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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human α-mannosidase for the treatment of α-mannosidosis

First publication	1 July 2005
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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 January 2005, orphan designation (EU/3/04/260) was granted by the European Commission to Zymenex A/S, Denmark, for recombinant human α-mannosidase for the treatment of α-mannosidosis.

The sponsorship was transferred to Chiesi Farmaceutici S.p.A., Italy, in January 2015.

What is α-mannosidosis?

α-mannosidosis is an inherited disease leading to an accumulation of oligosaccharides (small molecules composed of sugars) in the body. α-mannosidosis is due to a lack of α-mannosidase, an enzyme (a protein that speed up the conversion of certain substances into other substances) needed for the breaking down of oligosaccharides. The most severe form (type 1) occurs before 1 year of age. Clinical features include deafness and profound mental retardation. The juvenile/adult form (type 2) is less severe. Milder mental retardation occurs later, but hearing loss is a prominent feature. α-mannosidosis is chronically debilitating and life-threatening.

What is the estimated number of patients?

At the time of designation, α-mannosidosis affected approximately 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of around 470 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).

*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 466,600,000 (Eurostat 2005).

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What treatments are available?

At the time of submission of the application for the orphan drug designation there was no treatment authorised in the European Union. Treatment of α-mannosidosis included supportive care and bone-marrow transplantation.

How is this medicine expected to work?

It is assumed that, once admininistrated to the patient, recombinant human α-mannosidase will act as the original α-mannosidase and break down the oligosaccharides, thus preventing the negative effects due to their accumulation in the body.

What is the stage of development of this medicine?

The evaluation of the effects of recombinant human α-mannosidase in experimental models is ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients with α-mannosidosis were initiated.

Recombinant human α-mannosidase was not marketed anywhere worldwide for α-mannosidosis or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 December 2004 recommending the granting of this designation.

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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	Recombinant human α-mannosidase	Treatment of α-Mannosidosis
Bulgarian	Рекомбинантна човешка α-манозидаза	Лечение на α-манозидоза
Croatian	Rekombinantna ljudska α-manozidaza	Liječenje α-manozidoze
Czech	Rekombinantní lidská α- mannosidasa	Léčení nedostatku enzymu α-mannosidasy
Danish	Recombinant human α-Mannosidase	Behandling af α-Mannosidosis
Dutch	Recombinant humaan α-Mannosidase	Behandeling van α-Mannosidose
Estonian	Rekombinantne inimese α-Mannosidaas	α-Mannosidoosi ravi
Finnish	Rekombinantti humaani α-mannosidaasi	α-mannosidoosin hoito
French	α-Mannosidase humaine (origine recombinante)	Traitement de l'α-Mannosidose
German	Rekombinante humane α-Mannosidase	Behandlung des α-Mannosidasemangelsyndroms
Greek	Ανθρώπινη ανασυνδυασμένη α-μαννοσιδάση	Θεραπεία της α-μαννοσίδωσης
Hungarian	Rekombináns humán alfa-mannozidáz	Alfa-mannozidozis kezelése
Italian	α-Mannosidasi ricombinante umana	Trattamento della α-mannosidosi
Latvian	Rekombinēta cilvēku α -mannozidāze	α-mannozidozes ārstēšana
Lithuanian	Rekombinantinė žmogaus α-manozidozė	α-manozidozės gydymas
Maltese	α-Mannosidase uman rikombinanti	Kura ta' α-Mannosidosi
Polish	Rekombinowana ludzka α-mannozydaza	Leczenie α-mannosydozy
Portuguese	Recombinante humano α-Manosidase	Tratamento da α-Manosidose
Romanian	Alfa-manosidaza umană recombinantă	Tratamentul alfa-manosidozei
Slovak	Rekombinantná ľudská α-manozidáza	Liečba α-manozidózy
Slovenian	Rekombinantna humana α-manozidaza	zdravljenje α- manozidoze
Spanish	α-manosidasa humana recombinante	Tratamiento de la α-manosidosis
Swedish	Rekombinant humant alfa-mannosidas	Behandling av alfa-mannosidos
Norwegian	Rekombinant human alfa-mannosidase	Behandling av alfa-mannosidose
Icelandic	Raðbrigða α-mannósídasi manna	Meðferð gegn α-mannósíðkvilla

[1] At the time of transfer of sponsorship