



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Arimoclomol citrate for the treatment of inclusion body myositis

On 30 May 2016, orphan designation (EU/3/16/1659) was granted by the European Commission to Orphazyme ApS, Denmark, for arimoclomol citrate for the treatment of inclusion body myositis.

What is inclusion body myositis?

Inclusion body myositis is a condition in which muscle cells become damaged and inflamed, leading to progressive muscle weakness in the legs and arms and difficulty in walking and swallowing. The disease mainly affects people over 50 and is more common in men than in women.

Inclusion body myositis is a long-term debilitating condition due to the development of disabling muscle weakness and problems with swallowing.

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

At the time of designation, inclusion body myositis affected approximately 0.05 in 10,000 people in the European Union (EU). This was equivalent to a total of around 2,500 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 inclusion body myositis. Patients were often given treatment to suppress the immune system although the benefit was unclear. Supportive measures included physical therapy and exercise, and the use of devices and aids to mobility including motorised wheelchairs.

How is this medicine expected to work?

The mechanisms that lead to the disease are not well understood, but seem to include abnormalities in the way that proteins in muscle cells are folded, leading them to become tangled and clump together

*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).



inside cells. Arimoclomol is a medicine that stimulates the cell's natural mechanisms for coping with damage, by activating a class of proteins called 'heat shock proteins' in cells that are under stress. Heat shock proteins aid the correct folding of damaged proteins. By activating these proteins in muscle cells that are at risk of damage, the medicine is expected to reduce protein misfolding and so improve muscle weakness and other symptoms of the disease.

What is the stage of development of this medicine?

The effects of arimoclomol citrate 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 inclusion body myositis were ongoing.

At the time of submission, arimoclomol citrate was not authorised anywhere in the EU for inclusion body myositis. Orphan designation of the medicine had been granted in the EU and the United States for two other conditions associated with muscle weakness and difficulty moving, called amyotrophic lateral sclerosis and Niemann-Pick disease, type C.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 April 2016 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Arimoclomol citrate	Treatment of inclusion body myositis
Bulgarian	Аримокломол цитрат	Лечение на миозит с включвания
Croatian	Arimoklomolcitrata	Liječenje miozitisa s inkluzijskim tjelešcima
Czech	Arimoklomol citrát	Léčba myozitidy s inkluzními tělisky
Danish	Arimoclomol citrat	Behandling af inklusionslegeme myositis
Dutch	Arimoclomolcitraat	Behandeling van inclusielichaammyositis
Estonian	Arimoklomoolsitraat	Inklusioonkeha müosiidi ravi
Finnish	Arimoklomolisitraatti	Inklusiokappalemyosiitin hoito
French	Citrate d' arimoclomol	Traitement de la myosite à inclusions
German	Arimoclomolcitrat	Behandlung der Einschlußkörpermyositis
Greek	Κιτρική αριμοκλομόλη	Θεραπεία της μυοσίτιδας εξ εγκλειστών
Hungarian	Arimoklomol-citrát	Zárványtestes myositis kezelése
Italian	Citrato di arimoclomol	Trattamento della miosite da corpi inclusi
Latvian	Arimoklomola citrāts	Ieslēgto ķermenīšu miozīta ārstēšana
Lithuanian	Arimoklomolio citratas	Įterptinių kūnelių miozito gydymas
Maltese	Arimoclomol citrate	Kura tal-mijosite ta' korpi ta' inkluzjoni
Polish	Cytrynian arimoklomolu	Leczenie wtrętowego zapalenia mięśni
Portuguese	Citrato de arimoclomol	Tratamento da miosite de corpos de inclusão
Romanian	Citrat de arimoclomol	Tratamentul miozitei cu corpi de incluziune
Slovak	Arimoklomol citrátate	Liečba myozitídy s inklúznymi telieskami
Slovenian	Arimoklomol citrat	Zdravljenje miozitisa z inkluzijskimi telesci
Spanish	Citrato de arimoclomol	Tratamiento de la miositis por cuerpos de inclusión
Swedish	Arimoklomolcitrat	Behandling av inklusionskroppsmysositt
Norwegian	Arimoklomolsitrat	Behandling av inklusjonslegememyositt
Icelandic	Arímóklómól sítrat	Meðferð innlyksu vöðvabólgu

¹ At the time of designation