The safety and efficacy of Alpha-1 Antitrypsin (AAT) for the prevention of graftversus-host disease (GVHD) in patients receiving hematopoietic cell transplant.

Estado	**Tipo de Participantes**	**Rangos de Edad**
EC Finalizado	Sujetos incapaces de otorgar consentimiento	Menores de 18 , Mayores de 64 , Adultos (18-64 años) , Adolescentes (12-17 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase II , Fase III	265
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	profilaxis, seguridad, eficacia, farmacocinética, farmacodinamica, dosis	NO

Información

Identificador
2024-511164-92-00 (CTIS)

Cod. Protocolo
CSL964_2001

Transicionado 2018-000329-29

Área terapéutica
Procesos fisiológicos [G] - Sistema inmunitario [G12]|

Enfermedad investigada
Acute Graft versus host disease

Título Científico
A Phase 2/3, Multicenter, randOmized, Double-blind, placebo-controlled, stUdy to evaLuate the safety and efficacy of Alpha-1 AntiTrypsin for the prEvention of graft versus-host disease in patients receiving hematopoietic cell transplant

(MODULAATE Study)

Justificación

No aportado

Objetivo Principal

Primary objective is to evaluate the efficacy of AAT at the selected dose for the prevention of acute GVHD following HCT.

Variables de Evaluación Primaria

The time to Grade II-IV aGVHD or death through 180 days after hematopoietic cell transplantation (HCT).

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

Key Secondary Objective is To evaluate the efficacy of AAT for the prevention of severe aGVHD and infections following HCT. Also, 1. To further evaluate the efficacy of AAT for the prevention of complications after HCT. 2. To evaluate the safety of AAT based on investigational product- (IP)- related AEs. 3. To evaluate the steady state PK of AAT in HCT recipients.

Variables de Evaluación Secundaria

Proportion of subjects with lower GI aGVHD or Grade III-IV aGVHD in any organ. Through 180 days after HCT.
Proportion of subjects with severe infections defined by NCI-CTCAE Grade 3. Through Day 60 after HCT.
Proportion of subjects with Grade II-IV aGVHD or death. Through 100 and 180 days after HCT.
Proportion of subjects with lower GI aGVHD. Through Days 60, 100 and 180 after HCT.
Deaths (relapse and nonrelapse-related). Within 180, 365, and 730 days after HCT.
Proportion of subjects with severe infections defined by NCI-CTCAE Grade 3. Through 100 and 180 days after HCT.
Proportion of subjects with Grade III-IV aGVHD or death. Through Days 60, 100, and 180 days after HCT.
Proportion of subjects with moderate-to-severe chronic GVHD. Within 180, 365, 545, and 730 days after HCT.
Proportion of subjects who have discontinued immune suppression therapies including standard-of-care GVHD prophylaxis and steroid treatment. Within 180 and 365 days after HCT.
Time to neutrophil engraftment. Through 365 days after HCT.
Time to GVHD relapse-free survival. Within 365 and 730 days after HCT.
Proportion of subjects with relapse of primary malignancies. Through 180, 365, and 730 days after HCT.
Proportion of subjects with Grade II-IV aGVHD with an overall (complete + partial) response, complete response and partial response. Approximately 4 weeks after the initiation of systemic steroids during 8-week Treatment Period

Percent of subjects with study drug-related adverse events. Up to 365 days after HCT.
Maximum concentration (Cmax) of AAT. Before and up to 72 after infusion of AAT.
Area under the concentration curve for AAT. Before and up to 72 after infusion of AAT.
Clearance of AAT. Before and up to 72 after infusion of AAT.
Volume of distribution for AAT. Before and up to 72 after infusion of AAT.
Ctrough of AAT. Before and up to 72 after infusion of AAT.

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Male or female subjects, 12 years of age (18 years of age for subjects at German sites only), undergoing HCT for hematological malignancies, including leukemia, lymphoma multiple myeloma, myelodysplastic syndrome and myeloproliferative neoplasms.
Planned myeloablative conditioning regimen.
Participants must have a related or unrelated donor as follows: - Related donor must be a 6 / 6 match for human leukocyte antigen (HLA)-A, -B, at intermediate (or higher) resolution, and -DR beta 1 (DRB1) at high resolution using deoxyribonucleic acid (DNA)-based typing. - Unrelated donor must be 7 / 8 or 8 / 8 match for HLA-A, -B, and -C at intermediate (or higher) resolution, and -DRB1 at high resolution using DNA-based typing.

Criterios de Exclusión

Prior autologous or allogeneic HCT.
T-cell depleted transplant or planned use of anti-T cell antibody therapy either ex vivo or in vivo (ie, antithymocyte globulin [ATG], alemtuzumab) for GVHD prophylaxis.
Planned umbilical cord blood transplant.
Planned use of cyclophosphamide after HCT for GVHD prophylaxis.
Planned haploidentical donor.

Calendario

(Última actualización: 16/07/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
28/01/2019	17/05/2019	08/10/2019	13/01/2021	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
No aportado	27/11/2025	No aportado	No aportado	No aportado

Promotor

CSL Behring LLC United States

1020 1st Avenue

Contact Person

Study Director

+16108784697

clinicaltrials@cslbehring.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Hospital Universitari de Bellvitge

Feixa Llarga, s/n - Antiguo Módulo Banco de Santander 08907 Hospitalet de Llobregat

presidenciaceic@bellvitgehospital.cat

Centros

Cerrado (08/07/2026)

Hospital Universitario De Salamanca

Salamanca

SALAMANCA

Hematology

Cerrado (12/05/2026)

HOSPITAL UNIVERSITARIO MARQUÉS DE VALDECILLA

Santander

CANTABRIA

Hematology

Cerrado (21/05/2026)

Vall D'hebron Institut De Recerca

Barcelona

BARCELONA

Hematology

Medicamentos

Respreeza 1,000 mg powder and solvent for solution for infusion.

SOLUTION FOR INFUSION

INTRAVENOUS

Código ATC: B02AB02 - ALFA1 ANTITRIPSINA

Principios Activos: N/A

Experimental

Sin resultados

The safety and efficacy of Alpha-1 Antitrypsin (AAT) for the prevention of graftversus-host disease (GVHD) in patients receiving hematopoietic cell transplant.

State	Type of participants	Age Ranges
Finished CT	Incapable subjects of giving consent	Less than 18 , Older than 64 , Adults (18-64 years) , Teens (12-17 years)
Gender	**Phases**	**Expected Participants**
Both	Phase II , Phase III	265
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	prophylaxis, safety, effectiveness, pharmacokinetics, pharmacodynamics, dose	NO

Information

Identifier
2024-511164-92-00 (CTIS)

Protocol Code
CSL964_2001

Transitioned 2018-000329-29

Therapeutic area
Body processes [G] - Immune system processes [G12]|

Investigated Disease
Acute Graft versus host disease

Scientific Title
A Phase 2/3, Multicenter, randOmized, Double-blind, placebo-controlled, stUdy to evaLuate the safety and efficacy of Alpha-1 AntiTrypsin for the prEvention of graft versus-host disease in patients receiving hematopoietic cell transplant

(MODULAATE Study)

Rationale

Not provided

Main Objective

Primary objective is to evaluate the efficacy of AAT at the selected dose for the prevention of acute GVHD following HCT.

Primary Endpoints

The time to Grade II-IV aGVHD or death through 180 days after hematopoietic cell transplantation (HCT).

Temporary moments of secondary assessment

Not provided

Secondary Objective

Key Secondary Objective is To evaluate the efficacy of AAT for the prevention of severe aGVHD and infections following HCT. Also, 1. To further evaluate the efficacy of AAT for the prevention of complications after HCT. 2. To evaluate the safety of AAT based on investigational product- (IP)- related AEs. 3. To evaluate the steady state PK of AAT in HCT recipients.

Secondary Endpoints

Proportion of subjects with lower GI aGVHD or Grade III-IV aGVHD in any organ. Through 180 days after HCT.
Proportion of subjects with severe infections defined by NCI-CTCAE Grade 3. Through Day 60 after HCT.
Proportion of subjects with Grade II-IV aGVHD or death. Through 100 and 180 days after HCT.
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Clearance of AAT. Before and up to 72 after infusion of AAT.
Volume of distribution for AAT. Before and up to 72 after infusion of AAT.
Ctrough of AAT. Before and up to 72 after infusion of AAT.

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Male or female subjects, 12 years of age (18 years of age for subjects at German sites only), undergoing HCT for hematological malignancies, including leukemia, lymphoma multiple myeloma, myelodysplastic syndrome and myeloproliferative neoplasms.
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Exclusion criteria

Prior autologous or allogeneic HCT.
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Planned umbilical cord blood transplant.
Planned use of cyclophosphamide after HCT for GVHD prophylaxis.
Planned haploidentical donor.

Calendar

(Last Update: 16/07/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
28/01/2019	17/05/2019	08/10/2019	13/01/2021	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
Not aported	27/11/2025	Not aported	Not aported	Not aported

Sponsor

CSL Behring LLC United States

1020 1st Avenue

Contact Person

Study Director

+16108784697

clinicaltrials@cslbehring.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

CEIm Hospital Universitari de Bellvitge

Feixa Llarga, s/n - Antiguo Módulo Banco de Santander 08907 Hospitalet de Llobregat

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Sites

Closed (08/07/2026)

Hospital Universitario De Salamanca

Salamanca

SALAMANCA

Hematology

Closed (12/05/2026)

HOSPITAL UNIVERSITARIO MARQUÉS DE VALDECILLA

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Hematology

Closed (21/05/2026)

Vall D'hebron Institut De Recerca

Barcelona

BARCELONA

Hematology

Medication

Respreeza 1,000 mg powder and solvent for solution for infusion.

SOLUTION FOR INFUSION

INTRAVENOUS

ATC code: B02AB02 - ALFA1 ANTITRIPSINA

Active Principles: N/A

Experimental

No results