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Public summary of opinion on orphan designation

Efpegsomatropin for the treatment of growth hormone deficiency

On 27 June 2018, orphan designation (EU/3/18/2035) was granted by the European Commission to Hanmi Europe Limited, United Kingdom, for efpegsomatropin for the treatment of growth hormone deficiency.

What is growth hormone deficiency?

Growth hormone deficiency is a condition where the patient lacks a sufficient amount of growth hormone, which is normally secreted by the pituitary gland (at the base of the brain). Growth hormone promotes growth during childhood and adolescence, and also acts on the way the body handles proteins, fat and carbohydrates (sugars).

The condition can be caused by a genetic mutation (change) or other factors such as trauma and inflammation, or it may have no known cause. It can affect people of any age. In childhood, the main signs include failure to grow normally and impaired development of bones and skeletal muscle. In adulthood, the condition can affect the heart, muscles and bones, and cause psychological symptoms such as anxiety and depression.

Growth-hormone deficiency is a long-term debilitating condition that includes decreased bone mass, bone fractures and psychological symptoms. The disease can be life-threatening due to the risk of problems with the heart and blood circulation.

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

At the time of designation, growth hormone deficiency affected approximately 4.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 243,000 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).

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 517,400,000 (Eurostat 2018).

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What treatments are available?

At the time of designation, medicines containing recombinant human growth hormone were authorised in the EU to treat growth hormone deficiency. These were given to patients by daily injection.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with growth hormone deficiency. Early results in patients with the condition indicate that the medicine has a more prolonged effect than existing treatments and may improve patient outcomes. 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?

Efpegsomatropin is a modified version of synthetic human growth hormone. Normal growth hormone is quickly broken down in the body, which makes its effects short-lived. In efpegsomatropin, the growth hormone has been attached to a part of another protein, IgG4, which is found in the blood and has a longer life in the body. This helps protect the medicine from being broken down quickly, and allows the growth hormone to have a prolonged action over a week or more.

What is the stage of development of this medicine?

The effects of efpegsomatropin 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, efpegsomatropin was not authorised anywhere in the EU for growth hormone deficiency or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 24 May 2018 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Efpegsomatropin	Treatment of growth hormone deficiency
Bulgarian	Ефпегсоматропин	Лечение на дефицит на растежния хормон
Croatian	Efpegsomatropin	Liječenje manjka hormona rasta
Czech	Efpegsomatropin	Léčba deficitu růstového hormonu
Danish	Efpegsomatropin	Behandling af væksthormonmangel
Dutch	Efpegsomatropine	Behandeling van groeihormoondeficiëntie
Estonian	Efpegsomatropiini	Kasvuhormooni puudulikkuse ravi
Finnish	Efpegsomatropiini	Kasvuhormonin puutoksen hoito
French	Efpegsomatropine	Le traitement de la déficience en hormone de croissance
German	Efpegsomatropin	Behandlung eines Wachstumshormonmangels
Greek	Εφπεγκσοματροπίνη	Θεραπεία της ανεπάρκειας της αυξητικής ορμόνης
Hungarian	Efpegszomatropin	Növekedési hormon hiány kezelése
Italian	Efpegsomatropina	Trattamento del deficit di ormone della crescita
Latvian	Efpegsomatropīns	Augšanas hormona deficīta ārstēšana
Lithuanian	Efpegsomatropinas	Augimo hormono stokos gydymas
Maltese	Efpegsomatropin	Kura ta' nuqqas tal-ormon tat-tkabbir
Polish	Efpegsomatropina	Leczenie niedoboru hormonu wzrostu
Portuguese	Efpegsomatropina	Tratamento do défice de hormona de crescimento
Romanian	Efpegsomatropină	Tratamentul deficienţei de hormon de creştere
Slovak	Efpegsomatropín	Liečba nedostatku rastového hormónu
Slovenian	Efpegsomatropin	Zdravljenje pomanjkanja rastnega hormona
Spanish	Efpegsomatropina	Tratamiento de la deficiencia de la hormona del crecimiento
Swedish	Efpegsomatropin	Behandling av tillväxthormonbrist
Norwegian	Efpegsomatropin	Behandling av veksthormonmangel
Icelandic	Efpegsómatrópíni	Meðferð við vaxtarhormónskorti

[1] At the time of designation