EvAluation of the efficacy of MaaT013 as salvage theRapy in acute GVHD patiEntS with gastrointestinal involvement, refractory to ruxolitinib; a multi-center open-label phase III trial.

Estado	**Tipo de Participantes**	**Rangos de Edad**
EC Finalizado	No aportado	Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase III	73
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Multicéntrico internacional	tratamiento, seguridad, eficacia	NO

Información

Identificador
2024-513023-17-00 (CTIS)

Cod. Protocolo
MPOH06

Transicionado 2021-001841-11

Área terapéutica
Enfermedades [C] - Patologías digestivas [C06]|

Enfermedad investigada
Treatment of acute graft-versus host disease (aGvHD) to the gastrointestinal (GI) tract of patients who are resistant or intolerant to ruxolitinib.

Título Científico
EvAluation of the efficacy of MaaT013 as salvage theRapy in acute GVHD patiEntS with gastrointestinal involvement, refractory to ruxolitinib; a multi-center open-label phase III trial.

Justificación

No aportado

Objetivo Principal

To evaluate the efficacy of MaaT013 assessed by the overall response rate (ORR) of gastrointestinal (GI) acute graft-versus-host disease (aGVHD) response at day 28 (D28).

Variables de Evaluación Primaria

To evaluate the efficacy of MaaT013 assessed by the overall response rate (ORR) of gastrointestinal (GI) acute grfat-versus-host disease (aGVHD) response at day 28 (D28).

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To evaluate MaaT013 Safety
To evaluate ORR for GI at D56 and M3
To evaluate ORR for all organs at D28, D56 and M3
To evaluate the best ORR (for GI and all organs) achieved between D0 and D28
To assess duration of response
To assess overall survival (OS)
To assess progression-free survival (PFS)
To assess time to progression
To assess steroid-free survival
To evaluate the frequency of patients that tapered off CS
To measure the incidence of chronic GvHD
To evaluate changes in patient reported outcomes (PROs)
To evaluate MaaT013 activity on immune markers

Variables de Evaluación Secundaria

To evaluate MaaT013 safety
To evalutae ORR for GI at D56 and M3
To evaluate ORR for all organs at D28, D56 and M3
To evaluate the best ORR (for GI and all organs) acheived between D0 and D28
To assess duration of response
To assess overall survival (OS)
To assess progression-free survival (PFS)
To assess time to progression

To assess steroid-free survival
To evaluate the frequency of patients that tapered off CS
To measure the incidence of chronic GvHD
To evaluate changes in patient reported outcomes (PROs)

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Age 18 years old
Allo-HSCT with any type of donor, stem cell source, GvHD prophylaxis or conditioning regimen.
Acute GvHD episode with GI involvement per MAGIC guidelines (= grades II to IV), with or without involvement of other organs (Harris et al. 2016).
Patients resistant to steroids AND either resistant to OR with intolerance to ruxolitinib (intolerant patients: who had grade 3 or higher treatment-emergent and ruxolitinib-attributed adverse event that did not resolve within 7 days of discontinuing ruxolitinib): Resistance to steroids is defined as any of the following: - Lack of improvement (i.e., no decrease in stage in at least 1 involved organ system) after 5 days of treatment with CS at 2mg/kg/d methylprednisolone equivalent dose, - Progression (i.e., increase in any organ system or any new organ involvement) after 3 days of treatment with CS at 2 mg/kg/d methylprednisolone equivalent dose, - Patients treated with 1 mg/kg/d of CS because the physician deemed they would not tolerate 2 mg/kg/d and who correspond to the definition of SR patients, - Patients who previously began CS therapy at a lower dose (at least 1 mg/kg/d methylprednisolone equivalent) for skin or upper GI GvHD but develop new GvHD in another organ system, - Patients who cannot tolerate corticosteroid tapering, i.e., begin of CS at 2.0 mg/kg/d, demonstrate response, but show disease progress before a 50% decrease from the initial starting dose of CS is achieved. Resistance to ruxolitinib is defined as any of the following (Mohty et al. 2020): - Progression of GvHD compared to baseline after at least 5 days of treatment with ruxolitinib, based either on objective increase in stage/grade, or new organ involvement; - Lack of improvement in GvHD (partial response or better) compared to baseline after at least 14 days of treatment with ruxolitinib; - Loss of response, defined as objective worsening of GvHD determined by increase in stage, grade or new organ involvement at any time after initial improvement - Absence of complete response or very good partial response at day 28 after ruxolitinib Intolerance to ruxolitinib is defined as: - GvHD manifestations that persist without improvement in patients who had grade 3 or higher treatment-emergent and ruxolitinib-attributed adverse event that did not resolve within 7 days of discontinuing ruxolitinib.
Signature of informed and written consent by the subject or by the subjects legally acceptable representative for patients under guardianship or trusteeship.

Criterios de Exclusión

Patients with known hypersensitivity to vancomycin or to any of the excipients listed in the corresponding SmPC Absolute neutrophil count <500/L, confirmed within 3 days prior to pre-treatment start. Use of growth factor supplementation is allowed. Absolute platelet count < 10 000/L, confirmed within 3 days prior to pre-treatment start. Use of platelet infusion is allowed. Patient with negative IgG EBV serology. Current or past (< 6 months) evidence of toxic megacolon, bowel obstruction or gastrointestinal perforation. Any condition that would, in the investigator's judgment, interfere with full participation in the study, including administration of study drug and attending required study visits; pose a significant risk to the subject; or interfere with interpretation of study data. Known allergy or intolerance to trehalose or maltodextrin. Vulnerable patients such as: minors, persons deprived of liberty, persons in Intensive Care Unit unable to provide informed consent prior to the intervention. Females of childbearing potential should have a negative urine or serum pregnancy test within 72 hours prior to receiving the first dose of study

medication. Breastfeeding females. Females of childbearing potential should be willing to use an acceptable method of birth control such as hormonal contraception (progestogen-only may be sufficient), male or female condom with or without spermicide, cap, diaphragm or sponge with spermicide for the course of the study. A combination of male condom with either cap, diaphragm or sponge with spermicide (double barrier methods) are also considered acceptable. Females of childbearing potential are those who have not been surgically sterilized or have not been free from menses for >1 year. Other ongoing interventional protocol that might interfere with the current studys primary endpoint. Patients with active CMV colitis Patients who had previously received other lines of systemic aGvHD treatment other than CS and ruxolitinib. Grade II-IV hyper-acute GvHD as defined by the MD Andersons criteria (Saliba et al. 2007) (aGvHD onset within 14 days after allo-HSCT) Overlap chronic GvHD as defined by the NIH Consensus Criteria (Jagasia et al. 2015) Relapsed/persistent malignancy requiring rapid immune suppression withdrawal Active uncontrolled infection according to the attending physician Severe organ dysfunction unrelated to underlying GvHD, including: - Cholestatic disorders or unresolved veno-occlusive disease of the liver (defined as persistent bilirubin abnormalities not attributable to GvHD and ongoing organ dysfunction, with the exception of Gilbert syndrome). - Clinically significant or uncontrolled cardiac disease including unstable angina, acute myocardial infarction within 6 months before Day 1 of study drug administration, New York Heart Association Class III or IV congestive heart failure, circulatory collapse requiring vasopressor or inotropic support that requires therapy. - Clinically significant respiratory disease that requires mechanical ventilation support or 50% oxygen. Current or past (< 6 months) veno-occlusive disease or other uncontrolled complication unless otherwise agreed in writing by the sponsor.

Calendario

(Última actualización: 09/10/2025)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
03/02/2022	16/02/2022	22/03/2022	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
14/10/2024	No aportado	No aportado	24/09/2025	No aportado

Promotor

MaaT PHARMA France

70 Avenue Tony Garnier

Contact Person

Maat-Pharma Contact Point

+33428291400

clinical@maat-pharma.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Fundacio de Gestio Sanitaria Hospital de la Santa Creu i Sant Pau

C/ Sant Quintí, 77-79. Planta 1a. Despacho 95 08041 Barcelona

ceimsantpau@santpau.cat

935537813

Centros

Activo (15/12/2022)

CLINICA UNIVERSIDAD DE NAVARRA

Pamplona/Iruña

NAVARRA

Hematología

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

Clinica Universidad De Navarra

Pamplona

NAVARRA

Hematology

Activo (11/05/2022)

Complejo Asistencial Universitario De Salamanca

Salamanca

SALAMANCA

Hematology

Activo (20/06/2022)

HOSPITAL CLINICO UNIVERSITARIO DE VALENCIA

València

VALENCIA

Hematology

Activo (02/12/2022)

HOSPITAL CLINICO UNIVERSITARIO VIRGEN DE LA ARRIXACA

Murcia

MURCIA

Hematologia

Activo (05/04/2022)

HOSPITAL DE LA SANTA CREU I SANT PAU

Barcelona

BARCELONA

Hematology

Activo (28/09/2022)

HOSPITAL G. UNIVERSITARIO J.M. MORALES MESEGUER

Murcia

MURCIA

Hematology

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

Hospital General Universitario Morales Meseguer

Murcia

MURCIA

Hematology and Medical Oncology Service

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

Hospital Universitario De Salamanca

Salamanca

SALAMANCA

Hematology

Activo (11/01/2023)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Hematología

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

Hospital Universitario La Paz

Madrid

MADRID

Hematology

Activo (06/05/2022)

HOSPITAL UNIVERSITARIO MARQUES DE VALDECILLA

Santander

CANTABRIA

Hematology

Activo (17/11/2022)

HOSPITAL UNIVERSITARIO RAMON Y CAJAL

Madrid

MADRID

Hematología

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

Hospital Universitario Ramon Y Cajal

Madrid

MADRID

Hematology

Activo (18/02/2022)

HOSPITAL UNIVERSITARIO VIRGEN DE LAS NIEVES

Granada

GRANADA

Hematology

Activo (16/02/2022)

HOSPITAL UNIVERSITARIO VIRGEN DEL ROCIO

Sevilla

SEVILLA

Hematology

Activo (25/02/2022)

HOSPITAL UNIVERSITARIO Y POLITECNICO LA FE

València

VALENCIA

Hematology

Activo (07/09/2022)

Institut Catala D'oncologia

Hospitalet de Llobregat, L'

BARCELONA

Hematology

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

University Clinical Hospital Virgen De La Arrixaca

Murcia

MURCIA

Hematology

Medicamentos

MaaT013

RECTAL SOLUTION

RECTAL USE

-

Principios Activos: N/A

Huérfano **Experimental**

Sin resultados

EvAluation of the efficacy of MaaT013 as salvage theRapy in acute GVHD patiEntS with gastrointestinal involvement, refractory to ruxolitinib; a multi-center open-label phase III trial.

State	**Type of participants**	**Age Ranges**
Finished CT	Not provided	Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase III	73
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
International multicenter	treatment, safety, effectiveness	NO

Information

Identifier

2024-513023-17-00 (CTIS)

Protocol Code

MPOH06

Transitioned 2021-001841-11

Therapeutic area

Diseases [C] - Digestive System Diseases [C06]|

Investigated Disease

Treatment of acute graft-versus host disease (aGvHD) to the gastrointestinal (GI) tract of patients who are resistant or intolerant to ruxolitinib.

Scientific Title

EvAluation of the efficacy of MaaT013 as salvage theRapy in acute GVHD patiEntS with gastrointestinal involvement, refractory to ruxolitinib; a multi-center open-label phase III trial.

Rationale

Not provided

Main Objective

To evaluate the efficacy of MaaT013 assessed by the overall response rate (ORR) of gastrointestinal (GI) acute graft-versus-host disease (aGVHD) response at day 28 (D28).

Primary Endpoints

To evaluate the efficacy of MaaT013 assessed by the overall response rate (ORR) of gastrointestinal (GI) acute grfat-versus-host disease (aGVHD) response at day 28 (D28).

Temporary moments of secondary assessment

Not provided

Secondary Objective

To evaluate MaaT013 Safety
To evaluate ORR for GI at D56 and M3
To evaluate ORR for all organs at D28, D56 and M3
To evaluate the best ORR (for GI and all organs) achieved between D0 and D28
To assess duration of response
To assess overall survival (OS)
To assess progression-free survival (PFS)
To assess time to progression
To assess steroid-free survival
To evaluate the frequency of patients that tapered off CS
To measure the incidence of chronic GvHD
To evaluate changes in patient reported outcomes (PROs)
To evaluate MaaT013 activity on immune markers

Secondary Endpoints

To evaluate MaaT013 safety
To evalutae ORR for GI at D56 and M3
To evaluate ORR for all organs at D28, D56 and M3
To evaluate the best ORR (for GI and all organs) acheived between D0 and D28
To assess duration of response
To assess overall survival (OS)
To assess progression-free survival (PFS)
To assess time to progression

To assess steroid-free survival
To evaluate the frequency of patients that tapered off CS
To measure the incidence of chronic GvHD
To evaluate changes in patient reported outcomes (PROs)

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Age 18 years old
Allo-HSCT with any type of donor, stem cell source, GvHD prophylaxis or conditioning regimen.
Acute GvHD episode with GI involvement per MAGIC guidelines (= grades II to IV), with or without involvement of other organs (Harris et al. 2016).
Patients resistant to steroids AND either resistant to OR with intolerance to ruxolitinib (intolerant patients: who had grade 3 or higher treatment-emergent and ruxolitinib-attributed adverse event that did not resolve within 7 days of discontinuing ruxolitinib): Resistance to steroids is defined as any of the following: - Lack of improvement (i.e., no decrease in stage in at least 1 involved organ system) after 5 days of treatment with CS at 2mg/kg/d methylprednisolone equivalent dose, - Progression (i.e., increase in any organ system or any new organ involvement) after 3 days of treatment with CS at 2 mg/kg/d methylprednisolone equivalent dose, - Patients treated with 1 mg/kg/d of CS because the physician deemed they would not tolerate 2 mg/kg/d and who correspond to the definition of SR patients, - Patients who previously began CS therapy at a lower dose (at least 1 mg/kg/d methylprednisolone equivalent) for skin or upper GI GvHD but develop new GvHD in another organ system, - Patients who cannot tolerate corticosteroid tapering, i.e., begin of CS at 2.0 mg/kg/d, demonstrate response, but show disease progress before a 50% decrease from the initial starting dose of CS is achieved. Resistance to ruxolitinib is defined as any of the following (Mohty et al. 2020): - Progression of GvHD compared to baseline after at least 5 days of treatment with ruxolitinib, based either on objective increase in stage/grade, or new organ involvement; - Lack of improvement in GvHD (partial response or better) compared to baseline after at least 14 days of treatment with ruxolitinib; - Loss of response, defined as objective worsening of GvHD determined by increase in stage, grade or new organ involvement at any time after initial improvement - Absence of complete response or very good partial response at day 28 after ruxolitinib Intolerance to ruxolitinib is defined as: - GvHD manifestations that persist without improvement in patients who had grade 3 or higher treatment-emergent and ruxolitinib-attributed adverse event that did not resolve within 7 days of discontinuing ruxolitinib.
Signature of informed and written consent by the subject or by the subjects legally acceptable representative for patients under guardianship or trusteeship.

Exclusion criteria

Patients with known hypersensitivity to vancomycin or to any of the excipients listed in the corresponding SmPC Absolute neutrophil count <500/L, confirmed within 3 days prior to pre-treatment start. Use of growth factor supplementation is allowed. Absolute platelet count < 10 000/L, confirmed within 3 days prior to pre-treatment start. Use of platelet infusion is allowed. Patient with negative IgG EBV serology. Current or past (< 6 months) evidence of toxic megacolon, bowel obstruction or gastrointestinal perforation. Any condition that would, in the investigator's judgment, interfere with full participation in the study, including administration of study drug and attending required study visits; pose a significant risk to the subject; or interfere with interpretation of study data. Known allergy or intolerance to trehalose or maltodextrin. Vulnerable patients such as: minors, persons deprived of liberty, persons in Intensive Care Unit unable to provide informed consent prior to the intervention. Females of childbearing potential should have a negative urine or serum pregnancy test within 72 hours prior to receiving the first dose of study

medication. Breastfeeding females. Females of childbearing potential should be willing to use an acceptable method of birth control such as hormonal contraception (progestogen-only may be sufficient), male or female condom with or without spermicide, cap, diaphragm or sponge with spermicide for the course of the study. A combination of male condom with either cap, diaphragm or sponge with spermicide (double barrier methods) are also considered acceptable. Females of childbearing potential are those who have not been surgically sterilized or have not been free from menses for >1 year. Other ongoing interventional protocol that might interfere with the current studys primary endpoint. Patients with active CMV colitis Patients who had previously received other lines of systemic aGvHD treatment other than CS and ruxolitinib. Grade II-IV hyper-acute GvHD as defined by the MD Andersons criteria (Saliba et al. 2007) (aGvHD onset within 14 days after allo-HSCT) Overlap chronic GvHD as defined by the NIH Consensus Criteria (Jagasia et al. 2015) Relapsed/persistent malignancy requiring rapid immune suppression withdrawal Active uncontrolled infection according to the attending physician Severe organ dysfunction unrelated to underlying GvHD, including: - Cholestatic disorders or unresolved veno-occlusive disease of the liver (defined as persistent bilirubin abnormalities not attributable to GvHD and ongoing organ dysfunction, with the exception of Gilbert syndrome). - Clinically significant or uncontrolled cardiac disease including unstable angina, acute myocardial infarction within 6 months before Day 1 of study drug administration, New York Heart Association Class III or IV congestive heart failure, circulatory collapse requiring vasopressor or inotropic support that requires therapy. - Clinically significant respiratory disease that requires mechanical ventilation support or 50% oxygen. Current or past (< 6 months) veno-occlusive disease or other uncontrolled complication unless otherwise agreed in writing by the sponsor.

Calendar

(Last Update: 09/10/2025)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
03/02/2022	16/02/2022	22/03/2022	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
14/10/2024	Not aported	Not aported	24/09/2025	Not aported

Sponsor

MaaT PHARMA France

70 Avenue Tony Garnier

Contact Person

Maat-Pharma Contact Point

+33428291400

clinical@maat-pharma.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

CEIm Fundacio de Gestio Sanitaria Hospital de la Santa Creu i Sant Pau

C/ Sant Quintí, 77-79. Planta 1a. Despacho 95 08041 Barcelona

ceimsantpau@santpau.cat

935537813

Sites

Active (15/12/2022)

CLINICA UNIVERSIDAD DE NAVARRA

Pamplona/Iruña

NAVARRA

Hematología

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

Clinica Universidad De Navarra

Pamplona

NAVARRA

Hematology

Active (11/05/2022)

Complejo Asistencial Universitario De Salamanca

Salamanca

SALAMANCA

Hematology

Active (20/06/2022)

HOSPITAL CLINICO UNIVERSITARIO DE VALENCIA

València

VALENCIA

Hematology

Active (02/12/2022)

HOSPITAL CLINICO UNIVERSITARIO VIRGEN DE LA ARRIXACA

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MURCIA

Hematologia

Active (05/04/2022)

HOSPITAL DE LA SANTA CREU I SANT PAU

Barcelona

BARCELONA

Hematology

Active (28/09/2022)

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Murcia

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Hematology

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

Hospital General Universitario Morales Meseguer

Murcia

MURCIA

Hematology and Medical Oncology Service

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Hospital Universitario De Salamanca

Salamanca

SALAMANCA

Hematology

Active (11/01/2023)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Hematología

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

Hospital Universitario La Paz

Madrid

MADRID

Hematology

Active (06/05/2022)

HOSPITAL UNIVERSITARIO MARQUES DE VALDECILLA

Santander

CANTABRIA

Hematology

Active (17/11/2022)

HOSPITAL UNIVERSITARIO RAMON Y CAJAL

Madrid

MADRID

Hematología

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

Hospital Universitario Ramon Y Cajal

Madrid

MADRID

Hematology

Active (18/02/2022)

HOSPITAL UNIVERSITARIO VIRGEN DE LAS NIEVES

Granada

GRANADA

Hematology

Active (16/02/2022)

HOSPITAL UNIVERSITARIO VIRGEN DEL ROCIO

Sevilla

SEVILLA

Hematology

Active (25/02/2022)

HOSPITAL UNIVERSITARIO Y POLITECNICO LA FE

València

VALENCIA

Hematology

Active (07/09/2022)

Institut Catala D'oncologia

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BARCELONA

Hematology

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

University Clinical Hospital Virgen De La Arrixaca

Murcia

MURCIA

Hematology

Medication

MaaT013

RECTAL SOLUTION

RECTAL USE

-

Active Principles: N/A

Orphan **Experimental**

No results