

Poster Number 61

Abstract 0240_0346_000080

Dental Data Review on Efficacy of Asfotase Alfa in Infants and Children 5 years of age with Hypophosphatasia

Catherine Long, University of Manitoba; **Robert J Schroth**, University of Manitoba; **Cheryl Rockman-Greenberg**, University of Manitoba

Background:

Hypophosphatasia (HPP) is a highly variable genetic disease that impedes the development of teeth and bones. Phenotype ranges from early lethality shortly after birth to mild expression in adult to a dental only form (odontoHPP). HPP is due to mutations in the *ALPL* gene which encodes the tissue non-specific form of alkaline phosphatase (TNSALP). Dental manifestations of HPP include painless premature exfoliation of primary teeth with intact roots. Until recently, there was no effective treatment for this disorder.

Objective:

The aim of this study is to review the development and exfoliation patterns of primary and permanent teeth in HPP patients with systemic HPP to examine the efficacy of the enzyme replacement therapy treatment (ERT) using asfotase alfa. This is a human recombinant bone-targeted ALP (Strensiq™)(Alexion Pharma).

Methods:

Available dental data were recorded from chart review of study files from scheduled visits as follows: exfoliation patterns, radiographic records, and development of deciduous and permanent teeth.

Results:

A total of 11 patients (7 females, 4 males) participated in the 72-month clinical trial. The average age of the patients at initial treatment was 2.5 years old. Although exfoliation patterns did not change in patients treated with ERT, results, as compared with data from the literature and untreated historical controls, showed improvement in the radiopacity of adult teeth developed after the treatment as well as patterns of increase in average number of teeth erupted starting from 0.2 at three months to 1.65 at 24 months of clinical study.

Conclusion:

Current published studies show sustained improvement in systemic HPP manifestations following ERT. This is the first demonstration, albeit on a small number of patients, that long term dental health may be improved in patients with systemic HPP and dental involvement.