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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Iduronate-2-sulfatase for the treatment of mucopolysaccharidosis, type II (Hunter Syndrome)

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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 11 December 2001, orphan designation (EU/3/01/078) was granted by the European Commission to TKT UK Limited, United Kingdom, for iduronate-2-sulfatase for the treatment of mucopolysaccharidosis, type II (Hunter Syndrome).

The sponsorship was transferred to TKT Europe AB, Sweden, in December 2005.

The sponsor changed its name to Shire Human Genetic Therapies AB in February 2010.

What is mucopolysaccharidosis, type II (Hunter Syndrome)?

Mucopolysaccharidosis is a so called lysosomal storage disease. It is caused by the absence of the enzyme iduronate-2-sulfatase, which is found in lysosomes. Lysosomes are small organelles that contain several enzymes important to break down substances like proteins and sugars. Normally, iduronate-2-sulfatase would break down two specific mucopolysaccharides. As it is absent in patients with Hunter Syndrome, these mucopolysaccharides build up in the lysosomes. The condition is chronically debilitating and life-threatening in particular due to neurologic decline, difficulties to breath and heart failure.

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

At the time of designation, mucopolysaccharidosis, type II (Hunter Syndrome) affected approximately 0.02 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 760 people, and is below the threshold 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 II (Hunter Syndrome) in the Community at the time of submission of the application for orphan drug designation. The treatment options for mucopolysaccharidosis type II (Hunter Syndrome) patients were limited to symptomatic care.

How is this medicine expected to work?

Iduronate -2- sulfatase is the deficient enzyme in patients with mucopolysaccharidosis type II (Hunter Syndrome). By replacing it with a functioning enzyme, it is expected that the normal lysosomal function is restored and ultimately improve the overall health of the patients.

What is the stage of development of this medicine?

The effects of iduronate -2- sulfatase were evaluated in experimental models. At the time of submission of the application for orphan designation, one clinical trial in patients with mucopolysaccharidosis type II (Hunter Syndrome) was ongoing.

Iduronate-2-sulfatase was not marketed anywhere worldwide for the treatment of mucopolysaccharidosis, type II (Hunter Syndrome) 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 26 October 2001 recommending the granting of this designation.

Update: iduronate-2-sulfatase (Elaprase) was authorised in the EU on 8 January 2007 for the long-term treatment of patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II).

Heterozygous females were not studied in the clinical trials.

More information on Elaprase 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.
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 European Union) 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	Iduronate-2-sulfatase	Treatment of Mucopolysaccharidosis, type II (Hunter Syndrome)
Czech	Iduronat- 2-sulfatása	Léčba mukopolysacharidózy typu II (Hunterův syndrom)
Danish	Iduronat-2-sulfatase	Behandling af mucopolysakkaridosis, Type II, (Hunters sygdom)
Dutch	Iduronaat-2-sulfatase	Behandeling van mucopolysaccharidosis, type II (Hunter-syndroom)
Estonian	Iduronaat-2-sulfataas	II tüübi mukopolüsahharidoosi (Hunteri sündroom) ravi
Finnish	Iduronaatti-2 -sulfataasi	Tyypin II mukopolysakkaridosin hoito (Hunterin oireyhtymä)
French	Iduronate 2 sulfatase	Traitement de la mucopolysaccharidose de type II (maladie de Hunter)
German	Iduronat-2-sulfatase-	Behandlung der Mucopolysaccharidose Typ II (Hunter-Syndrom)
Greek	Ιδουρονάτη-2-σουλφατάση	θεραπεία βλεννοπολυσακχαρίδωσης τύπου II (Σύνδρομο Hunter)
Hungarian	Iduronat-2-szulfatáz	II-típusú Mucopolysaccharidosis (Hunter szindróma)
Italian	Iduronato-2-solfatasi	Trattamento della mucopolisaccaridosi tipo II (sindrome di Hunter)
Latvian	Iduronāta-2-sulfatāze	Mukopolisaharodozes, II tips (Hantera sindroms), ārstēšana
Lithuanian	Iduronato-2-sulfatazė	II tipo mukopolisacharidozės (Hunter sindromo) gydymas
Polish	Iduronatu-2-sulfataza	Leczenie mukopolisacharydozy typu II (zespół Hunter'a)
Portuguese	Iduronato-2-sulfatase	Tratamento da mucopolissacaridose tipo II (Síndrome de Hunter)
Slovak	Iduronát-2-sulfatáza	Liečba mukopolysacharidózy, typ II (Hunterov syndróm)
Slovenian	Iduronat-2-sulfataza	Zdravljenje mukopolisaharidoze tipa II (Hunterjev sindrom)
Spanish	Iduronato-2-sulfatasa	Tratamiento de mucopolisacaridosis tipo II (enfermedad de Hunter)
Swedish	Iduronat-2-sulfatas	Behandling av mucopolysackaridos, typ II (Hunters sjukdom)

[1] At the time of transfer of sponsorship