An observational, multicenter study to evaluate the safety and tolerability of deferasirox in the treatment of pediatric patients with non-transfusion-dependent iron overload (NESO)

First published: 28/02/2014

Last updated: 02/07/2025

Study Finalised

Administrative details

EU PAS number

EUPAS5914

Study ID

47545

DARWIN EU® study

No

Study countries

- [ ] Egypt
- [ ] France

- [ ] Greece
- [ ] Lebanon
- [ ] Oman
- [ ] Saudi Arabia
- [ ] Thailand
- [ ] Türkiye
- [ ] United Arab Emirates
- [ ] United States

Study description

This was a pediatric registry in patients with non-transfusion dependent thalassemia who were aged ≥ 10 to <18 years at enrollment and treated with deferasirox. Patients were followed for up to 5 years from the start of deferasirox treatment. Retrospective data collection was conducted for patients who had started deferasirox 12 months or less prior to enrollment.

Study status

Finalised

Research institutions and networks

Institutions

Novartis Pharmaceuticals

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Institution

Contact details

Study institution contact

Clinical Disclosure Officer Novartis

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Study contact

trialandresults.registries@novartis.com

Primary lead investigator

Clinical Disclosure Officer Novartis

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 30/09/2012

Actual: 31/12/2013

Study start date

Planned: 30/06/2014

Actual: 28/07/2014

Data analysis start date

Planned: 06/01/2025

Actual: 08/01/2025

Date of final study report

Planned: 02/05/2025

Actual: 05/06/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Novartis Pharmaceuticals

Study protocol

ICL670E2422-v04--protocol_Redacted.pdf (2.58 MB)

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

CICL670E2422

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Main study objective:

The primary objective of this study was to characterize the long-term safety profile of deferasirox in pediatric patients with Non-transfusion-dependent thalassemia (NTDT) with exposure up to 5 years.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Non Interventional Safety

Study drug and medical condition

Medicinal product name

EXJADE

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

DEFERASIROX

Anatomical Therapeutic Chemical (ATC) code

(V03AC03) deferasirox

deferasirox

Medical condition to be studied

Iron overload

Population studied

Age groups

- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)

Estimated number of subjects

40

Study design details

Data analysis plan

All data summaries and analyses were descriptive only. Categorical data (e.g., gender, race, etc.) were summarized by means of contingency tables; a “missing” category was included as applicable.
Percentages were calculated using the number of patients in the relevant population or subgroup as the denominator. Quantitative data (e.g., age, body weight, etc.) was summarized by appropriate descriptive statistics (i.e. mean, standard deviation, median, minimum and maximum).

Documents

Study report

CICL670E2422 Report Component Report Body 1.0 (1)_Redacted.pdf (1.24 MB)

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)

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