EUROPEAN MEDICINES AGENCY
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Press release

First hormone replacement therapy for parathyroid disorder

Orphan medicine Natpar recommended by CHMP for conditional marketing authorisation

The European Medicines Agency (EMA) has recommended granting a conditional marketing authorisation in the European Union (EU) for Natpar (parathyroid hormone). Natpar is proposed as a treatment for patients with chronic hypoparathyroidism who cannot be adequately controlled with standard treatment with calcium and vitamin D. It is the first approved replacement therapy with parathyroid hormone for this rare condition.

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 during surgery. This results in too little calcium and too much phosphate in the blood, which affects the normal functioning of nerves and muscles leading to symptoms such as tingling sensations and muscle spasms or even seizures and heart rhythm disorders. In the longer term, uncontrolled hypoparathyroidism increases the risk of bone fractures and calcium deposits, particularly on the kidney, brain and eye lens.

Natpar, a hormonal injection administered once daily, is identical to human parathyroid hormone. Injection of Natpar replaces to some extent the missing hormone in patients with hypoparathyroidism, thus helping to restore calcium and phosphate levels in those patients where standard therapy with calcium and vitamin D is insufficient or poorly tolerated. Currently, no treatment options are available for these patients.

EMA's Committee for Medicinal Products for Human Use (CHMP) recommended conditional approval for Natpar. Conditional approval is one of the Agency's main mechanisms to facilitate earlier access by patients to medicines that fulfil unmet medical needs. Conditional approval allows EMA to recommend a medicine for marketing authorisation before all data from clinical trials become available, if the benefits of making this medicine available to patients immediately outweigh the risks inherent in the lack of comprehensive data.

The safety and effectiveness of Natpar were evaluated in a clinical trial of 124 participants who were randomly assigned to receive Natpar or a placebo (dummy treatment), in addition to the standard treatment with calcium and vitamin D. The trial was designed to determine whether Natpar can be

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used to help reduce the amount of calcium or vitamin D taken by the participants, while maintaining acceptable calcium and phosphate serum levels. Results showed that 54.8% of participants treated with Natpar were able to reduce the doses of calcium and vitamin D supplements by more than 50% while maintaining acceptable blood-calcium levels, compared to 2.5% of participants who received the placebo treatment.

As part of the conditional marketing authorisation, the applicant for Natpar is required to conduct a 26-week clinical trial to further study the safety and efficacy of the medicine, confirm the dosing schedule and assess the effects of treatment on symptoms of the disease and on patients' quality of life. The study will also look at how calcium and phosphate are processed in the body during treatment.

Because hypoparathyroidism is rare, Natpar received an orphan designation from the Committee for Orphan Medicinal Products (COMP) in 2013. Orphan designation is the key instrument available in the EU to encourage the development of medicines for patients with rare diseases. Orphan-designated medicines qualify for ten years' market exclusivity. In addition, orphan designation gives medicine developers access to incentives, such as fee reductions for marketing authorisation applications and for scientific advice.

The opinion adopted by the CHMP at its February 2017 meeting is an intermediary step on Natpar's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of Natpar in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Natpar is Shire Pharmaceuticals Ireland Limited.
3. Following this positive CHMP opinion, the COMP will assess whether the orphan designation for Natpar should be maintained.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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