A Post-Authorisation Safety Study Patient Registry of patients with high-risk neuroblastoma being treated with the monoclonal antibody dinutuximab beta (EUSA DB 0001)

First published: 31/10/2019

Last updated: 08/07/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS31973

Study ID

45710

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ France

- [ ] Germany
- [ ] Italy
- [ ] Poland
- [ ] Spain
- [ ] United Kingdom

Study description

EUSA DB 0001 is a non-interventional, multi-national, observational, prospective registry designed to capture data from real-world clinical practice to further evaluate efficacy and safety of dinutuximab beta

Study status

Ongoing

Research institutions and networks

Institutions

EUSA Pharma

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Charité-Universitätsmedizin

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Gustave Roussy

France

First published: 01/02/2024

Last updated: 01/02/2024

Institution | Educational Institution | Hospital/Clinic/Other health care facility

St. Anna Kinderspital Austria, Hospital Universitario y Politécnico la Fe Valencia, Spain, Institut Gustave Roussy Villejulf, France, Institut Curie Paris, France, Centre Oscar Lambret Lille, France, Hopital de la Timone Enfants, Marseille Marseille, France, Universitätsmedizin Greifswald Greifswald, Germany, Charité – Universitätsmedizin Berlin Berlin, Germany, Szpital Uniwersytecki Krakowie Ul. Wielicka 265 Krakow, Poland, IRCCS Istituto Giannina Gaslini Genova, Italy

Networks

Paediatric Oncology for the Treatment of Neuroblastoma (SIOPEN)

Contact details

Study institution contact

Jose-Luis Garcia PASS@eusapharma.com

Study contact

PASS@eusapharma.com

Primary lead investigator

Jose-Luis Garcia

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 28/09/2015

Actual: 28/09/2015

Study start date

Planned: 30/09/2019

Actual: 30/09/2019

Data analysis start date
Planned: 30/10/2031

Date of interim report, if expected
Planned: 31/12/2025

Date of final study report
Planned: 15/01/2033

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

EUSA Pharma

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:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Drug utilisation

Effectiveness study (incl. comparative)

Main study objective:

The efficacy and safety of dinutuximab beta will further be evaluated in this registry that will provide information on survival, QOL/Burden of care, pain severity and incidence of neurotoxicity, visual impairment, capillary leak syndrome, cardiovascular events, hypersensitivity reactions and longterm safety.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

QARZIBA

Name of medicine, other

Qarziba 4.5 mg/mL concentrate for solution for infusion

Medical condition to be studied

Neuroblastoma

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

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

---

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. 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.

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

No