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

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Study | Planned

Administrative details

PURI
https://redirect.ema.europa.eu/resource/1000000046

EU PAS number
EUPAS49370

Study ID
1000000046

DARWIN EU® study
No

Study countries
Austria
Brazil
Germany

Study status
Planned

Research institution and networks

Institutions

Johnson & Johnson

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Institution

40 centres are involved in this study

Contact details

Study institution contact
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Study contact

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Primary lead investigator
Silva Koskinen

Primary lead investigator

Study timelines

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

Study start date
Planned:
28/06/2024

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. (50%); Legend Biotech (50%)

Study protocol

23May2024-REDACTED_Protocol-FD-68284528MMY4009-588202_946738 (1).pdf(3.87 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

Methodological aspects

Study type

Study type list

Study topic:
Human medicinal product

Study type:
Non-interventional study

Scope of the study:
Assessment of risk minimisation measure implementation or effectiveness
Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Study design:
This is a non-interventional PASS to describe the safety profile of cilta-cel in the treatment of adult patients with multiple myeloma, primarily in the European Union (EU) region, with the option to expand to other regions/countries.

Main study objective:
This study aims to document the short- and long-term safety of adult patients with multiple myeloma receiving ciltacabtagene autoleucel in the post-authorization setting per the health authority approved product information in the respective country/region.

Study Design

Non-interventional study design
Other

Non-interventional study design, other
Prospective, non-interventional post-authorization safety study, registry

Study drug and medical condition

Name of medicine
CARVYKTI

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

Anatomical Therapeutic Chemical (ATC) code
200000030588
ciltacabtagene autoleucel

Additional medical condition(s)
Relapsed/refractory multiple myeloma

Population studied

Age groups
Adults (18 to < 65 years)
Adults (18 to < 46 years)
Adults (46 to < 65 years)

Elderly (? 65 years)
Adults (65 to < 75 years)
Adults (75 to < 85 years)
Adults (85 years and over)

Estimated number of subjects
300

Study design details

Outcomes
Primary outcomes: Safety will be measured through the rate of selected adverse events associated with the administration of cilta-cel regardless of causality and seriousness. Selected adverse events include but are not limited to subsequent malignancies, neurotoxicity, hematologic disorders, hypogammaglobulinemia, clinically significant infections, cytokine release syndrome, graft-versus-host disease and others.

Secondary outcomes: The effectiveness of cilta-cel measured through the following parameters: Overall survival (OS), Progression-free survival (PFS), Duration of response (DOR), Overall response rate (ORR), 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. All documented adverse events (AEs) will be analyzed. All AEs regardless of causality to cilta-cel beginning from product administration on Day 0 until Day 100 following CAR-T infusion will be analyzed. Thereafter, only nonserious AEs related to cilta-cel (with some exceptions) and all SAEs regardless of causality up to End of Study 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. 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

Data sources

Data sources (types)
Other
Spontaneous reporting system

Data sources (types), other
- Prospective patient-based data collection
- The primary data source for this study will be the medical records of each patient who provided a signed informed consent form. Other data sources may also include analyses from tumor samples of patients developing second primary malignancies or adverse events spontaneously reported to the sponsor, where available.

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