



Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of mecasermin for the treatment of primary growth hormone insensitivity syndrome

On 26 August 2005, orphan designation (EU/3/05/307) was granted by the European Commission to MDS Pharma Services GB Limited, United Kingdom, for mecasermin for the treatment of primary growth hormone insensitivity syndrome.

The sponsorship was transferred to Tercica Europe Limited, Ireland, in December 2005 and subsequently to Ipsen Pharma, France, in September 2009.

What is primary growth hormone insensitivity syndrome?

Primary growth hormone insensitivity syndrome is a growth disorder. The growth hormone (GH) is a natural hormone secreted (produced) in the body and responsible for the maturation and growth of the human body. In order to activate the cascade of molecular reactions which induce (stimulate) the biological activity of growth, the GH needs to bind to specific structures on the surface of cells: the GH receptors. Sometimes these GH receptors are defective and unable to respond to GH, in other words, the receptor is "insensitive" to the hormone, which is the case in patients with primary growth hormone insensitivity syndrome. The reason why these receptor defects occur is unknown. Other reactions which are normally induced by the link of the GH to its receptor, and which are thus fundamental for the growth of the human body, can be defective in primary growth hormone insensitivity syndrome patients. The insulin-like growth factor-1 (IGF-1) production is one of these reactions. IGF-1, in fact, is crucial for stimulating body growth. Primary growth hormone insensitivity syndrome is a serious chronically debilitating condition.

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

At the time of designation, primary growth hormone insensitivity syndrome affected not more than 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 46,000 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 knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

There were no approved treatments available for primary growth hormone insensitivity syndrome at the time the application was made.

How is this medicine expected to work?

Mecasermin is a recombinant (artificially synthesised) human insulin-like growth factor-1. Once administered in primary growth hormone insensitivity syndrome patients, mecasermin is expected to

*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. This represents a population of 459,700,000 (Eurostat 2004).

replace the natural IGF-1 in the body, thus compensating the lack of this factor. Mecasermin could thereby produce similar effects on body growth to IGF-1's.

What is the stage of development of this medicine?

The effects of mecasermin were evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials in patients were ongoing.

Mecasermin was not authorised anywhere worldwide for primary growth hormone insensitivity syndrome, at the time of submission. Orphan designation of mecasermin was granted in the United States for growth hormone insensitivity syndrome in December 1995.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 13 July 2005 a positive opinion recommending the grant of the above-mentioned designation.

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 Community) 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.

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**Translations of the active ingredient and indication in all EU languages
and Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Mecasermin	Treatment of primary growth hormone insensitivity syndrome
Czech	Mecasermin	Léčba syndromu primární necitlivosti růstového hormonu
Danish	Mecasermin	Behandling af primær væksthormon insensitivitetssyndrom
Dutch	Mecasermin	Behandeling van primair groeihormoon insensitiviteitssyndroom
Estonian	Mekasermiin	Primaarse kasvuhormooni insensitiivsuse sündroomi ravil
Finnish	Mekasermiini	Primaarisen kasvuhormoni-insensitiivisyssyndrooman hoito
French	Mécasermine	Traitement du syndrome primaire de l'insensibilité à l'hormone de croissance
German	Mecasermin	Behandlung des primären Wachstumshormon-insensitivitätssyndroms
Greek	Μηκασερμίνη	Αγωγή κατά του συνδρόμου πρωτοπαθούς έλλειψης ευαισθησίας στην αυξητική ορμόνη
Hungarian	Mecasermin	Primer inzulinszerű növekedési faktor (IGF-1) hiány (primer IGF-1-hiány) okozta növekedési rendellenességben szenvedő gyermekek tartós kezelésére
Italian	Mecasermina	Trattamento della sindrome da resistenza primaria all'ormone della crescita
Latvian	Mekasermins	Primāras augšanas hormona rezistences sindroma ārstēšana
Lithuanian	Mekaserminas	Pirminio augimo hormono nejautrumo sindromo gydymas
Polish	Mecasermin	Leczenie zespołu pierwotnej niewrażliwości na hormon wzrostu
Portuguese	Mecasermina	Tratamento do síndrome de insensibilidade primária à hormona do crescimento
Slovak	Mecasermin	Liečba syndrómu primárnej necitlivosti rastového hormónu
Slovenian	Mekasermin	Zdravljenje primarnega sindroma neobčutljivosti na rastni hormon
Spanish	Mecasermina	Tratamiento del síndrome de resistencia primaria a la hormona de crecimiento
Swedish	Mekasermin	Behandling av primärt tillväxthormon känslolöshets syndrom
Norwegian	Mecasermin	Behandling av primært veksthormoninsensitivitetssyndrom
Icelandic	Mecasermín	Meðferð við frumkomnu vaxtarhormón ónæmisheilkenni