LONG-TERM TERIPARATIDE (rhPTH 1-34) TREATMENT IN CHILDREN WITH SYNDROMIC HYPOPARATHYROIDISM

Raffaele Buganza¹, Gerdi Tuli¹, Patrizia Matarazzo¹, Daniele Tessaris¹, Luisa de Sanctis¹

¹Department of Paediatric Endocrinology, Regina Margherita Children’s Hospital, Turin, Italy

Topic: Bone, growth plate and mineral metabolism

Background

Hypoparathyroidism is characterized by absence or inadequately low circulating concentrations of the parathyroid hormone, resulting in hypocalcemia, hyperphosphatemia and elevated fractional excretion of calcium in the urine. The use of activated vitamin D analogues and calcium supplements represents the conventional therapy. Subcutaneous recombinant human parathormone (rhPTH (1-34)) has been proposed for hypoparathyroidism treatment, even to avoid side effects of vitamin D and calcium.

Objective

To evaluate rhPTH (1-34) long term safety and efficacy in pediatric patients with genetically proven syndromic hypoparathyroidism.

Patients and methods

The study is a 9.2-year self-controlled trial on six pediatric patients (four males, two females, age 9.4±2.2 years) with syndromic hypoparathyroidism: three subjects with autoimmune polyendocrinopathy candidiasis ectodermal dysplasia syndrome (APECED) syndrome (one of those with intestinal malabsorption), two with DiGeorge syndrome and one with hypoparathyroidism-deafness-renal dysplasia syndrome. Hypocalcemic clinical signs and biochemical parameters (blood calcium, phosphate, alkaline phosphatase and urinary calcium-to-creatinine ratio) were compared during conventional treatment and on rhPTH (1-34) (teriparatide, 12.5 μg twice a day).

Results

The rhPTH (1-34) treatment allowed a marked reduction, although not always a complete suspension, of calcium supplementation and a slight reduction of calcitriol therapy. During rhPTH (1-34), mean blood calcium and alkaline phosphatase were not significantly modified, whereas significant reduction of the urinary calcium-to-creatinine ratio (0.55±0.32 vs. 0.16±0.09, p=0.02) and blood phosphate (2.01±0.23 vs. 1.69±0.01, p=0.21) was obtained. The number of tetanic episodes was reduced in four patients during teriparatide treatment. Renal ultrasound findings worsened in 3 patients (with nephrocalcinosis in 2) and was unmodified in the other 3. No adverse effects were detected during the observation time.

Conclusions

In the presented children with syndromic hypoparathyroidism, substitutive treatment with rhPTH (1-34) allowed to maintain adequate blood calcium and phosphorus levels, to normalize urinary calcium excretion, to reduce the tetanic episodes. In patients with low compliance or with intestinal malabsorption, its utilization should be considered, even to reduce vitamin D and calcium treatment side effects.

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