A Phase 2 Study of Momelotinib in Participants with low-risk myelodysplastic syndrome

Estado	Tipo de Participantes	Rangos de Edad
Reclutando	No aportado	Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase II	35
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	Sin determinar	NO

Información

Identificador
2024-519928-24-00 (CTIS)

Cod. Protocolo
223584

Área terapéutica
Enfermedades [C] - Cáncer [C04]|

Enfermedad investigada
Myelodysplastic Syndrome

Título Científico
A Phase 2, Randomized, Open-label, Study of Momelotinib in Participants with Anemia due to Low-risk Myelodysplastic Syndrome.

Justificación

No aportado

Objetivo Principal

To identify the RP3D To assess the effect of momelotinib on RBC TI response by Week 24

Variables de Evaluación Primaria

1. Percentage of participants with rolling 12 weeks of RBC-TI by the end of Week 24
2. Selected safety endpoints: Grade 3 AE, AEs leading to treatment discontinuation and/or dose modifications
3. Pharmacokinetic parameters of momelotinib and M21 (e.g., Cmax, AUC), as data permit

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

- To assess the effect of momelotinib on RBC-TI response by Week 24
- To characterize the safety and tolerability of momelotinib at mg and mg in participants with LR-MDS
- To describe the exposure of momelotinib and M21
- To assess the effect of momelotinib on other anemia-related improvement measures

Variables de Evaluación Secundaria

3. Percentage of participants with 24 weeks RBC-TI by the end of Week 48
6. Incidence and severity of AEs and SAEs, and AEs leading to treatment discontinuation or dose modifications by the end of Week 24 and longer term
7. Clinically important changes in laboratory parameters and vital signs by the end of Week 24 and longer term
8. Plasma concentration of momelotinib and M21
1. Percentage of participants with rolling 16 weeks of RBC-TI by the end of Week 24
2. Increase in hemoglobin of 1.5 g/dL
4. HIE response per International Working Group 2018 criteria
5. Time to RBC-TI by Week 24 and Week 48

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Participants are eligible to be included in the study only if all of the following criteria apply. 1. Age 18 years OR of legal age of consent in the jurisdiction in which the study is taking place, at the time of signing the ICF. 2. Documented diagnosis of MDS according to the World Health Organization classifications [Arber, 2016] with an IPSS-R classification of very low, low, or intermediate risk disease, with an overall risk score 3.5 [Greenberg, 2012] and bone marrow blasts <5%. 3. Received ESA OR luspatercept and/or other therapies approved by health authorities and available for the treatment of LR-MDS-related anemia that is relapsed/refractory or intolerant to the most recent therapy prior to screening. Relapse/refractory to most recent prior treatment: documentation of loss of erythroid (E) response or never achieved hematologic improvement (HI-E) response as defined by the IWG 2018 criteria [Platzbecker, 2019]. If the most recent prior treatment is ESA or Luspatercept. 4. Red blood cell transfusion dependence, defined as requiring 3 units of pRBC transfused over 16-week period in at least 2 transfusion episodes during the 16 weeks preceding randomization. Documentation of a participants transfusion policy during this 16week period is required. 5. A female participant is eligible to participate if she is not pregnant or breastfeeding and one of the following conditions applies: a. Is a woman of non-childbearing potential (WONCBP) (as defined in Section 10.4), OR b. Is a woman of childbearing potential (WOCBP) and using a contraceptive method that is highly effective (with a failure rate of <1% per year), preferably with low user dependency (as described in Section 10.4) 28 days prior to and during the study intervention period and for at least 1 week after the last dose of study intervention. The investigator should evaluate the potential for contraceptive method failure (e.g., noncompliance, recently initiated) in relationship to the first dose of study intervention. 6. Is capable of giving signed informed consent as described in Section 10.1, including compliance with the requirements and restrictions listed in the ICF and in this protocol. 7. Eastern Cooperative Oncology Group performance status 2 (see Section 10.7) 8. Adequate organ function as defined in Table 8.

Criterios de Exclusión

Participants are excluded from the study if any of the following criteria apply. d. Hypomethylating agents or other disease modifying agents (i.e., IMiDs) and immunosuppressive therapy for MDS Note: Exception to this exclusion is if 1 week of treatment received; provided last dose was 8 weeks from randomization 19. Has any clinically significant gastrointestinal conditions or abnormalities that may alter absorption, e.g., uncontrolled nausea, vomiting, malabsorption syndrome or major resection of the stomach and/or bowels. 20. Is unable to swallow and/or retain oral medications 2. Use of the following treatments within the noted time periods referenced from date of randomization: a. ESA within 4 weeks, or 8 weeks for long-acting ESA b. Growth factors (i.e., G-CSF, GM-CSF) within 4 weeks c. Luspatercept within 8 weeks d. Imetelstat within 8 weeks e. Investigational agents within 4 weeks or 5 half-lives, whichever is longer f. Corticosteroids for treatment of the underlying disease within 28 days. Supportive care use of steroids for non-MDS indications may be used provided participant is on a stable dose equivalent to 10 mg prednisone per day g. Other active anti-MDS therapy not otherwise listed within 28 days or 5 halflives whichever is longer h. Potent cytochrome P450 3A4 (CYP3A4) inducers, except for rifampin and rifampicin, within 14 days prior to the first dose of momelotinib i. Has received a live vaccine within 30 days 21. Known contraindication or hypersensitivity to momelotinib and its metabolites, or any of their excipients. 3. Prior allogeneic or autologous stem cell transplant 4. Has had any major surgery within 28 days prior to randomization 5. Ongoing adverse reaction(s) from prior therapy that have not recovered to Grade 1 or to the baseline status preceding prior therapy, except if the investigator, with the agreement of the sponsor, considers to be not clinically relevant for the tolerability of study intervention in the current clinical study. 6. MDS associated with del 5q cytogenetic abnormality 8. Secondary MDS (i.e., MDS that is known to have arisen as the result of chemical injury, treatment with chemotherapy, and/or radiation for other diseases). 9. Known history of diagnosis of acute myeloid leukemia. 11. Diagnosis of invasive malignancy or history of invasive malignancy other than the disease under study within the last 5 years, except as noted below: a. History of an invasive malignancy for which the participant was definitively treated, and in which the participant has been disease free for at least 2 years, and which, in the opinion of the principal investigator and medical monitor, is not expected to affect the evaluation of the effects of the study intervention on the currently targeted disease under study b. Curatively treated basal cell carcinoma of the skin, superficial bladder cancer, squamous cell carcinoma of the skin, in situ cervical cancer, and/or in situ breast cancer may be enrolled c. Incidental histologic finding of prostate cancer (T1a or T1b using the TNM clinical staging system) 10. Known

clinically significant anemia due to iron, vitamin B12, or folate deficiencies, or autoimmune or hereditary hemolytic anemia, gastrointestinal bleeding, or thalassemia. 7. MDS/MPN overlap disorders (e.g., CMML). 12. Uncontrolled intercurrent illness including, but not limited to: a. Active uncontrolled infection (participants receiving outpatient antibacterial and/or antiviral treatments for infection that is under control or as infection prophylaxis may be included in the trial); or b. Significant active or chronic bleeding event Grade 2 per CTCAE v5.0 within 4 weeks prior randomization c. Uncontrolled acute and chronic liver disease (e.g., Child-Pugh score 10) OR has current unstable liver or biliary disease per investigator assessment defined by the presence of ascites, encephalopathy, coagulopathy, hypoalbuminemia, esophageal or gastric varices, persistent jaundice, or cirrhosis. NOTE: Stable chronic liver disease (including Gilbert's syndrome or asymptomatic gallstones) is acceptable if participant otherwise meets entry criteria 13. Any of the following conditions within 6 months prior to randomization: a. Unstable angina pectoris b. Symptomatic congestive heart failure c. Uncontrolled cardiac arrhythmia 14. QTc interval >480 msec (corrected using Fridericia formula). 15. Psychiatric illness, social situation, or any other condition that would limit compliance with trial requirements or may interfere with the interpretation of study results, as judged by investigator or sponsor. 16. Presence of peripheral neuropathy Grade 2 per CTCAE v5.0. 17. Known positive status for HIV. 18. Hepatitis B, or C status as defined below: a. Active Hepatitis B infection indicated by the presence of hepatitis B surface antigen (HBsAg) at screening or within 3 months prior to the first dose of study intervention. b. Positive hepatitis C antibody test result at screening or within 3 months before the first dose of study intervention. NOTE: Participants with positive hepatitis C antibody due to prior resolved disease can be enrolled, only if a confirmatory negative hepatitis C RNA test is obtained.

Calendario

(Última actualización: 17/07/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
08/08/2025	21/08/2025	02/09/2025	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

Glaxosmithkline Research & Development Limited United Kingdom

79 New Oxford Street

Contact Person

EU GSK Clinical Trials Call Center

+442089904466

GSKClinicalSupportHD@gsk.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Hospital Universitari Germans Trias i Pujol

Ctra. Canyet, s/n Edificio General - 1a planta / Módulos urgencias - 1a planta 08916 Badalona

ceic.germanstrias@gencat.cat

934978956

Centros

Activo (26/08/2025)

Clinica Universidad De Navarra

Pamplona

NAVARRA

Hematology

Activo (12/09/2025)

Clinica Universidad De Navarra

Madrid

MADRID

Hematology

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

Complejo Hospitalario Universitario De Ourense

Ourense

OURENSE

Hematology

Activo (29/09/2025)

Hospital Clinico Universitario De Valencia

Valencia

VALENCIA

Hematology

Activo (29/09/2025)

Hospital Universitari Vall D Hebron

Barcelona

BARCELONA

Hematology

Activo (21/08/2025)

Hospital Universitario De Salamanca

Salamanca

SALAMANCA

Hematology

Activo (16/09/2025)

Hospital Universitario Virgen De La Victoria

Malaga

MÁLAGA

Hematology & Hemotherapy

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

Institut Catala D´oncologia

Badalona

BARCELONA

Hematology

Activo (28/11/2025)

Institut Catala D´oncologia

L'hospitalet De Llobregat

BARCELONA

Hematology

Medicamentos

PRD11032047
TABLET
ORAL USE

-

Principios Activos: N/A

Huérfano **Experimental**

PRD11032121
TABLET
ORAL USE

-

Principios Activos: N/A

Huérfano **Experimental**

PRD11032087
TABLET
ORAL USE

-

Principios Activos: N/A

Huérfano **Experimental**

Sin resultados

A Phase 2 Study of Momelotinib in Participants with low-risk myelodysplastic syndrome

State	Type of participants	Age Ranges
Recruiting	Not provided	Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase II	35
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	Undetermined	NO

Information

Identifier
2024-519928-24-00 (CTIS)

Protocol Code
223584

Therapeutic area
Diseases [C] - Cancer [C04]|

Investigated Disease
Myelodysplastic Syndrome

Scientific Title
A Phase 2, Randomized, Open-label, Study of Momelotinib in Participants with Anemia due to Low-risk Myelodysplastic Syndrome.

Rationale

Not provided

Main Objective

To identify the RP3D To assess the effect of momelotinib on RBC TI response by Week 24

Primary Endpoints

1. Percentage of participants with rolling 12 weeks of RBC-TI by the end of Week 24
2. Selected safety endpoints: Grade 3 AE, AEs leading to treatment discontinuation and/or dose modifications
3. Pharmacokinetic parameters of momelotinib and M21 (e.g., Cmax, AUC), as data permit

Temporary moments of secondary assessment

Not provided

Secondary Objective

- To assess the effect of momelotinib on RBC-TI response by Week 24
- To characterize the safety and tolerability of momelotinib at mg and mg in participants with LR-MDS
- To describe the exposure of momelotinib and M21
- To assess the effect of momelotinib on other anemia-related improvement measures

Secondary Endpoints

3. Percentage of participants with 24 weeks RBC-TI by the end of Week 48
6. Incidence and severity of AEs and SAEs, and AEs leading to treatment discontinuation or dose modifications by the end of Week 24 and longer term
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1. Percentage of participants with rolling 16 weeks of RBC-TI by the end of Week 24
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Temporary moments of secondary assessment

Not provided

Inclusion criteria

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clinically significant anemia due to iron, vitamin B12, or folate deficiencies, or autoimmune or hereditary hemolytic anemia, gastrointestinal bleeding, or thalassemia. 7. MDS/MPN overlap disorders (e.g., CMML). 12. Uncontrolled intercurrent illness including, but not limited to: a. Active uncontrolled infection (participants receiving outpatient antibacterial and/or antiviral treatments for infection that is under control or as infection prophylaxis may be included in the trial); or b. Significant active or chronic bleeding event Grade 2 per CTCAE v5.0 within 4 weeks prior randomization c. Uncontrolled acute and chronic liver disease (e.g., Child-Pugh score 10) OR has current unstable liver or biliary disease per investigator assessment defined by the presence of ascites, encephalopathy, coagulopathy, hypoalbuminemia, esophageal or gastric varices, persistent jaundice, or cirrhosis. NOTE: Stable chronic liver disease (including Gilbert's syndrome or asymptomatic gallstones) is acceptable if participant otherwise meets entry criteria 13. Any of the following conditions within 6 months prior to randomization: a. Unstable angina pectoris b. Symptomatic congestive heart failure c. Uncontrolled cardiac arrhythmia 14. QTc interval >480 msec (corrected using Fridericia formula). 15. Psychiatric illness, social situation, or any other condition that would limit compliance with trial requirements or may interfere with the interpretation of study results, as judged by investigator or sponsor. 16. Presence of peripheral neuropathy Grade 2 per CTCAE v5.0. 17. Known positive status for HIV. 18. Hepatitis B, or C status as defined below: a. Active Hepatitis B infection indicated by the presence of hepatitis B surface antigen (HBsAg) at screening or within 3 months prior to the first dose of study intervention. b. Positive hepatitis C antibody test result at screening or within 3 months before the first dose of study intervention. NOTE: Participants with positive hepatitis C antibody due to prior resolved disease can be enrolled, only if a confirmatory negative hepatitis C RNA test is obtained.

Calendar

(Last Update: 17/07/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
08/08/2025	21/08/2025	02/09/2025	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

Glaxosmithkline Research & Development Limited United Kingdom

79 New Oxford Street

Contact Person

EU GSK Clinical Trials Call Center

+442089904466

GSKClinicalSupportHD@gsk.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

CEIm Hospital Universitari Germans Trias i Pujol

Ctra. Canyet, s/n Edificio General - 1a planta / Módulos urgencias - 1a planta 08916 Badalona

ceic.germanstrias@gencat.cat

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Sites

Active (26/08/2025)

Clinica Universidad De Navarra

Pamplona

NAVARRA

Hematology

Active (12/09/2025)

Clinica Universidad De Navarra

Madrid

MADRID

Hematology

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

Complejo Hospitalario Universitario De Ourense

Ourense

OURENSE

Hematology

Active (29/09/2025)

Hospital Clinico Universitario De Valencia

Valencia

VALENCIA

Hematology

Active (29/09/2025)

Hospital Universitari Vall D Hebron

Barcelona

BARCELONA

Hematology

Active (21/08/2025)

Hospital Universitario De Salamanca

Salamanca

SALAMANCA

Hematology

Active (16/09/2025)

Hospital Universitario Virgen De La Victoria

Malaga

MÁLAGA

Hematology & Hemotherapy

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

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Badalona

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Hematology

Active (28/11/2025)

Institut Catala D´oncologia

L'hospitalet De Llobregat

BARCELONA

Hematology

Medication

PRD11032047
TABLET
ORAL USE

-

Active Principles: N/A

Orphan **Experimental**

PRD11032121
TABLET
ORAL USE

-

Active Principles: N/A

Orphan **Experimental**

PRD11032087
TABLET
ORAL USE

-

Active Principles: N/A

Orphan **Experimental**

No results