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SCIENCE MEDICINES HEALTH

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Lonapegsomatropin Ascendis Pharma (*lonapegsomatropin*)

An overview of Lonapegsomatropin Ascendis Pharma and why it is authorised in the EU

What is Lonapegsomatropin Ascendis Pharma and what is it used for?

Lonapegsomatropin Ascendis Pharma is a medicine that is used to improve growth in children and adolescents who do not produce enough growth hormone (growth hormone deficiency or GHD). The medicine is intended for children and adolescents from 3 up to 18 years of age.

GHD is rare, and Lonapegsomatropin Ascendis Pharma was designated an 'orphan medicine' (a medicine used in rare diseases) on 17 October 2019. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3192213.

Lonapegsomatropin Ascendis Pharma contains the active substance lonapegsomatropin.

How is Lonapegsomatropin Ascendis Pharma used?

Lonapegsomatropin Ascendis Pharma can only be obtained with a prescription and treatment should be started and monitored by a doctor who is qualified and experienced in the diagnosis and treatment of GHD in children.

The medicine is available as an injection of various strengths, to be given under the skin once a week. The starting dose depends on body weight and is then adjusted individually by the doctor based on response. Patients or their caregivers can inject the dose themselves after appropriate training.

For more information about using Lonapegsomatropin Ascendis Pharma, see the package leaflet or contact your doctor or pharmacist.

How does Lonapegsomatropin Ascendis Pharma work?

Growth hormone is released by the pituitary gland (a gland at the base of the brain). It is important for growth during childhood and adolescence, and it also affects how the body handles proteins, fat and carbohydrates. The active substance in the medicine, lonapegsomatropin, is a version of human growth hormone (somatropin) that has been attached to a 'carrier' that protects it from being removed from

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the body too quickly. The medicine slowly releases growth hormone into the body after injection, meaning that injections do not have to be given every day

What benefits of Lonapegsomatropin Ascendis Pharma have been shown in studies?

Lonapegsomatropin Ascendis Pharma given once weekly in equivalent doses has been shown to be as effective as daily somatropin injections in a main study involving 161 patients aged 3 to 12 years with previously untreated GHD. The study measured the average rate of growth (height velocity) over a year, which was 11.2 cm per year in the group given lonapegsomatropin and 10.3 cm per year in those given daily somatropin (average growth rate in both groups before treatment was 3.9 cm per year). The company also presented the results of supportive studies including patients who had previously had growth hormone treatment.

What are the risks associated with Lonapegsomatropin Ascendis Pharma?

The most common side effects with Lonapegsomatropin Ascendis Pharma (which may affect up to around 1 in 10 people) are headache, joint pain, secondary hypothyroidism (a type of low thyroid function) and reactions at the injection site such as redness, pain, itching or swelling.

For the full list of side effects of Lonapegsomatropin Ascendis Pharma, see the package leaflet.

Growth hormone medicines like Lonapegsomatropin Ascendis Pharma must not be used if the patient has an active tumour or an acute life-threatening illness. The medicine must also not be used for promoting growth in children with closed epiphyses (when the large bones have finished growing). For the full list of restrictions, see the package leaflet.

Why is Lonapegsomatropin Ascendis Pharma authorised in the EU?

The European Medicines Agency decided that Lonapegsomatropin Ascendis Pharma's benefits are greater than its risks and it can be authorised for use in the EU. The medicine was shown to be effective both in patients previously treated with growth hormone and newly diagnosed patients who had not yet been treated, and the weekly injections were preferred by most patients. The short-term safety appears to be in line with other growth hormone products; although any longer-term risk from the carrier part of the active substance seems unlikely, this will be closely monitored after marketing.

What measures are being taken to ensure the safe and effective use of Lonapegsomatropin Ascendis Pharma?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Lonapegsomatropin Ascendis Pharma have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Lonapegsomatropin Ascendis Pharma are continuously monitored. Suspected side effects reported with Lonapegsomatropin Ascendis Pharma are carefully evaluated and any necessary action taken to protect patients.

Other information about Lonapegsomatropin Ascendis Pharma

Further information on Lonapegsomatropin Ascendis Pharma can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/lonapegsomatropin-ascendis-pharma.