A Study of ASTX030 (Cedazuridine in Combination with Azacitidine) in MDS, CMML, or AML

Estado	Tipo de Participantes	Rangos de Edad
Reclutando	No aportado	Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase III	200
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	tratamiento, seguridad, eficacia, farmacocinética, farmacodinamica, dosis	NO

Información

Identificador
2024-515098-93-00 (CTIS)

Cod. Protocolo
ASTX030-01

Área terapéutica
Enfermedades [C] - Hematología [C15]|

Enfermedad investigada
Myelodysplastic Syndromes (MDS), Chronic Myelomonocytic Leukemia (CMML)

Título Científico
A Multi-phase, Dose-Escalation followed by an Open-label, Randomized, Crossover Study of Oral ASTX030 (Cedazuridine and Azacitidine Given in Combination) Versus Subcutaneous Azacitidine in Subjects with Myelodysplastic Syndromes (MDS), Chronic Myelomonocytic Leukemia (CMML), or Acute Myeloid Leukemia (AML)

Justificación

No aportado

Objetivo Principal

Establish azacitidine AUC equivalence (90% CI 0.8, 1.25) between the final selected dose of ASTX030 from Phase 2 using FDC and SC azacitidine at 75 mg/m2/day across BSA ranges

Variables de Evaluación Primaria

Ratio of azacitidine total cycle AUC 0-24 exposures after oral ASTX030 over SC azacitidine at 75 mg/m2/day

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

Assess safety and tolerability of ASTX030
Evaluate changes in DNA methylation of ASTX030 compared to SC azacitidine
Assess clinical activity of ASTX030
Evaluate other PK parameters of azacitidine, cedazuridine, and cedazuridine-epimer

Variables de Evaluación Secundaria

1 TEAEs including SAEs
2 Percent LINE-1 demethylation change from baseline between SC azacitidine and ASTX030 in Cycles 1 and 2
3 Participants with MDS or CMML: - Clinical response rate - AML-free survival - Duration of response - OS - Time to response - Transfusion independence

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

1. Must be 18 years 8. Able to swallow the number of capsules required within 10-mins and can tolerate 4 hours of fasting. 9. Life expectancy >= 12 weeks. 10. Follow contraceptive barrier requirements per protocol 11. Capable of giving legally effective informed consent. and willing to participate in the study. 2. Confirmed MDS or CMML and a

candidate to receive and benefit from single-agent azacitidine according to French-American-British myelodysplastic syndrome subtypes: or MDS with intermediate-2 or high risk MDS according to the IPSS (Greenberg P, et al. Blood. 1997;89:2079-2088) 3. ECOG 0 or 1 4. Adequate organ function : a. Total or direct bilirubin 2 × ULN; AST/ SGOT and ALT/SGPT 2.5 × ULN. b. Calculated CrCl >50 mL/min by Cockcroft-Gault formula or other medically acceptable formulas. 5. For participants with prior allogeneic stem cell transplant, has no evidence of graft vs host disease and must be 3 weeks off systemic immunosuppressive therapy before study start. 6. No major surgery within 3 weeks before treatment initiation. 7. No cytotoxic chemotherapy (excluding hydroxyurea) within 4 weeks before treatment initiation

Criterios de Exclusión

1. Active, uncontrolled gastric or duodenal ulcer. 4. Known positive for Hepatitis B or C infection with the exception of those with an undetectable viral load within 3 months of screening 5. Life-threatening illness, uncontrolled medical condition or organ system dysfunction, or other reasons that, could compromise the participant's safety, interfere with the absorption or metabolism of ASTX030, or the integrity of study outcomes. 6.History of other malignancies, except certain adequately treated and/or controlled cancers, or early-stage cases not requiring therapy. 7. Extramedullary disease in MDS/MPN, including palpable hepatomegaly or splenomegaly 8. Prior treatment with > 1 cycle of decitabine or azacitidine 9. Treated with any investigational therapy within 2 weeks, or 5 half-lives, whichever is longer, before treatment initiation , or has ongoing clinically significant AEs from previous treatment with investigational agent 10. Cannot discontinue treatment with drugs that delay gastric emptying (GLP-1 and/or GIP agonists in Cycles 1 & 2. 11. Known hypersensitivity to cedazuridine or azacitidine or any excipients. 2. Poor medical risk due to any uncontrolled systemic diseases or active uncontrolled bacterial, viral, or fungal infections. 3. Known HIV infection.

Calendario

(Última actualización: 25/05/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
03/06/2025	25/07/2025	05/08/2025	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
No aportado	No aportado	No aportado	No aportado	No aportado

Promotor

Taiho Oncology Inc. United States

101 Carnegie Center Suite 300

Contact Person

Yuri Sano

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medicalinformation@taihooncology.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Hospital General Universitario Gregorio Marañón

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915867007

Centros

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

Clinica Universidad De Navarra

Madrid

MADRID

Haematology

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

Clinica Universidad De Navarra

Pamplona

NAVARRA

Haematology

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

Hospital Clinic De Barcelona

Barcelona

BARCELONA

Haematology

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

Hospital General Universitario Gregorio Maranon

Madrid

MADRID

Haematology and haemotherapy

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

Hospital Quironsalud Malaga

Malaga

MÁLAGA

Haematology

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

Hospital San Pedro De Alcantara

Caceres

CÁCERES

Haematology

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

Hospital Universitari De Girona Doctor Josep Trueta

Girona

GERONA

Haematology and haemotherapy

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

Hospital Universitari Vall D Hebron

Barcelona

BARCELONA

Haematology

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

Hospital Universitario Central De Asturias

Oviedo

ASTURIAS

Haematology

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

Hospital Universitario De Salamanca

Salamanca

SALAMANCA

Haematology

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

Hospital Universitario La Paz

Madrid

MADRID

Haematology

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

Hospital Universitario Virgen De Las Nieves

Granada

GRANADA

Haematology

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

Hospital Universitario Y Politecnico La Fe

Valencia

VALENCIA

Haematology

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

Institut Catala D´oncologia

Badalona

BARCELONA

Haematology

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

Institut Catala D´oncologia

L'hospitalet De Llobregat

BARCELONA

Haematology

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

MD Anderson Cancer Center

Madrid

MADRID

Haematology

Medicamentos

Azacitidine, Cedazuridine
CAPSULE
ORAL

-

Principios Activos: N/A

Experimental

Vidaza 25 mg/ml powder for suspension for injection
SUSPENSION FOR INJECTION
SUBCUTANEOUS USE

Código ATC: L01BC07 - AZACITIDINA

Principios Activos: N/A

Experimental

Azacitidine, Cedazuridine
CAPSULE
ORAL

-

Principios Activos: N/A

Experimental

Azacitidine
CAPSULE
ORAL

-

Principios Activos: N/A

Experimental

Sin resultados

A Study of ASTX030 (Cedazuridine in Combination with Azacitidine) in MDS, CMML, or AML

State	Type of participants	Age Ranges
Recruiting	Not provided	Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase III	200
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	treatment, safety, effectiveness, pharmacokinetics, pharmacodynamics, dose	NO

Information

Identifier
2024-515098-93-00 (CTIS)

Protocol Code
ASTX030-01

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

Investigated Disease
Myelodysplastic Syndromes (MDS), Chronic Myelomonocytic Leukemia (CMML)

Scientific Title
A Multi-phase, Dose-Escalation followed by an Open-label, Randomized, Crossover Study of Oral ASTX030 (Cedazuridine and Azacitidine Given in Combination) Versus Subcutaneous Azacitidine in Subjects with Myelodysplastic Syndromes (MDS), Chronic Myelomonocytic Leukemia (CMML), or Acute Myeloid Leukemia (AML)

Rationale

Not provided

Main Objective

Establish azacitidine AUC equivalence (90% CI 0.8, 1.25) between the final selected dose of ASTX030 from Phase 2 using FDC and SC azacitidine at 75 mg/m2/day across BSA ranges

Primary Endpoints

Ratio of azacitidine total cycle AUC 0-24 exposures after oral ASTX030 over SC azacitidine at 75 mg/m2/day

Temporary moments of secondary assessment

Not provided

Secondary Objective

Assess safety and tolerability of ASTX030
Evaluate changes in DNA methylation of ASTX030 compared to SC azacitidine
Assess clinical activity of ASTX030
Evaluate other PK parameters of azacitidine, cedazuridine, and cedazuridine-epimer

Secondary Endpoints

1 TEAEs including SAEs
2 Percent LINE-1 demethylation change from baseline between SC azacitidine and ASTX030 in Cycles 1 and 2
3 Participants with MDS or CMML: - Clinical response rate - AML-free survival - Duration of response - OS - Time to response - Transfusion independence

Temporary moments of secondary assessment

Not provided

Inclusion criteria

1. Must be 18 years 8. Able to swallow the number of capsules required within 10-mins and can tolerate 4 hours of fasting. 9. Life expectancy >= 12 weeks. 10. Follow contraceptive barrier requirements per protocol 11. Capable of giving legally effective informed consent. and willing to participate in the study. 2. Confirmed MDS or CMML and a

candidate to receive and benefit from single-agent azacitidine according to French-American-British myelodysplastic syndrome subtypes: or MDS with intermediate-2 or high risk MDS according to the IPSS (Greenberg P, et al. Blood. 1997;89:2079-2088) 3. ECOG 0 or 1 4. Adequate organ function : a. Total or direct bilirubin 2 × ULN; AST/ SGOT and ALT/SGPT 2.5 × ULN. b. Calculated CrCl >50 mL/min by Cockcroft-Gault formula or other medically acceptable formulas. 5. For participants with prior allogeneic stem cell transplant, has no evidence of graft vs host disease and must be 3 weeks off systemic immunosuppressive therapy before study start. 6. No major surgery within 3 weeks before treatment initiation. 7. No cytotoxic chemotherapy (excluding hydroxyurea) within 4 weeks before treatment initiation

Exclusion criteria

1. Active, uncontrolled gastric or duodenal ulcer. 4. Known positive for Hepatitis B or C infection with the exception of those with an undetectable viral load within 3 months of screening 5. Life-threatening illness, uncontrolled medical condition or organ system dysfunction, or other reasons that, could compromise the participant's safety, interfere with the absorption or metabolism of ASTX030, or the integrity of study outcomes. 6.History of other malignancies, except certain adequately treated and/or controlled cancers, or early-stage cases not requiring therapy. 7. Extramedullary disease in MDS/MPN, including palpable hepatomegaly or splenomegaly 8. Prior treatment with > 1 cycle of decitabine or azacitidine 9. Treated with any investigational therapy within 2 weeks, or 5 half-lives, whichever is longer, before treatment initiation , or has ongoing clinically significant AEs from previous treatment with investigational agent 10. Cannot discontinue treatment with drugs that delay gastric emptying (GLP-1 and/or GIP agonists in Cycles 1 & 2. 11. Known hypersensitivity to cedazuridine or azacitidine or any excipients. 2. Poor medical risk due to any uncontrolled systemic diseases or active uncontrolled bacterial, viral, or fungal infections. 3. Known HIV infection.

Calendar

(Last Update: 25/05/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
03/06/2025	25/07/2025	05/08/2025	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
Not aported	Not aported	Not aported	Not aported	Not aported

Sponsor

Taiho Oncology Inc. United States

101 Carnegie Center Suite 300

Contact Person

Yuri Sano

+18448782446

medicalinformation@taihooncology.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

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Sites

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

Clinica Universidad De Navarra

Madrid

MADRID

Haematology

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

Clinica Universidad De Navarra

Pamplona

NAVARRA

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Hospital Clinic De Barcelona

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Malaga

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Hospital Universitari De Girona Doctor Josep Trueta

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Hospital Universitario Virgen De Las Nieves

Granada

GRANADA

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Hospital Universitario Y Politecnico La Fe

Valencia

VALENCIA

Haematology

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BARCELONA

Haematology

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

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Madrid

MADRID

Haematology

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Azacitidine, Cedazuridine
CAPSULE
ORAL

-

Active Principles: N/A

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SUSPENSION FOR INJECTION
SUBCUTANEOUS USE

ATC code: L01BC07 - AZACITIDINA

Active Principles: N/A

Experimental

Azacitidine, Cedazuridine
CAPSULE
ORAL

-

Active Principles: N/A

Experimental

Azacitidine
CAPSULE
ORAL

-

Active Principles: N/A

Experimental

No results