A Phase 1/2, Open-Label Study to Evaluate the Safety and Efficacy of Autologous CD19-specific Chimeric Antigen Receptor T cells (CABA-201) in Subjects with Active Systemic Lupus Erythematosus

Estado	Tipo de Participantes	Rangos de Edad
Reclutando	No aportado	Mayores de 64 , Adultos (18-64 años) , Adolescentes (12-17 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase I , Fase II	8
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	No
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	seguridad, eficacia	Terapia génica

Información

Identificador
2023-507613-10-01 (CTIS)

Cod. Protocolo
CAB-201-001

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

Enfermedad investigada
Active Systemic Lupus Erythematosus;Systemic lupus erythematosus (SLE) is a chronic autoimmune disorder characterized by autoantibody production and abnormal B cell function. SLE presents with fluctuating severity and may cause tissue damage in a variety of organs over time. Lupus nephritis (LN) is a common severe manifestation of SLE, which can lead to significant morbidity and mortality.

Título Científico

A Phase 1/2 Open-Label Study to Evaluate the Safety and Efficacy of Autologous CD19-specific Chimeric Antigen Receptor T cells (CABA-201) in Subjects with Active Systemic Lupus Erythematosus

Justificación

No aportado

Objetivo Principal

To evaluate the safety and tolerability of CABA-201 in subjects with active SLE over 28 days

Variables de Evaluación Primaria

AEs occurring within 28 days after CABA-201 infusion, including DLTs and AEs related to CABA-201.

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To evaluate the safety and tolerability of CABA-201 in subjects with active SLE over 156 weeks.
To evaluate the effects of CABA-201 on WBC, including B cell counts.
To evaluate CABA-201 manufacturing success rates.
To characterize manufacturing process using subject cells.
To evaluate CABA-201 persistence and kinetics after infusion.
To evaluate the effects of CABA-201 on SLE serology.
To evaluate the effects of CABA-201 on SLE disease activity.
To evaluate the time to achieve disease response after CABA-201 infusion.
To evaluate the effect of CABA-201 on concomitant steroid use and other SLE-related therapy.
To evaluate the effect of CABA-201 on PROs and health-related QOL.
To evaluate the effect of CABA-201 on disease flare.
To evaluate the effect of CABA-201 on drug-free response.
To evaluate the time to achieve drug-free response after CABA-201 infusion.

Variables de Evaluación Secundaria

AEs, vital signs, physical examination, and clinical laboratory tests occurring within 156 weeks after CABA-201 infusion.
Changes from baselinea in WBC with differential, including B cell counts.
Frequency of reaching released product at target dose.
Fold cell expansion, transduction efficiency, vector copy number per cell, and product phenotype; Total number of CABA-201-positive cells in each manufacturing run; Percent of CAR-transduced cells in the total number of cells for

infusion.
Number and percentage of CABA-201-positive cells in the peripheral blood of subjects over time.
Changes from baseline in anti-dsDNA antibodies, C3, C4, and CH50.
Changes in UPCR, eGFR, SLEDAI-2K score, BILAG-2004 score, PGA score, joint counts, CLASI score, and SDI.; Proportions of subjects achieving complete renal response (for LN cohort only), SRI-4, BICLA responses, and LLDAS and DORIS criteria.
Time to achieve complete renal response (for LN cohort only), SRI-4, SRI-5, SRI-6, BICLA responses, LLDAS, and DORIS /Additional endpoint for adolescents: time to achieve modified PRINTO/ACR juvenile response criteria
Change from baseline in the dose of concomitant corticosteroids and other SLE-related therapies.; Proportion of subjects achieving a dose of 5 mg/day oral prednisone or equivalent.; Proportion of subjects who require no SLE-related therapies.
Adults:Changes from baselinea in SF-36v2, pain NRS, FACIT-F, PtGA, Lupus QoL, and EQ-5D-5L scores. Adolescents: Changes from baselinea in pain NRS, PtGA, PedsQL, and PedsQL multidimensional Fatigue Scale
Incidences of mild/moderate and severe disease flare per BILAG-2004 and SFI.; Time to mild/moderate and severe disease flare.
Proportions of subjects achieving drug free complete renal response (for LN cohort only), SRI-4, BICLA responses and LLDAS, and DORIS/ Additional endpoint for adolescents: proportion of subjects achieving drug-free complete modified PINTO/ACR juvenile response criteria
Time to achievement of drug-free complete renal response (for LN cohort only), SRI-4, BICLA responses, LLDAS and DORIS. Additional endpoint for adolescents: time to achievement of drug-free modified PINTO/ACR juvenile response criteria

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Able to provide informed consent.
Diagnosed with active SLE. Subjects with either LN or without LN will be eligible, if they meet the following criteria: -
For LN subjects: urine protein-to-creatinine ratio (UPCR) 1 mg/mg despite prior or current treatment with standard of care therapy
Adequate renal function
Adequate hepatic function
Have received all recommended vaccinations, including against COVID-19/severe acute respiratory syndrome coronavirus 2 (SARS-CoV-2), per the Centers for Disease Control and Prevention (CDC) or local/institutional guidelines for immunocompromised individuals before or during Screening. o Live vaccines must be administered at least 30 days prior to the Pre-Infusion Visit. o Non-live vaccines should be administered to subjects at least 2 weeks prior to the start of study drug infusion. If possible, non-live vaccines should also be administered at least 2 weeks prior to Leukapheresis.
Clinical stability by vital signs assessment at the time of screening
Women of reproductive potential (Section 9.4) who are sexually active must agree to use 1 highly effective method of contraception
Age 18 to 65 years.
Eastern Cooperative Oncology Group (ECOG) performance status 0 to 1.
A clinical diagnosis of SLE, based on the 2019 European League Against Rheumatism (EULAR)/American College of Rheumatology (ACR) classification criteria for adult SLE.
Positive antinuclear antibody (ANA) titer or positive anti-dsDNA antibody at Screening.

Criterios de Exclusión

Treatment with rituximab or other B cell-depleting agent within 26 weeks prior to Screening Previous CAR T cell therapy. Prior solid organ (heart, liver, kidney, lung) transplant or hematopoietic cell transplant. For LN subjects only: Evidence of severe chronicity on kidney biopsy, defined as a modified National Institute of Health chronicity index score of 3+ for any of the following individual biopsy features: total glomerulosclerosis score, fibrous crescents, tubular atrophy, or interstitial fibrosis. Pregnant or lactating woman, or plan to become pregnant within 52 weeks following CABA-201 infusion. Men of reproductive potential (see Section 9.4) who plan to father a child in the 52 weeks following CABA-201 infusion. Unable or unwilling to comply with protocol. Treatment with any investigational agent within 4 weeks or 5 half-lives, whichever is longer. Note: Subjects who had received a C5 inhibitor (e.g., eculizumab, ravulizumab) may be eligible earlier than 5 half-lives after the last C5 inhibitor administration if, at Screening, they have evidence of complement lab testing (i.e., total hemolytic complement [CH50] measurement) that has normalized or returned to pre-treatment levels. Diagnosis of cancer, except basal or squamous cell skin cancer or carcinoma in situ of the cervix that has been excised and cured and at least 5 years since excision. Planned major surgery (including joint surgery) within 52 weeks following the CABA-201 infusion. Contraindication to Leukapheresis. Active infection requiring medical intervention at Screening. History of anaphylactic or severe systemic reaction to FLU, CY, any of their metabolites A diagnosis of antiphospholipid antibody syndrome Positive human immunodeficiency virus (HIV), hepatitis C antibody, or hepatitis B surface antigen test, or evidence of active or chronic tuberculosis at Screening. Autoimmune disorder other than SLE requiring immunosuppressive therapies. The presence of kidney disease other than active lupus nephritis Current symptoms of severe, progressive, or uncontrolled renal, hepatic, hematological, gastrointestinal, pulmonary, psychiatric, cardiac, neurological, or cerebral disease, including severe and uncontrolled infections, such as sepsis and opportunistic infections. Concomitant medical conditions that, in the opinion of the investigator, might place the subject at unacceptable risk for participation in this study, interfere with the assessment of the effects or safety of the investigational product or with the study procedures. The presence of severe edema or anasarca at Screening Moderate-to-severe chronic pulmonary disease Impaired cardiac function or clinically significant cardiac disease, including: a. Unstable angina or myocardial infarction or coronary artery bypass graft within 26 weeks prior to Leukapheresis. b. New York Heart Association stage III or IV congestive heart failure. c. History of clinically significant cardiac arrhythmia (e.g., ventricular tachycardia), complete left bundle branch block, high-grade atrioventricular block d. History of severe non-ischemic cardiomyopathy. e. Left ventricular ejection fraction <45% as assessed by echocardiogram or multi-gated acquisition scan (if performed) 8 weeks of Leukapheresis. f. Active, severe cardiac manifestations of SLE, including constrictive pericarditis, hemodynamically significant pericardial effusions, and myocarditis at the time of screening.

Calendario

(Última actualización: 11/06/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
18/09/2024	31/03/2025	02/04/2025	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

Cabaletta Bio Inc. United States

2929 Arch Street Suite 600

Contact Person

Cabaletta EU Regulatory

+12677593100

EURreg@cabalettabio.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm de la Comunidad Foral de Navarra

C/ Irunlarrea, 3 31008 Pamplona

ceic@cfnavarra.es

Centros

Activo (19/12/2024)

Clinica Universidad De Navarra

Madrid

MADRID

Unidad de Ensayos Clínicos

Activo (19/12/2024)

Clinica Universidad De Navarra

Pamplona

NAVARRA

Unidad de Ensayos Clínicos

Medicamentos

FLUDARABINE ACCORD 25 mg/ml, solution à diluer pour solution injectable/pour perfusion
SOLUTION FOR INJECTION/INFUSION
INTRAVENIOUS INFUSION

Código ATC: L01BB05 - FLUDARABINA
Principios Activos: N/A

Experimental

PRD11086249
INFUSION
INFUSION

-

Principios Activos: N/A

Experimental

Genoxal 1.000 mg polvo para solución inyectable y para perfusión
SOLUTION FOR INJECTION/INFUSION
INTRAVENIOUS INFUSION

Código ATC: L01AA01 - CICLOFOSFAMIDA
Principios Activos: N/A

Experimental

CYCLOPHOSPHAMIDE SANDOZ 1000 mg, poudre pour solution injectable ou pour perfusion
SOLUTION FOR INJECTION/INFUSION
INTRAVENIOUS INFUSION

Código ATC: L01AA01 - CICLOFOSFAMIDA
Principios Activos: N/A

Experimental

Fludarabina Teva 25 mg/ml concentrado para solución para perfusión o inyección EFG
INJECTION
INTRAVENIOUS INFUSION

Código ATC: L01BB05 - FLUDARABINA
Principios Activos: N/A

Experimental

Sin resultados

A Phase 1/2, Open-Label Study to Evaluate the Safety and Efficacy of Autologous CD19-specific Chimeric Antigen Receptor T cells (CABA-201) in Subjects with Active Systemic Lupus Erythematosus

State	**Type of participants**	**Age Ranges**
Recruiting	Not provided	Older than 64 , Adults (18-64 years) , Teens (12-17 years)
Gender	**Phases**	**Expected Participants**
Both	Phase I , Phase II	8
Results	**Low level of intervention**	**Rare disease**
No results	No	No
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	safety, effectiveness	Gene therapy

Information

Identifier
2023-507613-10-01 (CTIS)

Protocol Code
CAB-201-001

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

Investigated Disease
Active Systemic Lupus Erythematosus;Systemic lupus erythematosus (SLE) is a chronic autoimmune disorder characterized by autoantibody production and abnormal B cell function. SLE presents with fluctuating severity and may cause tissue damage in a variety of organs over time. Lupus nephritis (LN) is a common severe manifestation of SLE, which can lead to significant morbidity and mortality.

Scientific Title

A Phase 1/2 Open-Label Study to Evaluate the Safety and Efficacy of Autologous CD19-specific Chimeric Antigen Receptor T cells (CABA-201) in Subjects with Active Systemic Lupus Erythematosus

Rationale

Not provided

Main Objective

To evaluate the safety and tolerability of CABA-201 in subjects with active SLE over 28 days

Primary Endpoints

AEs occurring within 28 days after CABA-201 infusion, including DLTs and AEs related to CABA-201.

Temporary moments of secondary assessment

Not provided

Secondary Objective

To evaluate the safety and tolerability of CABA-201 in subjects with active SLE over 156 weeks.
To evaluate the effects of CABA-201 on WBC, including B cell counts.
To evaluate CABA-201 manufacturing success rates.
To characterize manufacturing process using subject cells.
To evaluate CABA-201 persistence and kinetics after infusion.
To evaluate the effects of CABA-201 on SLE serology.
To evaluate the effects of CABA-201 on SLE disease activity.
To evaluate the time to achieve disease response after CABA-201 infusion.
To evaluate the effect of CABA-201 on concomitant steroid use and other SLE-related therapy.
To evaluate the effect of CABA-201 on PROs and health-related QOL.
To evaluate the effect of CABA-201 on disease flare.
To evaluate the effect of CABA-201 on drug-free response.
To evaluate the time to achieve drug-free response after CABA-201 infusion.

Secondary Endpoints

AEs, vital signs, physical examination, and clinical laboratory tests occurring within 156 weeks after CABA-201 infusion.
Changes from baselinea in WBC with differential, including B cell counts.
Frequency of reaching released product at target dose.
Fold cell expansion, transduction efficiency, vector copy number per cell, and product phenotype; Total number of CABA-201-positive cells in each manufacturing run; Percent of CAR-transduced cells in the total number of cells for

infusion.
Number and percentage of CABA-201-positive cells in the peripheral blood of subjects over time.
Changes from baseline in anti-dsDNA antibodies, C3, C4, and CH50.
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Temporary moments of secondary assessment

Not provided

Inclusion criteria

Able to provide informed consent.
Diagnosed with active SLE. Subjects with either LN or without LN will be eligible, if they meet the following criteria: -
For LN subjects: urine protein-to-creatinine ratio (UPCR) 1 mg/mg despite prior or current treatment with standard of care therapy
Adequate renal function
Adequate hepatic function
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Exclusion criteria

Treatment with rituximab or other B cell-depleting agent within 26 weeks prior to Screening Previous CAR T cell therapy. Prior solid organ (heart, liver, kidney, lung) transplant or hematopoietic cell transplant. For LN subjects only: Evidence of severe chronicity on kidney biopsy, defined as a modified National Institute of Health chronicity index score of 3+ for any of the following individual biopsy features: total glomerulosclerosis score, fibrous crescents, tubular atrophy, or interstitial fibrosis. Pregnant or lactating woman, or plan to become pregnant within 52 weeks following CABA-201 infusion. Men of reproductive potential (see Section 9.4) who plan to father a child in the 52 weeks following CABA-201 infusion. Unable or unwilling to comply with protocol. Treatment with any investigational agent within 4 weeks or 5 half-lives, whichever is longer. Note: Subjects who had received a C5 inhibitor (e.g., eculizumab, ravulizumab) may be eligible earlier than 5 half-lives after the last C5 inhibitor administration if, at Screening, they have evidence of complement lab testing (i.e., total hemolytic complement [CH50] measurement) that has normalized or returned to pre-treatment levels. Diagnosis of cancer, except basal or squamous cell skin cancer or carcinoma in situ of the cervix that has been excised and cured and at least 5 years since excision. Planned major surgery (including joint surgery) within 52 weeks following the CABA-201 infusion. Contraindication to Leukapheresis. Active infection requiring medical intervention at Screening. History of anaphylactic or severe systemic reaction to FLU, CY, any of their metabolites A diagnosis of antiphospholipid antibody syndrome Positive human immunodeficiency virus (HIV), hepatitis C antibody, or hepatitis B surface antigen test, or evidence of active or chronic tuberculosis at Screening. Autoimmune disorder other than SLE requiring immunosuppressive therapies. The presence of kidney disease other than active lupus nephritis Current symptoms of severe, progressive, or uncontrolled renal, hepatic, hematological, gastrointestinal, pulmonary, psychiatric, cardiac, neurological, or cerebral disease, including severe and uncontrolled infections, such as sepsis and opportunistic infections. Concomitant medical conditions that, in the opinion of the investigator, might place the subject at unacceptable risk for participation in this study, interfere with the assessment of the effects or safety of the investigational product or with the study procedures. The presence of severe edema or anasarca at Screening Moderate-to-severe chronic pulmonary disease Impaired cardiac function or clinically significant cardiac disease, including: a. Unstable angina or myocardial infarction or coronary artery bypass graft within 26 weeks prior to Leukapheresis. b. New York Heart Association stage III or IV congestive heart failure. c. History of clinically significant cardiac arrhythmia (e.g., ventricular tachycardia), complete left bundle branch block, high-grade atrioventricular block d. History of severe non-ischemic cardiomyopathy. e. Left ventricular ejection fraction <45% as assessed by echocardiogram or multi-gated acquisition scan (if performed) 8 weeks of Leukapheresis. f. Active, severe cardiac manifestations of SLE, including constrictive pericarditis, hemodynamically significant pericardial effusions, and myocarditis at the time of screening.

Calendar

(Last Update: 11/06/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
18/09/2024	31/03/2025	02/04/2025	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

Cabaletta Bio Inc. United States

2929 Arch Street Suite 600

Contact Person

Cabaletta EU Regulatory

+12677593100

EURreg@cabalettabio.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

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Sites

Active (19/12/2024)

Clinica Universidad De Navarra

Madrid

MADRID

Unidad de Ensayos Clínicos

Active (19/12/2024)

Clinica Universidad De Navarra

Pamplona

NAVARRA

Unidad de Ensayos Clínicos

Medication

FLUDARABINE ACCORD 25 mg/ml, solution à diluer pour solution injectable/pour perfusion

SOLUTION FOR INJECTION/INFUSION

INTRAVENIOUS INFUSION

ATC code: L01BB05 - FLUDARABINA

Active Principles: N/A

Experimental

PRD11086249

INFUSION

INFUSION

-

Active Principles: N/A

Experimental

Genoxal 1.000 mg polvo para solución inyectable y para perfusión

SOLUTION FOR INJECTION/INFUSION

INTRAVENIOUS INFUSION

ATC code: L01AA01 - CICLOFOSFAMIDA

Active Principles: N/A

Experimental

CYCLOPHOSPHAMIDE SANDOZ 1000 mg, poudre pour solution injectable ou pour perfusion

SOLUTION FOR INJECTION/INFUSION

INTRAVENIOUS INFUSION

ATC code: L01AA01 - CICLOFOSFAMIDA

Active Principles: N/A

Experimental

Fludarabina Teva 25 mg/ml concentrado para solución para perfusión o inyección EFG

INJECTION

INTRAVENIOUS INFUSION

ATC code: L01BB05 - FLUDARABINA

Active Principles: N/A

Experimental

No results