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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Rituximab for treatment in solid organ transplantation

On 20 April 2017, orphan designation (EU/3/17/1869) was granted by the European Commission to Hôpital Foch, France, for rituximab for treatment in solid organ transplantation.

What is solid organ transplantation?

Solid organ transplantation is a surgical procedure in which a diseased organ, such as the heart, lungs, liver or kidney, is replaced with an organ from a donor.

Transplantation is a very complex procedure. During transplantation, the organ to be transplanted can become damaged because of the interruption and restoration of blood supply to the organ. In addition, graft rejection can occur after transplantation, when the recipient's body rejects the transplanted organ. Graft rejection is caused by the patient's immune system (the body's natural defences) recognising the transplanted graft as 'foreign' and attacking it.

These complications can be debilitating and life-threatening because they may result in the transplanted organ not working properly.

What is the estimated number of patients receiving solid organ transplants?

At the time of designation, approximately 1 in 10,000 people per year in the European Union (EU) have solid organ transplants. This was equivalent to a total of around 52,000 people per year*, and is below the ceiling for orphan designation. 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 EU to treat graft rejection in solid organ transplantation. These include antibodies such as antilymphocyte immunoglobulin and thymoglobulin, medicines that suppress the immune process such as azathioprine, ciclosporin,

*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 (European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).

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mycophenolate mofetil and tacrolimus, and corticosteroids such as prednisolone or methylprednisolone.

The sponsor has provided sufficient information to show that rituximab might be of significant benefit for patients undergoing solid organ transplantation because published and unpublished studies indicate that this medicine may improve survival of the transplanted organ. 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?

Rituximab is a monoclonal antibody (a type of protein) designed to recognise and attach to a protein called CD20 on the surface of B cells (types of white blood cells). When rituximab attaches to CD20, it causes the destruction of B cells. Destroying the B cells lowers the production of antibodies, which stops the patient's immune system from recognising the transplanted organ as foreign, thereby reducing the immune system's attack on the transplanted organ. This is expected to decrease organ damage and lower the risk of the transplanted organ not working.

What is the stage of development of this medicine?

The effects of rituximab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with rituximab in patients undergoing solid organ transplantation were ongoing.

At the time of submission, rituximab was authorised in the EU for treatment of certain blood cancers and inflammatory conditions.

At the time of submission, rituximab was not authorised anywhere in the EU for use in solid organ transplantation 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 15 March 2017 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:

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Rituximab	Treatment in solid organ transplantation
Bulgarian	Ритуксимаб	Лечение при трансплантация на солиден орган
Croatian	Rituksimab	Liječenje u transplantaciji solidnih organa
Czech	Rituximab	Léčba pro transplantaci solidních orgánů
Danish	Rituximab	Behandling i organ transplantation
Dutch	Rituximab	Behandeling bij soliede orgaantransplantatie
Estonian	Rituksimaab	Kasutamiseks elundite siirdamise ravis
Finnish	Rituksimabi	Hoito kiinteän elimen siirron yhteydessä
French	Rituximab	Traitement pour la transplantation d'organes solides
German	Rituximab	Behandlung in Organtransplantation
Greek	Ριτουξιμάμπη	Θεραπεία στη μεταμόσχευση συμπαγών οργάνων
Hungarian	Rituximab	Szervtranszplantáció esetén alkalmazandó
Italian	Rituximab	Trattamento nel trapianto di organi solidi
Latvian	Rituksimabs	Ārstēšanai norobežoto orgānu transplantācijā
Lithuanian	Rituksimabas	Transplantacijos parenchiminiame organe gydymas
Maltese	Ritussimab	Kura fi trapjant ta' organi solidi
Polish	Rytuksymab	Leczenie w przebiegu transplantacji organów
Portuguese	Rituximab	Tratamento em transplante de órgãos sólidos
Romanian	Rituximab	Tratament în transplantul de organe solide
Slovak	Rituximab	Liečba pri transplantácii celého orgánu
Slovenian	Rituksimab	Zdravljenje pri transplantaciji organov
Spanish	Rituximab	Tratamiento del transplante de organo solido
Swedish	Rituximab	Behandling vid organtransplantation
Norwegian	Rituksimab	Behandling ved transplantasjon av solide organer
Icelandic	Rítúxímab	Meðferð við lífæraígræðslu

[1] At the time of designation