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EMA/COMP/810031/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant modified human growth hormone for the treatment of growth hormone deficiency

On 24 January 2013, orphan designation (EU/3/12/1087) was granted by the European Commission to Richardson Associates Regulatory Affairs Ltd, United Kingdom, for recombinant modified human growth hormone for the treatment of growth hormone deficiency.

What is growth hormone deficiency?

Growth hormone deficiency is a disease caused by the pituitary gland (a gland located at the base of the brain) not producing enough growth hormone, a hormone responsible for body growth in children and for the control of the correct composition of fat, muscle and bone in adults. As a result, children fail to grow normally and have delayed tooth development and puberty. In adults, low or absent growth hormone can cause reduced muscle and bone mass, low energy, weight gain, heart problems and psychological symptoms such as anxiety and depression. The causes of growth hormone deficiency are varied and often unknown.

Growth hormone deficiency is a long-term debilitating and life-threatening condition particularly because of problems with the heart and bones, and psychological symptoms.

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

At the time of designation, growth hormone deficiency affected approximately 4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 204,000 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 designation, several medicines containing growth hormone were authorised in the EU for the treatment of growth hormone deficiency. These medicines are given by daily injection.

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 509,000,000 (Eurostat 2013).

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The sponsor has provided sufficient information to show that recombinant modified human growth hormone might be of significant benefit for patients with growth hormone deficiency because it is expected to be given less often than current treatments. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Recombinant modified human growth hormone is expected to work in the body in the same way as human growth hormone. When injected into the patient, it is expected to replace the missing hormone, thereby correcting the deficiency.

This medicine is produced by a method known as 'recombinant DNA technology': it is made by cells that have received a gene (DNA) that makes them able to produce growth hormone. The growth hormone in this medicine has also been modified to decrease the rate at which it is removed from the body, allowing the medicine to be given less often (possibly once a week or once every other week).

What is the stage of development of this medicine?

The effects of recombinant modified human growth hormone have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with growth hormone deficiency were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for growth hormone deficiency. Orphan designation of the medicine had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 December 2012 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 modified human growth hormone	Treatment of growth hormone deficiency
Bulgarian	Рекомбинантен, модифициран човешки растежен хормон	Лечение на дефицит на растежния хормон
Czech	Rekombinantní modifikovaný lidský růstový hormon	Léčba deficitu růstového hormonu
Danish	Rekombinant modificeret humant væksthormon	Behandling af væksthormonmangel
Dutch	Recombinant gemodificeerd humaan groeihormoon	Behandeling van groeihormoondeficiëntie
Estonian	Rekombinantne modifitseeritud inimese kasvuhormoon.	Kasvuhormooni puudulikkuse ravi
Finnish	Rekombinantti muunneltu ihmisen kasvuhormoni	Kasvuhormonin puutoksen hoito
French	Hormone de croissance recombinante humaine modifiée	Le traitement de la déficience en hormone de croissance
German	Rekombinantes modifiziertes humanes Wachstumshormon	Behandlung eines Wachstumshormonmangels
Greek	Ανασυνδυασμένη τροποποιημένη ανθρώπινη αυξητική ορμόνη	Θεραπεία της ανεπάρκειας της αυξητικής ορμόνης
Hungarian	Módosított rekombináns humán növekedési hormon	Növekedési hormon hiány kezelése
Italian	Ormone della crescita umano ricombinante, modificato	Per il trattamento del deficit di ormone della crescita
Latvian	Rekombinēts modificēts cilvēka augšanas hormons	Augšanas hormona deficīta ārstēšana
Lithuanian	Rekombinantinis modifikuotas žmogaus augimo hormonas	Augimo hormono stokos gydymas
Maltese	Ormon tat-tkabbir uman modifikat rikombinanti	Kura ta' nuqqas tal-ormon tat-tkabbir
Polish	Rekombinowany zmodyfikowany ludzki hormon wzrostu	Leczenie niedoboru hormonu wzrostu
Portuguese	Hormona de crescimento humana modificada recombinante	Tratamento do défice de hormona de crescimento
Romanian	Hormon de creştere uman recombinant modificat	Tratamentul deficienţei de hormon de creştere
Slovak	Rekombinantný modifikovaný ľudský rastový hormón	Liečba nedostatku rastového hormónu
Slovenian	Rekombinantni spremenjen človeški rastni hormon	Zdravljenje pomanjkanja rastnega hormona

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Hormona de crecimiento humana modificada recombinante	Tratamiento de la deficiencia de la hormona del crecimiento
Swedish	Rekombinant modifierat humant tillväxthormon	Behandling av tillväxthormonbrist
Norwegian	Rekombinant modifisert humant veksthormon	Behandling av veksthormonmangel
Icelandic	Raðbrigða umbreytt manna vaxtarhormón	Meðferð við vaxtarhormónskorti