10 February 2021
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Public summary of opinion on orphan designation

Poly(oxy-1,2-ethanediyl), alpha-hydro-omega-methoxy, ether with N-[[[2-[[6-[[1-[3-[[3-(2,3-dihydroxypropoxy)propyl]amino]-3-oxopropyl]-2,5-dioxo-3-pyrrolidinyl]thio]hexyl]amino]ethyl]amino]carbonyl]-2-methylalanyl-teriparatide (2:1) for the treatment of hypoparathyroidism

On 19 October 2020, orphan designation EU/3/20/2350 was granted by the European Commission to Ascendis Pharma Bone Diseases A/S, Denmark, for poly(oxy-1,2-ethanediyl), alpha-hydro-omega-methoxy, ether with N-[[[2-[[6-[[1-[3-[[3-(2,3-dihydroxypropoxy)propyl]amino]-3-oxopropyl]-2,5-dioxo-3-pyrrolidinyl]thio]hexyl]amino]ethyl]amino]carbonyl]-2-methylalanyl-teriparatide (2:1) (also known as TransConPTH) for the treatment of hypoparathyroidism.

What is hypoparathyroidism?

Hypoparathyroidism is a hormone disorder where the parathyroid glands in the neck produce too little parathyroid hormone, in most cases because of damage to the parathyroid glands occurring during surgery. Parathyroid hormone helps to regulate levels of calcium and phosphate in the body; too little of it may result in too little calcium and too much phosphate in the blood, which can affect bones, nerves and muscles, causing problems such as paraesthesia (tingling sensations), weakening of the bones, muscle spasms, seizures (fits), irregular heartbeat and damage to heart muscle.

Hypoparathyroidism is a debilitating disease that is long lasting and may be life threatening due to the effects of low blood calcium.

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

At the time of designation, Hypoparathyroidism affected approximately 3.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 166,000 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).

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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What treatments are available?

At the time of designation, hypoparathyroidism was treated in the EU with calcium and vitamin D supplements, with Natpar (parathyroid hormone) as an add-on when these treatments have not worked well enough. Teriparatide was also used to prevent the loss of calcium in the bones.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with hypoparathyroidism because preliminary data shows that it can reduce the need for vitamin D supplements. 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?

The medicine is a prodrug that is expected to be given by injection under the skin once a day. It is converted inside the body to a shortened form of parathyroid hormone, which has similar effects to naturally occurring parathyroid hormone. It is expected to replace the missing hormone in patients with hypoparathyroidism, thereby helping to correct the body's levels of calcium and phosphate.

What is the stage of development of this medicine?

The effects of the medicine 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 hypoparathyroidism were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of hypoparathyroidism 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 10 September 2020, 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

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.