An Open Label Extension Study of PTG-300 in Non-Transfusion Dependent (NTD) and Transfusion-Dependent (TD) β-Thalassemia Subjects

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Main Information

Primary registry identifying number
LBCTR2019070220

Protocol number
PTG300-03

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
19/06/2019

Primary sponsor
Protagonist Therapeutics Inc

Primary sponsor: Country of origin
USA

Date of registration in primary registry
08/07/2019

Date of registration in national regulatory agency
19/06/2019

Public title
An Open Label Extension Study of PTG-300 in Non-Transfusion Dependent (NTD) and Transfusion-Dependent (TD) β-Thalassemia Subjects

Acronym

Scientific title
An Open Label Extension Study of PTG-300 in Non-Transfusion Dependent (NTD) and Transfusion-Dependent (TD) β-Thalassemia Subjects

Acronym

Brief summary of the study: English

This is an open-label, long term extension study for subjects completing study PTG-300-02. After completing the previous study, eligible subjects who choose to continue treatment may enroll in the PTG-300-03 study. The safety evaluation done at the end of study PTG-300-02 will be used to confirm subject eligibility for this study (see Screening in Study Procedure section). No interruption of PTG -300 treatment is expected with the transition between studies.

Brief summary of the study: Arabic

PTG-300-. بعد الانتهاء من الدراسة السابقة, قد يتم تسجيل الاشخاص المؤهلين الذين يختارون مواصلة العلاج في دراسة PTG-300-03 . دراسة تمديدية مفتوحة التسمية للاشخاص الذين اكملوا دراسة 02
لتاكيد اهلية الموضوع لهذه الدراسة .لا يتوقع اي انقطاع للعلاج مع الانتقال بين الدراسات.
سيتم استخدام تقييم السلامة الذي تم في نهاية الدراسة PTG-300-02

Health conditions/problem studied: Specify

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

Interventions: Specify

Subjects rolling over from the PTG-300-02 study, who meet the response criteria defined for this study at the last dose received in study PTG-300-02, will continue to receive the same dose in PTG-300-03.
Subjects who did not meet response criteria defined for this study at the last dose received in study PTG-300-02, will start PTG-300-03 at the next higher dose level.

Key inclusion and exclusion criteria: Inclusion criteria

1. NTD and TD β-thalassemia subjects who completed Week 12 and Week 16 respectively in Study PTG-300-02.
2. Women of childbearing potential (WOCBP) and men agree to use a highly effective contraceptive measure (base on the Clinical Trial Facilitation
Group [CTFG]) during the duration of the study and for 4 weeks after the last dose of study drug in the case of women and 90 days after the last
dose of study drug in the case of men.
3. For WOCBP, a negative urine pregnancy test within 24 hours prior to the first dose of study medication in this study.
4. Subjects or legal guardians (in the case of minors) understand the study procedures and agree to participate in the study by giving written informed consent.
5. Subjects, or legal representative (in the case of minors) are willing and able to adhere to the study visit schedule and other protocol requirements.
6. Subjects between 12-<18 years of age understand and provide the assent to participate in the study, according to local guidelines.

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. Subjects who discontinued prematurely from study 300-02 (before Week 12 in NTD and Week 16 in TD)
2. Clinically meaningful laboratory abnormalities at Screening.
3. Pregnant or lactating females.
4. Current history of alcohol dependence or illicit drug use.
5. Subject has a concurrent clinically significant, unstable or uncontrolled cardiovascular, pulmonary, hepatic, renal, gastrointestinal, genitourinary,
hematological, coagulation, immunological, endocrine/metabolic or other medical disorder that, in the opinion of the Investigator, might confound the results of the study or pose additional risk to the subject by their participation in the study.
6. Subject is mentally or legally incapacitated at the time of Screening visit or has a history of clinically significant psychiatric disorders that would impact the subject's ability to participate in the trial according to the Investigator.
7. Concurrent participation in any other interventional study.

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

N/A

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

Year of authorization

Month of authorization

PTG-300

Type of IMP

Cell therapy

Pharmaceutical class

PTG-300 is a peptidic agent structurally related to natural hepcidin that mimics its inhibitory activity on ferroportin.

Therapeutic indication

β thalassemia

Therapeutic benefit

Administration of PTG-300 may result in iron redistribution in β-thalassemia subjects with potentially beneficial effects on erythropoiesis and consequently improvements in chronic anemia. This improvement in ineffective erythropoiesis may result in a clinical benefit both in NTD and in TD β-thalassemia subjects, by improving the symptomatology of the chronic anemia and the complications of the extramedullary hematopoiesis in the first group and by decreasing the need for transfusions in the latter.

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

Blood samples taken throughout the study will be shipped to ICON lab in Ireland for analysis. These samples will be then stored at ICON Lab by Protagonist Therapeutics for up to 10 years.

Target sample size

84

Actual enrollment target size

Date of first enrollment: Type

Anticipated

Date of first enrollment: Date

05/08/2019

Date of study closure: Type

Anticipated

Date of study closure: Date

27/06/2022

Recruitment status
Pending

Recruitment status: Specify

Date of completion
25/06/2020

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
Food and Drug Administration	IND

Sources of Monetary or Material Support

Name
Protagonist Therapeutics.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@mct-cro.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	Hematology/Oncology	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Chronic Care Center	01/06/2019	Michele Abi Saad	cccmas@chroniccare.org.lb	05-455101

Countries of Recruitment

Name
Lebanon
Thailand
United Kingdom
United States of America
Turkey
Tunisia
Malaysia
Greece
Italy

Health Conditions or Problems Studied

Condition	Code	Keyword
Thalassemia	Thalassaemia (D56)	Thalassemia

Interventions

Intervention	Description	Keyword
PTG-300	3mg/week	NA
PTG-300	10mg/week	NA
PTG-300	20mg/week	NA
PTG-300	40mg/week	NA
PTG-300	40 mg every 2 weeks	NA
PTG-300	80 mg/week	NA
PTG-300	80 mg every 2 weeks	NA

Primary Outcomes

Name	Time Points	Measure
NTD Subjects who achieve an increase in Hgb ≥1.0 g/dL	from pre-treatment baseline without transfusion	Hemoglobin Test
NTD patients: Hgb change	from pre-treatment baseline	Hemoglobin Test
TD Subjects who achieve a ≥20% reduction in the red blood cell (RBC) units transfused over 8-week period compared to pre-treatment baseline	over an 8-week period	RBC units transfused
TD patients: Change in the number of units of RBC required	from pre-treatment baseline	RBC units transfused

Key Secondary Outcomes

Name	Time Points	Measure
NTD patients: Proportion of subjects who achieve an increase in Hgb ≥1.5 g/dL	from pre-treatment baseline without transfusion	Hgb test
Proportion of subjects who achieve a maintenance dose	from pre-treatment baseline	Dose
Hgb level	from pre-treatment baseline	Hgb test
NTD Patients: Duration of Hgb change of ≥1.0 g/dL	from pre-treatment baseline without transfusion	Hgb test
NTD Patients: Duration of Hgb change of ≥1.5 g/dL from	from pre-treatment baseline without transfusion	Hgb test
Change in the following PD parameters	from pre-treatment baseline	serum iron, ferritin, transferrin saturation (TSAT)
TD Patients: Proportion of subjects who achieve ≥ 33% reduction in the RBC units required over an 8-week period	over an 8-week period	RBC units transfused
TD Patients: Duration of response defined as ≥ 20% reduction in the RBC units	over an 8-week period	RBC units transfused
TD Patients: Number of RBC units required	from pre-treatment baseline	RBC units transfused
TD Patients: Percent change in the RBC units required	from pre-treatment baseline	RBC units transfused
TD: Hgb change	from pre-treatment baseline	Hgb test

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