



EUROPEAN MEDICINES AGENCY
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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Hydroxy-propyl-beta-cyclodextrin for the treatment of Niemann-Pick disease, type C

First publication	20 September 2011
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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 30 August 2011, orphan designation (EU/3/11/895) was granted by the European Commission to Susan French, United Kingdom, for hydroxy-propyl-beta-cyclodextrin for the treatment of Niemann-Pick disease, type C.

The sponsorship was transferred to Medical Need Europe AB, Sweden, in March 2015.

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 (part of the body's cells which 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 in the cells, with the result that fats such as cholesterol build up within cells 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 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. The sponsor has provided sufficient information to show that hydroxy-propyl-beta-cyclodextrin might be of significant benefit for patients with the disease, because early studies indicate that it may improve the outcome of patients compared with the existing 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?

Hydroxy-propyl-beta-cyclodextrin is a cyclic oligosaccharide. In Niemann-Pick disease type C, it is expected to reduce the accumulation of fats in the lysosomes by forming a complex with the cholesterol inside the lysosomes, which allows the fats to leave the lysosomes and enter into the wider cell, where they can be processed normally.

What is the stage of development of this medicine?

The effects of hydroxy-propyl-beta-cyclodextrin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with hydroxy-propyl-beta-cyclodextrin in patients with Niemann-Pick disease type C had been started.

At the time of submission, hydroxy-propyl-beta-cyclodextrin was not authorised anywhere in the EU for Niemann-Pick disease type C. Orphan designation of hydroxy-propyl-beta-cyclodextrin had been granted in the United States for Niemann-Pick disease type C.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 June 2011 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.

*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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).

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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<http://medicalneed.com/contact-us/>

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	Hydroxy-propyl-beta-cyclodextrin	Treatment of Niemann-Pick disease, type C
Bulgarian	Хидрокси-пропил-бета-циклодекстрин	Лечение на болест на Нийман-Пик, тип C
Croatian	Hidroksi-propil-beta-ciklodekstrin	Liječenje Niemann-Pickove bolesti, tip C
Czech	Hydroxy-propyl-beta-cyclodextrin	Léčba Niemann-Pickovy choroby, typ C
Danish	Hydroxy-propyl-beta-cyclodextrin	Behandling af Niemann-Picks sygdom, type C
Dutch	Hydroxy-propyl-beta-cyclodextrin	Behandeling van de ziekte van Niemann-Pick, type C
Estonian	Hüdroksü-propüül-beeta-tsüklodekstriin	C tüüpi Niemann-Pick'i tõve ravi
Finnish	Hydroksi-propyyli-beeta-syklodekstriini	Niemann-Pick'n taudin tyyppi C:n hoito
French	Hydroxy-propyl-beta-cyclodextrine	Traitement de la maladie de Niemann-Pick de type C
German	Hydroxy-propyl-beta-cyclodextrin	Zur Behandlung des Morbus Niemann-Pick Typ C
Greek	Υδροξυ-προπουλο-β-κυκλοδεξτρίνη	Θεραπεία της Νόσου Niemann-Pick τύπου C
Hungarian	Hydroxy-propyl-beta-cyclodextrin	C típusú Niemann-Pick betegség kezelése
Italian	Hydroxy-propyl-beta-cyclodestrina	Trattamento della Malattia di Niemann-Pick, tipo C
Latvian	Hidroksi-propil-beta-ciklodekstrīns	C tipa Nīmaņa-Pika slimības ārstēšanai
Lithuanian	Hidroksi-propil-beta-ciklodekstrinas	C tipo Niemann-Picko ligos gydymas
Maltese	Hydroxy-propyl-beta-cyclodextrin	Kura tal-marda ta' Niemann-Pick tat-tip Ċ
Polish	Hydroksypropylo-beta-cyklodekstryna	Leczenie choroby Niemann-Picka typu C
Portuguese	Hidroxi-propil-beta-ciclodextrin	Tratamento da Doença de Niemann-Pick, tipo C
Romanian	Hidroxi-propil-beta-ciclodextrină	Tratamentul bolii Niemann-Pick tip C
Slovak	Hydroxy-propyl-beta-cyklodextrín	Liečba Niemann Pick ochorenia, typ C
Slovenian	Hidroksi-propil-beta-ciklodekstrin	Zdravljenje Niemann-Pickove bolezni, tipa C
Spanish	Hidroxi-propil-beta-ciclodestrina	Tratamiento de la Enfermedad de Niemann-Pick, tipo C
Swedish	Hydroxypropylbetacyclodextrin	Behandling av Niemann-Picks sjukdom, typ C
Norwegian	Hydroksypropylbetasyklodekstrin	Behandling av Niemann-Picks sykdom, type C
Icelandic	Hýdroxý-própýl-beta-cýklódestrín	Til meðferðar á Niemann-Pick sjúkdómi, gerð C

¹ At the time of transfer of sponsorship