WHAT IS HYPOPHOSPHATASIA?

Hypophosphatasia (HPP) is a rare, genetic (inherited), metabolic disease that reduces the normal mineralization ("calcification") of the process that hardens and strengthens bones. This can lead to poor growth and development, weakness and deformity of bones and other skeletal abnormalities, and premature loss of teeth with the root intact. As a result, the disease can have a debilitating impact, including heart and lung function. HPP is caused by a defect in the gene that is responsible for making an enzyme known as alkaline phosphatase (ALP), which is important for building healthy bones.

The severity of HPP can be wide-ranging and may present in many ways. All patients, including those whose signs or symptoms are not recognized until childhood or adulthood, may experience significant disease burden that impacts their daily life, including the ability to perform daily tasks or work.

HOW IS HPP DIAGNOSED?

Since HPP shares symptoms with other, more common diseases, it can be misdiagnosed, and diagnosis is often delayed. New symptoms can appear at any age and worsen over time, causing significant disability or life-threatening complications.

Once a person shows signs and symptoms of a full clinical assessment and blood test, low ALP can help lead to a correct diagnosis. Genetic testing may also be helpful in confirming HPP.

HOW HAS HPP TREATMENT EVOLVED?

Initially, treatment for HPP relied only on supportive care and symptoms management, but research has led to more options. Clinical studies in HPP have shown that replacing deficient ALP can improve the body’s ability to mineralize bone by allowing phosphate and calcium to bind together. This has proven to improve bone health and mobility, as well as survival in infants.

We continue to innovate for patients with HPP and accelerate the development of life-changing therapies. Alexion is progressing our next generation alkaline phosphatase enzyme replacement therapy in clinical trials in adults and children without an underlying cause of HPP for patients with signs and symptoms of the disease during childhood.

HOW IS ALEXION CONTINUING TO INNOVATE FOR HPP PATIENTS?

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