13 March 2015
EMA/COMP/439730/2013 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Eculizumab for the treatment of neuromyelitis optica

First publication	12 September 2013
Rev.1: sponsor's change of address	13 March 2015
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.	

On 5 August 2013, orphan designation (EU/3/13/1185) was granted by the European Commission to Alexion Europe SAS, France, for eculizumab for the treatment of neuromyelitis optica.

What is neuromyelitis optica?

Neuromyelitis optica is an inflammatory disease of the optic nerve and the spinal cord that can lead to the reduction or loss of vision, loss of sensation, bladder dysfunction, weakness and paralysis of the arms and legs.

The cause of the disease is not known but it is thought to involve the complement system, a group of proteins in the blood that help the immune system (the body's natural defences) to fight infections. In patients with neuromyelitis optica, the complement system gets activated and results in damage to the nerve cells.

Neuromyelitis optica is a debilitating disease that can be life threatening due to damage to the nervous system function.

What is the estimated number of patients by the condition?

At the time of designation, neuromyelitis optica affected approximately 0.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 20,000 people*, and is below the ceiling

*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 512,200,000 (Eurostat 2013).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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. Treatments for this disease were aimed at reducing inflammation. They included glucocorticoids, immunosuppressants and plasmapheresis, also called plasma exchange (or passage of the blood plasma through a filter).

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, eculizumab is expected to attach to the C5 complement protein. By attaching and blocking the C5 complement protein, eculizumab prevents the complement system activation, 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 were planned.

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, it was not authorised anywhere in the EU for neuromyelitis optica 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 11 July 2013 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:

Alexion Europe SAS
1-15, avenue Edouard Belin
92500 Rueil-Malmaison
France
Tel. +33 1 47 32 36 21
Fax +33 1 47 10 24 46
E-mail: medicalinformation.europe@alxn.com

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
Bulgarian	Екулизумаб	Лечение на невромиелитис оптика
Croatian	Ekulizumab	Liječenje optičkog neuromijelitisa
Czech	Eculizumab	Léčba optické neuromyelitidy
Danish	Eculizumab	Behandling af Neuromyelitis optica
Dutch	Eculizumab	Behandeling van neuromyelitis optica
Estonian	Ekulizumab	Nägemisnärvi neuromüeliidi ravi
Finnish	Ekulitsumabi	Neuromyelitis optican hoito
French	Eculizumab	Traitement de la neuromyélite optique (NMO)
German	Eculizumab	Behandlung von Neuromyelitis Optica
Greek	Εκουλιζουμάμπη	Θεραπεία της Οπτικής Νευρομυελίτιδας
Hungarian	Eculizumab	Neuromyelitis optica kezelése
Italian	Eculizumab	Trattamento della Neuromielite Ottica
Latvian	Ekulizumabs	Optiskā neiromielīta ārstēšana
Lithuanian	Ekulizumabas	Optinio neuromielito gydymas
Maltese	Eculizumab	Kura tan-newromelite optika
Polish	Ekulizumab	Leczenie zapalenia rdzenia i nerwów wzrokowych (zespołu Devica)
Portuguese	Eculizumab	Tratamento da neuromielite óptica
Romanian	Eculizumab	Tratamentul neuromielitei optice
Slovak	Ekulizumab	Liečba optickej neuromyelitídy
Slovenian	Ekulizumab	zdravljenje nevromielitisa vidnega živca
Spanish	Eculizumab	Tratamiento para la neuromielitis óptica
Swedish	Eculizumab	Behandling av neuromyelitis optica
Norwegian	Ekulizumab	Behandling av neuromyelitis optica
Icelandic	Ecúlízúmab	Meðhöndlun á sjóntaugar- og mænubólgu

[1] At the time of designation