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: 03/02/2025

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/47545

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 is a pediatric registry in patient with non-transfusion dependent thalassemia who are aged ≥ 10 to <18 years at enrollment and taking deferasirox. 40 patients will be enrolled and evaluated for safety for a 5-years follow-up period.

Study status

Ongoing

Research institutions and networks

Institutions

Novartis Pharmaceuticals

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Institution

Contact details

Study institution contact

Clinical Disclosure Officer Novartis

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

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Novartis Pharmaceuticals

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:

Primary objective: To characterize the long term safety profile of deferasirox in pediatric patients with 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

Name of medicine

EXJADE

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

Data will be collected on 40 eligible pediatric patients aged ≥ 10 to <18 years at enrollment with non-transfusion dependent thalassemia treated with deferasirox and will be followed for up to 5 years from the start of deferasirox treatment. Retrospective data collection will be done for patients whom have started deferasirox 12 months or less prior to enrollment, i.e. data will be collected from the start of deferasirox treatment (baseline) and then on for five years.

Data management

Data sources

Data sources (types)

Disease registry

Drug dispensing/prescription data

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