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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Inebilizumab for the treatment of neuromyelitis optica spectrum disorders

On 20 March 2017, orphan designation (EU/3/17/1856) was granted by the European Commission to AstraZeneca AB, Sweden, for inebilizumab (also known as MEDI-551) for the treatment of neuromyelitis optica spectrum disorders.

What are neuromyelitis optica spectrum disorders?

Neuromyelitis optica spectrum disorders are inflammatory disorders that mainly affect 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) mistakenly producing antibodies that attack the 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 were aimed at reducing inflammation. They

*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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included medicines such as corticosteroids and immunosuppressants, as well as plasmapheresis (also called plasma exchange, a procedure to remove antibodies from the liquid part of the blood).

How is this medicine expected to work?

Inebilizumab is a monoclonal antibody (a type of protein) that attaches to a type of immune cell called CD19+ B cells and causes their death. CD19+ B cells produce antibodies responsible for attacking the nerves in neuromyelitis optica spectrum disorders. By reducing the numbers of B cells, the medicine is expected to prevent damage to nerve cells and reduce the symptoms of the condition.

What is the stage of development of this medicine?

The effects of inebilizumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with inebilizumab in patients with neuromyelitis optica spectrum disorders were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 February 2017 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:

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

[1] At the time of designation