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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Murine monoclonal antibody against CD26 for the treatment of graft-versus-host disease

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Rev.1: sponsor's name and address change	20 January 2014

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 26 November 2010, orphan designation (EU/3/10/808) was granted by the European Commission to ADIENNE S.r.l., Italy, for murine monoclonal antibody against CD26 for the treatment of graft-versus-host disease.

In January 2014, ADIENNE S.r.l. changed name to ADIENNE S.r.l.S.U.

What is graft-versus-host disease?

Graft-versus-host disease (GvHD) is a complication that can affect patients who have received allogeneic haematopoietic (blood) stem-cell transplantation (HCT). This is a complex procedure used to treat diseases such as leukaemia or multiple myeloma (cancers of the white blood cells), whereby a patient receives stem cells from a matched donor to help restore the bone marrow, which produces new blood cells.

In GvHD, the transplanted cells recognise the patient as 'foreign' and attack the patients' organs, such as the stomach, gut, skin and liver, leading to organ damage. GvHD may happen shortly after transplantation or later on, in which case a wider range of organs can be involved. GvHD is a serious and life-threatening disease with a high mortality rate.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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

At the time of designation, GvHD affected less than 0.4 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 20,000 people*, and is below the threshold 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?

At the time of submission of the application for orphan drug designation, several medicines were authorised in the European Union (EU) for GvHD, such as ciclosporin and corticosteroids. Treatment aimed to reduce the activity of immune cells involved in GvHD, thereby reducing their ability to attack the patient's organs.

The sponsor has provided sufficient information to show that murine monoclonal antibody against CD26 might be of significant benefit for patients with GvHD because early studies indicate that it might improve the treatment particularly of patients who do not respond to corticosteroids. 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 a monoclonal antibody (a type of protein) that has been designed to attach to a receptor called CD26, which is present on the surface of T-cells. These are cells of the immune system that are involved in GvHD.

CD26 is responsible for stimulating the T-cells to divide. By attaching to CD26, this medicine is expected to block its activity, reducing the rate at which the T-cells multiply. This reduces the number of activated T-cells and lowers the risk of these cells attacking the patient's organs.

What is the stage of development of this medicine?

The effects of murine monoclonal antibody against CD26 have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with GvHD were planned.

At the time of submission, murine monoclonal antibody against CD26 was not authorised anywhere in the EU for GvHD 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 September 2010 recommending the granting of this designation.

*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 506,300,000 (Eurostat 2010).

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:

ADIENNE S.r.l.S.U.
Via Galileo Galilei, 19
20867 Caponago (MB)
Italy
Tel.: +39 02 4070 0445
Fax: +39 02 9574 5179
E-mail: adienne@adienne.com

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	Murine monoclonal antibody against CD26	Treatment of graft-versus-host disease
Bulgarian	Мише моноклонално антитяло срещу CD26	Лечение на реакция на присадката срещу приемателя
Czech	Myší monoklonální protilátka proti CD26	Léčba reakce štěpu proti hostiteli
Danish	Murint monoklonalt antistof mod CD26	Behandling af graft versus host reaktion
Dutch	Murien monoklonaal antilichaam tegen CD26	Behandeling van graft versus host ziekte
Estonian	Hiire monoklonaalne antikeha CD26 vastu	Graft versus host haiguse ravi
Finnish	Hiiren monoklonaalinen anti-CD26-vasta-aine	Käänteishyljintäreaktion hoito
French	Anticorps monoclonal murin anti-CD26	Traitement de la réaction du greffon contre l'hôte
German	Muriner monoklonaler anti-CD26-Antikörper	Behandlung der Graft-versus-Host-Reaktion
Greek	Μονοκλωνικό αντίσωμα τρωκτικού κατά του CD26	Θεραπεία της αντίδρασης του μοσχεύματος
Hungarian	CD26 elleni egér/patkány monoklonális antitest	Graft-versus-host betegség kezelése
Italian	Anticorpo monoclonale murino anti CD26	Trattamento della reazione del trapianto contro l'ospite
Latvian	Peļu monoklonālās antivielas pret CD26	Saimnieka-transplantāta slimības ārstēšana
Lithuanian	Pelės monokloninis antikūnas prieš CD26	Transplantato atmetimo ligos gydymas
Maltese	Antikorp monoklonali tal-ġrieden għall-CD26	Kura tal-marda tat-tessut għat-trapjant kontra dak li jirċievih
Polish	Mysie przeciwciało monoklonalne przeciw CD26	Leczenie choroby przeszczep przeciw gospodarzowi
Portuguese	Anticorpo monoclonal murino anti-CD26	Tratamento da reacção do enxerto contra o hospedeiro
Romanian	Anticorp monoclonal murin anti CD26	Tratamentul reacţiei grefei contra gazdei
Slovak	Myšia monoklonálna protilátka proti CD26	Liečba reakcie štepu proti hostiteľovi
Slovenian	Mišje monoklonsko protitelo proti antigenu CD26	Zdravljenje bolezni presadka proti gostitelju
Spanish	Anticuerpo monoclonal murino anti-CD26	Tratamiento de la enfermedad de injerto contra huésped
Swedish	Murin monoklonal antikropp mot CD26	Behandling av graft-värd host reaktion
Norwegian	Murint monoklonalt antistoff mot CD26	Behandling av graft-versus-host -reaksjon
Icelandic	Músa einstofna mótefni gegn CD26	Til meðferðar á hýsilssótt

[1] At the time of designation