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	Tipo de Participantes	Rangos de Edad
Reclutando	Sujetos incapaces de otorgar consentimiento	Menores de 18 , Adolescentes (12-17 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase I , Fase II	9
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Unicéntrico nacional	tratamiento, seguridad, eficacia	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	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
14/03/2020	28/05/2020	01/06/2020	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

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

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Avda. Menéndez Pelayo, 65 28009 Madrid

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Centros

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

Hospital Infantil Universitario Nino Jesus

Madrid

MADRID

Hematology

Activo (01/06/2020)

HOSPITAL INFANTIL UNIVERSITARIO NIÑO JESUS

Madrid

MADRID

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	**Type of participants**	**Age Ranges**
Recruiting	Incapable subjects of giving consent	Less than 18 , Teens (12-17 years)
Gender	**Phases**	**Expected Participants**
Both	Phase I , Phase II	9
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
National One-center	treatment, safety, effectiveness	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	Start of Trial	First patient inclusion	Halted	Restarted
14/03/2020	28/05/2020	01/06/2020	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

Rocket Pharmaceuticals Inc. United States

9 Cedarbrook Drive

Contact Person

Clinical Trials

+16464409100

clinicaltrials@rocketpharma.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

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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