A Non-Interventional Registry Study of Patients with β-thalassemia to Characterise and Contextualise the Safety and Effectiveness of Betibeglogene Autotemcel

First published: 12/07/2021

Last updated: 23/04/2024

Study Planned

Administrative details

EU PAS number

EUPAS41950

Study ID

46901

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ Belgium
- ☐ Bulgaria

- [ ] Croatia
- [ ] Cyprus
- [ ] Czechia
- [ ] Denmark
- [ ] Estonia
- [ ] Finland
- [ ] France
- [ ] Germany
- [ ] Greece
- [ ] Hungary
- [ ] Iceland
- [ ] Ireland
- [ ] Italy
- [ ] Latvia
- [ ] Lithuania
- [ ] Luxembourg
- [ ] Malta
- [ ] Netherlands
- [ ] Norway
- [ ] Poland
- [ ] Portugal
- [ ] Romania
- [ ] Slovakia
- [ ] Slovenia
- [ ] Spain
- [ ] Sweden
- [ ] Switzerland
- [ ] United Kingdom
- [ ] United States

Study description

This study has been cancelled. The European Commission withdrew the marketing authorisation for Zynteglo (betibeglogene autotemcel) in the European Union (EU). The withdrawal was at the request of the marketing authorisation holder, bluebird bio (Netherlands) B.V., which notified the European Commission of its decision to no longer market the product in the EU for commercial reasons. The withdrawal occurred before any patients were enrolled in REG-504 and, as this study was only planned to be conducted in the EU, it will no longer be conducted. REG-504 was intended to be a non-interventional, descriptive cohort study involving secondary use of product registry and transplant registry data to evaluate endpoints related to the safety and effectiveness of betibeglogene autotemcel and allogeneic-hematopoietic stem cell transplant (allo-HSCT). The study was intended to include two cohorts of patients, a betibeglogene autotemcel cohort and an allo-HSCT cohort.

Study status

Planned

Research institutions and networks

Institutions

Bluebird bio

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Institution

Contact details

Study institution contact

bluebird bio Contact regulatory@bluebirdbio.com

Study contact

regulatory@bluebirdbio.com

Primary lead investigator

bluebird bio Contact

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 31/08/2021

Study start date

Planned: 30/09/2021

Date of interim report, if expected

Planned: 31/03/2022

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:

Describe the safety outcomes of patients with β-thalassaemia treated with betibeglogene autotemcel or allo-HCST in Europe

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

325

Study design details

Outcomes

- Describe the safety outcomes of patients with treated with betibeglogene autotemcel or allo-HCST in Europe, - Describe the effectiveness outcomes of patients with β-thalassaemia treated with betibeglogene autotemcel or allo-HCST in Europe - Describe the baseline and pretreatment characteristics of patients with β-thalassaemia treated with betibeglogene autotemcel or allo-HCST in Europe

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Data analysis plan

Given the study design, expected sample size, low event rates, and strong chance of unmeasured confounding, analyses will be descriptive in nature, although any known confounding will be addressed in sensitivity analyses, to the extent possible. Descriptive statistics of central tendency (medians and arithmetic or geometric means) and dispersion (standard deviation, interquartile range IQR, range) will be presented for continuous variables. For categorical variables, frequencies and percentages will be reported, where appropriate, mode, median, IQR, and range will also be reported.

Data management

Data sources

Data source(s), other

EBMT

Data sources (types)

Other

Data sources (types), other

Exposure registry

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