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Public summary of opinion on orphan designation

Eculizumab for the treatment of neuromyelitis optica spectrum disorders

On 24 April 2019, orphan designation (EU/3/13/1185) was granted by the European Commission to Alexion Europe S.A.S., France, for eculizumab for the treatment of neuromyelitis optica spectrum disorders.

What are neuromyelitis optica spectrum disorders?

Neuromyelitis optica spectrum disorders are inflammatory disorders that affect mostly the optic (eye) nerve and the spinal cord. They can lead to reduction or loss of vision, loss of sensation, loss of bladder control, weakness and paralysis of the arms and legs.

The disorders occur more frequently in women than in men. They are thought to be caused by the immune system (the body's natural defences) damaging nerve cells.

Neuromyelitis optica spectrum disorders are debilitating and life threatening due to damage to the nervous system function.

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

At the time of designation, neuromyelitis optica spectrum disorders 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, no satisfactory methods were authorised in the EU for the treatment of neuromyelitis optica spectrum disorders. Treatments aimed at reducing inflammation. They included glucocorticoids, immunosuppressants and plasmapheresis (also called plasma exchange, a procedure to remove antibodies from the liquid part of the blood).

*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 518,400,000 (Eurostat 2019).

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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. In patients with neuromyelitis optica spectrum disorders, eculizumab is expected to attach to a protein called C5 complement protein that plays a key role in the immune system. By attaching to and blocking the C5 complement protein, eculizumab prevents part of the immune system becoming activated, thereby reducing the damage to nerve cells and relieving the symptoms of the disease.

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 with neuromyelitis optica spectrum disorders were ongoing.

At the time of submission, eculizumab was authorised in the EU for paroxysmal nocturnal haemoglobinuria and atypical haemolytic uraemic syndrome.

At the time of submission, eculizumab was not authorised anywhere in the EU for the treatment of neuromyelitis optica spectrum disorders. Orphan designation of eculizumab had been granted in the United States for treatment of neuromyelitis optica.

This medicine had been designated orphan on 5 August 2013 for the treatment of neuromyelitis optica. At the request of the sponsor and having assessed the additional information submitted, the COMP adopted an opinion on 21 March 2019 recommending the change of the orphan condition from neuromyelitis optica to neuromyelitis optica spectrum disorders, in accordance with the most recent disease classification.

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.

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	Eculizumab	Treatment of neuromyelitis optica spectrum disorders
Bulgarian	Екулизумаб	Лечение на невромиелитис оптика и подобни нарушения
Croatian	Ekulizumab	Liječenje spektra poremećaja optičkog neuromijelitisa
Czech	Eculizumab	Léčba chorob v rámci neuromyelitis optica
Danish	Eculizumab	Behandling af neuromyelitis optica spektrum forstyrrelser
Dutch	Eculizumab	Behandeling van neuromyelitis optica spectrum aandoeningen
Estonian	Ekulizumab	Nägemisnärvi neuromüeliidi spektrumi häirete ravi
Finnish	Ekulitsumabi	Neuromyelitis optican tautikirjon hoito
French	Eculizumab	Traitement des désordres du spectre de la neuromyélite optique (NMO)
German	Eculizumab	Neuromyelitis optica-Spektrum-Erkrankung
Greek	Εκουλιζουμάμπη	Θεραπεία των διαταραχών του φάσματος της Οπτικής Νευρομυελίτιδας
Hungarian	Eculizumab	Neuromyelitis optica spektrum betegségek kezelése
Italian	Eculizumab	Trattamento dei disturbi dello spettro della neuromielite ottica
Latvian	Ekulizumabs	Optiskā neiromielīta spektra traucējumu ārstēšana
Lithuanian	Ekulizumabas	Optinio neuromielito ligų spektro gydymas
Maltese	Eculizumab	Kura ta' mard tal-firxa ta' newromelite optika
Polish	Ekulizumab	Leczenie chorób ze spektrum zapalenia rdzenia i nerwów wzrokowych
Portuguese	Eculizumab	Tratamento de doenças do espectro da neuromielite óptica
Romanian	Eculizumab	Tratamentul spectrului de boli al neuromielitei optice
Slovak	Ekulizumab	Liečba spektra porúch pri optickej neuromyelitíde
Slovenian	Ekulizumab	Zdravljenje spektra motenj nevromielitisa vidnega živca
Spanish	Eculizumab	Tratamiento para el espectro de desordenes de la neuromielitis óptica
Swedish	Eculizumab	Behandling av neuromyelitis optica spektrumtillstånd
Norwegian	Ekulizumab	Behandling av neuromyelitis optica spekter forstyrrelser
Icelandic	Ecúlízúmab	Meðferð neuromyelitis Optica litróf sjúkdóma

[1] At the time of designation