

# Investigational hormone replacement promising treatment for rare disorder

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An investigational parathyroid hormone replacement effectively treated a rare disorder characterized by low calcium and high phosphate levels in the blood, a new study finds. The results will be presented at The Endocrine Society's 94th Annual Meeting in Houston.

The [parathyroid](#) hormone replacement is called recombinant human [parathyroid hormone](#), or rhPTH(1-84). It is produced in the lab using a replica of the [human parathyroid hormone](#) gene. rhPTH(1-84) is identical to the naturally occurring parathyroid hormone and acts to regulate calcium levels in the same way. Calcium is an essential mineral for bone, teeth, and muscle health, among other things.

In the rare and complex disorder known as hypoparathyroidism, or HypoPARA, the parathyroid gland produces insufficient parathyroid hormone. As a result, calcium levels drop, while the amount of phosphate, another mineral, increases in the body. This mineral imbalance can cause a number of symptoms, including muscle twitching, spasms, and pain; seizures; cramps in the hands and feet; fatigue; anxiety; depression; and problems with bone and [tooth development](#).

Current options are limited to symptom management with calcium and active vitamin D in high doses to increase [calcium levels](#). The problem with this approach is that long-term use of high-dose calcium and vitamin D can cause dangerous calcium formations in [vital organs](#), including the kidneys, heart, and brain.

"Hypoparathyroidism is the only classic endocrine deficiency disease for which the missing hormone, in this case parathyroid hormone, is not an approved therapy," said lead study investigator, John P. Bilezikian, professor of medicine, Division of Endocrinology, Columbia University College of Physicians and Surgeons. "This study has the potential not only to fill in this major therapeutic gap by showing the efficacy and safety of parathyroid hormone, but also to markedly improve the management of this disease."

In this study, the largest of its kind, rhPTH(1-84) helped regulate calcium and reduced the amount of calcium and active vitamin D required in more than half of patients, while well tolerated. In contrast, only 2 percent of those who received placebo improved. Also, by the study's end, 41 percent of patients who received rhPTH(1-84) no longer required additional vitamin D and needed  $\leq 500$ mg calcium per day.

"These are important findings for patients with this rare and complex disorder who need more options for care, especially considering the potential threat to the kidneys, heart, and brain associated with the long-term use of high-dose calcium and vitamin D," Bilezikian said.

Investigators randomly assigned 134 patients to receive either the parathyroid hormone replacement or placebo. Patients were 78 percent female, 96 percent white, and their average age was 48 years.

After undergoing adjustments of calcium and active vitamin D doses to normalize blood levels of calcium for at least two weeks, patients received daily injections of rhPTH(1-84) or placebo for 24 weeks. Study visits occurred every four weeks, and neither patients nor investigators knew who was receiving study drug versus placebo in this double-blinded study.

Hypoparathyroidism is believed to affect about 100,000 people in the

United States. This rare and complex disorder is most often the result of injury to the parathyroid gland during surgery, but also may be hereditary.

Provided by The Endocrine Society

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