ESCORT-HU : European Sickle Cell Disease Cohort - Hydroxyurea

First published: 16/12/2015

Last updated: 02/07/2024

Study Finalised

Administrative details

EU PAS number

EUPAS10565

Study ID

33716

DARWIN EU® study

No

Study countries

France

Germany

Greece

Italy

Study description

In the context of the Risk Management Plan (RMP), as requested from Addmedica by the EMEA, to collect information about long-term safety of Siklos® (hydroxycarbamide) when used in patients with Sickle Cell Disease.

Study status

Finalised

Research institutions and networks

Institutions

Addmedica

Multiple centres: 48 centres are involved in the study

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Frédéric Galacteros

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 11/12/2007

Actual: 11/12/2007

Study start date
Planned: 01/06/2008

Actual: 21/01/2009

Data analysis start date
Planned: 31/01/2019

Actual: 31/01/2019

Date of final study report
Planned: 16/12/2019

Actual: 16/12/2019

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Addmedica

Study protocol

Protocol ESCORT-HU _amended final version_20080618.pdf (193.06 KB)

ESCORT Protocol amendment 2 - 20102017 (EN).pdf (903.38 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Data collection methods:

Primary data collection

Main study objective:

To collect information about long-term safety of Siklos® when used in patients with Sickle Cell Disease, assessed on overall mortality and survival time, frequency of malignancies, skin ulceration and impaired postnatal development (growth), myelosuppression and amenorrhea, subgroups (young patients, elderly...)

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

SIKLOS

Medical condition to be studied

Sickle cell anaemia with crisis

Population studied

Short description of the study population

Patients with Sickle Cell Disease (SCD) treated with Siklos®.

Inclusion criteria:

1. Male or female ambulatory patients, aged 2 years and more (children, adolescents or adults)
2. With symptomatic sickle cell syndrome
3. Justifying a treatment with Siklos® according to the product indications
4. Having been informed of the study by the initiating physician and consenting to participate to the cohort.

Age groups

- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)
- Adults (18 to < 46 years)
- Adults (46 to < 65 years)
- Adults (65 to < 75 years)
- Adults (75 to < 85 years)
- Adults (85 years and over)

Special population of interest

Hepatic impaired

Pregnant women

Renal impaired

Estimated number of subjects

2.000

Study design details

Outcomes

frequency of malignancies, skin ulceration and impaired postnatal development (growth), myelosuppression, Effects of Siklos® on growth development, outcome of pregnancies, occurrence of adverse events and serious adverse events

Data analysis plan

All statistical analyses will be performed at the 5% significance.Parameters will be summarized using mean, median, standard deviation, range for continuous data and counts or percentages for categorical data.Descriptive statistics will be used to report the prevalence of primary and secondary parameters, describe the global population and the sub-populationsAppropriate multivariate analysis will be used to describe the determinants of the observed prevalence.

Documents

Study results

ADD_12304_ESCORT-HU_Final synopsis_20191216.pdf (190.47 KB)

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)

Other

Data sources (types), other

Prospective patient-based data collection

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

Unknown