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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allogeneic human dendritic cells derived from a CD34+ progenitor cell line for the treatment of acute myeloid leukaemia

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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.	

On 22 May 2012, orphan designation (EU/3/12/969) was granted by the European Commission to DCPrime BV, the Netherlands, for allogeneic human dendritic cells derived from a CD34+ progenitor cell line 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 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.

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

At the time of designation, AML affected less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 102,000 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).

*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 509,000,000 (Eurostat 2012).

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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 allogeneic human dendritic cells derived from a CD34+ progenitor cell line might be of significant benefit for patients with AML because it works in a different way to existing medicines which may allow it to be used in combination with other treatments. 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 a type of white blood cell called 'dendritic cells' which are produced from immature bone marrow cells (called CD34+ cells) from a donor with AML. These dendritic cells have on their surface specific molecules that are found in large amounts on cancer cells but are not found on normal cells. When the dendritic cells are injected into the patient, the immune system is expected to recognise them as 'foreign'. This is expected to stimulate an immune response against the AML cells in the body, resulting in the immune system killing the AML cells.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the effects of this medicine had not been evaluated in experimental models.

At the time of submission, clinical trials with the medicine in patients with AML were 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 11 January 2012 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 human dendritic cells derived from a CD34+ progenitor cell line	Treatment of acute myeloid leukaemia
Bulgarian	Алогенни човешки дендритни клетки, получени от прогениторни клетки от + CD34 линия	Лечение на остра миелоидна левкемия
Czech	Alogenní lidské dendritické buňky odvozené z CD34 + progenitorových buněčných linií	Léčba akutní myeloidní leukémie
Danish	Allogene humane dendritiske celler, der stammer fra en CD34 +-stamcelle cellelinie	Behandling af akut myeloid leukæmi
Dutch	Allogene humane dendritische cellen, afkomstig van een CD34 + progenitor cellijn	Behandeling van acute myeloïde leukemie
Estonian	Allogeensed inimese dendriitrakud, mis on saadud CD34 + tüvirakkude liinist	Akuutse müeloidse leukeemia ravi
Finnish	Allogeeniset CD34+ kantasolulinjasta johdetut ihmisen dendriittisolut	Akuutin myelooisen leukemian hoito
French	Cellules dendritiques allogeniques humaines dérivées d'une lignée cellulaire progénitrice de CD34+	Traitement de la leucémie aiguë myéloïde
German	Aus einer CD34 + Vorläuferzellen Zelllinie abgeleitete allogene humane dendritische Zellen	Behandlung der akuten myeloischen Leukämie
Greek	Αλλογενή ανθρώπινα δενδριτικά κύτταρα που προέρχονται από μια CD34 + γραμμή προγονικών κυττάρων	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	CD34+ őssejt-vonalból nyert allogén emberi dendritikus sejtek	Akut myeloid leukaemia kezelése
Italian	Trapianto allogenico di cellule dendritiche umane derivate da una linea + CD34 delle cellule progenitrici	Trattamento della leucemia mieloide acuta
Latvian	Alogēna cilvēka dendrītiskajās šūnām, kas iegūti no CD34 + cilmes šūnu līnijas	Akūtas mieloleikozes ārstēšana
Lithuanian	Alogeninės žmogaus dendritinės ląstelės, gautos iš CD34 + pirmtako ląstelių linijos	Ūmios mieloleukozės gydymas
Maltese	Alloġeniku ċelloli tal-bniedem dendritic derivat minn CD34 + linja ta 'ċelloli proġenituri	Kura tal-lewkimja mjelojda akuta
Polish	Allogeniczne ludzkie komórki dendrytyczne uzyskane z komórek CD34+ linii komórek macierzystych	Leczenie ostrej białaczki szpikowej
Portuguese	Alogênico células dendríticas derivadas de uma linhagem de células CD34 + progenitoras	Tratamento da leucémia mielóide aguda
Romanian	Celule dendritice umane alogenice derivate dintr-o linie de celule progenitoare CD34 +	Tratamentul leucemiei mieloide acute
Slovak	Alogénne ľudské dendritické bunky odvodené z CD34 + progenitorovej bunkovej línie	Liečba akútnej myeloickej leukémie

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Alogenska človeških dendritičnih celic, pridobljenih iz CD34 + matičnih celic line	Zdravljenje akutne mieloične levkemije
Spanish	Células dendríticas humanas alogénicas derivadas de una línea de células progenitoras CD34 positivas	Tratamiento de la leucemia mieloide aguda
Swedish	Allogena humana dendritiska celler som härrör från en CD34+ stamcellslinje	Behandling av akut myeloisk leukemi
Norwegian	Allogene humane dendrittiske celler avledet fra en CD34 + progenitor cellelinje	Behandling av akutt myelogen leukemi
Icelandic	Ósamgena frumur úr mönnum dendritic fengin frá CD34 + stofnfrumur frumulína	Meðferð við bráðu kyrningahvítblæði