطفال المصابين بمرض الخلايا المنجلية

Health conditions/problem studied: Specify
Sickle Cell Disease

Interventions: Specify
Drug: GBT440 administered as oral capsules, tablets or dispersible tablets

Key inclusion and exclusion criteria: Inclusion criteria

1. Male or female participants with homozygous hemoglobin SS (HbSS) or hemoglobin S beta0 thalassemia (HbS β0 thal).
2. Age:
• Part A – 6 to 17 years of age. (Cohort 1 [12 to 17] and Cohort 2 [6 to 11] as defined in the Study Design)
• Part B – 12 to 17 years of age
• Part C – 4 to 17 years of age
3. A participant taking hydroxyurea (HU) may be enrolled if the dose has been stable for at least 3 months with no anticipated need for dose adjustments during the study and no sign of hematological toxicity.
4. Hemoglobin (Hb):
• Part A – No restriction
• Part B – Hb ≤10.5 g/dL
• Part C – Hb ≤10.5 g/dL
5. Written informed parental/guardian consent and participant assent has been obtained per institutional review board (IRB)/Ethics Committee (EC) policy and requirements, consistent with ICH guidelines.

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

17

Key inclusion and exclusion criteria: Exclusion criteria

1. Any one of the following requiring medical attention within 14 days prior to signing the Informed Consent Form (ICF):
• Vaso-occlusive crisis (VOC)
• Acute chest syndrome (ACS)
• Splenic sequestration crisis
• Dactylitis
2. Requires chronic transfusion therapy
3. History of stroke or meeting criteria for primary stroke prophylaxis (history of two TCD measurements ≥200 cm/sec)
4. Transfusion within 30 days prior to signing the ICF

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: Single arm study

Study design: Masking

Open (masking not used)

Study design: Control

Dose comparison

Study phase

2

Study design: Purpose

Treatment

Study design: Specify purpose

N/A

Study design: Assignment

Single

Study design: Specify assignment

N/A

IMP has market authorization

No

IMP has market authorization: Specify

Name of IMP

GBT440 (Voxelotor)

Year of authorization

Month of authorization

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

32

Date of first enrollment: Type

Actual

Date of first enrollment: Date

21/07/2016

Date of study closure: Type

Actual

Date of study closure: Date

31/01/2021

Recruitment status

Recruiting

Recruitment status: Specify

Date of completion

IPD sharing statement plan
No

IPD sharing statement description
N/A

Additional data URL
https://clinicaltrials.gov/ct2/show/NCT02850406

Admin comments

Trial status
Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
Clinicaltrials.gov	NCT02850406
EU Clinical Trials Registry	EudraCT: 2016-004209-15

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	Dan Rudin	171 Oyster Point Blvd., Suite 300 South San Francisco, CA 94080	United States of America	(650) 534-2574	drudin@gbt.com	Global Blood Therapeutics
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-Oncology	Approved
Rafik Hariri University Hospital	Dr. Adlette Inati	Pediatric Hematology-Oncology	Approved
Nini Hospital	Dr. Adlette Inati	Pediatric Hematology-Oncology	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
American University of Beirut Medical Center	09/07/2018	Dr. Fuad Ziyadeh	irb@aub.edu.lb	9611738025
Rafic Hariri University Hospital	31/08/2018	Dr. Iyad Issa	NA	9611830000
Nini Hospital	31/08/2018	Dr. Nabil Kabbara	NA	9616431400

Countries of Recruitment

Name
Lebanon
United States of America
United Kingdom

Health Conditions or Problems Studied

Condition	Code	Keyword
Sickle Cell Disease	Sickle-cell disorders (D57)	Sickle Cell, Anemia, Hemolytic, Congenital, Hematologic Diseases

Interventions

Intervention	Description	Keyword
Drug	GBT440	Oral Capsule, Tablet or Dispersible Tablet

Primary Outcomes

Name	Time Points	Measure
Part A: Pharmacokinetic profile of GBT440 including maximum concentration	Pre-dose to Day 15	Pharmacokinetic profile
Part A: Pharmacokinetic profile of GBT440 including the time taken to reach the maximum concentration	Pre-dose to Day 15	Pharmacokinetic profile
Part A: Pharmacokinetic profile of GBT440 including the total drug concentration over time	Pre-dose to Day 15	Pharmacokinetic profile
Part B: Change in hemoglobin	Baseline to Week 24	Hemoglobin in Blood
Part C: Change in cerebral blood flow	Baseline to Week 48	TAMM TCD velocity

Key Secondary Outcomes

Name	Time Points	Measure
Part A: Number of participants with treatment-related adverse events	Days 1 - 15	Assessed by CTCAE v4.03
Part B: Multiple Dose effect on Clinical Measures of Hemolysis	Day 1 - Week 24	Clinical Measures of Hemolysis
Part B: Pharmacokinetic profile of GBT440 including maximum concentration	Pre-dose to Week 24	Pharmacokinetic profile
Part B: Pharmacokinetic profile of GBT440 including the time taken to reach the maximum concentration	Pre-dose to Week 24	Pharmacokinetic profile
Part B: Pharmacokinetic profile of GBT440 including the total drug concentration over time	Pre-dose to Week 24	Pharmacokinetic profile
Part C: Multiple dose effect on clinical measures of hemolysis	Baseline to Week 24 and Week 48	Clinical Measures of Hemolysis
Part C: Change in cerebral blood flow	Baseline to Week 24	Measured by the TAMM TCD velocity
Part C: Pharmacokinetic profile of GBT440 including maximum concentration	Pre-Dose to Week 48	Pharmacokinetic profile
Part C: Pharmacokinetic profile of GBT440 including the time taken to reach the maximum concentration	Pre-Dose to Week 48	Pharmacokinetic profile
Part C: Pharmacokinetic profile of GBT440 including the total drug concentration over time	Pre-Dose to Week 48	Pharmacokinetic profile

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