Efficacy, safety and pharmacokinetics of rilzabrutinib in patients with warm autoimmune hemolytic anemia

Estado	**Tipo de Participantes**	**Rangos de Edad**
EC Finalizado	No aportado	Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase II	7
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	No
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	tratamiento, seguridad, eficacia, farmacocinética, farmacodinamica	NO

Información

Identificador
2023-509441-13-00 (CTIS)

Cod. Protocolo
ACT17209

Transicionado 2021-001671-16

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

Enfermedad investigada
Warm autoimmune hemolytic anemia

Título Científico
A multicenter, open-label, Phase IIb study to evaluate the efficacy, safety and pharmacokinetics of rilzabrutinib in patients with warm autoimmune hemolytic anemia (LUMINA 2)

Justificación

No aportado

Objetivo Principal

Part A: To evaluate the efficacy of rilzabrutinib in adult patients with wAIHA
Part B: To evaluate the long-term efficacy of rilzabrutinib in patients with wAIHA

Variables de Evaluación Primaria

Proportion of participants with overall hemoglobin response
Proportion of participants who maintain durable response achieved during Part A or achieve a durable response during Part B and have a hemoglobin response

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

Part A: To evaluate the durable hemoglobin response in adult patients with wAIHA
Part A: To assess time to response (TTR)
Parts A and B: To assess the effect of treatment with rilzabrutinib on rescue medication requirement in patients with wAIHA
Parts A and B: To evaluate the impact of rilzabrutinib treatment on fatigue
Parts A and B: To evaluate the safety and tolerability of rilzabrutinib in patients with wAIHA

Variables de Evaluación Secundaria

Proportion of participants with durable hemoglobin response
Median time from baseline to first hemoglobin response
Frequency of rescue therapy (any wAIHA-directed therapy other than predniso[lo]ne or transfusion) received
Change from baseline in FACIT-Fatigue scale score
Safety assessments

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Male and female patients with a confirmed diagnosis of primary wAIHA or systemic lupus erythematosus (SLE)-associated wAIHA (without other SLE-related manifestations apart from cutaneous and musculoskeletal manifestations) Participants who have previously failed to maintain a sustained response after treatment with corticosteroids Eastern Cooperative Oncology Group (ECOG) performance status grade 2 or lower Up-to-date vaccination status as per local guideline Body mass index (BMI) >17.5 and <40 kg/m2 All contraceptive use by men and women should be consistent with local regulations regarding the methods of contraception for those participating in clinical studies Core Part B: Evidence of treatment efficacy to rilzabrutinib as defined by achieving overall response during Part A Core Part B: - Completion of Part A treatment period (24 weeks) Extended Part B: Completion of Core Part B period.

Criterios de Exclusión

Clinically significant medical history or ongoing chronic illness that would jeopardize the safety of the participant or compromise the quality of the data derived from his or her participation in the study as determined by the Investigator
Part B only: Presence of unacceptable side effect(s) or toxicity associated with rilzabrutinib such that there is an unfavorable risk-benefit assessment for continued treatment with rilzabrutinib in the opinion of the Investigator and/or Sponsor
Participants with medical history of lymphoma, leukemia, or any malignancy within the past 5 years except for basal cell or squamous epithelial carcinomas of the skin that have been resected with no evidence of metastatic disease for the past 3 years
Secondary wAIHA from any cause including drugs, lymphoproliferative disorders (low-count monoclonal B-cell lymphocytosis is allowed), infectious or autoimmune disease, or active hematologic malignancies. Participants with positive antinuclear antibodies but without a definitive diagnosis of an autoimmune disease are allowed
Myelodysplastic syndrome
Uncontrolled or active HBV infection: Patients with positive HBsAg and/or HBV DNA
HIV infection
Concurrent treatment with other experimental drugs or participation in another clinical trial with any investigational drug within 30 days or 5 half-lives, whichever is greater, prior to treatment start
Known allergy to any of the study medications, their analogues, or excipients in the various formulations of any agent
Part B only: Participants who receive any therapy during Part A known to be active in wAIHA

Calendario

(Última actualización: 27/04/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
08/10/2021	03/01/2023	03/01/2023	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
06/11/2023	No aportado	No aportado	10/06/2025	No aportado

Promotor

Sanofi-Aventis Recherche & Developpement France

82 Avenue Raspail

Contact Person

Clinical Sciences and Operations

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Monetary support: N/A

Tipo de promotor: Comercial

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Centros

Cerrado (10/06/2025)

HOSPITAL CLINIC DE BARCELONA

Barcelona

BARCELONA

Servicio de Hematología

Cerrado (17/02/2025)

HOSPITAL UNIVERSITARIO CRUCES

Barakaldo

VIZCAYA/BIZKAIA

Cerrado (15/02/2024)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Servicio de Hematología

Cerrado (20/02/2024)

HOSPITAL UNIVERSITARIO VIRGEN DEL ROCIO

Sevilla

SEVILLA

Servicio de Hematología

Medicamentos

Rilzabrutinib
TABLET
ORAL USE

-

Principios Activos: N/A

Experimental

Sin resultados

Efficacy, safety and pharmacokinetics of rilzabrutinib in patients with warm autoimmune hemolytic anemia

State	Type of participants	Age Ranges
Finished CT	Not provided	Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase II	7
Results	**Low level of intervention**	**Rare disease**
No results	No	No
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	treatment, safety, effectiveness, pharmacokinetics, pharmacodynamics	NO

Information

Identifier
2023-509441-13-00 (CTIS)

Protocol Code
ACT17209

Transitioned 2021-001671-16

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

Investigated Disease
Warm autoimmune hemolytic anemia

Scientific Title
A multicenter, open-label, Phase IIb study to evaluate the efficacy, safety and pharmacokinetics of rilzabrutinib in patients with warm autoimmune hemolytic anemia (LUMINA 2)

Rationale

Not provided

Main Objective

Part A: To evaluate the efficacy of rilzabrutinib in adult patients with wAIHA
Part B: To evaluate the long-term efficacy of rilzabrutinib in patients with wAIHA

Primary Endpoints

Proportion of participants with overall hemoglobin response
Proportion of participants who maintain durable response achieved during Part A or achieve a durable response during Part B and have a hemoglobin response

Temporary moments of secondary assessment

Not provided

Secondary Objective

Part A: To evaluate the durable hemoglobin response in adult patients with wAIHA
Part A: To assess time to response (TTR)
Parts A and B: To assess the effect of treatment with rilzabrutinib on rescue medication requirement in patients with wAIHA
Parts A and B: To evaluate the impact of rilzabrutinib treatment on fatigue
Parts A and B: To evaluate the safety and tolerability of rilzabrutinib in patients with wAIHA

Secondary Endpoints

Proportion of participants with durable hemoglobin response
Median time from baseline to first hemoglobin response
Frequency of rescue therapy (any wAIHA-directed therapy other than predniso[lo]ne or transfusion) received
Change from baseline in FACIT-Fatigue scale score
Safety assessments

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Male and female patients with a confirmed diagnosis of primary wAIHA or systemic lupus erythematosus (SLE)-associated wAIHA (without other SLE-related manifestations apart from cutaneous and musculoskeletal manifestations) Participants who have previously failed to maintain a sustained response after treatment with corticosteroids Eastern Cooperative Oncology Group (ECOG) performance status grade 2 or lower Up-to-date vaccination status as per local guideline Body mass index (BMI) >17.5 and <40 kg/m2 All contraceptive use by men and women should be consistent with local regulations regarding the methods of contraception for those participating in clinical studies Core Part B: Evidence of treatment efficacy to rilzabrutinib as defined by achieving overall response during Part A Core Part B: - Completion of Part A treatment period (24 weeks) Extended Part B: Completion of Core Part B period.

Exclusion criteria

Clinically significant medical history or ongoing chronic illness that would jeopardize the safety of the participant or compromise the quality of the data derived from his or her participation in the study as determined by the Investigator
Part B only: Presence of unacceptable side effect(s) or toxicity associated with rilzabrutinib such that there is an unfavorable risk-benefit assessment for continued treatment with rilzabrutinib in the opinion of the Investigator and/or Sponsor
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Part B only: Participants who receive any therapy during Part A known to be active in wAIHA

Calendar

(Last Update: 27/04/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
08/10/2021	03/01/2023	03/01/2023	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
06/11/2023	Not aported	Not aported	10/06/2025	Not aported

Sponsor

Sanofi-Aventis Recherche & Developpement France

82 Avenue Raspail

Contact Person

Clinical Sciences and Operations

8006331610

contact-us@sanofi.com

Monetary support: N/A

Sponsor type: Commercial

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Sites

Closed (10/06/2025)

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Sevilla

SEVILLA

Servicio de Hematología

Medication

Rilzabrutinib

TABLET

ORAL USE

-

Active Principles: N/A

Experimental

No results