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EMA/COMP/637387/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Arimoclomol citrate for the treatment of Niemann-Pick disease, type C

On 19 November 2014, orphan designation (EU/3/14/1376) was granted by the European Commission to Orphazyme ApS, Denmark, for arimoclomol citrate for the treatment of Niemann-Pick disease, type C.

What is Niemann-Pick disease, type C?

Niemann-Pick disease is a group of inherited disorders belonging to the larger family of metabolic disorders called 'lysosomal storage diseases', in which fats accumulate within lysosomes (parts of the body's cells that break down nutrients and other materials).

In Niemann-Pick disease type C, there is a lack of transporter proteins that are needed to move fatty substances such as cholesterol and other fats within cells, leading to the build-up of these fats in the brain and elsewhere in the body (such as the spleen and liver). This causes a wide range of symptoms, including behavioural problems, learning disabilities and difficulty moving and speaking.

Niemann-Pick disease type C is chronically debilitating and life threatening since the build-up of fatty substances can cause brain damage and swelling of organs such as the spleen and the liver.

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

At the time of designation, Niemann-Pick disease type C affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 5,000 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, Zavesca (miglustat) was authorised in the EU to treat Niemann-Pick disease, type C.

*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 511,100,000 (Eurostat 2014).

The sponsor has provided sufficient information to show that arimoclomol citrate might be of significant benefit for patients with Niemann-Pick disease type C because it works in a different way to miglustat and experimental studies suggest it might be of benefit in reducing 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 Niemann-Pick disease type C the build-up of fatty substances in the lysosomes reduces their ability to function normally and makes the membrane around the lysosome unstable. Arimoclomol is a medicine that stimulates the cell's natural mechanisms for coping with damage, by increasing the production of proteins called heat shock proteins in cells that are under stress. These proteins help to stabilise the membrane around the lysosomes and improve the ability of the lysosome to function and break down certain fats. This is expected to help reduce the 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, no clinical trials with arimoclomol citrate in patients with Niemann-Pick disease, type C had been started.

At the time of submission, arimoclomol citrate was not authorised anywhere in the EU for Niemann-Pick disease type C or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 October 2014 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:

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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 Niemann-Pick disease, type C
Bulgarian	Аримокломол цитрат	Лечение на болест на Нийман-Пик, тип C
Croatian	Arimoklomolcitrate	Liječenje Niemann-Pickove bolesti, tip C
Czech	Arimoklomol citrát	Léčba Niemann-Pickovy choroby, typ C
Danish	Arimoclomol citrat	Behandling af Niemann-Picks sygdom, type C
Dutch	Arimoclomolcitraat	Behandeling van de ziekte van Niemann-Pick, type C
Estonian	Arimoklomoolsitraat	C tüüpi Niemann-Pick'i tõve ravi
Finnish	Arimoklomolisitraatti	Niemann-Pick'n taudin tyypin C:n hoito
French	Citrate d' arimoclomol	Traitement de la maladie de Niemann-Pick de type C
German	Arimoclomolcitrat	Zur Behandlung des Morbus Niemann-Pick Typ C
Greek	Κιτρική αριμοκλομόλη	Θεραπεία της Νόσου Niemann-Pick τύπου C
Hungarian	Arimoklomol-citrát	C típusú Niemann-Pick betegség kezelése
Italian	Citrato di arimoclomol	Trattamento della Malattia di Niemann-Pick, tipo C
Latvian	Arimoklomola citrāts	C tipa Nīmaņa-Pika slimības ārstēšanai
Lithuanian	Arimoklomolio citratas	C tipo Niemann-Picko ligos gydymas
Maltese	Arimoclomol citrate	Kura tal-marda ta' Niemann-Pick tat-tip Ċ
Polish	Cytrynian arimoklomolu	Leczenie choroby Niemann-Picka typu C
Portuguese	Citrato de arimoclomol	Tratamento da Doença de Niemann-Pick, tipo C
Romanian	Citrat de arimoclomol	Tratamentul bolii Niemann-Pick tip C
Slovak	Arimoklomol citrate	Liečba Niemannovej-Pickovej choroby, typ C
Slovenian	Arimoklomol citrat	Zdravljenje Niemann-Pickove bolezni, tipa C
Spanish	Citrato de arimoclomol	Tratamiento de la Enfermedad de Niemann-Pick, tipo C
Swedish	Arimoklomolcitrat	Behandling av Niemann-Picks sjukdom, typ C
Norwegian	Arimoklomolsitrat	Behandling av Niemann-Picks sykdom, type C
Icelandic	Arímóklómól sítrat	Til meðferðar á Niemann-Pick sjúkdómi, gerð C

¹ At the time of designation