Analysis of patients with arginase 1 deficiency treated with Loargys in standard clinical care enrolled in a European, non-interventional, multicentre registry

First published: 07/07/2025

Last updated: 22/10/2026

Study Ongoing

Administrative details

EU PAS number

EUPAS1000000495

Study ID

1000000495

DARWIN EU® study

No

Study countries

Austria

France

Germany

Netherlands

Portugal

Study description

This is a post-authorisation efficacy study (PAES) conducted in collaboration with the European Registry and Network for Intoxication Type Metabolic Diseases (E-IMD) that will analyse the ARG1-D population of the pre-existing and active registry held by the E-IMD.

The E-IMD international, multicentric, observational registry collects health data generated during the routine care of affected patients with urea cycle disorders, including ARG1-D.

Study status

Ongoing

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

Last updated: 01/02/2024

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

Actual: 04/08/2025

Study start date
Planned: 01/05/2026

Actual: 02/06/2026

Data analysis start date
Planned: 14/12/2026

Date of interim report, if expected
Planned: 01/02/2027

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

Sources of funding

- Pharmaceutical company and other private sector

Study protocol

IMM-PEG-003 Study Protocol v1.0 24Jun2025_Redacted.pdf (444.91 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/PAM/0000264956

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Effectiveness study (incl. comparative)

Data collection methods:

Primary data collection

Study design:

This PAES is conducted in collaboration with E-IMD that will analyse the ARG1-D population of the pre-existing and active registry held by the E-IMD.The E-IMD international, multicentric, observational registry collects health data generated

during the routine care of patients with UCD, incl. ARG1-D

Main study objective:

The objective of the study is to evaluate the long-term effectiveness of Loargys treatment in patients with ARG1-D in standard clinical care

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 enrolled in the E-IMD registry with a confirmed diagnosis of ARG1-D

Age groups

- **Paediatric Population (< 18 years)**
  - Infants and toddlers (28 days – 23 months)
  - 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 treating patients with ARG1-D.

Outcomes

Study endpoints

Primary endpoint:

• Description of plasma arginine levels over time

Secondary endpoints:

• Clinical neuromotor outcomes over time (GMFCS level, muscle strength and tone, gross motor exam, six gross motor development milestones, fine motor exam, balance exam, movement exam)

• Occurrence of seizures over time

• Change in EEG

• Clinical neurocognitive outcomes over time (BSID-II/III, Denver Test, WAIS-IV, WISC-IV, WPPSI-III)
• Change in quality of Life (PedsQL, WHOQOL)
• Hospitalisations (number, length, reason) over time
• Change in growth (height, weight)
• Plasma ammonia levels over time
• Occurrence of hyperammonaemic episodes over time (as defined below).
• Description of Loargys prescribed dose, route and exposure (start/stop; reason for stopping)
• Change in dietary protein (natural amino acid supplements) and calories intake and correlation with Loargys treatment
• Change in nitrogen scavenger treatment, dose, duration of exposure and correlation with Loargys treatment.

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

Data sources (types)

Disease registry

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