A prospective non-interventional post-authorization safety study (PASS), designed as a disease registry of patients with transfusion dependent IPSS low or intermediate-1-risk myelodysplastic syndromes (MDS) and isolated del(5q) (CC-5013-MDS-010)

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Last updated: 02/07/2024

Study Finalised

Administrative details

EU PAS number

EUPAS7412

Study ID

46350

DARWIN EU® study

No

Study countries

- Belgium
- Denmark
- France
- Germany
- Greece
- Italy
- Luxembourg
- Netherlands
- Norway
- Spain
- Sweden
- United Kingdom

Study description

This is a prospective non-interventional post-authorization safety study (PASS), designed as a disease registry. No deviation from the routine clinical practice of enrolled patients is expected as a result of this study. Patients with transfusion-dependent IPSS low or intermediate-1 myelodysplastic syndromes (MDS) and isolated del(5q) who meet the inclusion/exclusion criteria will be eligible for enrollment. This study will be conducted in countries in the EU, where it is expected that lenalidomide will be marketed in the MDS indication. Details of implementation of this condition to the marketing authorization will be agreed with each NCA in the Member States where the registry is planned. Exposure studies with uniform data collection procedures for antecedent and outcome variables can provide robust benefit/risk information through the enrollment of large numbers of patients with an uncommon condition. The enrollment will not be limited to patients receiving lenalidomide within the indication approved in the EU but will aim to include additional MDS patients receiving treatments or treatment modalities other than lenalidomide. Primary endpoints for this study

include product-limit estimators of AML progression and survival for all MDS patients in the MDS registry and separately for patients in the primary population and the non-primary populations. Hazard ratios derived from Cox proportional hazards models will quantify the magnitude of risk associated with lenalidomide treatment, other risk factors, and any interaction effects derived from data obtained from patients in the primary population. Measures of effectiveness among patients in the primary population will be described as the proportion of patients who achieve the effectiveness endpoints (i.e. erythroid response, transfusion independence, and cytogenetic response).

Study status

Finalised

Research institutions and networks

Institutions

Celgene International

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Institution

Multiple centres: 162 centres are involved in the study

Contact details

Study institution contact

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Primary lead investigator

Medical Affairs Celgene International Sarl

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 01/12/2013

Study start date

Planned: 01/12/2014

Actual: 17/12/2014

Date of final study report

Planned: 31/03/2023

Actual: 21/03/2023

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Celgene International Sarl

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 topic:

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Other

Safety study (incl. comparative)

If ‘other’, further details on the scope of the study

Patient-based data collection

Data collection methods:

Primary data collection

Main study objective:

Whether the 2-year cumulative incidence of acute myeloid leukemia (AML) progression and mortality among transfusion-dependent, IPSS low or intermediate-1-risk MDS and isolated del(5q) patients treated with lenalidomide in a routine-care setting will differ from the incidence observed in Studies MDS-003 and MDS-004 combined.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Prospective, post-authorisation safety study (PASS)

Study drug and medical condition

Medical condition to be studied

Myelodysplastic syndrome

Population studied

Short description of the study population

The study population included 18 years or older aged patients with transfusion-dependent International Prognostic Scoring System (IPSS) low or intermediate-1-risk myelodysplastic syndromes (MDS) and isolated del(5q) cytogenetic abnormality.

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)

Special population of interest

Other

Special population of interest, other

Myelodysplastic syndrome patients

Estimated number of subjects

375

Study design details

Outcomes

To ascertain the progression to AML and survival (through calculation of product-limit estimators and incidence rates, as well as the attributable risk AR and AR percentage AR%) among 603 patients (primary population) with

transfusion dependent IPSS low- or int-1-risk MDS with del (5q) as an isolated cytogenetic abnormality who have been treated with lenalidomide, To describe the progression to AML and survival among patients with MDS who have never been exposed to lenalidomide. This will include explorative analyses of progression to AML and survival in patients receiving treatments or treatment modalities other than lenalidomide whenever possible. To further characterise the safety profile of lenalidomide among MDS patients treated with lenalidomide

Data analysis plan

The formal multivariate analysis of AML progression risk and OS will be conducted using the primary population of 603 MDS patients with a single del (5q) aberration who have received at least 1 complete cycle of lenalidomide. -For purposes of estimating the incidence of hematologic and nonhematologic AEs, as well as uncommon events not previously documented, all MDS patients treated with at least 1 dose of lenalidomide will be included. -Safety analyses will be conducted separately for the primary population and the safety populations. In addition, explorative analyses of progression to AML and survival in patients receiving treatments or treatment modalities other than lenalidomide, will be run whenever possible.

Documents

Study results

cc-5013-mds-010-pass-synopsis-redacted.pdf (2.61 MB)

Study, other information

CC-5013-MDS-010_Site List_2022-03-21.pdf (95.18 KB)

MDS-010_Approved sites for ENCePP_20Mar15_SitesOnly.pdf (11.52 KB)

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 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