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Alexion Announces Presentations at 2015 American Society for Bone and Mineral Research Annual Meeting, Including New Data on Strensiq™ (asfotase alfa) in Children with Hypophosphatasia (HPP) Treated for Five Years

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that researchers will present new data on the long-term efficacy and tolerability of Strensiq™ (asfotase alfa) in children with hypophosphatasia (HPP) who were treated for up to five years, as well as new data comparing the functional mobility of children with HPP treated with Strensiq compared to untreated historical control patients. Researchers will also present new data on the dosing for Strensiq in patients with HPP and the use of the Radiographic Global Impression of Change (RGI-C) scale for assessing skeletal manifestations of HPP in infants and children, along with a case literature review of HPP manifestations in adults with pediatric-onset HPP. The data will be presented at the 2015 American Society for Bone and Mineral Research (ASBMR) Annual Meeting being held October 9-12, 2015, in Seattle.

HPP is a genetic and progressive metabolic disease in which patients experience devastating effects on multiple systems of the body, leading to debilitating or life-threatening complications. It is an ultra-rare disease, which is defined as a disease that affects fewer than 20 patients per one million in the general population.¹ HPP is characterized by defective bone mineralization that can lead to deformity of bones and other skeletal abnormalities, as well as systemic complications such as profound muscle weakness, seizures, pain, and respiratory failure leading to premature death in infants.²⁻⁶

Strensiq is approved in the European Union, Japan, and Canada as a treatment for patients with HPP. The U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation for Strensiq and accepted Alexion's Biologics License Application (BLA) for Priority Review.

Abstracts summarizing these presentations have been published on the ASBMR website and are available to conference registrants and ASBMR members at: <http://www.asbmr.org/ASBMR-abstracts>.

The following abstract will be presented in an oral session on Saturday, October 10, 2015, from 5:00 to 5:15 p.m., Pacific Daylight Time (PDT):

- Abstract 1071: "Asfotase Alfa: Sustained Efficacy and Tolerability in Children with Hypophosphatasia Treated for 5 Years," Rockman-Greenberg, et al.

The following abstract will be presented in a poster session on Saturday, October 10, 2015, from 12:30 to 2:30 p.m., Pacific Daylight Time (PDT):

- Abstract SA0376: "A Longitudinal, Prospective, Long-Term Registry of Patients with Hypophosphatasia," Seefried, et al.

The following abstracts will be presented in a poster session on Sunday, October 11, 2015, from 12:30 to 2:30 p.m., Pacific Daylight Time (PDT):

- Abstract SU0380: "Exposure-Response Modeling and Simulation to Support Evaluation of Efficacious and Safe Exposure and Dose Range for Asfotase Alfa in Patients with Hypophosphatasia," Pradhan, et al.
- Abstract SU0381: "Manifestations of Hypophosphatasia in Adults with Pediatric Onset of Symptoms: A Review of the Case Literature," Sawyer, et al.

The following abstracts will be presented in a poster session on Monday, October 12, 2015, from 12:30 to 2:30 p.m., Pacific Daylight Time (PDT):

- Abstract MO0382: "Improved Functional Mobility with Asfotase Alfa Treatment in Childhood Hypophosphatasia," Madson, et al.
- Abstract MO0059: "Validation of a Novel Scoring System, the Radiographic Global Impression of Change (RGI-C) Scale, for Assessing Skeletal Manifestations of Hypophosphatasia in Infants and Children," Whyte, et al.

About Hypophosphatasia (HPP)

HPP is a genetic, chronic, and progressive ultra-rare metabolic disease characterized by defective bone mineralization that can lead to destruction and deformity of bones and other skeletal abnormalities, as well as systemic complications such as profound muscle weakness, seizures, pain, and respiratory failure leading to premature death in infants.²⁻⁶

HPP is caused by mutations in the gene encoding an enzyme known as tissue non-specific alkaline phosphatase (TNSALP).^{2,3} The genetic deficiency in HPP can affect people of all ages.² HPP is classified by the age of the patient at the onset of symptoms of the disease, with infantile- and juvenile-onset HPP defined as manifestation of the first symptom prior to 18 years of age.

HPP can have devastating consequences for patients at any stage of life.² In a natural history study, infants who had their first symptom of HPP within the first 6 months of life had high mortality, with an overall mortality rate of 73% at 5 years.⁷ In these patients, mortality is primarily due to respiratory failure.^{2,6,8} In patients surviving to adolescence and adulthood, long-term clinical sequelae include recurrent and non-healing fractures, profound muscle weakness, debilitating pain, and the requirement for ambulatory assistive devices such as wheelchairs, wheeled walkers, and canes.^{2,5}

About Strensiq™ (asfotase alfa)

Strensiq™ (asfotase alfa) is an innovative enzyme replacement therapy designed to address the underlying cause of HPP—a deficiency of TNSALP activity. By replacing the defective enzyme, treatment with Strensiq aims to prevent or reverse the mineralization defects of the skeleton, thereby preventing serious skeletal and systemic morbidity and premature death.

About Alexion

Alexion is a global biopharmaceutical company focused on developing and delivering life-transforming therapies for patients with devastating and rare disorders. Alexion developed and commercializes Soliris® (eculizumab), the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), two life-threatening ultra-rare disorders. Alexion is also establishing a premier global metabolic rare disease franchise, which includes Kanuma™ (sebelipase alfa) for patients with lysosomal acid lipase deficiency (LAD), and Strensiq™ (asfotase alfa) for patients with hypophosphatasia (HPP). In addition, Alexion is advancing the most robust rare disease pipeline in the biotech industry, with highly innovative product candidates in multiple therapeutic areas. As the global leader in complement inhibition, the Company is strengthening and broadening its portfolio of complement inhibitors across diverse platforms, including evaluating potential indications for Soliris in additional severe and ultra-rare disorders. This press release and further information about Alexion can be found at: www.alexion.com.

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References

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3. Whyte MP. Hypophosphatasia: nature's window on alkaline phosphatase function in humans. In: Bilezikian JP, Raisz LG, Martin TJ, eds. *Principles of Bone Biology*. Vol 1. 3rd ed. San Diego, CA: Academic Press; 2008:1573-1598.
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6. Baumgartner-Sigl S, Haberlandt E, Mumm S, et al. Pyridoxine-responsive seizures as the first symptom of infantile hypophosphatasia caused by two novel missense mutations (c.677T > C, p.M226T; c.1112C > T, p.T371I) of the tissue-nonspecific alkaline phosphatase gene. *Bone.* 2007; 40(6):1655-1661.
7. Whyte MP, Leung E, Wilcox W, et al. Hypophosphatasia: a retrospective natural history study of the severe perinatal and infantile forms. Poster presented at the 2014 Pediatric Academic Societies and Asian Society for Pediatric Research Joint Meeting, Vancouver, B.C., Canada, May 5, 2014. Abstract 752416.

8. Whyte MP, Rockman-Greenberg C, Hofmann C, et al. Improved survival with asfotase alfa treatment in pediatric patients with hypophosphatasia at high risk of death. Poster presented at the American Society for Bone and Mineral Research (ASBMR) 2014 Annual Meeting, Houston, September 14, 2014. Abstract 1097.

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Alexion Pharmaceuticals, Inc.

Media

Stephanie Fagan, 203-271-8223

Senior Vice President, Corporate Communications

or

Kim Diamond, 203-439-9600

Executive Director, Corporate Communications

or

Investors

Elena Ridloff, CFA, 203-699-7722

Executive Director, Investor Relations

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