Long-term efficacy and safety follow-up of MLD patients treated with atidarsagene autotemcel (LongTERM-MLD)

First published: 07/09/2022

Last updated: 12/12/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS48374

Study ID

48375

DARWIN EU® study

No

Study countries

- France
- Germany
- Italy
- Netherlands

Sweden

United Kingdom

Study description

The aim of this study is to ensure that efficacy and safety of patients treated with Libmeldy in the Clinical Development Program (CDP) and in post-authorisation setting are assessed for up to 15 years following treatment in line with regulatory requirements.

Study status

Ongoing

Research institutions and networks

Institutions

Orchard Therapeutics

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Institution

Contact details

Study institution contact

Therapeutics Orchard info@orchard-tx.com

Study contact

info@orchard-tx.com

Primary lead investigator

Therapeutics Orchard

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 19/05/2022

Actual: 19/05/2022

Study start date

Planned: 30/12/2022

Actual: 21/12/2022

Date of final study report

Planned: 29/03/2041

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Orchard Therapeutics

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)

Other study registration identification numbers and links

OTL-200-10

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Main study objective:

Evaluate the durability of clinical efficacy and long-term safety following treatment with Libmeldy.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Observational long term follow up

Study drug and medical condition

Medicinal product name

LIBMELDY

Anatomical Therapeutic Chemical (ATC) code

(A16AB21) atidarsagene autotemcel

atidarsagene autotemcel

Medical condition to be studied

Metachromatic leukodystrophy

Population studied

Age groups

- 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

72

Study design details

Data analysis plan

Full statistical methods are detailed in a separate statistical analysis plan (SAP).

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

Data sources (types), other

Prospective patient-based data collection, Retrospective data collection for group 2 patients

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