

# 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)

**First published:** 19/01/2017

**Last updated:** 06/11/2025

Study

Ongoing

## Administrative details

### EU PAS number

EUPAS15795

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### Study ID

48875

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### DARWIN EU® study

No

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### Study countries

## 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.

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## 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

[cvezzali@fondazionetelethon.it](mailto:cvezzali@fondazionetelethon.it)

### 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

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### Study start date

Planned: 28/02/2017

Actual: 31/03/2017

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**Date of interim report, if expected**

Planned: 31/03/2021

Actual: 30/03/2021

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**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

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**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

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**Study type:**

Non-interventional study

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## Study Design

**Non-interventional study design, other**

Registry

## Study drug and medical condition

**Medicinal product name**

STRIMVELIS

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**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)
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### **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.

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### **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

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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

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### **Check completeness**

Unknown

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### **Check stability**

Unknown

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### **Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

Unknown