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

- [ ] Austria
- [ ] Belgium
- [ ] Bulgaria
- [ ] Croatia
- [ ] Cyprus
- [ ] Denmark
- [ ] Estonia
- [ ] Finland
- [ ] France
- [ ] Germany
- [ ] Greece
- [ ] Hungary
- [ ] Ireland
- [ ] Italy
- [ ] Latvia
- [ ] Lithuania
- [ ] Luxembourg
- [ ] Malta
- [ ] Netherlands
- [ ] Poland
- [ ] Portugal
- [ ] Romania
- [ ] Slovakia
- [ ] Slovenia
- [ ] Spain
- [ ] Sweden
- [ ] United Kingdom

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, 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. Safety and effectiveness will be assessed for a target number of 50 patients who will have received Strimvelis.™

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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Study contact

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Primary lead investigator

Ospedale San Raffaele (SR-Tiget)

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: 31/12/2037

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 type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Main study objective:

The objective of the registry is to collect long term safety and effectiveness outcomes for patients that have received Strimvelis™.

Study Design

Non-interventional study design

Other

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

Data sources

Data sources (types)

Electronic healthcare records (EHR)

Other

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

No