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Public summary of opinion on orphan designation

Siplizumab for the treatment in solid organ transplantation

On 16 October 2017, orphan designation (EU/3/17/1931) was granted by the European Commission to ITB-MED AB, Sweden, for siplizumab for the 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 in the European Union (EU) were undergoing solid organ transplantation every year. 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 prevent or treat graft rejection in solid organ transplantation. These include antibodies such as antilymphocyte immunoglobulin and thymoglobulin and other medicines that suppress immune processes such as

*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 515,700,000 (Eurostat 2017).

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

The sponsor has provided sufficient information to show that siplizumab might be of significant benefit for patients undergoing solid organ transplantation. Early studies indicate that the medicine could help the recipient's body accept the transplant in the long term, allowing the recipient to stop taking medicines that suppress the immune system.

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?

Siplizumab is a monoclonal antibody (a type of protein) designed to recognise and attach to a protein called CD2 on the surface of T-lymphocytes, a type of white blood cell. When siplizumab attaches to CD2, it stops the white blood cells 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 siplizumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with siplizumab in patients with solid organ transplants were ongoing.

At the time of submission, siplizumab was not authorised anywhere in the EU for use in patients with solid organ transplants or designated as an orphan medicinal product elsewhere for this use.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 September 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	Siplizumab	Treatment in solid organ transplantation
Bulgarian	Сиплизумаб	Лечение при трансплантация на солиден орган
Croatian	Siplizumab	Liječenje u transplantaciji solidnih organa
Czech	Siplizumab	Léčba pro transplantaci solidních orgánů
Danish	Siplizumab	Behandling i organ transplantation
Dutch	Siplizumab	Behandeling bij soliede orgaantransplantatie
Estonian	Siplizumab	Kasutamiseks elundite siirdamise ravis
Finnish	Siplitsumabi	Hoito kiinteän elimen siirron yhteydessä
French	Siplizumab	Traitement pour la transplantation d'organes solides
German	Siplizumab	Behandlung in Organtransplantation
Greek	Σιπλιζουμάβη	Θεραπεία στη μεταμόσχευση συμπαγών οργάνων
Hungarian	Siplizumab	Szervtranszplantáció esetén alkalmazandó
Italian	Siplizumab	Trattamento nel trapianto di organi solidi
Latvian	Siplizumabs	Ārstēšanai norobežoto orgānu transplantācijā
Lithuanian	Siplizumabas	Transplantacijos parenchiminiame organe gydymas
Maltese	Siplizumab	Kura fi trapjant ta' organi solidi
Polish	Siplizumab	Leczenie w przebiegu transplantacji organów
Portuguese	Siplizumab	Tratamento em transplante de órgãos sólidos
Romanian	Siplizumab	Tratament în transplantul de organe solide
Slovak	Siplizumab	Liečba pri transplantácii celého orgánu
Slovenian	Siplizumab	Zdravljenje pri transplantaciji organov
Spanish	Siplizumab	Tratamiento del trasplante de órgano sólido
Swedish	Siplizumab	Behandling vid organtransplantation.
Norwegian	Siplizumab	Behandling ved transplantasjon av solide organer
Icelandic	Siplízúmab	Meðferð við líffæraígræðslu

[1] At the time of designation