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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Plerixafor for adjunctive treatment to cytotoxic therapy in acute myeloid leukaemia

First publication	20 December 2011
Rev.1: withdrawal from the Community Register	9 February 2015
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in September 2014 on request of the Sponsor.

On 9 December 2011, orphan designation (EU/3/11/931) was granted by the European Commission to Genzyme Europe BV, the Netherlands, for plerixafor for adjunctive treatment to cytotoxic therapy in acute myeloid leukaemia.

What is acute myeloid leukaemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells (cells that fight against infections). In patients with AML, the bone marrow (the spongy tissue inside the large bones where blood cells are produced) produces large numbers of abnormal, immature white blood cells. These abnormal cells quickly build up in large numbers in the bone marrow and are found in the blood.

AML is a life-threatening disease because these abnormal immature cells take the place of the normal white blood cells, reducing the patient's ability to fight infections.

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What is the estimated number of patients affected by the condition?

At the time of designation, acute myeloid leukaemia affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

Treatment of AML is complex and depends on a number of factors including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. At the time of designation, the main treatments for AML were chemotherapy (medicines to treat cancer) and haematopoietic (blood) stem-cell transplantation, a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow. Haematopoietic stem cells are cells in the bone marrow that can develop into different types of blood cells.

The sponsor has provided sufficient information to show that plerixafor might be of significant benefit for patients with AML because it works in a different way to existing treatments and early studies show that it might improve the treatment of patients when used in combination with chemotherapy. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Plerixafor is already authorised in the EU as a treatment to mobilise haematopoietic stem cells from the bone marrow into the blood stream, in order to collect them prior to stem cell transplantation. Plerixafor works by blocking the activity of a protein called CXCR4, which helps to keep stem cells within the bone marrow. By blocking its activity, plerixafor allows the stem cells to be released into the blood stream.

In AML plerixafor is expected to mobilise the leukaemia cells from the bone marrow into the blood stream, by blocking the activity of CXCR4. When the leukaemia cells are released into the blood stream they are expected to become more susceptible to chemotherapy. Therefore, plerixafor is expected to be used together with chemotherapy. By blocking CXCR4, plerixafor is also expected to reduce the survival of leukaemia cells.

What is the stage of development of this medicine?

The effects of plerixafor have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with plerixafor in patients with AML were ongoing.

At the time of submission, plerixafor was authorised in the EU for use in patients with lymphoma and multiple myeloma to mobilise haematopoietic stem cells for autologous stem cell transplantation.

At the time of submission, orphan designation of plerixafor had been granted in the United States of America for use in the treatment of AML.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 October 2011 recommending the granting of this designation.

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Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

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For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Plerixafor	Adjunctive treatment to cytotoxic therapy in acute myeloid leukaemia
Bulgarian	Плериксафор	Допълнително лечение към цитотоксичната терапия на остра миелоидна левкемия
Czech	Plerixafor	Doplňková léčba k cytotoxické terapii akutní myeloidní leukemie
Danish	Plerixafor	Understøttende behandling til cytotoksisk terapi i akut myeloid leukæmi
Dutch	Plerixafor	Toegevoegde behandeling aan cytotoxische therapie bij acute myeloïde leukemie
Estonian	Pleriksafoor	Täiendava ravimina ägeda müeloidse leukeemia tsütotoksilises ravis
Finnish	Pleriksafori	Lisähoito sytostaattihoitoon akuutissa myelooisessa leukemiassa
French	Plérixafor	Traitement adjuvant à la thérapie cytotoxique de la leucémie aiguë myéloïde
German	Plerixafor	Ergänzende Behandlung im Rahmen einer zytotoxischen Therapie bei Akuter Myeloischer Leukämie
Greek	Πλεριξαφόρη	Ενισχυτική αγωγή στη θεραπεία της οξείας μυελοειδούς λευχαιμίας.
Hungarian	Plerixafor	Akut myeloid leukémia citotoxikus terápájának kiegészítő kezelése
Italian	Plerixafor	Trattamento adiuvante della terapia citotossica nella leucemia mieloide acuta
Latvian	Pleriksafors	Papildus ārstēšana akūtas mieloleikozes citotoksiskas terapijas gadījumā
Lithuanian	Pleriksaforas	Papildomas gydymas prie citotoksinės terapijos ūmiai mieloidinei leukemijai
Maltese	Plerixafor	Kura miżjuda mal-kura ċitotossika fil-lewkimja mjelojda akuta
Polish	Pleryksafor	Terapia wspomagająca w leczeniu cytotoksycznym ostrej białaczki szpikowej
Portuguese	Plerixafor	Tratamento adjuvante da terapêutica citotóxica da leucemia mielóide aguda
Romanian	Plerixafor	Tratament adjuvant al terapiei citotoxice în leucemia mieloidă acută
Slovak	Plerixafor	Adjuvantná liečba k cytotoxickej terapii pri akútnej myeloidnej leukémii
Slovenian	Pleriksafor	Adjuvantno zdravljenje k citostatični terapiji akutne mieloične levkemije
Spanish	Plerixafor	Tratamiento adyuvante a la terapia citotóxica en la leucemia mieloide aguda

[1] At the time of designation

Language	Active ingredient	Indication
Swedish	Plerixafor	Tilläggsbehandling till cellgiftsbehandling vid akut myeloisk leukemi
Norwegian	Pleriksafor	Tilleggsbehandling til cellegiftterapi ved akutt myelogen leukemi
Icelandic	Plerixafór	Viðbótarmeðferð við frumudrepandi lyfjameðferð við bráðu kyrninga hvitblæði