A Patient Registry of patients with high-risk neuroblastoma being treated with the monoclonal antibody dinutuximab beta

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Study Ongoing

Administrative details

EU PAS number

EUPAS31973

Study ID

45710

DARWIN EU® study

No

Study countries

- Austria
- France
- Germany
- Hungary

Italy

Poland

Spain

United Kingdom

Study description

EUSA DB 0001 is a long-term non-interventional, multi-national, observational and prospective registry designed to capture data from real-world clinical practice to further evaluate efficacy and safety of the monoclonal antibody dinutuximab beta. This registry will provide information on pain severity and use of analgesics during treatment, incidence of neurotoxicity, visual impairment, capillary leak syndrome, cardiovascular events, hypersensitivity reactions, long-term safety and survival.

Study status

Ongoing

Research institutions and networks

Institutions

Recordati

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Institution

Austria

St. Anna Kinderspital

France

Institut Gustave Roussy

Centre Oscar Lambret

Hôpital de la Timone

Germany

Universitätsmedizin Greifswald

Universitätsmedizin Berlin

Italy

IRCCS Istituto Giannina Gaslini

Poland

Uniwersytecki Szpital Dzieciecy w Krakowie

Spain

Hospital Universitario I Politècnico de La Fe

Hospital Universitari Vall d'Hebron

United Kingdom

Southampton University Hospital NHS Foundation Trust

University Hospitals Birmingham NHS Foundation Trust

Tweddle Newcastle Hospitals NHS Foundation Trust

Hungary

Debreceni Egyetem Klinikai Kozpont

Networks

Paediatric Oncology for the Treatment of Neuroblastoma (SIOPEN)

Contact details

Study institution contact

Ignacio Alvarez Rojo PASS@eusapharma.com

Study contact

PASS@eusapharma.com

Primary lead investigator

Ignacio Alvarez Rojo

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 28/09/2015

Actual: 28/09/2015

Study start date
Planned: 19/09/2019

Actual: 30/09/2019

Data analysis start date
Planned: 01/07/2036

Date of interim report, if expected
Planned: 17/09/2025

Date of final study report
Planned: 15/12/2036

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Recordati UK Ltd.

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)

Other study registration identification numbers and links

Protocol number EUSA DB 0001

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Data collection methods:

Primary data collection

Study design:

Long-term non-interventional, multi-national, observational and prospective registry

Main study objective:

The efficacy and safety of dinutuximab beta will further be evaluated in this registry that will provide information on pain severity and use of analgesics during treatment, incidence of neurotoxicity, visual impairment, capillary leak syndrome, cardiovascular events, hypersensitivity reactions and long-term safety and survival.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Patient Registry

Study drug and medical condition

Medicinal product name

QARZIBA

Medicinal product name, other

Qarziba 4.5 mg/mL concentrate for solution for infusion

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

DINUTUXIMAB BETA

Medical condition to be studied

Neuroblastoma

Population studied

Short description of the study population

Patients with high-risk neuroblastoma being treated with the monoclonal antibody dinutuximab beta

Age groups

- Infants and toddlers (28 days – 23 months)
- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)

Estimated number of subjects

125

Study design details

Outcomes

- Pain severity and use of analgesics during treatment - Incidence of neurotoxicity, visual impairment, capillary leak syndrome, cardiovascular events and hypersensitivity reactions. - Long term safety, - Progression Free

Survival (PFS) in patients treated with dinutuximab beta. - Event Free Survival (EFS) in patients treated with dinutuximab beta - Overall Survival (OS) in patients treated with dinutuximab beta

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Data analysis plan

The safety analysis set, containing all patients treated with dinutuximab beta at least once will be considered for safety and efficacy analyses.

Data will be presented in individual listings and summarized if appropriate over time - according to their data type:

- Continuous data by mean, standard deviation, minimum, median, maximum
- Qualitative (nominal) data by absolute and/or relative frequencies
- Time to event (death or progression of disease) using Kaplan Meier methods
- Endpoints addressing primary and secondary analysis will also include 95% CI including the Clopper Pearson method for binomial, log-log transform for survival.
- Confidence intervals (CIs) for continuous outcome variables were calculated based on normal distribution assumption.

Patient listings of efficacy outcome will be provided separately for the different patient subgroups (relapsed, refractory, first line). Efficacy tables will be repeated for the different patient subsets (relapsed, refractory, first line) and overall. Analyses will be performed by visit/ time point, if not stated otherwise. For time to event analyses censoring information will be used for statistical analyses.

Prior treatments for neuroblastoma (induction chemotherapy and high dose chemotherapy) and used analgesics during treatment were coded using the most recent version of WHO-DRUG.

AEs, prior surgeries, and relevant medical history were coded using MedDRA v23.1.

Data management

Data sources

Data source(s)

Other data source

Data source(s), other

Data will be collected from physicians using an electronic data capture (EDC) system. The electronic case report forms (eCRFs) will be designed to gather data from the medical records at baseline, during treatment and normal clinical practice follow up visits

Prospective patient-based data collection

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

Yes

Check completeness

Yes

Check stability

Unknown

Check logical consistency

Yes

Data characterisation

Data characterisation conducted

No