Estudio para investigar la seguridad y la eficacia del pirtobrutinib en adultos con trombocitopenia inmunitaria

Estado	Tipo de Participantes	Rangos de Edad
Reclutando	No aportado	Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase I , Fase II	33
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	seguridad, eficacia, farmacocinética, dosis	NO

Información

Identificador
2024-518502-40-00 (CTIS)

Cod. Protocolo
J2N-MC-JZNZ

Área terapéutica
Enfermedades [C] - Patologías del sistema inmunitario [C20]|

Enfermedad investigada
Trombocitopenia inmunitaria

Título Científico
Estudio de fase I/II de determinación de la dosis para investigar la seguridad y la eficacia del pirtobrutinib en adultos con trombocitopenia inmunitaria

Justificación

No aportado

Objetivo Principal

Fase I: evaluar el perfil de seguridad y la tolerabilidad del pirtobrutinib en participantes con TI y seleccionar las dosis para la parte de fase II de este estudio.
Fase II: evaluar la eficacia del pirtobrutinib en comparación con el placebo en participantes con TI.

Variables de Evaluación Primaria

Fase I: TLD.
Fase I: otros criterios de valoración de la seguridad, incluidos, entre otros, AADT, AAG, análisis clínicos, constantes vitales y ECG.
Fase II: tasa de respuesta plaquetaria estable, definida como la proporción de participantes que logran una cifra de trombocitos de ¿50 k/¿l en, al menos, 4 de las 6 visitas cada dos semanas consecutivas entre las semanas 14 y 24 en ausencia de tratamiento de rescate y medicamentos simultáneos prohibidos que puedan influir en la eficacia si se toman entre las semanas 14 y 24

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

Fase I: evaluar la eficacia preliminar del pirtobrutinib en participantes con TI.
Fase I: evaluar el grado de control de la enfermedad.
Fase I: describir la farmacocinética del pirtobrutinib en participantes con TI.
Fase II: realizar una evaluación adicional de la eficacia del pirtobrutinib en comparación con el placebo en participantes con TI.
Fase II: evaluar el grado de control de la enfermedad con el pirtobrutinib en comparación con el placebo en participantes con TI.
Fase II: describir el uso de medicación de rescate con el pirtobrutinib en comparación con el placebo en participantes con TI.
Fase II: evaluar la seguridad del pirtobrutinib en participantes con TI.
Fase II: describir la farmacocinética del pirtobrutinib en participantes con TI

Variables de Evaluación Secundaria

Phase 1: Platelet response rate defined as proportion of participants who achieve at least 2 consecutive platelet counts of 50 k/L and an increase from baseline of 20 k/L to any time during treatment without the use of rescue medication or prohibited concomitant medication that may impact efficacy within 4 weeks prior to the latest elevated platelet count

Phase 1: Number of cumulative weeks with platelet counts 50 k/L by Week 12
Phase 1: Plasma concentrations of pirtobrutinib
Phase 2: Platelet response rate is defined as the proportion of participants with 2 consecutive platelet counts 50 k/L and an increase of platelet count of 20 k/L from baseline to any time during treatment or follow-up without the use of rescue medication or prohibited concomitant medication that may impact efficacy within 4 weeks prior to the latest elevated platelet count (i.e. second consecutive platelet count 50 k/L)
Phase 2: Number of cumulative weeks with platelet counts 50 k/L . Number of cumulative weeks with platelet counts 100 k/L
Phase 2: Proportion of participants requiring rescue therapy
Phase 2: Summary of safety data, including but not limited to the number and incidence of TEAEs, SAEs, and discontinuations due to AEs
Phase 2: Plasma concentrations of pirtobrutinib
Phase 2: Platelet response as defined by the IWG as the proportion of participants with platelet count 30 k/L and at least doubled from baseline in the absence of bleeding for 50% of assessments between Week 1 and Week 24.

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Tener 18 años o más.
Contar con un diagnóstico confirmado de TI primaria.
Tener antecedentes documentados de respuesta a, al menos, 1 tratamiento para la TI anterior.
No haber respondido a tratamientos para la TI primaria anteriores

Criterios de Exclusión

Tener antecedentes de coágulos sanguíneos u obstrucciones en vasos sanguíneos en los últimos 12 meses.
Haber recibido una transfusión de sangre o hemoderivados o plasmaféresis en los 14 días anteriores a la parte de fase I del estudio o en los 28 días anteriores a la parte de fase II del estudio.
Tener antecedentes de una vasculopatía significativa.
Contar con un diagnóstico o antecedentes de un cáncer relacionado con la sangre

Calendario

(Última actualización: 04/09/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
27/06/2025	13/08/2025	21/08/2025	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

Eli Lilly & Co. United States

1 Lilly Corporate Center

Contact Person

Lilly Clinical Trials information desk

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Monetary support: N/A

Tipo de promotor: Comercial

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Activo (13/08/2025)

Clinica Universidad De Navarra

Madrid

MADRID

Hematology

Activo (13/08/2025)

Clinica Universidad De Navarra

Pamplona

NAVARRA

Hematology

Activo (23/09/2025)

Hospital Clinic De Barcelona

Barcelona

BARCELONA

Hematology

Activo (29/08/2025)

Hospital General Universitario Morales Meseguer

Murcia

MURCIA

Hematology

Activo (18/08/2025)

Hospital Universitario De Burgos

Burgos

BURGOS

Hematology

Medicamentos

Pirtobrutinib
TABLET
ORAL USE

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Principios Activos: N/A

Huérfano Experimental

Sin resultados

A Study to Investigate the Safety and Efficacy of Pirtobrutinib in Adults with Immune Thrombocytopenia

State	Type of participants	Age Ranges
Recruiting	Not provided	Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase I , Phase II	33
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	safety, effectiveness, pharmacokinetics, dose	NO

Information

Identifier
2024-518502-40-00 (CTIS)

Protocol Code
J2N-MC-JZNZ

Therapeutic area
Diseases [C] - Immune System Diseases [C20]|

Investigated Disease
Immune Thrombocytopenia

Scientific Title
A Phase 1/2, Dose-finding Study Investigating the Safety and Efficacy of Pirtobrutinib in Adults with Immune Thrombocytopenia

Rationale

Not provided

Main Objective

Phase 1: To assess the safety profile and tolerability of pirtobrutinib in participants with ITP and select the doses for the Phase 2 part of this study
Phase 2: To evaluate the efficacy of pirtobrutinib versus placebo in participants with ITP

Primary Endpoints

Phase 1: DLTs
Phase 1: Other safety endpoints, including, but not limited to, TEAEs, SAEs, clinical laboratory tests, vital signs, and ECGs
Phase 2: Stable platelet response rate is defined as the proportion of participants achieving platelet count of 50 k/L on at least 4 of the 6 consecutive biweekly visits between Weeks 14 and 24 in the absence of rescue therapy and prohibited concomitant medication that may impact efficacy taken between Weeks 14-24

Temporary moments of secondary assessment

Not provided

Secondary Objective

Phase 1: To evaluate the preliminary efficacy of pirtobrutinib in participants with ITP
Phase 1: To evaluate the extent of disease control
Phase 1: To describe the pharmacokinetics of pirtobrutinib in participants with ITP
Phase 2: To assess additional efficacy of pirtobrutinib versus placebo in participants with ITP
Phase 2: To evaluate the extent of disease control of pirtobrutinib versus placebo in participants with ITP
Phase 2: To describe the use of rescue medications of pirtobrutinib versus placebo in participants with ITP
Phase 2: To assess the safety of pirtobrutinib in participants with ITP
Phase 2: To describe the pharmacokinetics of pirtobrutinib in participants with ITP

Secondary Endpoints

Phase 1: Platelet response rate defined as proportion of participants who achieve at least 2 consecutive platelet counts of 50 k/L and an increase from baseline of 20 k/L to any time during treatment without the use of rescue medication or prohibited concomitant medication that may impact efficacy within 4 weeks prior to the latest elevated platelet count
Phase 1: Number of cumulative weeks with platelet counts 50 k/L by Week 12
Phase 1: Plasma concentrations of pirtobrutinib
Phase 2: Platelet response rate is defined as the proportion of participants with 2 consecutive platelet counts 50 k/L and an increase of platelet count of 20 k/L from baseline to any time during treatment or follow-up without the use of

rescue medication or prohibited concomitant medication that may impact efficacy within 4 weeks prior to the latest elevated platelet count (i.e. second consecutive platelet count 50 k/L)
Phase 2: Number of cumulative weeks with platelet counts 50 k/L . Number of cumulative weeks with platelet counts 100 k/L
Phase 2: Proportion of participants requiring rescue therapy
Phase 2: Summary of safety data, including but not limited to the number and incidence of TEAEs, SAEs, and discontinuations due to AEs
Phase 2: Plasma concentrations of pirtobrutinib
Phase 2: Platelet response as defined by the IWG as the proportion of participants with platelet count 30 k/L and at least doubled from baseline in the absence of bleeding for 50% of assessments between Week 1 and Week 24.

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Are 18 years of age or older
Have a confirmed diagnosis of primary ITP
Have documented history of response to at least 1 previous treatment for ITP
Have not responded to previous treatments for primary ITP

Exclusion criteria

Have a history of any blood clots or blockages in their blood vessels in the last 12 months
Had a transfusion with blood or blood products or plasmapheresis within 14 days of the Phase 1 part of the study or within 28 days for the Phase 2 part of the study
Have a history of significant cardiovascular disease
Have a diagnosis or history of a blood-related cancer

Calendar

(Last Update: 04/09/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
27/06/2025	13/08/2025	21/08/2025	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

Eli Lilly & Co. United States

1 Lilly Corporate Center

Contact Person

Lilly Clinical Trials information desk

00353214232400

EU_lilly_clinical_trials@lilly.com

Monetary support: N/A

Sponsor type: Commercial

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Sites

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Hematology

Active (29/08/2025)

Hospital General Universitario Morales Meseguer

Murcia

MURCIA

Hematology

Active (18/08/2025)

Hospital Universitario De Burgos

Burgos

BURGOS

Hematology

Medication

Pirtobrutinib
TABLET
ORAL USE

-

Active Principles: N/A

Orphan Experimental

No results