



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Lona pegsomatropin for the treatment of growth hormone deficiency

On 17 October 2019, orphan designation EU/3/19/2213 was granted by the European Commission to Ascendis Pharma Endocrinology Division A/S, Denmark, for lona pegsomatropin for the treatment of growth hormone deficiency.

What is growth hormone deficiency?

Growth hormone deficiency is a condition where the patient lacks sufficient growth hormone, which is normally released by the pituitary gland at the base of the brain.

Growth hormone promotes growth during childhood and adolescence, and also affects how the body handles proteins, fat and carbohydrates (sugars). In children, the main signs of growth hormone deficiency include failure to grow normally and impaired development of bones and skeletal muscle. In adults, the condition can affect the heart, muscles and bones, alter metabolism and cause mental problems such as anxiety and depression.

Growth hormone deficiency is a long-term debilitating condition that includes decreased bone mass, bone fractures and mental problems. 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 in 10,000 people in the European Union (EU). This was equivalent to a total of around 207,000 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, medicines containing recombinant human growth hormone were authorised in the EU to treat growth hormone deficiency. These were given to patients 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 28), Norway, Iceland and Liechtenstein. This represents a population of 518,400,000 (Eurostat 2019).



The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with growth hormone deficiency. Early clinical data indicate that the use of lonapegsomatropin leads to faster growth than existing 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?

Lonapegsomatropin is a 'prodrug' of somatropin (human growth hormone), meaning that it is converted to somatropin in the body. The somatropin in the medicine is attached to polyethylene glycol (PEG), with a linker that attaches the two. The linker is expected to break down gradually in the blood to release the somatropin over a long period. In this way the somatropin is expected to remain in the body for longer and it can be injected less frequently than other growth hormone medicines.

What is the stage of development of this medicine?

The effects of lonapegsomatropin have been evaluated in experimental models.

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

At the time of submission, lonapegsomatropin 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, the COMP adopted a positive opinion on 12 September 2019, recommending the granting of this 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 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](#).

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Lona pegsomatropin	Treatment of growth hormone deficiency
Bulgarian	Лонапегсоматропин	Лечение на дефицит на растежния хормон
Croatian	Lona pegsomatropin	Liječenje manjka hormona rasta
Czech	Lona pegsomatropin	Léčba deficitu růstového hormonu
Danish	Lona pegsomatropin	Behandling af væksthormonmangel
Dutch	Lona pegsomatropine	Behandeling van groeihormoondeficiëntie
Estonian	Lona pegsomatropiin	Kasvuhormooni puudulikkuse ravi
Finnish	Lona pegsomatropiini	Kasvuhormonin puutoksen hoito
French	Lona pegsomatropine	Le traitement de la déficience en hormone de croissance
German	Lona pegsomatropin	Behandlung eines Wachstumshormonmangels
Greek	Λοναπεγκσοματροπίνη	Θεραπεία της ανεπάρκειας της αυξητικής ορμόνης
Hungarian	Lona pegszomatropin	Növekedési hormon hiány kezelése
Italian	Lona pegsomatropina	Trattamento del deficit di ormone della crescita
Latvian	Lona pegsomatropīns	Augšanas hormona deficīta ārstēšana
Lithuanian	Lona pegsomatropinas	Augimo hormono stokos gydymas
Maltese	Lona pegsomatropin	Kura ta' nuqqas tal-ormon tat-tkabbir
Polish	Lona pegsomatropina	Leczenie niedoboru hormonu wzrostu
Portuguese	Lona pegsomatropina	Tratamento do déficit de hormona de crescimento
Romanian	Lona pegsomatropină	Tratamentul deficienței de hormon de creștere
Slovak	Lona pegsomatropín	Liečba nedostatku rastového hormónu
Slovenian	Lona pegsomatropin	Zdravljenje pomanjkanja rastnega hormona
Spanish	Lona pegsomatropina	Tratamiento de la deficiencia de la hormona del crecimiento
Swedish	Lona pegsomatropin	Behandling av tillväxthormonbrist
Norwegian	Lona pegsomatropin	Behandling av veksthormonmangel
Icelandic	Lona pegsomatropin	Meðferð við vaxtarhormónskorti

¹ At the time of designation