Evaluation of referring HCPs’ and parents’/carers’ understanding of specific risks associated with Strimvelis™ treatment (STRIM-001)

First published: 21/07/2017

Last updated: 11/03/2024

Study Finalised

Administrative details

EU PAS number

EUPAS19951

Study ID

48878

DARWIN EU® study

No

Study countries

☐ Italy

Study description

To evaluate the effectiveness of routine and additional risk minimization measures by assessing the understanding of referring HCPs and parents/carers with regard to specific risks associated with Strimvelis. Surveys will be provided to referring HCPs and parents/carers of children approximately six months after treatment with Strimvelis.

Study status

Finalised

Research institutions and networks

Institutions

Orchard Therapeutics

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

Ospedale San Raffaele (SR-Tiget) Ospedale San Raffaele (SR-Tiget) cvezzali@telethon.it

Study contact

cvezzali@telethon.it

Primary lead investigator

Orhard Therapeutics Orchard Therapeutics

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 31/03/2017

Actual: 24/03/2017

Study start date
Planned: 16/04/2018

Actual: 12/04/2018

Data analysis start date
Planned: 01/10/2020

Actual: 30/08/2021

Date of final study report
Planned: 30/09/2023

Actual: 18/11/2021

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Orchard Therapeutics

Study protocol

gsk-205881-protocol-redact.pdf (635.93 KB)

STRIM-001_Protocol_Final_v2.0_05March2019_Signed_redacted.pdf (418.6 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Safety study (incl. comparative)

Data collection methods:

Primary data collection

Main study objective:

To Evaluate the effectiveness of routine and additional risk minimization measures (e.g. Summary of Product Characteristics/Patient Information Leaflet, educational materials) by assessing the understanding of HCPs and parents/carers with regard to specific risks associated with Strimvelis.

Study Design

Non-interventional study design

Cross-sectional

Other

Non-interventional study design, other

Survey

Study drug and medical condition

Medicinal product name

STRIMVELIS

Medical condition to be studied

Adenosine deaminase decreased

Population studied

Short description of the study population

A survey of healthcare professionals (HCPs) and parents/carers of patients enrolled within the registry or other patients outside of the registry receiving treatment with Strimvelis.

Inclusion criteria for HCPs

• HCPs or HCPs' close family members may not have been employees of Orchard Therapeutics, GlaxoSmithKline, Pharmaceutical Product Development LLC (PPD), the Food and Drug Administration (FDA), or the European Medicines Agency (EMA).

• HCPs must be licensed.

• An HCP must not have previously completed a survey regarding Strimvelis educational materials.

• An HCP must have previously referred a patient for Strimvelis treatment.

Inclusion criteria for Parent/Carer survey, parents or carers:

• Parents/carers or parents'/carers' close family members may not have been employees of Orchard Therapeutics, GlaxoSmithKline, Pharmaceutical Product Development LLC (PPD), the Food and Drug Administration (FDA), or the European Medicines Agency (EMA).

• A parent/carer must not have previously completed a survey regarding Strimvelis educational materials.

• A parent's/carer's child must have received treatment with Strimvelis approximately twelve months prior to participation.

Age groups

- Children (2 to < 12 years)

Estimated number of subjects

20

Study design details

Outcomes

Safety Concerns (malignancy due to insertional oncogenesis), autoimmunity, unsuccessful response to gene therapy, pregnancy, Requirement for long-term monitoring, patient alert card

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Data analysis plan

Data from the survey respondents will be analysed and reported as descriptive statistics. A frequency distribution of responses to each question will be presented. Summary statistics will be prepared describing the proportion of referring HCPs and parents/carers for whom questionnaires were returned relative to total number of referring HCPs and parents/carers of patient eligible for enrolment into the study.

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)

Patient surveys

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