A Study to Evaluate Overall Health, Physical Activity and Joint Outcomes, in Participants with Severe or Moderate Hemophilia A without FVIII Inhibitors on Emicizumab Prophylaxis

Estado	**Tipo de Participantes**	**Rangos de Edad**
EC Finalizado	No aportado	Mayores de 64 , Adultos (18-64 años) , Adolescentes (12-17 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase IV	91
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	No
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	seguridad, eficacia	NO

Información

Identificador

2023-505747-40-00 (CTIS)

Cod. Protocolo

MO42623

Transicionado 2020-005092-13

Área terapéutica

Enfermedades [C] - Hematología [C15]|

Enfermedad investigada

Severe or Moderate Hemophilia A

Título Científico

A Multicenter, Open-Label Phase IV Study to Evaluate Overall Health, Physical Activity, and Joint Outcomes, in Participants Aged 13 and < 70 Years with Severe or Moderate Hemophilia a Without FVIII Inhibitors on Emicizumab Prophylaxis

Justificación

No aportado

Objetivo Principal

To evaluate the impact of emicizumab treatment on joint health and health-related quality of life (HRQoL) outcomes as well as physical activity of participants with hemophilia A

Variables de Evaluación Primaria

1. Joint status over time based on centrally reviewed Haemophilia Early Arthropathy Detection with Ultrasound (HEAD-US) scores with a specific focus on the synovitis score in participants with synovitis
2. Clinical joint status over time based on the Hemophilia Joint Health Score (HJHS v2.1), excluding gait assessment
3. Joint status at screening and month 36 based on centrally reviewed International Prophylaxis Study Group (IPSG) score (with MRI)
4. Number of problem joints and proportion of problem joints, defined as joints having chronic joint pain and/or limited range of movement due to compromised joint integrity (i.e., chronic synovitis and/or hemophilic arthropathy) with or without persistent bleeding, over time
5. Number of target joint bleeds over time (target joints are defined as joints with 3 bleeds occurring in the same joint during the last 24 weeks)
6. HRQoL, as assessed through use of the Comprehensive Assessment Tool of Challenges in Hemophilia (CATCH) Questionnaire over time with a focus on the following domains: risk perception of recreational activities, restrictions experienced in recreational activities, preoccupation with disease, impact of treatment burden on HRQoL, and pain severity
7. Change in the level of physical activity during the study as measured with a wearable activity tracker (Fitbit)
8. Change in daily step count, active minutes metabolic equivalents of tasks (METs), moderate to vigorous physical activity (MVPA; as per activity tracker default categorization), and type of physical activities
9. Change in the time and intensity level of physical activity as measured by the International Physical Activity Questionnaire Short Format (IPAQ-SF)
10. Number of all bleeds (i.e., those treated and untreated with FVIII), treated bleeds, spontaneous bleeds, joint bleeds, treated joint bleeds, and target joint bleeds (i.e., bleed rate) over time (ABR) as assessed through use of the Bleed and Medication Questionnaire (BMQ)
11. Participant preference for emicizumab compared with previous FVIII regimen, as assessed through use of the Emicizumab Preference Survey (EmiPref) at month 6

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To evaluate the safety of emicizumab
To evaluate the immune response to emicizumab

Variables de Evaluación Secundaria

1. Incidence and severity of adverse events, with severity determined according to World Health Organization (WHO) toxicity scale
2. Incidence of thromboembolic events
3. Incidence of thrombotic microangiopathy
4. Incidence of severe hypersensitivity, anaphylaxis, and anaphylactoid events
5. Incidence and severity of injection-site reactions
6. Prevalence of anti-drug antibodies (ADAs) against emicizumab at baseline and incidence of ADAs against emicizumab during the study
7. Number and proportion of participants who develop anti-FVIII inhibitors (titer 0.6 BU/mL) at specified timepoints

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Diagnosis of severe congenital hemophilia A (intrinsic FVIII level <1%) or moderate congenital hemophilia A (intrinsic FVIII level 5%) if previously prescribed prophylaxis A negative test for FVIII inhibitor (i.e., <0.6 BU) during screening period No history of FVIII inhibitory antibodies (<0.6 BU/mL using the Bethesda assay) in the last 5 years. Participants who completed successful immune tolerance induction (ITI) at least 5 years before screening are eligible, provided they have had no evidence of inhibitor recurrence (permanent or temporary) as may be indicated by detection of an inhibitor, FVIII half-life <6 hours, or FVIII recovery < 66% since completing ITI Participants who were on standard FVIII prophylaxis, defined as the regular administration of FVIII to prevent bleeding, for at least the last 24 weeks, can be enrolled regardless of the number of bleeds during this period Adequate hematologic, hepatic and renal function For women of childbearing potential: agreement to remain abstinent (refrain from heterosexual intercourse) or use contraception during the treatment period and for at least 24 weeks after the final dose of emicizumab

Criterios de Exclusión

Inherited or acquired bleeding disorder other than severe congenital hemophilia A (intrinsic FVIII level <1%) or moderate congenital hemophilia A (intrinsic FVIII level 5%) without FVIII inhibitors who were previously prescribed prophylaxis for at least 24 weeks Participants who have previously received emicizumab prophylaxis Participants that plan to have joint replacement, joint procedure, synovectomy or synoviorthesis at screening Participants who had joint replacement, joint procedure, synovectomy or synoviorthesis: Less than 2 years ago OR More than 3 years ago and are still experiencing pain in the joint For participants who had joint replacement, joint procedure, synovectomy or synoviorthesis more than 2 years ago who are not experiencing pain in the joint, the participant may be enrolled but the specific joint in which the procedure was conducted will be excluded from the study Participants who have conditions other than hemophilia A that can affect joint health and structure (e.g., osteoarthritis) or with severely impaired mobility due to conditions other than hemophilia A

Calendario

(Última actualización: 17/06/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
08/10/2021	05/05/2022	03/06/2022	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
04/12/2023	No aportado	No aportado	10/06/2026	No aportado

Promotor

F. Hoffmann-La Roche AG Switzerland

Grenzacherstrasse 124

Contact Person

Trial Information System - TISL

41616881111

global.eudract@roche.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Hospital Universitario La Paz

Paseo de la Castellana, 261 28046 Madrid

ceic.hulp@salud.madrid.org

917277413

Centros

No iniciado (06/10/2021)

Complejo Hospitalario La Paz

Madrid

MADRID

Servicio de Hematologia

No iniciado (23/12/2022)

COMPLEXO HOSPITALARIO UNIVERSITARIO A CORUÑA

Coruña, A

CORUÑA

Hematología

No iniciado (06/10/2021)

HOSPITAL DE LA SANTA CREU I SANT PAU

Barcelona

BARCELONA

Servicio de Hematología

Activo (05/05/2022)

Hospital Regional Universitario de Málaga

Málaga

MÁLAGA

Servicio de Hematología

No iniciado (14/02/2022)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

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

Hospital Universitario Regional De Malaga

Malaga

MÁLAGA

Hematologia

Medicamentos

Hemlibra 150 mg/mL solution for injection
SOLUTION FOR INJECTION
SUBCUTANEOUS INJECTION

Código ATC: B02BX06 - EMICIZUMAB
Principios Activos: N/A

Experimental

Sin resultados

A Study to Evaluate Overall Health, Physical Activity and Joint Outcomes, in Participants with Severe or Moderate Hemophilia A without FVIII Inhibitors on Emicizumab Prophylaxis

State	Type of participants	Age Ranges
Finished CT	Not provided	Older than 64 , Adults (18-64 years) , Teens (12-17 years)
Gender	**Phases**	**Expected Participants**
Both	Phase IV	91
Results	**Low level of intervention**	**Rare disease**
No results	No	No
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	safety, effectiveness	NO

Information

Identifier
2023-505747-40-00 (CTIS)

Protocol Code
MO42623

Transitioned 2020-005092-13

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

Investigated Disease
Severe or Moderate Hemophilia A

Scientific Title
A Multicenter, Open-Label Phase IV Study to Evaluate Overall Health, Physical Activity, and Joint Outcomes, in Participants Aged 13 and < 70 Years with Severe or Moderate Hemophilia a Without FVIII Inhibitors on Emicizumab Prophylaxis

Rationale

Not provided

Main Objective

To evaluate the impact of emicizumab treatment on joint health and health-related quality of life (HRQoL) outcomes as well as physical activity of participants with hemophilia A

Primary Endpoints

1. Joint status over time based on centrally reviewed Haemophilia Early Arthropathy Detection with Ultrasound (HEAD-US) scores with a specific focus on the synovitis score in participants with synovitis
2. Clinical joint status over time based on the Hemophilia Joint Health Score (HJHS v2.1), excluding gait assessment
3. Joint status at screening and month 36 based on centrally reviewed International Prophylaxis Study Group (IPSG) score (with MRI)
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6. HRQoL, as assessed through use of the Comprehensive Assessment Tool of Challenges in Hemophilia (CATCH) Questionnaire over time with a focus on the following domains: risk perception of recreational activities, restrictions experienced in recreational activities, preoccupation with disease, impact of treatment burden on HRQoL, and pain severity
7. Change in the level of physical activity during the study as measured with a wearable activity tracker (Fitbit)
8. Change in daily step count, active minutes metabolic equivalents of tasks (METs), moderate to vigorous physical activity (MVPA; as per activity tracker default categorization), and type of physical activities
9. Change in the time and intensity level of physical activity as measured by the International Physical Activity Questionnaire Short Format (IPAQ-SF)
10. Number of all bleeds (i.e., those treated and untreated with FVIII), treated bleeds, spontaneous bleeds, joint bleeds, treated joint bleeds, and target joint bleeds (i.e., bleed rate) over time (ABR) as assessed through use of the Bleed and Medication Questionnaire (BMQ)
11. Participant preference for emicizumab compared with previous FVIII regimen, as assessed through use of the Emicizumab Preference Survey (EmiPref) at month 6

Temporary moments of secondary assessment

Not provided

Secondary Objective

To evaluate the safety of emicizumab
To evaluate the immune response to emicizumab

Secondary Endpoints

1. Incidence and severity of adverse events, with severity determined according to World Health Organization (WHO) toxicity scale
2. Incidence of thromboembolic events
3. Incidence of thrombotic microangiopathy
4. Incidence of severe hypersensitivity, anaphylaxis, and anaphylactoid events
5. Incidence and severity of injection-site reactions
6. Prevalence of anti-drug antibodies (ADAs) against emicizumab at baseline and incidence of ADAs against emicizumab during the study
7. Number and proportion of participants who develop anti-FVIII inhibitors (titer 0.6 BU/mL) at specified timepoints

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Diagnosis of severe congenital hemophilia A (intrinsic FVIII level <1%) or moderate congenital hemophilia A (intrinsic FVIII level 5%) if previously prescribed prophylaxis A negative test for FVIII inhibitor (i.e., <0.6 BU) during screening period No history of FVIII inhibitory antibodies (<0.6 BU/mL using the Bethesda assay) in the last 5 years. Participants who completed successful immune tolerance induction (ITI) at least 5 years before screening are eligible, provided they have had no evidence of inhibitor recurrence (permanent or temporary) as may be indicated by detection of an inhibitor, FVIII half-life <6 hours, or FVIII recovery < 66% since completing ITI Participants who were on standard FVIII prophylaxis, defined as the regular administration of FVIII to prevent bleeding, for at least the last 24 weeks, can be enrolled regardless of the number of bleeds during this period Adequate hematologic, hepatic and renal function For women of childbearing potential: agreement to remain abstinent (refrain from heterosexual intercourse) or use contraception during the treatment period and for at least 24 weeks after the final dose of emicizumab

Exclusion criteria

Inherited or acquired bleeding disorder other than severe congenital hemophilia A (intrinsic FVIII level <1%) or moderate congenital hemophilia A (intrinsic FVIII level 5%) without FVIII inhibitors who were previously prescribed prophylaxis for at least 24 weeks Participants who have previously received emicizumab prophylaxis Participants that plan to have joint replacement, joint procedure, synovectomy or synoviorthesis at screening Participants who had joint replacement, joint procedure, synovectomy or synoviorthesis: Less than 2 years ago OR More than 3 years ago and are still experiencing pain in the joint For participants who had joint replacement, joint procedure, synovectomy or synoviorthesis more than 2 years ago who are not experiencing pain in the joint, the participant may be enrolled but the specific joint in which the procedure was conducted will be excluded from the study Participants who have conditions other than hemophilia A that can affect joint health and structure (e.g., osteoarthritis) or with severely impaired mobility due to conditions other than hemophilia A

Calendar

(Last Update: 17/06/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
08/10/2021	05/05/2022	03/06/2022	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
04/12/2023	Not aported	Not aported	10/06/2026	Not aported

Sponsor

F. Hoffmann-La Roche AG Switzerland

Grenzacherstrasse 124

Contact Person

Trial Information System - TISL

41616881111

global.eudract@roche.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

CEIm Hospital Universitario La Paz

Paseo de la Castellana, 261 28046 Madrid

ceic.hulp@salud.madrid.org

917277413

Sites

not initialized (06/10/2021)

Complejo Hospitalario La Paz

Madrid

MADRID

Servicio de Hematologia

not initialized (23/12/2022)

COMPLEXO HOSPITALARIO UNIVERSITARIO A CORUÑA

Coruña, A

CORUÑA

Hematología

not initialized (06/10/2021)

HOSPITAL DE LA SANTA CREU I SANT PAU

Barcelona

BARCELONA

Servicio de Hematología

Active (05/05/2022)

Hospital Regional Universitario de Málaga

Málaga

MÁLAGA

Servicio de Hematología

not initialized (14/02/2022)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

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

Hospital Universitario Regional De Malaga

Malaga

MÁLAGA

Hematologia

Medication

Hemlibra 150 mg/mL solution for injection
SOLUTION FOR INJECTION
SUBCUTANEOUS INJECTION

ATC code: B02BX06 - EMICIZUMAB
Active Principles: N/A

Experimental

No results