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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Eculizumab for the prevention of delayed graft function after solid organ transplantation

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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.

Withdrawn

On 19 February 2014, orphan designation (EU/3/14/1238) was granted by the European Commission to Alexion Europe SAS, France, for eculizumab for the prevention of delayed graft function after solid organ transplantation.

What is delayed graft function after solid organ transplantation?

Delayed graft function is the failure of a transplanted organ to start working properly in the first few days after the transplant. Delayed graft function following transplantation of a solid organ can occur as a result of damage to the organ caused by the interruption and restoration of blood flow. This is called 'ischaemia/reperfusion injury' and is associated with an inflammatory reaction, caused in part by the invasion of neutrophils (a type of white blood cell) into the transplanted organ.

Delayed graft function after solid organ transplantation is a debilitating and life-threatening condition because of the risk of losing the transplanted organ.

What is the estimated number of patients at risk of developing the condition?

At the time of designation, the number of patients at risk of delayed graft function after solid organ transplantation was estimated to be not more than 0.6 people in 10,000 in the European Union (EU).

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This was equivalent to a total of not more than 31,000 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 methods of prevention are available?

At the time of designation, no satisfactory methods were authorised in the EU for the prevention of delayed graft function after solid organ transplantation. Several preventative measures were commonly used to reduce the risk of delayed graft function, including careful selection of the organ donor and preservation of the organ during transport.

How is this medicine expected to work?

Eculizumab is a monoclonal antibody, a type of protein that has been designed to recognise and attach to a specific structure (called an antigen) that is found in the body. It is expected to work by attaching and blocking the action of a protein called the C5 complement protein, which is involved in the inflammatory processes that can cause injury to the transplanted organ. This is expected to reduce the risk of delayed graft function after solid organ transplantation.

What is the stage of development of this medicine?

The effects of eculizumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with eculizumab in patients who had undergone solid organ transplantation were ongoing.

At the time of submission, eculizumab was not authorised anywhere in the EU for preventing delayed function after solid organ transplantation or designated as an orphan medicinal product elsewhere for this indication.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 January 2014 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.

*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.
At the time of designation, this represented a population of 512,900,000 (Eurostat 2014).

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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.

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Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Eculizumab	Prevention of delayed graft function after solid organ transplantation
Bulgarian	Екулизумаб	Предотвратяване на забавено функциониране на присадката при органна трансплантация
Czech	Eculizumab	prevence opožděné funkce štěpu po transplantaci orgánu
Croatian	Ekulizumab	Prevencija odgođene funkcije presatka nakon transplantacije solidnih organa
Danish	Eculizumab	Forebyggelse af forsinket transplanteringsfunktion i forbindelse med organtransplantation
Dutch	Eculizumab	Preventie van vertraagde orgaan transplantaatfunctie
Estonian	Ekulizumab	Transplantaadi funktsiooni hilinemise tõkestamine pärast elundisiirdamist
Finnish	Ekulitsumabi	Siirrännäisen toimintaviiveen ehkäisy elinsiirrossa
French	Eculizumab	Prévention des retards fonctionnels du greffon dans la greffe d'organe.
German	Eculizumab	Vorbeugung der verzögerten Transplantatfunktion nach Organtransplantation
Greek	Εκουλιζουμάμπη	Πρόληψη της καθυστερημένης λειτουργίας του μοσχεύματος κατά τη μεταμόσχευση οργάνων
Hungarian	Ekulizumab	Szervtranszplantációt követő késői graftfunkció megelőzése
Italian	Eculizumab	Prevenzione del ritardo nella funzionalita' nell'organo trapiantato
Latvian	Ekulizumabs	Aizkavētas transplantāta funkcijas profilaksei pēc parenhimatozo orgānu transplantācijas
Lithuanian	Ekulizumabas	Vėlyvos transplantato atmetimo reakcijos prevencija po organo transplantavimo
Maltese	Eculizumab	Prevenzjoni ta' ttardjar fil-funzjonalità ta' trapjant wara trapjant ta' organu
Polish	Ekulizumab	Zapobieganie opóźnieniu podjęcia czynności przez przeszczepiony narząd
Portuguese	Eculizumab	Prevenção do atraso funcional do órgão transplantado
Romanian	Eculizumab	Prevenirea întârzierii funcţionării grefei de organ transplantat
Slovak	Ekulizumab	Prevencia oneskorenej funkcie štepu po transplantácii orgánu
Slovenian	Ekulizumab	Preprečevanje zapoznelega začetka delovanja presadka po presaditvi organa
Spanish	Eculizumab	Prevención del retraso de la función del órgano transplantado
Swedish	Eculizumab	Förebyggande av fördröjd transplantatfunktion i samband med organtransplantation
Norwegian	Ekulizumab	Forebyggelse av forsinket transplantatfunksjon i forbindelse med organtransplantasjon
Icelandic	Ecúlízúmab	Til að koma í veg fyrir síðkomna starfsbilun eftir líffæraígræðslu

[1] At the time of designation