European Commission Grants Marketing Authorization for Strensiq™ (asfotase alfa) for the Treatment of Patients with Pediatric-Onset Hypophosphatasia (HPP)

– Strensiq is the First Approved Treatment in Europe for Patients Suffering from HPP, a Life-Threatening Ultra-Rare Metabolic Disorder –

CHESHIRE, Conn. — September 1, 2015 — Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) announced today that the European Commission has approved Strensiq™ (asfotase alfa) for long-term enzyme replacement therapy in patients with pediatric-onset hypophosphatasia (HPP) to treat the bone manifestations of the disease. The Summary of Product Characteristics (SmPC) states that HPP is associated with multiple bone manifestations including rickets/osteomalacia, altered calcium and phosphate metabolism, impaired growth and mobility, respiratory compromise that may require ventilation, and vitamin B6-responsive seizures. Strensiq is the first therapy approved in the European Union (EU) for the treatment of patients with HPP, a life-threatening, ultra-rare metabolic disorder. Alexion expects to begin serving patients in Germany in October and is now commencing reimbursement processes with healthcare authorities in each of the major European countries.

“Hypophosphatasia is an extremely rare disorder that can have devastating consequences for patients and families. Without treatment, patients may face significant challenges related to development, growth, and mobility, with an extremely high risk of mortality in infants,” said PD Dr. med Christine Hofmann, Children’s Hospital, University of Würzburg, Pediatric Rheumatology and Osteology Section, Würzburg, Germany. “I am very pleased that patients with pediatric-onset HPP in Europe now have an approved treatment that addresses the underlying cause of their genetic, lifelong metabolic disease by replacing tissue non-specific alkaline phosphatase.”

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]

"The approval of Strensiq in Europe is a significant milestone for patients and their families who now have the first approved treatment for HPP, a devastating and life-threatening disease. We are pleased that the EU label will allow any patient who had symptoms of HPP prior to the age of 18 to be eligible for treatment,” said David Hallal, Chief Executive Officer of Alexion. “We are grateful to the investigators, patients, and their families who participated in the clinical trials that made this approval possible and we are now commencing reimbursement processes with healthcare authorities throughout Europe to ensure

that patients with pediatric-onset HPP have access to Strensiq, a life-transforming treatment, as quickly as possible."

The EC has granted marketing authorization for Strensiq for long-term enzyme replacement therapy in patients with pediatric-onset HPP to treat the bone manifestations of the disease. The SmPC states that HPP is associated with multiple bone manifestations including rickets/osteomalacia, altered calcium and phosphate metabolism, impaired growth and mobility, respiratory compromise that may require ventilation, and vitamin B6-responsive seizures. The natural history of untreated infant hypophosphatasia patients suggests high mortality if ventilation is required. The SmPC also indicates that 71% of infant patients treated with Strensiq who required ventilation support remain alive and continue on treatment.

The EC approval of Strensiq applies to all 28 EU member states as well as Iceland, Norway, and Lichtenstein and follows the June 2015 positive opinion granted by the Committee for Medicinal Products for Human Use (CHMP). Strensiq has also been approved for the treatment of HPP by the Japanese Ministry of Health, Labour and Welfare and by Health Canada. The FDA granted Breakthrough Therapy designation for Strensiq and accepted Alexion's Biologics License Application (BLA) for Priority Review.

Clinical Data

The approval of Strensiq in the EU was based on clinical data from four pivotal prospective studies and their extensions, comprising 68 patients with pediatric-onset HPP (ranging from newborns to 66 years of age). Study results showed that patients with pediatric-onset HPP treated with Strensiq demonstrated rapid and sustained improvements in bone mineralization, as measured by the Radiographic Global Impression of Change (RGI-C) scale, which evaluates the severity of rickets based on X-ray images. Patients in the clinical studies also had improvements in skeletal structure, as demonstrated by x-ray appearance of joints, by histological appearance of bone biopsy material, and by apparent catch-up height-gain.

The most common adverse reactions observed in clinical studies were injection site reactions and injection-associated adverse reactions. Most of these reactions were non-serious, mild to moderate in intensity.

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, profound muscle weakness, seizures, respiratory failure and premature death.[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 traditionally 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.

Strensiq is approved in Japan as a treatment for patients with HPP, and a Biologics License Application for Strensiq has been accepted for priority review by the U.S. Food and Drug Administration (FDA).

Important Safety Information

Severe allergic-type hypersensitivity reactions are possible in patients treated with Strensiq, including urticaria, difficulty breathing and/or cardiovascular collapse. Administration of Strensiq may result in local injection site reactions.

Craniosynostosis have been reported in patients less than 5 years of age. Ophthalmic (conjunctival and corneal) calcification and nephrocalcinosis have been reported in patients treated with Strensiq. There are insufficient data to establish a causal relationship between exposure to Strensiq and progression of craniosynostosis or between exposure to Strensiq and ectopic calcification.

Serum parathyroid hormone concentration may increase in patients administered Strensiq. Patients taking Strensiq may display disproportionate weight increase.

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 (LAL-D), 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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Forward Looking Statement

This news release contains forward-looking statements, including statements related to potential medical benefits of Strensiq™ (asfotase alfa) for hypophosphatasia (HPP). Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example,

decisions of regulatory authorities regarding marketing approval or material limitations on the marketing of Strensiq for HPP, delays in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure for Strensiq for HPP, the possibility that results of clinical trials are not predictive of safety and efficacy results of Strensiq in broader or different patient populations, the risk that third party payors (including governmental agencies) will not reimburse for the use of Strensiq at acceptable rates or at all, the risk that estimates regarding the number of patients with Strensiq and observations regarding the natural history of patients with Strensiq are inaccurate, and a variety of other risks set forth from time to time in Alexion's filings with the Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended June 30, 2015. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

References

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