A European, non-interventional, multicentre, registry-based post-authorisation safety study to evaluate the long-term safety of Loargys treatment in arginase 1 deficiency patients in standard clinical care

First published: 07/07/2025

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Study Planned

Administrative details

EU PAS number

EUPAS1000000555

Study ID

1000000555

DARWIN EU® study

No

Study countries

Austria

France

Study description

This is a non-interventional, non-comparative, multi-centre, prospective, registry-based PASS, which will be conducted in collaboration with the E-IMD. It will be based on registry data using observational methods to collect uniform data prospectively in patients with ARG1 D to monitor the long-term safety of Loargys following granting of the EU marketing authorisation.

Study status

Planned

Research institutions and networks

Institutions

Immedica Pharma AB

Sweden

First published: 30/06/2025

Last updated: 30/06/2025

Institution **Pharmaceutical company**

European registry and network for Intoxication type Metabolic Diseases (E-IMD)

First published: 01/02/2024

Last updated: 01/02/2024

Institution | Other

Heidelberg University Hospital

First published: 01/02/2024

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Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Mattias Rudebeck

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 30/07/2025

Study start date
Planned: 03/11/2025

Data analysis start date
Planned: 15/12/2025

Date of interim report, if expected
Planned: 02/02/2026

Date of final study report
Planned: 04/02/2036

Sources of funding

- Pharmaceutical company and other private sector

Study protocol

IMM-PEG-002 Study Protocol v1.0 28May2025_Redacted.pdf (596.15 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 2 (specific obligation of marketing authorisation)

Regulatory procedure number

EMA/PASS/0000258458

Methodological aspects

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Data collection methods:

Primary data collection

Study design:

A non-interventional, non-comparative, multi-centre, prospective, registry-based PASS, conducted in collaboration with the E-IMD. It will be based on registry data using observational methods to collect uniform data prospectively in patients with ARG1-D to monitor the long-term safety of Loargys.

Main study objective:

The objective of the study is to evaluate the long-term safety of Loargys treatment in patients with ARG1-D.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

LOARGYS

Study drug International non-proprietary name (INN) or common name

PEGZILARGINASE

Anatomical Therapeutic Chemical (ATC) code

(A16AB24) pegzilarginase

pegzilarginase

Medical condition to be studied

Arginase deficiency

Population studied

Short description of the study population

Adult and paediatric patients aged 2 years and older enrolled in the PASS with a confirmed diagnosis of ARG1-D and prescribed treatment with Loargys

Age groups

- **Paediatric Population (< 18 years)**
  - Children (2 to < 12 years)
  - Adolescents (12 to < 18 years)
- **Adult and elderly population (≥18 years)**

Estimated number of subjects

15

Study design details

Setting

European E-IMD centres in countries where Loargys is available.

Outcomes

• Occurrence of Adverse Events, Serious Adverse Events, Adverse Drug Reactions

• Occurrence of severe hypersensitivity reactions

• Occurrences of prolonged hypoargininaemia

• Occurrence of medication errors during non-healthcare professional administration

• Occurrence and clinical course of hypersensitivity reactions during non-healthcare professional administration

• Exposure and outcomes of pregnancy and lactation

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 source(s)

European registry and network for intoxication type metabolic diseases

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Data source(s), other

Sponsor’s global safety database

Data sources (types)

Non-interventional study

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