7 May 2018
EMA/139668/2018

Public summary of opinion on orphan designation

Efgartigimod alfa for the treatment of myasthenia gravis

On 21 March 2018, orphan designation (EU/3/18/1992) was granted by the European Commission to argenx BVBA, Belgium, for efgartigimod alfa (also known as ARGX-113) 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 'acetylcholine receptors' on the surface of muscle cells. 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, because of the damage to these receptors, 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.

In myasthenia gravis, 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 around 103,000 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).

[*]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 517,400,000 (Eurostat 2018).

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

What treatments are available?

At the time of designation, a number of medicines were authorised in the EU for the treatment of myasthenia gravis, including acetylcholine esterase 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. Studies have shown that the medicine, given in addition to standard of care, can reduce the symptoms of the disease.

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?

In patients with myasthenia gravis, the body produces antibodies against the acetylcholine receptors. This medicine works by blocking a protein called FcRn, which attaches to these antibodies and protects them from degradation. Blocking FcRn leads to the degradation of the antibodies that damages the acetylcholine receptors; this is expected to restore the normal contraction of the muscles.

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 myasthenia gravis were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for myasthenia gravis. Orphan designation of the medicine had been granted in the United States for myasthenia gravis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 February 2018 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	Efgartigimod alfa	Treatment of myasthenia gravis
Bulgarian	Ефгартигимод алфа	Лечение на миастения гравис
Croatian	Efgartigimod alfa	Liječenje miastenije gravis
Czech	Efgartigimod alfa	Léčba myasthenie gravis
Danish	Efgartigimod alfa	Behandling af myasthenia gravis
Dutch	Efgartigimod alfa	Behandeling van myasthenia gravis
Estonian	Alfaefgartigimood	Myasthenia Gravise ravi
Finnish	Efgartigimodi alfa	Myasthenia graviksen hoito
French	Efgartigimod alfa	Traitement de la myasthénie
German	Efgartigimod alfa	Behandlung der Myasthenia Gravis
Greek	Εφγαρτιγιμόδη άλφα	Θεραπεία της βαρείας μυασθένειας
Hungarian	Efgartigimod alfa	Myasthenia gravis kezelése
Italian	Efgartigimod alfa	Trattamento della miastenia grave
Latvian	Alfa efgartigimods	Myasthenia gravis ārstēšanai
Lithuanian	Efgartigimodas alfa	Generalizuotos miastenijos gydymas
Maltese	Efgartigimod alfa	Kura ta' myasthenia gravis
Polish	Efgartigimod alfa	Leczenie miastenii gravis
Portuguese	Efgartigimod alfa	Tratamento da miastenia gravis
Romanian	Efgartigimod alfa	Tratamentul miasteniei gravis
Slovak	Efgartigimod alfa	Liečba myasthenie gravis
Slovenian	Efgartigimod alfa	Zdravljenje miastenije gravis
Spanish	Efgartigimod alfa	Tratamiento de la miastenia gravis
Swedish	Efgartigimod alfa	Behandling av myasthenia gravis
Norwegian	Efgartigimod alfa	Behandling av myasthenia gravis
Icelandic	Efgartigimód alfa	Meðferð við vöðvaslensfári

[1] At the time of designation