Incidence of Hematologic and Non-Hematologic Malignancies, Thrombotic Events, and Autoimmune Disorders in Unrelated Normal Donors Undergoing Bone Marrow Harvest Versus Peripheral Blood Stem Cell Mobilization with Recombinant Human Granulocyte Colony-Stimulating Factor (20130209)

First published: 30/06/2017

Last updated: 14/09/2022

Study Finalised

Administrative details

EU PAS number

EUPAS19126

Study ID

48970

DARWIN EU® study

No

Study countries

United States

Study description

The National Marrow Donor Program (NMDP) in the USA, through its research program, the Center for International Blood and Marrow Transplant Research (CIBMTR), collects data on outcomes of alloHCT donors and recipients. Both unstimulated BM and filgrastim-mobilized PBSC donors are contacted by the donor center two days post-donation and then weekly until the donor states he or she has recovered. Since 2006, all donors are then contacted annually for as long as possible, unless the donor formally withdraws from long term follow-up. Primary Objective: To describe the long-term incidence of malignant myeloid haematologic disorders in donors who received and in those who did not receive filgrastim. Secondary Objectives: To describe the long-term incidence of haematologic and non-haematologic malignant disorders, thrombotic events, and autoimmune diseases. Study Design/Type: The study is an observational cohort study, including unrelated normal donors who donated haematopoietic cells July 1999 through October 2010 and prospectively enrolled unrelated normal donors who donated haematopoietic cells between 2010 and 2015. Follow-up is approximately five years post study activation. Sample Size: Approximately 90% of the eligible donors are projected to donate during the study accrual period: approximately 10,956 unstimulated BM and 21,172 filgrastim-mobilized PBSC donors. Assuming that the incidence rate of haematological malignancies in the normal population is 1 case per thousand person-years, then this study should have approximately 80% power to detect a rate ratio of 1.25 associated with filgrastim use.

Study status

Finalised

Research institutions and networks

Institutions

Amgen

United States

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Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Global Development Leader Amgen Inc.

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 17/12/2014

Actual: 17/12/2014

Study start date
Planned: 07/11/2016

Actual: 07/11/2016

Data analysis start date
Planned: 31/05/2022

Actual: 27/04/2022

Date of final study report
Planned: 01/09/2022

Actual: 13/09/2022

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Amgen

Study protocol

Redacted 20130209 LTDFU Study NMDP protocol v4 1 Watermarked Version with Amgen Header and Synopsis.pdf (405.32 KB)

Regulatory

Was the study required by a regulatory body?

Unknown

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Data collection methods:

Secondary use of data

Main study objective:

The primary goal is to evaluate the hypothesis that the incidence of targeted disorders, consisting of malignant, thrombotic and autoimmune disorders, after unrelated hematopoietic stem cell donation are similar between unstimulated BM and filgrastim-mobilized PBSC donors.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name, other

Neupogen

Medical condition to be studied

Haematopoietic stem cell mobilisation

Population studied

Short description of the study population

The study participants are unrelated donors from the U.S. who received unstimulated bone marrow or filgrastim-mobilized peripheral blood stem cell (PBSC) donation from the National Marrow Donor Program (NMDP) between July 1999 and about five years post-activation in 2010. The study also includes those who received at least one injection of filgrastim during this timeframe but did not collect the cells.

Inclusion criteria:

• Unrelated donor who donated either unstimulated BM or filgrastim-mobilized PBSC between July 1, 1999 and approximately five years post study activation

• Unrelated donor who received at least one injection of filgrastim or more, but did not donate filgrastim-mobilized PBSC between July 1, 1999 and approximately five years post study activation

• Donation was managed by a U.S. NMDP donor centre

• Signed informed consent from the donor for participation in this long-term donor follow-up study
• Concurrent enrolment on other studies is permitted

Exclusion criteria:
• Unrelated donor who donated filgrastim-mobilized bone marrow
• Donation was managed by a non-U.S. donor centre
• Donor is unable to verbally communicate in any of the following languages: English, Spanish, Mandarin Chinese, Cantonese Chinese, Vietnamese, Korean or Portuguese

Age groups

- Adults (18 to < 46 years)

Special population of interest

Other

Special population of interest, other

Patients with haematopoietic stem cell mobilisation

Estimated number of subjects

32128

Study design details

Outcomes

Malignant disorders include AML, MDS, CML, chronic myeloproliferative disorders, acute lymphoblastic leukemia, chronic lymphocytic leukemia, lymphomas and non-hematologic malignancies, as defined in the Surveillance

Epidemiology and End Results (SEER). thrombotic and autoimmune disorders. Autoimmune events include rheumatoid arthritis, psoriatic arthritis, systemic lupus erythematosus, scleroderma, vasculidities, multiple sclerosis and ITP

Data analysis plan

NA

Documents

Study results

20130209_ORSR_Abstract_Redacted.pdf (1010.39 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)

Disease registry

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