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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Laronidase for the treatment of mucopolysaccharidosis, type I

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Rev.1: withdrawal from the Community Register	1 July 2013
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.	

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in June 2013 at the end of the period of market exclusivity.

On 14 February 2001, orphan designation (EU/3/01/022) was granted by the European Commission to Genzyme BV, the Netherlands, for laronidase for the treatment of mucopolysaccharidosis, type I.

The sponsor changed name to Genzyme Europe BV in 2002.

What is mucopolysaccharidosis, type I?

The human cells contain various structures with specific functions known as organelles. Lysosomes are an example of such organelles. Lysosomes contain enzymes (biological catalysts that speed up chemical reactions in the body) essential for breaking down substances like proteins and sugars. Mucopolysaccharidosis type I belongs to a group of mucopolysaccharide storage disorders, characterised by deficiencies of certain lysosomal enzymes. Mucopolysaccharidosis type I is caused by the deficiency of the lysosomal enzyme α-L-iduronidase. Patients with mucopolysaccharidosis type I are classified into three clinical syndromes- Hurler, Hurler-Scheie and Scheie. Severe progressive skeletal disease and joint stiffness are two of the main features of mucopolysaccharidosis type I. Mucopolysaccharidosis type I is serious, chronically debilitating and in most cases life threatening.

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

At the time of designation, mucopolysaccharidosis, type I affected approximately 0.025 in 10,000 people in the European Union (EU). This was equivalent to a total of around 950 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?

No medicinal products were authorised for the treatment of mucopolysaccharidosis type I in the Community at the time of submission of the application for orphan drug designation. The treatment options for a majority of mucopolysaccharidosis type I patients were limited to symptomatic care and occasionally bone-marrow transplantation.

How is this medicine expected to work?

The active ingredient of laronidase is the enzyme α-L-iduronidase. α-L-iduronidase is the deficient enzyme in patients with mucopolysaccharidosis type I. By replacing the deficient enzyme with a functioning one, the sponsor hopes to restore the normal lysosomal function and ultimately improve the overall outcome of the patients.

What is the stage of development of this medicine?

The effects of laronidase were evaluated in experimental models.

At the time of submission of the application for orphan designation, one clinical trial in patients with mucopolysaccharidosis type I was completed.

Laronidase was not marketed anywhere worldwide for treatment of mucopolysaccharidosis type I, at the time of submission.

Orphan designation of laronidase was granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 December 2000 recommending the granting of this designation.

Update: laronidase (Aldurazyme) has been authorised in the EU since 10 June 2003 for long-term enzyme replacement therapy in patients with a confirmed diagnosis of Mucopolysaccharidosis I (MPS I; α-L-iduronidase deficiency) to treat the nonneurological manifestations of the disease.

More information on Aldurazyme 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

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*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.
At the time of designation, this represented a population of 378,800,000 (Eurostat 2001).

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	Laronidase	Treatment of Mucopolysaccharidosis, type I
Danish	Laronidase	Behandling af mucopolysaccharidose, type I
Dutch	Laronidase	Behandeling van mucopolysaccharidosis, type I
Finnish	*Laronidaasi*	Mukopolysakkaridoosi I potilaiden hoito
French	*Laronidase*	Traitement de la mucopolysaccharidose, type I
German	Laronidase	Behandlung der Mucopolysaccharidose I
Greek	Λαρονιδάση	Θεραπεία της Βλεννοπολυσακχαρίδωσης Ι
Italian	Laronidasi	Trattamento della mucopolisaccaridosi, tipo I
Portuguese	Laronidase	Tratamento de Mucopolissacaridose de tipe I
Spanish	Laronidasa	Tratamiento de la mucopolisacaridosis tipo I
Swedish	*Laronidas*	Behandling av mukopolysackaridos typ I

[1] At the time of designation