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

Estado	**Tipo de Participantes**	**Rangos de Edad**
Fin Reclutamiento	No aportado	Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase III	76
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	seguridad, eficacia, farmacocinética, farmacodinamica	NO

Información

Identificador

2024-512747-23-00 (CTIS)

Cod. Protocolo

AG348-C-018

Transicionado 2021-000212-34

Área terapéutica

Enfermedades [C] - Hematología [C15]|

Enfermedad investigada

Transfusion-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 Transfusion-Dependent Alpha or Beta Thalassemia (ENERGIZE-T)

Justificación

No aportado

Objetivo Principal

To compare the effect of mitapivat versus placebo on transfusion burden in subjects with - or -transfusiondependent thalassemia (TDT)

Variables de Evaluación Primaria

Transfusion reduction response (TRR), defined as a 50% reduction in transfused red blood cell (RBC) units with a reduction of 2 units of transfused RBCs in any consecutive 12-week period through Week 48 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 DNA analysis from the subjects medical record. If this information is not available from the subjects medical record, DNA analysis can be performed by a local laboratory during the Screening Period. If a local laboratory is unable to perform the test, results from the comprehensive - and -globin genotyping performed by the study central laboratory can be used.
Transfusion dependent, defined as 6 to 20 RBC units transfused and a 6-week transfusion-free period during the 24-week period before randomization.

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 induce 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) 6 months 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 treatment, 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 12 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 36 weeks before randomization. Currently receiving treatment with hematopoietic stimulating agents; the last dose must have been administered 36 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	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
11/10/2021	05/11/2021	30/11/2021	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
07/03/2023	No aportado	No aportado	No aportado	No aportado

Promotor

Agios Pharmaceuticals Inc. United States

88 Sidney Street

Contact Person

Scientific Communications

+18446332332

medinfo@agios.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIM Área de Salud de Burgos y Soria

Avenida de las Islas Baleares, 3 09006 Burgos

jlabradorg@saludcastillayleon.es

947281964

Centros

Activo (10/11/2021)

HOSPITAL CLINICO UNIVERSITARIO VIRGEN DE LA ARRIXACA

Murcia

MURCIA

No iniciado (--/--/----)

Hospital Universitari Vall D Hebron

Barcelona

BARCELONA

Hematology

Activo (19/10/2021)

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 Transfusion-Dependent Alpha- or Beta-Thalassemia (ENERGIZE-T)

State	**Type of participants**	**Age Ranges**
End of recruitment	Not provided	Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase III	76
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	safety, effectiveness, pharmacokinetics, pharmacodynamics	NO

Information

Identifier
2024-512747-23-00 (CTIS)

Protocol Code
AG348-C-018

Transitioned 2021-000212-34

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

Investigated Disease
Transfusion-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 Transfusion-Dependent Alpha or Beta Thalassemia (ENERGIZE-T)

Rationale

Not provided

Main Objective

To compare the effect of mitapivat versus placebo on transfusion burden in subjects with - or -transfusiondependent thalassemia (TDT)

Primary Endpoints

Transfusion reduction response (TRR), defined as a 50% reduction in transfused red blood cell (RBC) units with a reduction of 2 units of transfused RBCs in any consecutive 12-week period through Week 48 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 DNA analysis from the subjects medical record. If this information is not available from the subjects medical record, DNA analysis can be performed by a local laboratory during the Screening Period. If a local laboratory is unable to perform the test, 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	Start of Trial	First patient inclusion	Halted	Restarted
11/10/2021	05/11/2021	30/11/2021	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
07/03/2023	Not aported	Not aported	Not aported	Not aported

Sponsor

Agios Pharmaceuticals Inc. United States

88 Sidney Street

Contact Person

Scientific Communications

+18446332332

medinfo@agios.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

CEIM Área de Salud de Burgos y Soria

Avenida de las Islas Baleares, 3 09006 Burgos

jlabradorg@saludcastillayleon.es

947281964

Sites

Active (10/11/2021)

HOSPITAL CLINICO UNIVERSITARIO VIRGEN DE LA ARRIXACA

Murcia

MURCIA

not initialized (--/--/----)

Hospital Universitari Vall D Hebron

Barcelona

BARCELONA

Hematology

Active (19/10/2021)

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