Adenosine Deaminase Severe Combined Immunodeficiency (ADA-SCID) Registry for Patients Treated with Strimvelis (or GSK2696273) Gene Therapy: Long-Term Prospective, Non-Interventional Follow-up of Safety and Effectiveness (STRIM-003)

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Study Ongoing

Administrative details

EU PAS number

EUPAS15795

Study ID

48875

DARWIN EU® study

No

Study countries

Italy

Study description

ADA deficiency results in severe combined immunodeficiency (SCID), a fatal autosomal recessive inherited immune disorder.
Strimvelis™ is a gene therapy that restores ADA function in hematopoietic cell lineages, and in doing so prevents the pathology caused by purine metabolites (i.e., impaired immune function).
Strimvelis™ is intended for patients with ADA-SCID and for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.
The objective of this prospective and retrospective (depending on the category of enrolled patients), non-interventional registry is to collect long term safety and effectiveness outcomes for patients that have received Strimvelis™.
The registry does not have a comparator group and the product will have been given on a single occasion prior to entering this registry. In this study will be also included patients for whom the gene therapy medicinal product has been prepared starting from mobilized peripheral blood (mPB)-derived CD34+ cells (mPB-GT).
Safety and effectiveness will be assessed for a target number of 50 patients who will have received Strimvelis™ or GSK2696273 or mPB-GT.
The registry will close to enrollment when 50 patients have been enrolled but will not close completely until the 50th patient finishes their 15 year follow-up.

Study status

Ongoing

Research institutions and networks

Institutions

IRCCS Ospedale San Raffaele

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Institution

Contact details

Study institution contact

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Primary lead investigator

Maria Pia Cicalese

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 07/06/2016

Actual: 07/06/2016

Study start date

Planned: 28/02/2017

Actual: 31/03/2017

Date of interim report, if expected
Planned: 31/03/2021

Actual: 30/03/2021

Date of final study report
Planned: 30/06/2046

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Fondazione Telethon ETS

Regulatory

Was the study required by a regulatory body?

Yes

Is the study required by a Risk Management Plan (RMP)?

EU RMP category 1 (imposed as condition of marketing authorisation)

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Study Design

Non-interventional study design, other

Registry

Study drug and medical condition

Name of medicine

STRIMVELIS

Medical condition to be studied

Adenosine deaminase increased

Population studied

Age groups

Preterm newborn infants (0 – 27 days)

Term newborn infants (0 – 27 days)

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Adults (18 to < 46 years)

Estimated number of subjects

50

Study design details

Outcomes

Assessment of: Effectiveness: Survival, Treatments of interest, Immune reconstitution, metabolite detoxification, Vector copy number, severe infections, non-immunological manifestations, Pediatric development and PRO Safety: Reported AEs and SAEs, Laboratory blood test results, Fertility/pregnancy related outcomes, RIS analysis and replication competent retrovirus.

Data analysis plan

This is an exposure registry without a comparison group and no inferential hypothesis testing will be performed. All data, including patient demographics, laboratory values, and AE/SAE rates will be summarized using descriptive statistics.

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

Data sources

Data sources (types), other

Prospective patient-based data collection

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

Unknown