# REG-501: A Registry of Patients with $\beta$ Thalassemia Treated with Betibeglogene Autotemcel

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## Administrative details

<b>EU PAS number</b> 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  $\beta$ -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

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Institution

## Contact details

#### **Study institution contact**

Contact bluebird bio brehm@voisinconsulting.com

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  $\beta$ -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

#### **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

## **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

Rare Anemia Registry Germany	
Data sources (types)	
Other	
Data sources (types), other	
Prospective patient-based data collection	
Use of a Common Data Mo	odel (CDM)
CDM mapping	
No	
Data quality specifications	
Check conformance	
Unknown	

## **Check stability**

Unknown

# Check logical consistency

Unknown

# Data characterisation

#### **Data characterisation conducted**

No