

Enobia Presents Results from Hypophosphatasia Impact Patient Survey

Presentations at ACMG Annual Meeting Spotlight Clinical Progress and Documented Burden of Illness in Hypophosphatasia

Montreal, Canada – March 29, 2010 – Enobia Pharma Inc. unveiled findings from the first hypophosphatasia (HPP) self-reported patient survey intended to evaluate the burden of illness associated with HPP at the 2010 American College of Medical Genetics (ACMG) Annual Clinical Genetics Meeting. An update on the clinical program for ENB-0040, Enobia's investigational enzyme replacement therapy for HPP, was also presented at the meeting by Cheryl Rockman-Greenberg, MD, Medical Director, Child Health Programme, Winnipeg Regional Health Authority, Professor and Head, Department of Pediatrics & Child Health, University of Manitoba.

Hypophosphatasia is a rare, inherited, and sometimes fatal metabolic bone disease that affects individuals of all ages. The global Hypophosphatasia Impact Patient Survey (HIPS), fielded over the past six months, documents a wide range of serious and progressive symptoms in children and adults with HPP. The majority of adult respondents (69 percent) reported worsening of their HPP symptoms in the past five years. Health-related quality of life, as measured by the SF-12 (a health assessment questionnaire commonly used by clinicians), was significantly diminished compared to the normal population and comparable to data reported for patients with late-onset Pompe disease and Gaucher disease.^{1,2}

Also included among the findings presented today:

- Longer duration of HPP-related symptoms was associated with more bone fractures.
- Eighty-two percent of adults reported bone pain severe enough to limit activities of daily living, as well as joint pain (80 percent) and muscle pain (56 percent).
- Forty-seven percent of adults with onset of symptoms in the first year of life currently use a wheelchair as compared with 34 percent of children.
- Fifty-five percent of the adult respondents (and 75 percent of the adults with symptom onset in the first year of life) had surgical fracture repair requiring hardware, such as rods, plates or screws.
- Respondents with onset of symptoms in the first year of life had significantly more developmental delay, bone deformities, and fractures as compared to adults with onset of symptoms after the first year.

"HPP places a pronounced burden on patients of all ages," said Hal Landy, MD, Chief Medical Officer and Vice President, Medical Affairs at Enobia. "We are dedicated to improving knowledge about the impact HPP has on patients and their caregivers while we continue to advance our clinical program for ENB-0040 for all age groups."

In addition to the survey results, Enobia announced that enrollment is now complete in the third clinical trial of ENB-0040, a 13-patient Phase 2 study evaluating the safety, efficacy and pharmacokinetics of ENB-0040 in children with HPP between the ages of 5 and 12. The current study builds on positive effects seen in a Phase 2 study in infants, the results of which were presented last September at the Annual Meeting of the American Society for Bone and Mineral Research. The long-term extension phase of the infant trial continues. A total of 10 infants are currently being treated.

Thirty patients have now received ENB-0040 in clinical trials. To date no anti-ENB-0040 antibodies have been detected in any of the patients studied.

About Hypophosphatasia

Hypophosphatasia is a rare, inherited, and sometimes fatal metabolic bone disease. Affected individuals have low levels of the tissue non-specific form of alkaline phosphatase, an essential regulator of bone mineralization, leading to rickets in infants and children and osteomalacia (“soft bones” resulting from poor mineralization) in adults. Disease severity is inversely proportional to the age at symptom onset. Clinical severity ranges from the severe perinatal or infantile forms, with marked skeletal hypomineralization and respiratory compromise often causing death, to a persistent and debilitating osteomalacia in adults.

In the infantile form, infants may appear normal at birth but develop serious symptoms in the first six months of life. These can include failure to thrive, respiratory failure, fractures, and seizures. Radiographic findings include generalized hypomineralization and rickets. First year mortality in these patients is estimated at 50 percent. In the childhood form, patients have varying degrees of skeletal hypomineralization and may have frank rickets, short stature, bone pain, muscle weakness, delayed motor milestones, early loss of deciduous teeth, and may experience frequent, poorly-healing fractures. In the adult form, the underlying osteomalacia causes pathological fractures that impair ambulation.

About ENB-0040

ENB-0040, an investigational treatment for hypophosphatasia, is a subcutaneous enzyme replacement therapy of tissue non-specific alkaline phosphatase (TNSALP) fused to a patented bone targeting peptide. ENB-0040 is designed to directly target TNSALP to the bone in order to correct the enzyme deficiency, which could lead to restoration of normal bone mineralization. ENB-0040, awarded orphan designation in the U.S. and EU in 2008 and Fast Track status in 2009, is currently in Phase 2 clinical development.

About Enobia Pharma Inc.

Enobia Pharma Inc. is a private, Montreal based company focused on the development of therapeutics to treat serious bone disorders for which there are no drug therapies currently approved. ENB-0040, an investigational drug for the treatment of hypophosphatasia, is the Company’s lead program. For more information, please visit www.enobia.com.

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¹ Weinreb N, *et al.* Imiglucerase (Cerezyme) improves quality of life in patients with skeletal manifestations of Gaucher disease. *Clin Genet* 2007;71:576-588.

² Hagemans, *et al.* Late-onset Pompe disease primarily affects quality of life in physical health domains. *Neurology* 2004;63:1688-1692.