April 11, 2014

Researchers to Present New Data on Asfotase Alfa in Infants and Juveniles with Hypophosphatasia at the Joint Meeting of the Pediatric Academic Societies and the Asian Society for Pediatric Research

-- New data from a natural history study in infants with hypophosphatasia also to be presented --

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that researchers are scheduled to present new data from the extension phase of two clinical studies examining the long-term efficacy and safety of asfotase alfa in infants and juveniles with hypophosphatasia (HPP) at the joint meeting of the Pediatric Academic Societies (PAS) and the Asian Society for Pediatric Research, which takes place May 3-6, 2014 in Vancouver, B.C., Canada. The meeting will also feature the presentation of results from a retrospective natural history study of patients with perinatal and infantile HPP.

HPP is an inherited, ultra-rare metabolic disorder that can lead to progressive damage to multiple vital organs, destruction and deformity of bones, and death. The U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy designation for asfotase alfa in pediatric-onset HPP, defined as patients whose first signs or symptoms of HPP occurred prior to 18 years of age, including perinatal-, infantile-, and juvenile-onset forms of the disease.

Abstracts summarizing the asfotase alfa studies and the HPP natural history study were published today on the PAS website and are available to conference registrants at: http://www.pas-meeting.org/.

The following abstract will be presented in a poster session on May 4, 2014 from 4:15pm - 7:30pm Pacific Daylight Time (PDT):

- Abstract 752396: "Asfotase Alfa: Sustained Efficacy and Tolerability in Infants and Young Children with Life-Threatening Hypophosphatasia," Whyte, et al.

The following abstracts will be presented in a poster session on May 5, 2014 from 4:15pm - 7:30pm PDT:

- Abstract 752577: "Asfotase Alfa: Long-Term Safety and Efficacy in Children with Hypophosphatasia," Madson, et al.
- Abstract 752416: "Hypophosphatasia: A Retrospective Natural History Study of the Severe Perinatal and Infantile Forms," Whyte, et al.

About Hypophosphatasia (HPP)

Hypophosphatasia (HPP) is a chronic, life-threatening, genetic, and ultra-rare metabolic disease characterized by defective bone mineralization that can lead to destruction and deformity of bones, profound muscle weakness, seizures, and respiratory failure.[1-4]

HPP is caused by a genetic deficiency of an enzyme known as tissue non-specific alkaline phosphatase (TNSALP).[1]

The genetic deficiency in HPP can affect people of all ages.[1] HPP is traditionally classified by the age of the patient at the onset of symptoms of the disease. Patients with perinatal-onset HPP manifest their first signs of disease in utero or at birth. This form of the disease often leads to death at or soon after birth. Those patients who survive birth often have severe rickets and severely compromised respiratory function.[5]

Patients with infantile-onset HPP develop their first signs or symptoms of HPP before 6 months of age. Individuals with this form of disease develop rickets, skeletal abnormalities, fractures, failure to thrive and respiratory failure. The prognosis of these patients may be poor with mortality estimated to be as high as at 50%.[1]

Patients with juvenile-onset HPP exhibit their first signs or symptoms of HPP after 6 months of age and before 18 years of age. Individuals with this form of the disease are at risk for rickets, skeletal complications including fractures, and can have delayed acquisition of age-appropriate motor skills due to skeletal hypomineralization and muscle weakness leading to the need for walking assistance; some may never walk.[1]

About Asfotase Alfa

Asfotase alfa is an investigational, highly innovative, first-in-class targeted enzyme replacement therapy. Asfotase alfa is designed to address the underlying cause of HPP by normalizing the genetically defective metabolic process, and preventing or reversing the severe and potentially life-threatening complications of life-long dysregulated mineral metabolism.

According to the FDA, a Breakthrough Therapy designation is designed to expedite the development of a drug to treat a serious or life-threatening disease when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. The Breakthrough Therapy designation is part of the FDA Safety and Innovation Act (FDASIA) of 2012.[6]

About Alexion

Alexion is a biopharmaceutical company focused on serving patients with severe and rare disorders through the innovation, development and commercialization of life-transforming therapeutic products. Alexion is the global leader in complement inhibition and has developed and markets Soliris® (eculizumab) as a treatment for patients with PNH and aHUS, two debilitating, ultra-rare and life-threatening disorders caused by chronic uncontrolled complement activation. Soliris is currently approved in nearly 50 countries for the treatment of PNH, and in the United States, European Union, Japan and other countries for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris in additional severe and ultra-rare disorders beyond PNH and aHUS, and is developing other highly innovative biotechnology product candidates across multiple therapeutic areas. This press release and further information about Alexion can be found at: www.alexionpharma.com.

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References

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5. Whyte MP. Hypophosphatasia and the extracellular metabolism of inorganic pyrophosphate: Clinical and laboratory aspects. Crit Rev Clin Lab Sci. 1991; 23:175-195.

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