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EMA/COMP/395313/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised anti-IL-6 receptor monoclonal antibody for the treatment of neuromyelitis optica spectrum disorders

On 27 June 2016, orphan designation (EU/3/16/1680) was granted by the European Commission to Chugai Pharma Europe Ltd, UK, for humanised anti-IL-6 receptor monoclonal antibody (also known as SA237) 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 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 513,700,000 (Eurostat 2016).

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included glucocorticoids, immunosuppressants and plasmapheresis (also called plasma exchange, a procedure to remove certain substances from the liquid part of the blood).

How is this medicine expected to work?

Antibodies against the protein AQP4 are found in most patients with neuromyelitis optica spectrum disorders. AQP4 plays an important role in protecting nerve cells.

The medicine is a monoclonal antibody (a type of protein) that blocks the action of interleukin-6 (IL-6), a protein in the body involved in the production of antibodies against AQP4. By blocking IL-6, the medicine is expected to decrease the production of antibodies against AQP4 and therefore increase the activity of AQP4. This is expected to prevent damage to nerve cells and reduce the symptoms of neuromyelitis optica spectrum disorders.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 May 2016 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	Humanised anti-IL-6 receptor monoclonal antibody	Treatment of neuromyelitis optica spectrum disorders
Bulgarian	Хуманизирано моноклонално антитяло срещу IL-6 рецептор	Лечение на невромиелитис оптика и подобни нарушения
Croatian	Humanizirano monoklonsko antitijelo na receptor za IL-6	Liječenje spektra poremećaja optičkog neuromijelitisa
Czech	Humanizovaná monoklonální protilátka proti receptoru pro IL-6	Léčba chorob v rámci neuromyelitis optica
Danish	Humaniseret monoklonalt anti-IL-6-receptor antistof	Behandling af neuromyelitis optica spektrum forstyrrelser
Dutch	Gehumaniseerd anti-IL-6-receptor monoklonaal antilichaam	Behandeling van neuromyelitis optica spectrum aandoeningen
Estonian	Il-6 retseptori vastane humaniseeritud monokloonne antikeha	Nägemisnärvi neuromüeliidi spektrumi häirete ravi
Finnish	Humanisoitu anti-IL-6-reseptorin monoklonaalinen vasta-aine	Neuromyelitis optican tautikirjon hoito
French	Anticorps monoclonal humanisé dirigé contre le récepteur de l'IL6	Traitement des désordres du spectre de la neuromyélite optique (NMO)
German	Humanisierter monoklonaler Anti-IL-6-Rezeptor-Antikörper	Neuromyelitis optica-Spektrum-Erkrankung
Greek	Ανθρωποποιημένο μονοκλωνικό αντίσωμα κατά του υποδοχέα της ιντερλευκίνης 6 (IL-6)	Θεραπεία των διαταραχών του φάσματος της Οπτικής Νευρομυελίτιδας
Hungarian	Humanizált anti-IL-6 receptor monoklonális antitest	Neuromyelitis optica spektrum betegségek kezelése
Italian	Anticorpo monoclonale umanizzato contro il recettore di IL-6	Trattamento dei disturbi dello spettro della neuromielite ottica
Latvian	Humanizēta monoklonāla antiviela pret IL-6 receptoru	Optiskā neiromielīta spektra traucējumu ārstēšana
Lithuanian	Humanizuotas monokloninis antikūnas prieš IL-6 receptorių	Optinio neuromielito ligų spektro gydymas
Maltese	Antikorp monoklonali umanizzat kontra r-riċettur IL-6	Kura ta' mard tal-firxa ta' newromelite optika
Polish	Humanizowane przeciwciało monoklonalne skierowane przeciwko receptorowi dla IL-6	Leczenie chorób ze spektrum zapalenia rdzenia i nerwów wzrokowych
Portuguese	Anticorpo monoclonal humanizado anti-recetor IL-6	Tratamento de doenças do espectro da neuromielite óptica
Romanian	Anticorp monoclonal umanizat anti-receptor pentru IL -6	Tratamentul spectrului de boli al neuromielitei optice

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Humanizovaná monoklonálna protilátka proti receptoru pre interleukín IL-6	Liečba spektra porúch pri optickej neuromyelitíde
Slovenian	Humanizirano monoklonsko protitelo proti receptorju za IL-6	Zdravljenje spektra motenj nevromielitisa vidnega živca
Spanish	Anticuerpo monoclonal humanizado contra el receptor de IL-6	Tratamiento para el espectro de desordenes de la neuromielitis óptica
Swedish	Humaniserad monoklonal anti-IL-6-receptor antikropp	Behandling av neuromyelitis optica spektrumtillstånd
Norwegian	Humanisert monoklonalt anti-IL-6-reseptor antistoff	Behandling av neuromyelitis optica spekter forstyrrelser
Icelandic	Mannalagaður and-IL-6-viðtaka einstofna mótefnis	Meðferð neuromyelitis Optica litróf sjúkdóma