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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allogeneic bone marrow stem cells treated *ex vivo* with 16,16-dimethyl prostaglandin E2 for the treatment of acute myeloid leukaemia

On 13 May 2011, orphan designation (EU/3/11/866) was granted by the European Commission to Fate Therapeutics, LTD, United Kingdom, for allogeneic bone marrow stem cells treated *ex vivo* with 16,16-dimethyl prostaglandin E2 for the treatment of 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) 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.

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

At the time of designation, AML affected not more than 1.2 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of not more than 61,000 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?

The treatment for 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).

[*]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. This represents a population of 506,300,000 (Eurostat 2011).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with AML because early studies in experimental models show that it might improve the treatment of patients with this condition. 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?

This medicine is made up of bone marrow stem cells extracted from a donor that have been treated with a chemical called '16,16-dimethyl prostaglandin E2' (dmPGE2). Stem cells are cells that can develop into different types of cell. The bone marrow stem cells are used to replace the cells in the patient's bone marrow. Treatment with dmPGE2 is expected to improve the properties of the cells, making them better able to survive, multiply, and settle in the patient's bone marrow.

What is the stage of development of this medicine?

The effects of this medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with the medicine in patients with AML was ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for AML or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 February 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	Allogeneic bone marrow stem cells treated *ex vivo* with 16,16-dimethyl prostaglandin E2	Treatment of acute myeloid leukaemia
Bulgarian	Алогенни, костно-мозъчни стволови клетки, третирани *ex vivo* с 16,16-диметил простагландин Е2	Лечение на остра миелоидна левкемия
Czech	Allogenní kmenové buňky z kostní dřeně ošetřené *ex vivo* 16,16-dimetyl prostaglandinem E2	Léčba akutní myeloidní leukémie
Danish	Allogene knoglemarvsstamceller behandlet *ex vivo* med 16,16-dimethylprostaglandin E2	Behandling af akut myeloid leukæmi
Dutch	Allogene beenmergstamcellen *ex vivo* behandeld met 16,16-dimethyl-prostaglandine E2	Behandeling van acute myeloïde leukemie
Estonian	Allogeensed luuüdi tüvirakud, mida on *ex vivo* töödeldud 16,16-dimetüül prostaglandiin E2-ga	Akuutse müeloidse leukeemia ravi
Finnish	Allogeenisia luuytimen kantasoluja, jotka on käsitelty *ex vivo* 16,16-dimetyyliprostaglandiini E2:lla	Akuutin myelooisen leukemian hoito
French	Cellules souches médullaires allogéniques traitées *ex vivo* par la 16,16-diméthyl prostaglandine E2	Traitement de la leucémie aiguë myéloïde
German	Allogene Knochenmarkstammzellen, *ex vivo* mit 16,16-Dimethylprostaglandin E2 behandelt	Behandlung der akuten myeloischen Leukämie
Greek	Αλλογενή βλαστικά κύτταρα μυελού των οστών που έλαβαν επεξεργασία *ex vivo* με 16,16-διμεθυλο προσταγλαδίνη Ε2	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Allogén, csontvelőből származó őssejtek 16,16-dimetil-prosztaglandin-E2-vel, *ex vivo* kezelve	Akut myeloid leukaemia kezelése
Italian	Cellule staminali midollari allogeniche trattate *ex vivo* con 16,16-dimetil prostaglandina E2	Trattamento della leucemia mieloide acuta
Latvian	Alogēniskas kaulu smadzeņu cilmes šūnas, kas *ex vivo* apstrādātas ar 16,16-dimetilprostaglandīnu E2	Akūtas mieloleikozes ārstēšana
Lithuanian	Alogeninės kaulų čiulpų ląstelės *ex vivo* veiktos 16,16-dimetil-prostaglandinu E2	Ūmios mieloleukozės gydymas
Maltese	Ċelluli staminali alloġeniċi tal-mudullun ittrattati *ex vivo* b'16,16-dimethyl prostaglandin E2	Kura tal-lewkimja mjelojda akuta
Polish	Allogeniczne komórki macierzyste szpiku kostnego, poddane *ex vivo* działaniu 16,16-dimetyloprostaglandyny E2	Leczenie ostrej białaczki szpikowej
Portuguese	Células estaminais de medula óssea alogénicas tratadas *ex vivo* com 16,16-dimetilprostaglandina E2	Tratamento da leucémia mielóide aguda
Romanian	Celule stem medulare alogenice tratate *ex vivo* cu 16,16-dimetil prostaglandina E2	Tratamentul leucemiei mieloide acute
Slovak	Alogénne bunky kostnej drene ošetrené *ex vivo* 16,16-dimetyl prostaglandínom E2	Liečba akútnej myeloickej leukémie
Slovenian	Alogenske matične celice iz kostnega mozga, obdelane *ex vivo* s 16,16-dimetil prostaglandinom E2	Zdravljenje akutne mieloične levkemije

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Células madre de médula ósea alogénica tratadas *ex vivo* con 16,16-dimetil-PGE2	Tratamiento de la leucemia mieloide aguda
Swedish	Allogena stamceller från benmärgen behandlade *ex vivo* med 16,16-dimetylprostaglandin E2	Behandling av akut myeloisk leukemi
Norwegian	Allogene benmargsstamceller behandlet *ex vivo* med 16,16-dimetylprostaglandin E2	Behandling av akutt myelogen leukemi
Icelandic	Ósamgena beinmergsstofnfrumur meðhöndlaðar ex vivo með 16,16- dímetýl prostaglandíni E2	Meðferð við bráðu kyrningahvítblæði