

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)

**First published:** 14/10/2014

**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

**First published:** 01/02/2024

**Last updated:** 01/02/2024

**Institution**

**Multiple centres: 162 centres are involved in the study**

## Contact details

**Study institution contact**

Medical Affairs Celgene International Sarl  
ctt.group@bms.com

Study contact

[ctt.group@bms.com](mailto:ctt.group@bms.com)

**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\\_Aproved 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