

Long-Term Follow-up: Phase I/II clinical study to evaluate the safety and efficacy of the infusion of autologous CD34+ cells transduced with a lentiviral vector carrying the FANCA gene (orphan drug) in patients with Fanconi Anaemia Subtype A: FANCOLEN-I

Estado

Reclutando

Tipo de Participantes

Sujetos incapaces de otorgar consentimiento

Rangos de Edad

Menores de 18 , Adolescentes (12-17 años)

Género

Ambos

Fases

Fase I , Fase II

Participantes esperados

9

Resultados

Sin resultados

Bajo nivel intervención

No

Enfermedad rara

Si

Cobertura geográfica

Unicéntrico nacional

Ámbitos del ensayo

tratamiento, seguridad, eficacia

Terapia avanzada

Terapia génica, Terapia celular

Información

Identificador

2024-511523-33-00 (CTIS)

Cod. Protocolo

RP-L102-0116-LTFU

Transicionado 2019-004722-19

Área terapéutica

Enfermedades [C] - Anormalidades congénitas, hereditarias y neonatología [C16]

Enfermedad investigada

Fanconi anemia (subtype A)

Título Científico

Long-Term Follow-up: Phase I/II clinical study to evaluate the safety and efficacy of the infusion of autologous CD34+ cells transduced with a lentiviral vector carrying the FANCA gene (orphan drug) in patients with Fanconi

Anaemia Subtype A: FANCOLEN-I

Justificación

No aportado

Objetivo Principal

To assess survival in subjects treated in the parent study (FANCOLEN-1)
To evaluate long term (LT) safety following infusion of hematopoietic cells transduced with the therapeutic lentiviral LV vector.
To determine long term (LT) persistence of the therapeutic LV (provirus) in hematopoietic cells in the bone marrow (BM) & blood, & evaluate potential correlations between provirus/transgene persistence & hematologic stability
To determine long term (LT) clonality patterns beyond the 3-year follow-up stipulated in parent study.
To evaluate, when relevant, replication competent lentivirus (RCL) in serum and peripheral blood (PB) cells
To determine long-term (LT) stability & normalization of blood counts in subjects after RP-L102 infusion
To determine the phenotypic correction of BM and PB cells in in long-term follow-up after gene therapy.
To enable preliminary assessment of the incidence of hematologic malignancies and solid organ tumors.

Variables de Evaluación Primaria

Safety Assessments: will include blood-based evaluation of integration site analysis (ISA) in peripheral blood mononuclear cells (including lineage-specific subsets as determined by flow cytometry), assessment of RCL (serum and blood cells) when relevant, and detailed history regarding adverse events including hospitalizations, administration of medications or therapies for bone marrow failure, and development of hematologic and non-hematologic malignancies.

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

Not Applicable

Variables de Evaluación Secundaria

Importantly, in settings of hematologic malignancy development, a blood sample will be obtained to enable determination of whether the malignant clone developed from a gene-corrected lineage or an uncorrected FA hematopoietic population by means of PCR for the transgene within the malignant population. Efficacy Assessments: will include blood-based evaluation of peripheral blood counts, and ongoing assessment of phenotypic correction via

peripheral blood T-lymphocyte DEB chromosomal fragility

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Subjects must meet all the following criteria to be included in the study: 1. Was enrolled in the clinical phase 1/2 study FANCOLEN-I. 2. Received infusion of autologous CD34+ enriched gene corrected hematopoietic cells in clinical phase 1/2 study FANCOLEN-I. 3. Is willing and able to adhere to the study visit schedule and other protocol requirements. 4. Provided written informed consent and, as applicable, assent to participate in the current study in accordance with current regulatory requirements. Patients who have undergone allogeneic HSCT (either because of bone marrow failure or leukemia/MDS) will also be followed in this protocol. Evaluations for VCN in HSCT recipients will not be performed if 3 prior assessments did not indicate presence of provirus (transgene) in any evaluated cell population.

Criterios de Exclusión

There are no criteria for exclusion in this study.

Calendario

(Última actualización: 23/06/2025)

Autorización 14/03/2020	Inicio de Ensayo 28/05/2020	Inclusión Primer Paciente 01/06/2020	Interrumpido No aportado	Reiniciado No aportado
Fin de reclutamiento No aportado	Fin prematuro (España) No aportado	Fin prematuro (Global) No aportado	Fin del ensayo en España No aportado	Fin del ensayo global No aportado

Promotor

Rocket Pharmaceuticals Inc. United States

9 Cedarbrook Drive

Contact Person

Clinical Trials

+16464409100

clinicaltrials@rocketpharma.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Hospital Infantil Universitario del Niño Jesús

Avda. Menéndez Pelayo, 65 28009 Madrid

ceic.hnjs@salud.madrid.org

Centros

**Hospital Infantil Universitario Nino
Jesus**

Madrid

MADRID

Hematology

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

**HOSPITAL INFANTIL UNIVERSITARIO
NIÑO JESUS**

Madrid

MADRID

Activo (01/06/2020)

Medicamentos

Fancalen

INFUSION

INTRAVENOUS USE

Código ATC: B05AX - OTROS PRODUCTOS DE ORIGEN SANGUINEO

Principios Activos: N/A

Huérfano

Experimental

Sin resultados

Long-Term Follow-up: Phase I/II clinical study to evaluate the safety and efficacy of the infusion of autologous CD34+ cells transduced with a lentiviral vector carrying the FANCA gene (orphan drug) in patients with Fanconi Anaemia Subtype A: FANCOLEN-I

State
Recruiting

Type of participants
Incapable subjects of giving consent

Age Ranges
Less than 18 , Teens (12-17 years)

Gender
Both

Phases
Phase I , Phase II

Expected Participants
9

Results
No results

Low level of intervention
No

Rare disease
Yes

Geographic coverage
National One-center

Areas of the study
treatment, safety, effectiveness

Advanced therapy
Gene therapy, Cellular therapy

Information

Identifier
2024-511523-33-00 (CTIS)

Protocol Code
RP-L102-0116-LTFU

Transitioned 2019-004722-19

Therapeutic area
Diseases [C] - Congenital, Hereditary, and Neonatal Diseases and Abnormalities [C16]

Investigated Disease
Fanconi anemia (subtype A)

Scientific Title
Long-Term Follow-up: Phase I/II clinical study to evaluate the safety and efficacy of the infusion of autologous CD34+ cells transduced with a lentiviral vector carrying the FANCA gene (orphan drug) in patients with Fanconi

Anaemia Subtype A: FANCOLEN-I

Rationale

Not provided

Main Objective

To assess survival in subjects treated in the parent study (FANCOLEN-1)
To evaluate long term (LT) safety following infusion of hematopoietic cells transduced with the therapeutic lentiviral LV vector.
To determine long term (LT) persistence of the therapeutic LV (provirus) in hematopoietic cells in the bone marrow (BM) & blood, & evaluate potential correlations between provirus/transgene persistence & hematologic stability
To determine long term (LT) clonality patterns beyond the 3-year follow-up stipulated in parent study.
To evaluate, when relevant, replication competent lentivirus (RCL) in serum and peripheral blood (PB) cells
To determine long-term (LT) stability & normalization of blood counts in subjects after RP-L102 infusion
To determine the phenotypic correction of BM and PB cells in in long-term follow-up after gene therapy.
To enable preliminary assessment of the incidence of hematologic malignancies and solid organ tumors.

Primary Endpoints

Safety Assessments: will include blood-based evaluation of integration site analysis (ISA) in peripheral blood mononuclear cells (including lineage-specific subsets as determined by flow cytometry), assessment of RCL (serum and blood cells) when relevant, and detailed history regarding adverse events including hospitalizations, administration of medications or therapies for bone marrow failure, and development of hematologic and non-hematologic malignancies.

Temporary moments of secondary assessment

Not provided

Secondary Objective

Not Applicable

Secondary Endpoints

Importantly, in settings of hematologic malignancy development, a blood sample will be obtained to enable determination of whether the malignant clone developed from a gene-corrected lineage or an uncorrected FA hematopoietic population by means of PCR for the transgene within the malignant population. Efficacy Assessments: will include blood-based evaluation of peripheral blood counts, and ongoing assessment of phenotypic correction via

peripheral blood T-lymphocyte DEB chromosomal fragility

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Subjects must meet all the following criteria to be included in the study: 1. Was enrolled in the clinical phase 1/2 study FANCOLEN-I. 2. Received infusion of autologous CD34+ enriched gene corrected hematopoietic cells in clinical phase 1/2 study FANCOLEN-I. 3. Is willing and able to adhere to the study visit schedule and other protocol requirements. 4. Provided written informed consent and, as applicable, assent to participate in the current study in accordance with current regulatory requirements. Patients who have undergone allogeneic HSCT (either because of bone marrow failure or leukemia/MDS) will also be followed in this protocol. Evaluations for VCN in HSCT recipients will not be performed if 3 prior assessments did not indicate presence of provirus (transgene) in any evaluated cell population.

Exclusion criteria

There are no criteria for exclusion in this study.

Calendar

(Last Update: 23/06/2025)

Authorization 14/03/2020	Start of Trial 28/05/2020	First patient inclusion 01/06/2020	Halted Not aported	Restarted Not aported
End of recruitment Not aported	Premature end (Spain) Not aported	Premature End (Global) Not aported	Trial end (Spain) Not aported	Trial end (Global) Not aported

Sponsor

Rocket Pharmaceuticals Inc. United States

9 Cedarbrook Drive

Contact Person

Clinical Trials

+16464409100

clinicaltrials@rocketpharma.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

CEIm Hospital Infantil Universitario del Niño Jesús

Avda. Menéndez Pelayo, 65 28009 Madrid

ceic.hnjs@salud.madrid.org

Sites

not initialized (---/---/---)
Hospital Infantil Universitario Nino
Jesus

Madrid

MADRID

Hematology

Active (01/06/2020)
HOSPITAL INFANTIL UNIVERSITARIO
NIÑO JESUS

Madrid

MADRID

Medication

Fancalen

INFUSION

INTRAVENOUS USE

ATC code: B05AX - OTROS PRODUCTOS DE ORIGEN SANGUINEO

Active Principles: N/A

Orphan

Experimental

No results