

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Palopegteriparatide for treating hypoparathyroidism ID6380

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of palopegteriparatide within its marketing authorisation for treating hypoparathyroidism.

Background

Hypoparathyroidism is a condition in which the parathyroid glands in the neck do not produce enough parathyroid hormone, which regulates the levels of calcium and phosphate in the blood. This leads to low blood calcium levels (hypocalcaemia) and raised blood phosphorus levels (hyperphosphataemia). Symptoms include muscle cramps or pains, tiredness, and a tingling sensation in the hands, feet or around the mouth. People with hypoparathyroidism may also experience mood changes (such as irritability, anxiety or depression), blurred vision, dry and rough skin, and course hair and nails that break easily. Symptoms may worsen without treatment, and hypoparathyroidism as a whole is associated with an impaired quality of life and increased risk of depression.¹

Hypoparathyroidism may be temporary or permanent depending on the cause. Most cases of hypoparathyroidism are caused by accidental damage to or removal of the parathyroid glands following neck surgery, but other causes include autoimmune disease, radiotherapy, congenital issues with parathyroid glands, and low magnesium levels.¹

Estimates of prevalence of hypoparathyroidism range between 6 and 37 per 100,000 people, which equates to between 3,400 and 20,900 people across England with hypoparathyroidism based on current population estimates.^{2,3}

Treatment for hypoparathyroidism is focused on relief of symptoms and improvement in quality of life. Oral calcium supplements and vitamin D analogues such as alfacalcidol or calcitriol can be taken to keep calcium levels stable and within normal range. Where there are more acute and severe symptoms of hypocalcaemia, calcium may be given intravenously. Calcium and phosphorus levels may also be managed by following a high-calcium, low-phosphorus diet. Long-term supplementation for hypoparathyroidism may affect kidney function and therefore treatment should aim to keep calcium levels high enough to avoid symptoms of hypocalcaemia but low enough to avoid causing problems with the kidneys linked to high calcium levels.⁴

The technology

Palopegteriparatide (TransCon PTH, Ascendis Pharma) does not currently have a marketing authorisation in the UK for treatment of adult patients with hypoparathyroidism. It has been studied in clinical trials compared with placebo in adults with chronic hypoparathyroidism of at least 26 weeks in duration.

Intervention(s)	Palopegteriparatide
Population(s)	People with hypoparathyroidism lasting at least 6 months
Comparators	<ul style="list-style-type: none"> • Recombinant parathyroid hormone <p>Established clinical management without palopegteriparatide, which may include:</p> <ul style="list-style-type: none"> • Vitamin D analogues such as alfacalcidol or calcitrol • Calcium supplements
Outcomes	<p>The outcome measures to be considered include:</p> <ul style="list-style-type: none"> • reduction in symptoms such as fatigue • reduction in calcium supplements and vitamin D analogues • serum calcium levels • serum phosphate levels • adverse effects of treatment • health-related quality of life.
Economic analysis	<p>The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year.</p> <p>The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared.</p> <p>Costs will be considered from an NHS and Personal Social Services perspective.</p>
Other considerations	<p>Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.</p>
Related NICE recommendations	None
Related National Policy	<p>The NHS Long Term Plan (2019) NHS Long Term Plan</p> <p>NHS England (2018) NHS manual for prescribed specialist services (2018/2019)</p>

Questions for consultation

Is recombinant parathyroid hormone currently in use in clinical practice in England for the treatment of hypoparathyroidism?

Are there any particular symptoms of hypoparathyroidism for which a separate outcome related to reduction in symptoms should be listed?

Where do you consider palopegteriparatide will fit into the existing care pathway for hypoparathyroidism?

Would palopegteriparatide be a candidate for managed access?

Do you consider that the use of palopegteriparatide can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which palopegteriparatide will be licensed;
- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;
- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE intends to evaluate this technology through its Single Technology Appraisal process. (Information on NICE's health technology evaluation processes is available at <https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation>).

References

1. NHS (2021). [Hypoparathyroidism](#). Accessed February 2024.
2. Bjornsdottir S, Ing S, Mitchell DM, et al (2022). Epidemiology and financial burden of adult chronic hypoparathyroidism. *Journal of Bone and Mineral Research* 37(12):2602-2614.
3. Office for National Statistics (2022). [Population estimates for the UK, England and Wales, Scotland and Northern Ireland: mid-2021](#). Accessed March 2024.

4. Parathyroid UK (2023). [Hypoparathyroidism - Current treatment](#). Accessed February 2024.