A study to evaluate if VIT-2763 may be beneficial in the treatment of Nontransfusion Dependent Beta-thalassaemia.

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Main Information

Primary registry identifying number

LBCTR2020021295

Protocol number

VIT-2763-THAL-201

MOH registration number

Study registered at the country of origin

No

Study registered at the country of origin: Specify

No.Prevalence of the disease is low in the country of origin Switzerland

Type of registration

Prospective

Type of registration: Justify

N/A

Date of registration in national regulatory agency

Primary sponsor

Vifor (International) Inc.

Primary sponsor: Country of origin

Switzerland

Date of registration in primary registry

02/04/2020

Date of registration in national regulatory agency

Public title

A study to evaluate if VIT-2763 may be beneficial in the treatment of Nontransfusion Dependent Beta-thalassaemia.

Acronym

Scientific title

A Phase 2a, Double-blind, Randomised, Placebo-controlled, Parallel Group, Multicentre Study on Safety, Tolerability, Pharmacokinetics, Pharmacodynamics and Preliminary Efficacy of Multiple Doses of VIT-2763 in Subjects with Non-transfusion Dependent Beta-thalassaemia

Acronym

Brief summary of the study: English

This is a Phase 2a, Double-blind, Randomised, Placebo-controlled, Parallel Group,Multicentre Study. The primary objective is to assess the safety and tolerability of VIT-2763 versus placebo in adult and adolescent NTDT subjects over a 12-week treatment period.The secondary objectives are to assess Pharmacokinetics, Pharmacodynamics and Preliminary Efficacy of Multiple Doses of VIT-2763.

Brief summary of the study: Arabic

أ ، مزدوجة التعمية ، والعشوائية ، بالمقارنة مع دواء وهمي ، المجموعة الموازية ، دراسة متعددة المراكز . الهدف2هذه الدراسة في المرحلة للبالغين والمراهقين على مدى فترة علاج مدتها NTDT مقابل الدواء الوهمي في موضوعات VIT-2763 الأساسي هو تقييم سلامة التحمل و أسبوعًا. الأهداف الثانوية هي تقييم الحرائك الدوائية والديناميكا الدوائية والفعالية الأولية لجرعات متعددة من 12 VIT-276

Health conditions/problem studied: Specify

Chronic anemia due to ineffective erythropoiesis (IE) in subjects with β-thalassaemia

Interventions: Specify

The study will commence with enrolment and treatment of adult NTDT subjects(Cohort I). Adult subjects will be randomised in an 8:8:4 ratio to receive either VIT-2763 once daily (QD) or twice daily (BID) or placebo, at a dose of 120 mg for subjects with a body weight ≥60 kg or at dose of 60 mg for subjects with a body weight <60 kg.Following Cohort I review, enrolment of adolescent NTDT subjects into Cohort II. Adolescent

subjects will be randomised in a 4:4:2 ratio to receive either VIT-2763 QD or BID or placebo, at a dose of 120 mg for subjects with a body weight ≥60 kg or at a dose of 60 mg for subjects with a body weight <60 kg

Key inclusion and exclusion criteria: Inclusion criteria

1.Documented diagnosis of NTDT, including a β-thalassaemia intermedia-phenotype.

2.NTDT is defined as subjects having received <5 units of red blood cells (RBCs) during the 24-week period prior to randomisation/first drug administration of VIT-2763 or placebo (Day 1; 1 unit is defined as 200 to 350 ml of transfused packed RBCs and last RBC transfusion must have been received ≥14 days prior to randomisation).

Note: Subjects who are supposed to receive RBC transfusions after randomisation in the Investigator's opinion, and according to local practise, and having received at least 1 dose of VIT-2763, may be considered to stay on study treatment for safety reasons, and in case there are no tolerability concerns. Subjects will be censored for secondary efficacy.

3.Male and female adult NTDT subjects, 18-65 years of age inclusive (Cohort I only) at time of screening.

4.Male and female adolescent NTDT subjects, 12-17 years of age inclusive (Cohort II only) at time of screening.

5.Subjects must have a mean baseline Hb ≤11 g/dl, based on 2 consecutive measurements ≥1 week apart within 6 weeks prior to randomisation/baseline, and obtained Hb values show less than 10% relative difference (and equal or less than 1.0 g/dl absolute change between the highest and lowest value) between at least 2 measurements.

Note: If there is 1 retrospective Hb value available for the subject at maximum of 2 weeks prior to screening (Day -28), the Hb value can be taken into consideration. A subject not meeting this criterion would be excluded but can be rescreened at maximum 2 times at a later time point.

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

1.Documented diagnosis of transfusion dependent thalassaemia (TDT), including a beta-thalassaemia major phenotype (including β0/β0, β+/β+, β0/β+ genotype), and mixed compound heterozygous for sickling phenotype variants such as Hb S/β-thalassaemia, or transfusion dependent non-deletional Hb H disease (i.e., Hb constant spring) or Hb C disease.

2.Subjects on concomitant iron chelation therapy (ICT) or subjects on prior ICT when discontinued less than 4 weeks prior randomisation. Note: If ICT was discontinued ≥4 weeks prior randomisation the subject is eligible.

3.ICT naïve subjects with serum ferritin <150 ng/ml and documented LIC ≤1 mg/g liver dry weight assessed through MRI, or subjects on prior ICT with serum ferritin <300 ng/ml and documented LIC <3 mg/g liver dry weight assessed through MRI.
4.Subjects with TSAT <30%.

5.Subjects with documented LIC >15 mg/g liver dry weight assessed through MRI, or a documented myocardial T2-star (T2*) <20 ms.

6.Adult or adolescent subjects with body weight <40.0 kg or >100 kg at screening and/or randomisation.

7.Chronic liver disease and/or alanine transaminase (ALT), aspartate transaminase (AST) or gamma-glutamyl transpeptidase (GGT) above 3-fold the upper limit of normal (ULN) range at screening.
Note: A subject fulfilling this criterion will be excluded but can be rescreened
at a later time point (in order to fulfil eligibility, ≥2 values within ≥1 week
should be assessed and be within eligibility limits).

8.Estimated glomerular filtration rate (eGFR) <30 ml/min/1.73 m2 (according to chronic kidney disease classification Stage 4 or higher), and/or significant albuminuria >30 mg/mmol. eGFR should be estimated according to Cockcroft-Gault.

9.Newly diagnosed folate deficiency anaemia and/or Vitamin B12 megaloblastic anaemia. Subjects with known folate deficiency anaemia and/or Vitamin B12 megaloblastic anaemia who are on ≥12 weeks stable replacement therapy are eligible.
Note: A subject fulfilling this criterion will be excluded but can be rescreened
at a later time point.

10.Any history or clinically important finding of cardiac disorders, such as clinically relevant cardiac arrhythmia, cardiomyopathy, coronary disease, valve disorder, or heart failure according to New York Heart Association classification 3-4.

11.Subjects with partial or total splenectomy.

Type of study

Interventional

Type of intervention
Pharmaceutical

Type of intervention: Specify type
N/A

Trial scope
Other

Trial scope: Specify scope

Study design: Allocation
Randomized controlled trial

Study design: Masking
Blinded (masking used)

Study design: Control
Placebo

Study phase
2

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
VIT-2763

Year of authorization

Month of authorization

Type of IMP
Others

Pharmaceutical class
VIT-2763 is a Ferroportin (FPN) inhibitor and hepcidin-mimetic.

Therapeutic indication
Iron loading anaemias and thalassaemia

Therapeutic benefit
Administration of VIT-2763 may results in improvement of anaemia and amelioration of ineffective erythropoiesis in NTD beta-thalassemia patients, as it was already shown in nonclinical disease models. This improvement in ineffective erythropoiesis may result in a clinical benefit for NTD β-thalassemia subjects, by improving the symptomatology of the chronic anemia and the complications of the extramedullary hematopoiesis.

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

Urine will be taken for urinalysis (pH, protein, glucose, ketone, blood, spot urine for assessment of protein/creatinine and albumin/creatinine ratio) and urine drug and alcohol screen. blood samples for haematology and clinical chemistry and coagulation

Target sample size

36

Actual enrollment target size

Date of first enrollment: Type

Anticipated

Date of first enrollment: Date

11/05/2020

Date of study closure: Type

Anticipated

Date of study closure: Date

11/05/2021

Recruitment status

Pending

Recruitment status: Specify

Date of completion

IPD sharing statement plan

No

IPD sharing statement description

Not applicable

Additional data URL

none

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
EMA	2019-002221-29

Sources of Monetary or Material Support

Name
Vifor (International) Inc.

Secondary Sponsors

Name
Not Applicable

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Aziz Zoghbi	MCT-CRO, Berytech Technology and Health, 5th Floor Damascus Road, Beirut, Lebanon	Lebanon	009611612500	zog_az@mctcro.com	Regional Manager
Scientific	Ali Taher	Chronic Care Center, Hazmieh, Lebanon	Lebanon	009613755669	ataher@aub.edu.lb	PI

Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Chronic Care Center	Dr.Ali Taher	Professor of Medicine, Hematology & Oncology	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Chronic Care Center	30/01/2020	Michelle Abi Saad	cccmas@chroniccare.org.lb	05-455101

Countries of Recruitment

Name
Lebanon

Health Conditions or Problems Studied

Condition	Code	Keyword
NTDT Thalassemia	Thalassaemia (D56)	thalassemia

Interventions

Intervention	Description	Keyword
VIT-2763 60 mg capsules	Adult subjects will be randomised in an 8:8:4 ratio to receive either VIT-2763 QD or BID or placebo at a dose of 120 mg for subjects with a body weight ≥60 kg or at a dose of 60 mg for subjects with a body weight <60 kg.	cohort 1/2

Primary Outcomes

Name	Time Points	Measure
Reported or observed adverse events (AEs)	last study contact Visit 9/Week 16.	by SOC and PT MedDRA coded term, by severity and relation to study product in each treatment group.
Reported or observed serious adverse events (SAEs)	4 weeks (28+-4 days) following the last study drug administration.	by SOC and PT MedDRA coded term, by severity and relation to study product in each treatment group
Changes in vital signs	screening Visit V1 and on Visits V3 to V8. Vital signs should be performed at V3 to V8 before IMP dosing, after a resting period of at least 5 minutes.	Blood pressure and pulse rate
Changes in clinical laboratory safety tests	over 12 week treatment	haematology, serum biochemistry, coagulation, and urinalysis
12-Lead ECG	over 12 week treatment	ventricular rate, PR interval, QRS duration, QT interval and QTcF
Physical examination	Screening Visit V1 (i.e., Day -28 to -1) and on Visit V3 (Day 1), and V8 (Day 84)	general appearance, head (eyes, ears, nose and throat), cardiovascular, respiratory, abdominal, musculoskeletal, neurological, lymph nodes, and skin.

Key Secondary Outcomes

Name	Time Points	Measure
Assessment of iron parameters	from baseline over a 12-week period	total serum iron, serum ferritin, serum transferrin, calculated transferrin saturation (TSAT
PK parameters	from pre-dose trough to 3 hours or 4 hours post-dose at selected study visits	Cmax, clearance, distribution volume, area under the curve (AUC)

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