5 March 2015
EMA/COMP/25188/2006 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Miglustat for the treatment of Niemann-Pick disease, type C

First publication	24 August 2006
Rev.1: information about Marketing Authorisation	17 November 2009
Rev.2: sponsor's change of address	5 March 2015
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 16 February 2006, orphan designation (EU/3/06/351) was granted by the European Commission to Actelion Registration Ltd, United Kingdom, for miglustat for the treatment of Niemann-Pick disease, type C.

What is Niemann-Pick disease, type C?

Niemann-Pick disease comprises a group of inherited lysosomal storage disorders. Lysosomes are small vesicles within each cell containing enzymes, proteins that are able to destroy or transform different substances of the cell, such as other proteins, fats, nucleic acids (components of the genetic material) and sugars. When there is an alteration of one of the lysosomal enzymes there is abnormal accumulation of the product (the so called substrate) that is not transformed by this particular enzyme. This means that the cells are unable to destroy or eliminate these substrates, resulting in cell damage and malfunction of the organ where the product is accumulated. In Niemann-Pick type C the lysosomal enzyme alteration affects fatty products. The symptomatology and the severity of the disease depends very much on the level of accumulation of fatty substrates such as glycosphingolipids. This accumulation usually induces progressive degeneration of the nervous system and enlargement of some organs like the liver. Niemann-Pick disease type C is chronically debilitating and life threatening.

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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 47,000 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 submission of the application for orphan designation, no satisfactory method had been authorised in the European Union for treatment of the condition.

How is this medicine expected to work?

Miglustat is expected to block (inhibit) the action of an enzyme (glucosylceramide synthase) involved in the production of one of the substrates, the glycosphingolipids that accumulated in Niemann-Pick disease type C. It is expected that if the amount of the accumulated substance will decrease, it might help to limit the extent of the damage and the clinical consequences of the disease.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials in patients with Niemann-Pick type C were ongoing.

Miglustat was not authorised anywhere worldwide for Niemann-Pick type C, at the time of submission. Orphan designation of miglustat was previously granted in the EU for Gaucher disease.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 January 2006 recommending the granting of this designation.

Update: miglustat (Zavesca) has been authorised in the EU since 26 January 2009 for the treatment of progressive neurological manifestations in adult patients and paediatric patients with Niemann-Pick type C disease.

More information on Zavesca can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

*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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 468,900,000 (Eurostat 2006).

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 languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Miglustat	Treatment of Niemann-Pick Disease, type C
Bulgarian	Миглустат	Лечение на болест на Ниман-Пик, тип С
Czech	Miglustat	Léčba Niemann-Pickovy choroby, typ C
Danish	Miglustat	Behandling af Niemann-Picks sygdom, type C
Dutch	Miglustaat	Behandeling van de ziekte van Niemann-Pick, type C
Estonian	Miglustaat	C tüüpi Niemann-Pick'i tõve ravi
Finnish	Miglustaatti	Niemann-Pick'n taudin tyyppi C:n hoito
French	Miglustat	Traitement de la maladie de Niemann-Pick de type C
German	Miglustat	Zur Behandlung des Morbus Niemann-Pick Typ C
Greek	μιγλουστάτη	Θεραπεία της Νόσου Niemann-Pick τύπου C
Hungarian	Miglusztát	C típusú Niemann-Pick betegség kezelése
Italian	Miglustat	Trattamento della Malattia di Niemann-Pick, tipo C
Latvian	Miglustats	C tipa Nīmaņa-Pika slimības ārstēšanai
Lithuanian	Miglustatas	C tipo Niemann-Picko ligos gydymas
Maltese	Miglustat	Kura tal-marda ta' Niemann-Pick tat-tip Ċ
Polish	Miglustat	Leczenie choroby Niemanna-Picka typu C
Portuguese	Miglustat	Tratamento da Doença de Niemann-Pick, tipo C
Romanian	Miglustat	Tratamentul bolii Niemann-Pick, tip C
Slovak	Miglustat	Liečba Niemann Pick ochorenia, typ C
Slovenian	Miglustat	Zdravljenje Niemann-Pickove bolezni, tipa C
Spanish	Miglustat	Tratamiento de la Enfermedad de Niemann-Pick, tipo C
Swedish	Miglustat	Behandling av Niemann-Picks sjukdom, typ C
Norwegian	Miglustat	Behandling av Niemann-Picks sykdom, type C
Icelandic	Miglustat	Til meðferðar á Niemann-Pick sjúkdómi, gerð C

[1] At the time of marketing authorisation