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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

2-Hydroxypropyl- β -cyclodextrin for the treatment of Niemann-Pick disease, type C

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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 26 April 2013, orphan designation (EU/3/13/1124) was granted by the European Commission to International Niemann-Pick Disease Alliance (INPDA), the United Kingdom, for 2-hydroxypropyl- β -cyclodextrin for the treatment of Niemann-Pick disease, type C.

The sponsorship was transferred to Vtesse Europe Limited, United Kingdom, in February 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 such as cholesterol and other fats within cells, leading to their build-up 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.

The sponsor has provided sufficient information to show that 2-hydroxypropyl- β -cyclodextrin might be of significant benefit for patients with Niemann-Pick disease, type C because results from studies in experimental models show that it might improve survival of patients with this condition when used in combination with 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?

2-Hydroxypropyl- β -cyclodextrin is a cyclic oligosaccharide (a type of carbohydrate). 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 2-hydroxypropyl- β -cyclodextrin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with 2-hydroxypropyl- β -cyclodextrin in patients with Niemann-Pick disease type C had been started.

At the time of submission, 2-hydroxypropyl- β -cyclodextrin was not authorised anywhere in the EU for Niemann-Pick disease type C. Orphan designation of the medicine has been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 March 2013 recommending the granting of this designation.

*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. At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).

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	2-hydroxypropyl- β -cyclodextrin	Treatment of Niemann-Pick disease, type C
Bulgarian	2-хидроксипропил- β -циклодекстрин	Лечение на болест на Нийман-Пик, тип С
Croatian	2-hidroksipropil-beta-ciklodekstrin	Liječenje Niemann-Pickove bolesti, tip C
Czech	2-hydroxypropyl- β -cyklodextrin	Léčba Niemann-Pickovy choroby, typ C
Danish	2-hydroxypropyl- β -cyclodextrin	Behandling af Niemann-Picks sygdom, type C
Dutch	2-hydroxypropyl- β -cyclodextrine	Behandeling van de ziekte van Niemann-Pick, type C
Estonian	2-hüdroksüpropüül- β -tsüklodekstriin	C tüüpi Niemann-Pick'i tõve ravi
Finnish	2-hydroksipropyli- β -syklodekstriini	Niemann-Pick'n taudin tyyppi C:n hoito
French	2-hydroxypropyl- β -cyclodextrine	Traitement de la maladie de Niemann-Pick de type C
German	2-Hydroxypropyl-beta-Cyclodextrin	Zur Behandlung des Morbus Niemann-Pick Typ C
Greek	2-υδροξυπροπυλ- β -κυκλοδεξτρίνη	Θεραπεία της Νόσου Niemann-Pick τύπου C
Hungarian	2-hidroxí-propil- β -ciklodextrin	C típusú Niemann-Pick betegség kezelése
Italian	2-idrossipropil- β -ciclodestrina	Trattamento della Malattia di Niemann-Pick, tipo C
Latvian	2-hidroksipropil-beta-ciklodekstrīns	C tipa Nīmaņa-Pika slimības ārstēšanai
Lithuanian	2-hidroksipropil- β -ciklodekstrinas	C tipo Niemann-Picko ligos gydymas
Maltese	2-hydroxypropyl- β -cyclodextrin	Kura tal-marda ta' Niemann-Pick tat-tip Ċ
Polish	2-hydroksypropylo- β -cyklodekstryna	Leczenie choroby Niemann-Picka typu C
Portuguese	2-hidroxipropil- β -ciclodextrina	Tratamento da Doença de Niemann-Pick, tipo C
Romanian	2-hidroxipropil- β -ciclodextrina	Tratamentul bolii Niemann-Pick tip C
Slovak	2-hydroxypropyl- β -cyklodextrín	Liečba NiemannovejPickovej choroby, typ C
Slovenian	2-hidroksipropil- β -ciklodekstrin	Zdravljenje Niemann-Pickove bolezni, tipa C
Spanish	2-hidroxipropil- β -ciclodextrina	Tratamiento de la Enfermedad de Niemann-Pick, tipo C
Swedish	2-hydroxipropyl- β -cyklodextrin	Behandling av Niemann-Picks sjukdom, typ C
Norwegian	2-hydroksypropyl- β -syklodekstrin	Behandling av Niemann-Picks sykdom, type C
Icelandic	2-hýdroxýprópyl- β -sýklódextrín	Til meðferðar á Niemann-Pick sjúkdómi, gerð C

¹ At the time of transfer of sponsorship