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Public summary of opinion on orphan designation

Rozanolixizumab for the treatment of myasthenia gravis

On 22 April 2020, orphan designation EU/3/20/2272 was granted by the European Commission to UCB Pharma, Belgium, for rozanolixizumab for the treatment of myasthenia gravis.

What is myasthenia gravis?

Myasthenia gravis is a disease that leads to muscle weakness and tiredness. It is an autoimmune disorder in which the immune system (the body's natural defences) attacks and damages muscle cells, making them unable to contract normally. For a muscle to contract, a substance called acetylcholine is released from a nerve and attaches to the acetylcholine receptors on the muscle cells. In myasthenia gravis, an immune system protein called IgG antibody triggers the immune system to damage these receptors and the muscles are not able to contract as well as normal.

In most patients, the disease is associated with abnormalities of a gland in the chest called the thymus, which is part of the immune system.

The muscles involved in swallowing and those around the eyes are commonly affected first, causing difficulty in swallowing and the eyelids to drop. Muscle weakness typically worsens towards the end of the day and after exercise.

Myasthenia gravis is a long-term debilitating disease and may be life-threatening when the muscles involved in breathing are affected.

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

At the time of designation, myasthenia gravis affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of 104,000 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).

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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What treatments are available?

At the time of designation, several medicines were authorised in the EU for the treatment of myasthenia gravis, including acetylcholinesterase inhibitors (medicines that prevent breakdown of acetylcholine) and medicines that work on the immune system. Surgery to remove the thymus gland (thymectomy) was performed in some patients.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with myasthenia gravis because early data showed that rozanolixizumab improved the symptoms of the disease in patients who were not adequately controlled on standard treatment. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Rozanolixizumab is a monoclonal antibody (a type of protein) designed to recognise and attach to FcRn, a protein that keeps the IgG antibodies in the body for longer. By binding to and blocking FcRn, the medicine increases the removal of these antibodies, preventing them from triggering the attack on the acetylcholine receptors. This is expected to lead to an improvement in muscle function.

What is the stage of development of this medicine?

The effects of rozanolixizumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with rozanolixizumab in patients with myasthenia gravis were ongoing.

At the time of submission, rozanolixizumab was not authorised anywhere in the EU for the treatment of myasthenia gravis. Orphan designation of rozanolixizumab had been granted in the EU for the treatment of immune thrombocytopenia and in the USA for primary immune thrombocytopenic purpura and for myasthenia gravis.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 19 March 2020, 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.

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	Rozanolixizumab	Treatment of myasthenia gravis
Bulgarian	Розаноликсизумаб	Лечение на миастения гравис
Croatian	Rozanoliksizumab	Liječenje miastenije gravis
Czech	Rozanolixizumab	Léčba myasthenie gravis
Danish	Rozanolixizumab	Behandling af myasthenia gravis
Dutch	Rozanolixizumab	Behandeling van myasthenia gravis
Estonian	Rosanoliksizumab	Myasthenia Gravise ravi
Finnish	Rotsanoliksitsumabi	Myasthenia graviksen hoito
French	Rozanolixizumab	Traitement de la myasthénie
German	Rozanolixizumab	Behandlung der Myasthenia Gravis
Greek	Ροζανολιξιζουμάμπη	Θεραπεία της βαρείας μυασθένειας
Hungarian	Rozanolixizumab	Myasthenia gravis kezelése
Italian	Rozanolixizumab	Trattamento della miastenia grave
Latvian	Rozanoliksizumabs	Myasthenia gravis ārstēšanai
Lithuanian	Rozanoliksizumabas	Generalizuotos miastenijos gydymas
Maltese	Rożanolissiżumab	Kura ta’ myasthenia gravis
Polish	Rozanoliksyzumab	Leczenie miastenii gravis
Portuguese	Rozanolixizumab	Tratamento da miastenia gravis
Romanian	Rozanolixizumab	Tratamentul miasteniei gravis
Slovak	Rozanolizumab	Liečba myasthenie gravis
Slovenian	Rozanoliksizumab	Zdravljenje miastenije gravis
Spanish	Rozanolixizumab	Tratamiento de la miastenia gravis
Swedish	Rozanolixizumab	Behandling av myasthenia gravis
Norwegian	Rozanoliksizumab	Behandling av myasthenia gravis
Icelandic	Rozanolixizumab	Meðferð við vöðvaslensfári

[1] At the time of designation