Long-term observational safety study to evaluate the nature and incidence of adverse effects of deferiprone treatment in patients with beta-thalassaemia major aged from 1 month to less than 18 years. (DEEP-3)

First published: 11/04/2013

Last updated: 13/01/2016

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/12034

EU PAS number

EUPAS3803

Study ID

12034

DARWIN EU® study

No

Study countries

- ☐ Albania
- ☐ Cyprus
- ☐ Egypt
- ☐ Greece
- ☐ Italy
- ☐ Tunisia

Study description

The overall aim of this multi-centre, observational cohort study is to investigate the long-term safety of deferiprone in children and adolescent. The main objectives are to evaluate the incidence of serious and non-serious adverse drug reactions in patients with beta-thalassaemia receiving deferiprone and being aged between one month and 18 years at initiation of treatment. All patients being treated with deferiprone at the participating centres will be included. Data will be collected both retrospectively and prospectively. Adverse drug reactions will be identified using intensive chart review and by clinicians reporting. Patients will be followed up to October 2015 or until cessation of deferiprone treatment, whatever comes first. Cumulative ADR incidence and ADR incidence rate will be calculated to determine the safety of deferiprone use. Multivariate logistic regression will be used to identify risk factors for adverse drug reactions to deferiprone.

Study status

Ongoing

Research institutions and networks

Institutions

University Hospital Erlangen

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Paediatric Clinical Study Centre,Department of Paediatric and Adolescents Medicine

National and Kapodistrian University of Athens

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Cairo University Faculty of Medicine Egypt, National And Kapodistrian University Of Athens Greece, Qendra Spitalore Universitare "Nene Tereza" Tirane Albania, Cyprus Ministry of Health, Nicosia Thalassaemia Center Cyprus, Centre

national de Greffe de Moelle Osseuse Tunisia, Azienda Ospedaliera di Padova (Leading Centre in Italy) Italy, 10 additional centres Italy

Networks

TEDDY European Network of Excellence for Paediatric Clinical Research (TEDDY)

- [ ] Austria
- [ ] Cyprus
- [ ] Czechia
- [ ] France
- [ ] Germany
- [ ] Greece
- [ ] Italy
- [ ] Netherlands
- [ ] Poland
- [ ] Romania
- [ ] Spain
- [ ] Sweden
- [ ] United Kingdom

First published: 15/03/2022

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Network **ENCePP partner**

DEEP Consortium (Coordinator: Consorzio per le Valutazioni Biologiche e Farmacologiche, CVBF, Pavia, Italy)

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Antje Neubert

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 31/12/2010

Actual: 31/12/2010

Study start date

Planned: 01/05/2012

Actual: 01/02/2013

Data analysis start date
Planned: 01/03/2016

Date of interim report, if expected
Planned: 31/12/2013

Actual: 31/05/2014

Date of final study report
Planned: 30/04/2016

Sources of funding

- EU institutional research programme
- Other

More details on funding

FP7 Grant HEALTH-F4-2010-261483, Participating Centres

Regulatory

Was the study required by a regulatory body?

No

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Main study objective:

The main objective of the study is to investigate serious adverse reactions related to deferiprone treatment in children aged 1 month to less than 18 years diagnosed with beta-thalassaemia major.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

FERRIPROX

Name of medicine, other

Kelfer

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

DEFERIPRONE

Anatomical Therapeutic Chemical (ATC) code

(V03AC02) deferiprone

deferiprone

Medical condition to be studied

Thalassaemia beta

Population studied

Age groups

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Estimated number of subjects

330

Study design details

Outcomes

Primary outcome is the incidence of serious adverse drug reactions. Secondary outcome is the incidence of non-serious adverse drug reactions.

Data analysis plan

Descriptive analysis will be carried out on demographics, co-prescribing and co-morbidities as well as adverse drug reactions (ADRs).Data will be stratified by covariates such as age (e.g. <10 years, >10 years), gender and country. Cumulative ADR incidence and ADR incidence rate will be calculated Logistic regression will be used to investigate the risk factors for ADRs, Kaplan-Meier Survival and Cox regression analyses will be used to analyse time to withdrawal

from deferiprone treatment in order to identify the risk factors for withdrawal. All estimates will be presented with 95% confidence intervals.

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

Prospective patient-based data collection, Retrospective patient chart review

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