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Researchers to Present New Data at ENDO 2016 Advancing the Understanding of Strensiq® (asfotase alfa) in Infants, Children and Adult Patients with Hypophosphatasia (HPP)

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that researchers will present new functional and quality-of-life data in children with hypophosphatasia (HPP) treated with Strensiq® (asfotase alfa) for at least five years, as well as new data on the biochemical and physical function outcomes in juveniles and adults with HPP treated with Strensiq for up to four years. Researchers will also present new safety and efficacy data in infants with HPP treated with Strensiq for up to three and a half years. Data will be presented in two oral sessions and one poster presentation at the Endocrine Society's 98th Annual Meeting and Expo (ENDO), being held April 1-4, 2016, in Boston.

Strensiq is approved in the United States as a treatment for patients with perinatal-, infantile- or juvenile-onset HPP. Strensiq is also approved in the European Union, Japan and Canada.

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 low alkaline phosphatase (ALP) activity and 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]

Abstracts summarizing these studies were published on the ENDO website and can be accessed using the links below.

The following abstracts will be presented in an oral session on Sunday, April 3, 2016, from 11:45 a.m. to 1:15 p.m., Eastern Standard Time (EST):

- Abstract OR26-2: "Reduction in Pain and Improved Function and Activities of Daily Living in Children with Hypophosphatasia Treated with Asfotase Alfa for 5 Years," Phillips, et al.

  Accessible at: https://endo.confex.com/endo/2016endo/webprogram/Paper24241.html

- Abstract OR26-3: "Biochemical and Physical Function Outcomes in Adolescents and Adults with Hypophosphatasia Treated with Asfotase Alfa for up to 4 Years: Interim Results from a Phase II Study," Kishnani, et al.

  Accessible at: https://endo.confex.com/endo/2016endo/webprogram/Paper25979.html

The following abstract will be presented in a poster preview session on Sunday, April 3, 2016, from 11:30 to 11:45 a.m., as well as a poster session from 1:15 to 3:15 p.m., Eastern Standard Time (EST):

- Abstract PP26-3 and SUN-327: "Efficacy and Safety of Asfotase Alfa in Patients with Infantile Hypophosphatasia Treated for up to 3.5 Years: Results from a Phase II, Open-Label, Uncontrolled Study," Liese, et al.

  Accessible at: https://endo.confex.com/endo/2016endo/webprogram/Paper25983.html

The following abstract will be presented in an oral session on Monday, April 4, 2016, from 10:00 to 11:30 a.m., Eastern Standard Time (EST):

- Abstract OR35-3: "Muscular Function in Akp2$^{-/-}$ Mice and Evaluation of the Effect of Asfotase Alfa on the Akp2$^{-/-}$ Phenotype," Marozsan.

  Accessible at: https://endo.confex.com/endo/2016endo/webprogram/Paper25988.html

About Hypophosphatasia (HPP)

HPP is a genetic, chronic, progressive, and life-threatening ultra-rare metabolic disease characterized by low alkaline

phosphatase (ALP) activity and 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]Â HPP is traditionally classified by the age of the patient at the onset of symptoms of the disease, with perinatal-, infantile- and juvenile-onset HPP defined as patients who have their first symptom prior to 18 years of age.

HPP can have devastating consequences for patients at any stage of life.[2]Â 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 percent at 5 years.[7]Â In these patients, mortality is primarily due to respiratory failure.[2,6,8]Â In patients surviving and those with juvenile-onset HPP, 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®Â (asfotase alfa)

Strensiq® (asfotase alfa) is a highly innovative bone-targeted enzyme replacement therapy that treats the underlying cause of HPP by replacing the missing TNSALP enzyme. In clinical studies of patients with HPP who had their first symptom prior to the age of 18, treatment with Strensiq improved overall survival in infants, enhanced bone mineralization and improved height, weight and mobility.

Strensiq is approved in the United States, European Union, Japan and Canada.

Important Safety Information

Hypersensitivity reactions have been reported in STRENSIQ-treated patients. In clinical trials, 1 out of 99 treated patients (1%) experienced signs and symptoms consistent with anaphylaxis.

Localized lipodystrophy, including lipoatrophy and lipohypertrophy, has been reported at injection sites after several months in patients treated with STRENSIQ.

Patients with HPP are at increased risk for developing ectopic calcifications. In clinical trials, 14 cases (14%) of ectopic calcification of the eye including the cornea and conjunctiva, and the kidneys (nephrocalcinosis) were reported. There was insufficient information to determine whether or not the reported events were consistent with the disease or due to STRENSIQ. No visual changes or changes in renal function were reported.

The most common adverse reactions reported were injection site reactions, lipodystrophy, ectopic calcifications, and hypersensitivity reactions.

PleaseÂ click here for the full Prescribing Information.

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. As the global leader in complement inhibition, Alexion is strengthening and broadening its portfolio of complement inhibitors, including evaluating potential indications for eculizumab in additional severe and ultra-rare disorders. Alexion's metabolic franchise includes two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare disorders, Strensiq® (asfotase alfa) to treat patients with hypophosphatasia (HPP) and Kanuma™ (sebelipase alfa) to treat patients with lysosomal acid lipase deficiency (LAL-D). In addition, Alexion is advancing the most robust rare disease pipeline in the biotech industry, with highly innovative product candidates in multiple therapeutic areas. This press release and further information about Alexion can be found at: www.alexion.com.

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References

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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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