11 November 2021
EMA/CHMP/615593/2021
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Lonapegsomatropin Ascendis Pharma

lonapegsomatropin

On 11 November 2021, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Lonapegsomatropin Ascendis Pharma[2], intended for the treatment of growth hormone deficiency (GHD) in children and adolescents above 3 years of age.

The applicant for this medicinal product is Ascendis Pharma Endocrinology Division A/S.

Lonapegsomatropin Ascendis Pharma will be available as 3 mg, 3.6 mg, 4.3 mg, 5.2 mg, 6.3 mg, 7.6 mg, 9.1 mg, 11 mg and 13.3 mg powder and solvent for solution for injection. The active substance of Lonapegsomatropin Ascendis Pharma is lonapegsomatropin, a long-acting, once-weekly transiently pegylated somatropin that in the body dissociates into somatropin, also called human growth hormone (hGH), and carrier methoxypolyethylene glycol (mPEG). The ATC code is yet to be assigned.

The benefits of Lonapegsomatropin Ascendis Pharma are consistent, clinically relevant, improvements of growth-related parameters, such as annualised height velocity (AHV), height standard deviation score (height SDS), insulin-like growth factor-1 SDS (IGF-I SDS), in paediatric GHD patients who are either naïve or non-naïve to growth hormone.

The most common side effects are headache, arthralgia, secondary hypothyroidism, and injection site reactions.

The full indication is:

> Growth failure in children and adolescents aged from 3 years up to 18 years due to insufficient endogenous growth hormone secretion (growth hormone deficiency [GHD]).

Lonapegsomatropin Ascendis Pharma should be prescribed by physicians experienced in the diagnosis and management of paediatric patients with growth hormone deficiency.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.