A Post-authorization Safety Study to Evaluate the Safety of Multiple Myeloma Patients Treated with Ciltacabtagene Autoleucel

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Study Ongoing

Administrative details

EU PAS number

EUPAS49218

Study ID

50027

DARWIN EU® study

No

Study countries

Austria

Brazil

Germany

United States

Study status

Ongoing

Research institutions and networks

Institutions

Johnson & Johnson

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Institution

Contact details

Study institution contact

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Primary lead investigator

Denise De Wiest

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 03/06/2022

Actual: 03/06/2022

Study start date
Planned: 30/09/2022

Actual: 30/05/2022

Date of final study report
Planned: 30/06/2042

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Janssen Research & Development, LLC Legend Biotech USA, Inc

Study protocol

REDACTED_Protocol-FD-68284528MMY4004-67663_1643217.pdf (1.46 MB)

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

EMEA/H/C/005095/0000

Other study registration identification numbers and links

68284528MMY4004

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Study design:

This study is an observational PASS (68284528MMY4004; referred to throughout as MMY4004 or the “study”) to describe the data collection required to define the short- and long-term safety profile of cilta-cel in the treatment of patients with multiple myeloma.

Main study objective:

The primary objective of the study is to evaluate the short- and long-term safety including the risk of subsequent malignancy, of cilta-cel in adult patients with multiple myeloma.

Long-term data on replication-competent lentivirus (RCL) in patients who develop subsequent malignancies will also be collected, where allowed per local regulations in the context of a non-interventional study.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Prospective, secondary data analysis of databases

Study drug and medical condition

Medicinal product name

CARVYKTI

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

CILTACABTAGENE AUTOLEUCEL

Anatomical Therapeutic Chemical (ATC) code

(L01XL05) ciltacabtagene autoleucel

ciltacabtagene autoleucel

Medical condition to be studied

Plasma cell myeloma refractory

Population studied

Short description of the study population

The source population for this study will be those patients enrolled in the registry, who are receiving cilta-cel for multiple myeloma and who provided informed consent. Other data sources may also includeanalysis from tumor samples or spontaneously-reported adverse reactions to the Sponsor, where available.

Age groups

- 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

1700

Study design details

Outcomes

Primary outcomes of the study are the short- and long-term safety including the subsequent malignancy and replication-competent lentivirus (RCL) in patients who develop subsequent malignancies.

The secondary outcomes include: 1) Overall response rate (ORR) 2)Duration of response (DOR) 3)Progression-free survival 4) Overall survival (OS) 5)Cilta-cel's effect on myeloma-related comorbidities (amyloidosis and POEMS syndrome, if present)

Data analysis plan

Analysis set will include all patients who meet the selection criteria. Patient demographics, medical and disease history, current disease status and any previous therapies for multiple myeloma will be descriptively summarized at baseline.

Only selected toxicities will be collected in the registry and reported in this study. All documented AEs will be analyzed. All AEs regardless of causality to cilta-cel will be analyzed.

The verbatim terms used to identify AEs will be coded per MedDRA. For each AE, the percentage of patients who experience at least 1 occurrence of the given event will be summarized. Where appropriate, additional summaries, listings may be provided. Additionally, cumulative incidence estimates, or rates of AEs reported in person years may be used. The survival analysis will be performed for time-to-event variables (i.e. PFS, OS). Responses to cilta-cel will be summarized with count and percentage.

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 source(s), other

Center for International Blood & Marrow Transplant Research (CIMBTR) Cellular Therapy Registry United States, Janssen-sponsored PASS 68284528MMY4009 Belgium

Data sources (types)

Disease registry

Other

Data sources (types), other

Spontaneous reporting system, 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