Plerixafor Off-label Transplant Use

A Non-Interventional Study
31 July 2009 - 31 July 2014

EBMT
European Society for Blood and Marrow Transplantation

genzyme
A SANOFI COMPANY

Background and Objectives

Plerixafor (Mozobil©) received EU approval on 31 July 2009.

As a post–approval commitment, EMA have asked **Genzyme** to examine progression free survival, overall survival and relapse rate of patients with lymphoma or multiple myeloma who have received autologous transplants of stem cells mobilized with or without plerixafor. For this purpose, Genzyme and the EBMT have developed the CALM study (Collaboration to collect Autologous transplant outcomes in Lymphoma and Myeloma).

As a post-marketing commitment to the EMA, Genzyme will additionally monitor the off-label transplant use of plerixafor using data in the EBMT registry.

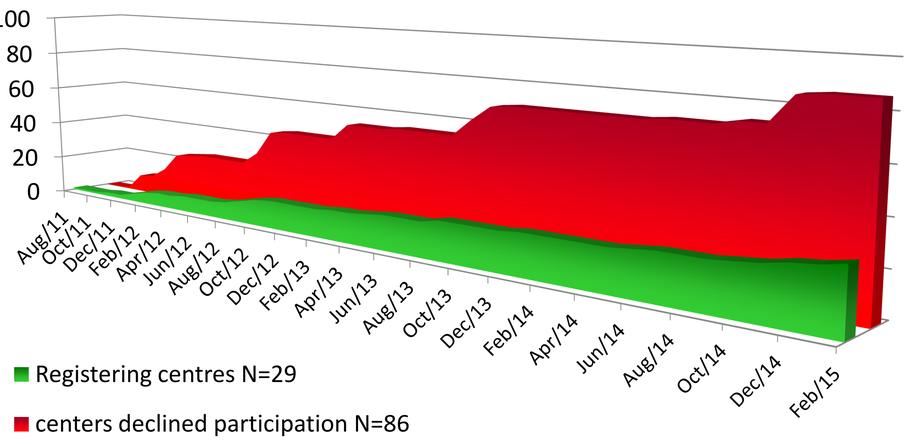
Study objectives Monitoring the off-label transplant use of plerixafor, using data entries in the EBMT registry over a 5-year period after the date of marketing authorization. The patient registration form collects only the off-label transplant use indication and patient identification items.

Inclusion period Transplants between **31 July 2009** and **31 July 2014**

Participating Centres

401 centres were invited by regular mail to register patients who received plerixafor in an off-label setting. Series of reminders were sent by e-mail.

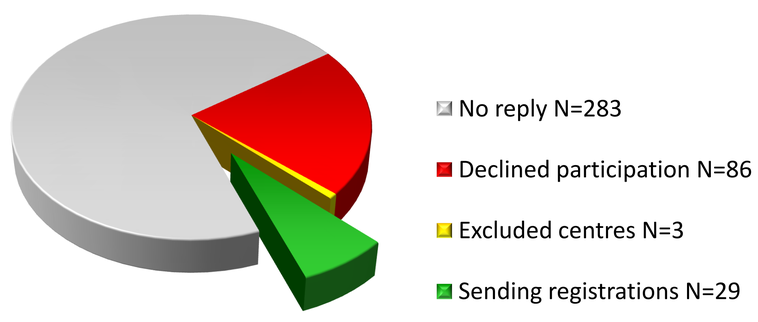
Progress of centre participation
31 July 2009 - 28 February 2015



29 centres included patients, while **3** centres were excluded because their registered patients later appeared to be not eligible.

86 centres declined participation.

401 centres invited



Call for final data submission

Plerixafor Off-label report planned: 1 May 2015

Contact

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Plerixafor Off-label Study Code 42206645

Inclusion Criteria

Eligible patients Patients treated with plerixafor, who have one or more of the following:

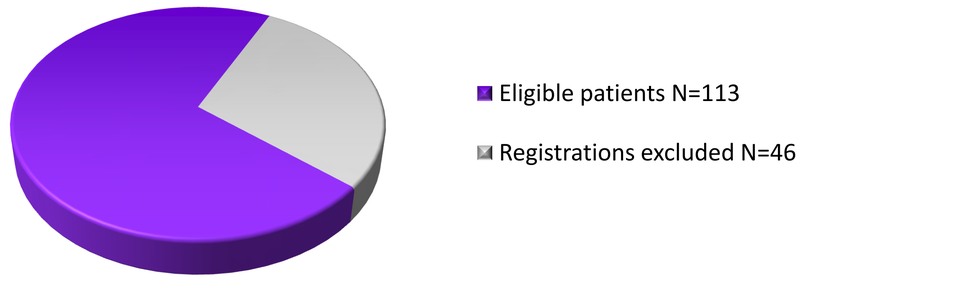
- ❑ Background disease other than lymphoma or multiple myeloma
- ❑ Younger than 18 years of age
- ❑ Transplant using ex-vivo plerixafor-mobilized cells (umbilical cord cell, peripheral blood, bone marrow cell collection)
- ❑ Treatment with plerixafor alone (i.e., without G-CSF)
- ❑ Contraindication for G-CSF
- ❑ Transplant using plerixafor-mobilized cells from allogeneic donor
- ❑ Transplant using plerixafor-mobilized BM cells
- ❑ Routes of plerixafor administration other than subcutaneous
- ❑ Do NOT have the diagnosis of poor mobilizer
- ❑ Other situations, e.g. plerixafor first-line use

Patient / Donor Inclusions

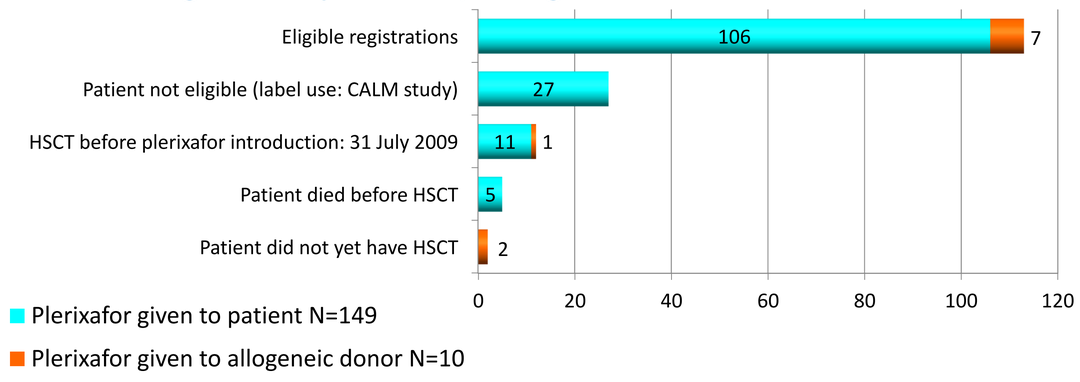
After data collection, while completing the data set, a number of registrations showed to be not eligible for the Plerixafor Off-label study: patients appeared to be eligible for the CALM study, they had a transplant before the inclusion date, they died before transplant, or they did not yet have the transplant.

Received registrations on 28 February 2015 **N=159**; eligible registrations **N=113**

159 registrations



Plerixafor given to patients / allogeneic donors



Participation per centre

Registrations on 28 Feb 2015, N=159