



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Rimiducid for treatment of graft-versus-host disease

On 30 May 2016, orphan designation (EU/3/16/1666) was granted by the European Commission to QRC Consultants Ltd., United Kingdom, for rimiducid (AP1903) for the treatment of graft-versus-host disease.

What is graft-versus-host disease?

Graft-versus-host disease is a complication that can affect patients who have had allogeneic haematopoietic (blood) stem-cell transplantation to treat diseases of the blood such as leukaemia (a cancer of the white blood cells). The procedure involves a patient receiving stem cells from a matched donor to help restore the bone marrow, which produces new blood cells.

Graft-versus-host disease occurs when transplanted cells recognise the patient's body as 'foreign' and attack the patient's organs, such as the stomach, gut, skin and liver, leading to organ damage. The disease may happen shortly after transplantation or later on, in which case a wider range of organs can be involved.

Graft-versus-host disease is a serious and life-threatening disease with a high mortality rate.

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

At the time of designation, graft-versus-host disease affected approximately 0.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 21,000 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?

At the time of designation, several medicines were authorised in the European Union (EU) for the treatment of graft-versus-host disease, such as ciclosporin and antilymphocyte immunoglobulins.

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).



Corticosteroids were also routinely used. Treatment aimed to reduce the activity of transplanted cells involved in graft-versus-host disease, thereby reducing their ability to attack the patient's organs.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with graft-versus-host disease because early studies showed that its use reduced the need for corticosteroid treatment. 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?

Before the patient receives a stem-cell transplant, T cells (a type of immune cell) are separated from the transplant donor and are genetically modified to include a 'suicide gene'. When the patient is given the transplant, the suicide gene in the T cells is switched off. The T cells are expected to help the patient to fight off virus infections while their immune system is being restored with the transplanted stem cells.

Rimiducid is only given to those patients who after transplantation develop graft-versus-host disease because the transplanted T cells attack the patient's organs. Rimiducid works by attaching to a molecule in the modified T cells and switching on the suicide gene, causing the cell to die, and so preventing further attack.

What is the stage of development of this medicine?

The effects of rimiducid have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with rimiducid in patients with graft-versus-host disease were ongoing.

At the time of submission, rimiducid was not authorised anywhere in the EU for graft-versus-host disease. Orphan designation of rimiducid had been granted in the United States for replacement T-cell therapy for the treatment of immunodeficiency and graft-versus-host disease after allogeneic hematopoietic stem cell transplant.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 April 2016 recommending the granting of this designation.

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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Rimiducid	Treatment of graft-versus-host disease
Bulgarian	Римидуцид	Лечение на болестта на присадката срещу приемателя
Croatian	Rimiducid	Liječenje reakcije presatka protiv primatelja
Czech	Rimiducid	Léčba reakce štěpu proti hostiteli
Danish	Rimiducid	Behandling af graft versus host reaktion
Dutch	Rimiducid	Behandeling van graft versus host ziekte
Estonian	Rimidutsiid	Graft versus host haiguse ravi
Finnish	Rimidusidi	Käänteishyljintäreaktion hoito
French	Rimiducid	Traitement de la réaction du greffon contre l'hôte
German	Rimiducid	Behandlung der Graft-versus-Host-Reaktion
Greek	Ριμιδουσιδη	Θεραπεία της αντίδρασης του μοσχεύματος
Hungarian	Rimiducid	Graft-versus-host betegség kezelése
Italian	Rimiducid	Trattamento della reazione del trapianto contro l'ospite
Latvian	Rimiducid	Saimnieka-transplantāta slimības ārstēšana
Lithuanian	Rimiducidas	Transplantato atmetimo ligos gydymas
Maltese	Rimiducid	Kura tal-marda tat-tessut għat-trapjant kontra dak li jirċievih
Polish	Rimiducid	Leczenie choroby przeszczep przeciw gospodarzowi
Portuguese	Rimiducida	Tratamento da reacção do enxerto contra o hospedeiro
Romanian	Rimiducid	Tratamentul reacției grefei contra gazdei
Slovak	Rimiducid	Liečba reakcie štepů proti hostiteľovi
Slovenian	Rimiducid	Zdravljenje bolezni presadka proti gostitelju
Spanish	Rimiducid	Tratamiento de la enfermedad de injerto contra huésped
Swedish	Rimiducid	Behandling av graft-värd host reaktion
Norwegian	Rimiducid	Behandling av graft-versus-host -reaksjon
Icelandic	Rímidúcið	Til meðferðar á hýsilssótt

¹ At the time of designation