REG-501: A Registry of Patients with β-Thalassemia Treated with Betibeglogene Autotemcel

First published: 27/07/2020

Last updated: 14/06/2024

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/42440

EU PAS number

EUPAS36487

Study ID

42440

DARWIN EU® study

No

Study countries

☐ Germany

Study description

REG-501-DE is a country-specific observational registry study conducted to collect longitudinal data on the clinical outcomes of patients with β-thalassemia treated in Germany with Betibeglogene Autotemcel (also known by the tradename Zynteglo or as LentiGlobin BB305 Drug Product).

Study status

Ongoing

Research institutions and networks

Institutions

Bluebird bio

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

Contact bluebird bio

Study contact

brehm@voisinconsulting.com

Primary lead investigator

Contact bluebird bio

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 24/05/2019

Actual: 24/05/2019

Study start date
Planned: 30/09/2020

Actual: 11/02/2021

Date of interim report, if expected
Planned: 31/12/2024

Date of final study report
Planned: 31/12/2042

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

bluebird bio, Inc

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)

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:

Evaluate the long-term and short-term safety of treatment with betibeglogene autotemcel (beti-cel) in patients with β-thalassaemia, including the risk of newly diagnosed malignancies. Evaluate the long-term effectiveness of treatment with betibeglogene autotemcel (beti-cel).

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

ZYNTEGLO

Medical condition to be studied

Thalassaemia beta

Population studied

Age groups

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)

Estimated number of subjects

8

Study design details

Outcomes

Incidence of Adverse Events of Interest (AEIs) through 15 years post-betibeglogene autotemcel (beti-cel) infusion, • Incidence of Serious Adverse Events (SAE) through 15 years post-beti-cel infusion (regardless of relatedness to beti-cel) • Incidence of beti-cel-related Adverse Events (AEs) through 15 years post-beti-cel infusion • Event-free survival through 15 years post-beti-cel infusion • Transfusion independence through 15 years post-beti-cel infusion

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Data analysis plan

Descriptive analyses will be performed to gain an understanding of the qualitative and quantitative nature of the data collected and the characteristics of the patient population. Formal statistical hypothesis testing will not be performed. Continuous variables will be reported as mean (and standard deviation), or median (and minimum, maximum) where appropriate. Categorical variables will be summarized as number and proportion of the total study population.

Data management

Data sources

Data source(s), other

Rare Anemia Registry Germany

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