An Observational, Ambispective Cohort Study Evaluating the Long-term Effectiveness and Safety of Dinutuximab Beta or Conventional Treatment as Maintenance Therapy in Patients With High Risk Neuroblastoma

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Study Ongoing

Administrative details

EU PAS number

EUPAS46562

Study ID

47638

DARWIN EU® study

No

Study countries

China

Study description

The aim of this study is to evaluate the long term effectiveness and safety of dinutuximab beta or conventional treatment as first-line maintenance therapy in participants with high risk neuroblastoma

Study status

Ongoing

Research institutions and networks

Institutions

BeiGene

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Institution

Beijing Children Hospital, Capital Medical University China, Children's Hospital affiliated to Capital Institute of Pediatrics China, Tianjin Cancer Hospital China, Guangzhou Women and Children's Medical Center China, The Children’s Hospital of

Fudan University China, Children’s Hospital of Nanjing Medical University China, Shenzhen Children's Hospital China, Hunan Provincial People's Hospital China, Shandong Cancer Hospital China, The Children's Hospital of Zhejiang University School of Medicine China

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Clinical Trials BeiGene

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 28/12/2021

Actual: 28/12/2021

Study start date
Planned: 30/04/2022

Actual: 03/06/2022

Data analysis start date
Planned: 30/04/2022

Actual: 03/06/2022

Date of interim report, if expected
Planned: 30/12/2023

Date of final study report
Planned: 31/08/2026

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BeiGene (Beijing) Co, Ltd

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)

Regulatory procedure number

DINUTUXIMAB BETA EU RMP,NMPA RMP in Chinese

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Main study objective:

This is a multi-center, observational, ambispective cohort study with the aim of evaluating the long-term effectiveness and safety of dinutuximab beta or conventional treatment as first-line maintenance therapy in patients with HR NB. The primary endpoint of this study is measured by the 3-year rwEFS rate of patients received dinutuximab beta or conventional treatment.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

QARZIBA

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

DINUTUXIMAB BETA

Medical condition to be studied

Neuroblastoma

Additional medical condition(s)

High-Risk 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

150

Study design details

Outcomes

Title: 3-year real-world event free survival (rwEFS) rate. The survival curves for rwEFS will be plotted using the Kaplan-Meier method, and the 3-year rwEFS rate will be estimated. The two-sided 95% CIs will be calculated using the

Greenwood formula. Real world event free survival (rwEFS) is defined as the time from first dose of maintenance therapy to the date of earliest occurrence, 2-year Real-World Event-Free Survival (rwEFS) rate 1-year Real-World Event-Free Survival (rwEFS) rate 3-year overall survival OS rate Percentage of Participants With an Adverse Event (AE), Serious AE, or Death Due to an AE

Data analysis plan

There are two stages for this study: 1. Case Collection Stage: Collecting eligible patients in 10 study centers and using the electronic data collection system to record demographic, clinical characteristics, treatment, concomitant medication and safety information. This will be from when they received dinutuximab beta until two years after. The case information is reported retrospectively and after recruitment, the data is collected at the site prospectively. 2. Follow Up Stage: all patients will be followed up with for at least two years to collection endpoint data.

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)

Disease registry

Electronic healthcare records (EHR)

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