

A study to Evaluate the Efficacy and Safety of Mitapivat in Subjects With Non Transfusion-Dependent Alpha- or Beta-Thalassemia (ENERGIZE)

Estado
Fin Reclutamiento

Tipo de Participantes
No aportado

Rangos de Edad
Mayores de 64 , Adultos (18-64 años)

Género
Ambos

Fases
Fase III

Participantes esperados
86

Resultados
Sin resultados

Bajo nivel intervención
No

Enfermedad rara
Si

Cobertura geográfica
Sin determinar

Ámbitos del ensayo
seguridad, eficacia, farmacocinética,
farmacodinámica

Terapia avanzada
NO

Información

Identificador
2024-512745-16-00 (CTIS)

Cod. Protocolo
AG348-C-017

Transicionado 2021-000211-23

Área terapéutica
Enfermedades [C] - Hematología [C15]

Enfermedad investigada
NonTransfusion-Dependent Alpha- or Beta-Thalassemia

Título Científico
A Phase 3, Double-blind, Randomized, Placebo-Controlled, Multicenter Study Evaluating the Efficacy and Safety of Mitapivat in Subjects With NonTransfusion-Dependent Alpha or Beta Thalassemia (ENERGIZE)

Justificación

No aportado

Objetivo Principal

To compare the effect of mitapivat versus placebo on anemia in subjects with - or - nontransfusion-dependent thalassemia (NTDT)

Variables de Evaluación Primaria

Hemoglobin (Hb) response, defined as a 1.0 g/dL increase in average Hb concentration from Week 12 through Week 24 compared with baseline

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

No aportado

Variables de Evaluación Secundaria

No aportado

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

18 years of age at the time of providing informed consent.
Documented diagnosis of thalassemia (-thalassemia with or without -globin gene mutations, HbE/-thalassemia, or -thalassemia/HbH disease) based on Hb electrophoresis, Hb high-performance liquid chromatography, and/or DNA analysis from the subjects medical record. If this information is not available from the subjects medical record, the test(s) can be performed by a local laboratory during the Screening Period. If a local laboratory is unable to perform the test(s), results from the comprehensive - and -globin genotyping performed by the study central laboratory can be used.
Hb concentration 10.0 g/dL (100.0 g/L), based on an average of at least 2 Hb concentration measurements

(separated by 7 days) collected during the Screening Period.

Nontransfusion dependent, defined as: 5 red blood cell (RBC) units during the 24-week period before randomization and no RBC transfusions 8 weeks before providing informed consent and no RBC transfusions during the Screening Period.

If taking hydroxyurea, the hydroxyurea dose must be stable for 16 weeks before randomization.

Women of childbearing potential (WOCBP) must be abstinent of sexual activities that may result in pregnancy as part of their usual lifestyle or agree to use 2 forms of contraception, 1 of which must be considered highly effective, from the time of providing informed consent, throughout the study, and for 28 days after the last dose of study drug. The second form of contraception can be an acceptable barrier method.

Written informed consent before any study-related procedures are conducted and willing to comply with all study procedures for the duration of the study.

Criterios de Exclusión

Pregnant, breastfeeding, or parturient. Nonfasting triglycerides >440 mg/dL (5 mmol/L). Active infection requiring systemic antimicrobial therapy at the time of providing informed consent. If antimicrobial therapy is required during the Screening Period, screening procedures should not be performed while antimicrobial therapy is being administered, and the last dose of antimicrobial therapy must be administered 7 days before randomization. Positive test for hepatitis C virus (HCV) antibody (Ab) with evidence of active HCV infection, or positive test for hepatitis B surface antigen. Positive test for HIV-1 Ab or HIV-2 Ab. History of major surgery (including splenectomy) 16 weeks before providing informed consent and/or a major surgical procedure planned during the study. Current enrollment or past participation (12 weeks before administration of the first dose of study drug or a time frame equivalent to 5 half-lives of the investigational study drug, whichever is longer) in any other clinical study involving an investigational treatment or device. Receiving strong cytochrome P450 (CYP)3A4/5 inhibitors that have not been stopped for 5 days or a time frame equivalent to 5 half-lives (whichever is longer), or strong CYP3A4 inducers that have not been stopped for 4 weeks or a time frame equivalent to 5 half-lives (whichever is longer), before randomization. Receiving anabolic steroids, that have not been stopped for at least 4 weeks before randomization. Testosterone replacement therapy to treat hypogonadism is allowed; the testosterone dose and preparation must be stable for 10 weeks before randomization. Known allergy, or other contraindication, to mitapivat or its excipients (microcrystalline cellulose, croscarmellose sodium, sodium stearyl fumarate, mannitol, magnesium stearate, and Opadry® II Blue [hypromellose, titanium dioxide, lactose monohydrate, triacetin, and FD&C Blue #2]). Any medical, hematological, psychological, or behavioral condition(s) or prior or current therapy that, in the opinion of the Investigator, may confer an unacceptable risk to participating in the study and/or could confound the interpretation of the study data. Also excluded are: Subjects who are institutionalized by regulatory or court order Subjects with any condition(s) that could create undue influence (including but not limited to incarceration, involuntary psychiatric confinement, and financial or familial affiliation with the Investigator or Sponsor). Documented history of homozygous or heterozygous HbS or HbC. Prior exposure to gene therapy or prior bone marrow or stem cell transplantation. Currently receiving treatment with luspatercept; the last dose must have been administered 18 weeks before randomization. Currently receiving treatment with hematopoietic stimulating agents; the last dose must have been administered 18 weeks before randomization. History of malignancy (active or treated) 5 years before providing informed consent, except for nonmelanomatous skin cancer in situ, cervical carcinoma in situ, or breast carcinoma in situ. History of active and/or uncontrolled cardiac or pulmonary disease 6 months before providing informed consent, including but not limited to: a. New York Heart Association Class III or IV heart failure or clinically significant dysrhythmia b. Myocardial infarction or unstable angina pectoris; hemorrhagic, embolic, or thrombotic stroke; deep venous thrombosis; or pulmonary or arterial embolism c. Heart ratecorrected QT interval using Fridericias method 450 milliseconds (males) or 470 milliseconds (females), except for right or left bundle branch block d. Severe pulmonary fibrosis as defined by severe hypoxia, evidence of right-sided heart failure, and radiographic pulmonary fibrosis >50% e. Severe pulmonary hypertension as defined by severe symptoms associated with hypoxia, right-sided heart failure, and oxygen indicated Hepatobiliary disorders, including but not limited to: a. Liver disease with histopathological evidence of cirrhosis or severe fibrosis b. Clinically symptomatic cholelithiasis or cholecystitis (prior cholecystectomy is not exclusionary) c. History of drug-induced cholestatic hepatitis d. Aspartate aminotransferase >2.5 × upper limit of normal (ULN); unless due to hemolysis and hepatic iron deposition) and alanine aminotransferase >2.5 × ULN (unless due to hepatic iron deposition). Estimated glomerular filtration rate <45 mL/min/1.73 m² by Chronic Kidney Disease Epidemiology Collaboration creatinine equation.

Calendario

(Última actualización: 13/08/2026)

Autorización 15/10/2021	Inicio de Ensayo 05/11/2021	Inclusión Primer Paciente 28/12/2021	Interrumpido No aportado	Reiniciado No aportado
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Fin de reclutamiento 31/03/2023	Fin prematuro (España) No aportado	Fin prematuro (Global) No aportado	Fin del ensayo en España No aportado	Fin del ensayo global No aportado
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Promotor

Agios Pharmaceuticals Inc. United States

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Contact Person

Scientific Communications

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Monetary support: N/A

Tipo de promotor: Comercial

Ceim

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947281964

Centros

Activo (16/11/2021)	HOSPITAL CLINICO UNIVERSITARIO VIRGEN DE LA ARRIXACA	Murcia	MURCIA	
No iniciado (---/---/----)	Hospital Universitari Vall D Hebron	Barcelona	BARCELONA	Hematology
Activo (22/10/2021)	HOSPITAL UNIVERSITARI VALL D'HEBRON	Barcelona	BARCELONA	
Activo (03/11/2021)	HOSPITAL UNIVERSITARIO LA PAZ	Madrid	MADRID	
No iniciado (---/---/----)	Hospital Universitario La Paz	Madrid	MADRID	Hematology
Cerrado (12/08/2026)	HOSPITAL UNIVERSITARIO VIRGEN DEL ROCIO	Sevilla	SEVILLA	
No iniciado (---/---/----)	University Clinical Hospital Virgen De La Arrixaca	Murcia	MURCIA	Hematology
No iniciado (---/---/----)	University Hospital Virgen Del Rocio S.L.	Sevilla	SEVILLA	Hematology

Medicamentos

MITAPIVAT

TABLET

ORAL USE

Principios Activos: N/A

Experimental

Sin resultados

A study to Evaluate the Efficacy and Safety of Mitapivat in Subjects With Non Transfusion-Dependent Alpha- or Beta-Thalassemia (ENERGIZE)

State
End of recruitment

Type of participants
Not provided

Age Ranges
Older than 64 , Adults (18-64 years)

Gender
Both

Phases
Phase III

Expected Participants
86

Results
No results

Low level of intervention
No

Rare disease
Yes

Geographic coverage
Undetermined

Areas of the study
safety, effectiveness, pharmacokinetics,
pharmacodynamics

Advanced therapy
NO

Information

Identifier
2024-512745-16-00 (CTIS)

Protocol Code
AG348-C-017

Transitioned 2021-000211-23

Therapeutic area
Diseases [C] - Hemic and Lymphatic Diseases [C15]

Investigated Disease
NonTransfusion-Dependent Alpha- or Beta-Thalassemia

Scientific Title
A Phase 3, Double-blind, Randomized, Placebo-Controlled, Multicenter Study Evaluating the Efficacy and Safety of Mitapivat in Subjects With NonTransfusion-Dependent Alpha or Beta Thalassemia (ENERGIZE)

Rationale

Not provided

Main Objective

To compare the effect of mitapivat versus placebo on anemia in subjects with - or - nontransfusion-dependent thalassemia (NTDT)

Primary Endpoints

Hemoglobin (Hb) response, defined as a 1.0 g/dL increase in average Hb concentration from Week 12 through Week 24 compared with baseline

Temporary moments of secondary assessment

Not provided

Secondary Objective

Not provided

Secondary Endpoints

Not provided

Temporary moments of secondary assessment

Not provided

Inclusion criteria

18 years of age at the time of providing informed consent.

Documented diagnosis of thalassemia (-thalassemia with or without -globin gene mutations, HbE/-thalassemia, or -thalassemia/HbH disease) based on Hb electrophoresis, Hb high-performance liquid chromatography, and/or DNA analysis from the subjects medical record. If this information is not available from the subjects medical record, the test(s) can be performed by a local laboratory during the Screening Period. If a local laboratory is unable to perform the test(s), results from the comprehensive - and -globin genotyping performed by the study central laboratory can be used.

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Exclusion criteria

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Calendar

(Last Update: 13/08/2026)

Authorization 15/10/2021	Start of Trial 05/11/2021	First patient inclusion 28/12/2021	Halted Not aported	Restarted Not aported
End of recruitment 31/03/2023	Premature end (Spain) Not aported	Premature End (Global) Not aported	Trial end (Spain) Not aported	Trial end (Global) Not aported

Sponsor

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Contact Person

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Monetary support: N/A

Sponsor type: Commercial

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Sites

Active (16/11/2021)	HOSPITAL CLINICO UNIVERSITARIO VIRGEN DE LA ARRIXACA Murcia MURCIA
not initialized (---/---/----)	Hospital Universitari Vall D Hebron Barcelona BARCELONA Hematology
Active (22/10/2021)	HOSPITAL UNIVERSITARI VALL D'HEBRON Barcelona BARCELONA
Active (03/11/2021)	HOSPITAL UNIVERSITARIO LA PAZ Madrid MADRID
not initialized (---/---/----)	Hospital Universitario La Paz Madrid MADRID Hematology
Closed (12/08/2026)	HOSPITAL UNIVERSITARIO VIRGEN DEL ROCIO Sevilla SEVILLA
not initialized (---/---/----)	University Clinical Hospital Virgen De La Arrixaca Murcia MURCIA Hematology
not initialized (---/---/----)	University Hospital Virgen Del Rocio S.L. Sevilla SEVILLA Hematology

Medication

MITAPIVAT

TABLET

ORAL USE

Active Principles: N/A

Experimental

No results