EMA/599869/2013

European Medicines Agency decision

P/0253/2013

of 29 October 2013

on the acceptance of a modification of an agreed paediatric investigation plan for plerixafor (Mozobil) (EMEA-000174-PIP01-07-M03) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This Decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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on the acceptance of a modification of an agreed paediatric investigation plan for plerixafor (Mozobil) (EMEA-000174-PIP01-07-M03) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/27/2009 issued on 23 February 2009, the decision P/232/2009 issued on 27 November 2009, and the decision P/48/2010 issued on 6 April 2010,

Having regard to the application submitted by Genzyme Europe B.V. on 24 June 2013 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 September 2013, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for plerixafor (Mozobil), solution for injection, subcutaneous use, intravenous use are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Genzyme Europe B.V., Gooimeer 10, 1411 DD – Naarden, The Netherlands.

Done at London, 29 October 2013

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/408155/2013

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-000174-PIP01-07-M03

Scope of the application

Active substance(s):

Plerixafor

Invented name:

Mozobil

Condition(s):

Myelosuppression caused by chemotherapy to treat malignant disorders, which requires an autologous haematopoietic stem cell transplant.

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Intravenous use

Name/corporate name of the PIP applicant:

Genzyme Europe B.V.

Information about the authorised medicinal product

See Annex II

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Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Genzyme Europe B.V. submitted to the European Medicines Agency on 24 June 2013 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/27/2009 issued on 23 February 2009, the decision P/232/2009 issued on 27 November 2009, and the decision P/48/2010 issued on 6 April 2010.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 17 July 2013.

Scope of the modification

Some measures have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

   The Icelandic and the Norwegian Paediatric Committee members agree with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

London, 13 September 2013

On behalf of the Paediatric Committee
Dr Dirk Mentzer, Chairman
(Signature on file)

Annex I

The measures and timelines of the agreed paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver

1. Waiver

1.1. Condition: Myelosuppression caused by chemotherapy to treat malignant disorders, which requires an autologous haematopoietic stem cell transplant.

The waiver applies to:

- Preterm newborn infants;
- term newborn infants from birth to less than 28 days;
- infants and toddlers from 28 days to less than 12 months;
- for solution for injection for subcutaneous and intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan

2.1. Condition to be investigated

Myelosuppression caused by chemotherapy to treat malignant disorders, which requires an autologous haematopoietic stem cell transplant.

2.1.1. Indication targeted by the PIP

Mobilisation of stem cells into the peripheral blood for harvesting by aphaeresis and subsequent autologous transplantation.

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 1 to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Solution for injection for subcutaneous and intravenous use.

2.1.4. Studies

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	1	Study No.1: Juvenile animal development study.
Clinical	1	Study No.2: Combined dose ranging and randomised, open label, comparative study

Area	Number of studies	Description
		of the efficacy and safety of plerixafor in addition to standard regimens for mobilisation of haematopoietic stem cells into peripheral blood, and subsequent collection by apheresis, versus standard mobilisation regimens alone in paediatric patients, aged 1 to less than 18 years, with solid tumours eligible for autologous transplants.

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety issues in relation to paediatric use:	Yes.
Date of completion of the paediatric investigation plan:	By June 2017.
Deferral for one or more studies contained in the paediatric investigation plan:	Yes.

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Myelosuppression caused by chemotherapy to treat malignant disorders, which requires an autologous haematopoietic stem cell transplant

   Authorised indication(s):

   - Mozobil is indicated in combination with G-CSF to enhance mobilisation of haematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in patients with lymphoma and multiple myeloma whose cells mobilise poorly.

Authorised pharmaceutical form(s):

Solution for injection

Authorised route(s) of administration:

Subcutaneous use