Health Canada Approves Strensiq™ (asfotase alfa), the First Therapy for Patients with Hypophosphatasia (HPP), an Ultra-rare, Life-threatening Disease

VAUGHAN, ONTARIO, August 17, 2015 -- Alexion Pharma Canada, a subsidiary of Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN), today announced that Health Canada has approved Strensiq™ (asfotase alfa) as enzyme replacement therapy for patients with confirmed diagnosis of paediatric-onset hypophosphatasia (HPP). Strensiq is the first approved treatment for HPP, an ultra-rare, genetic, metabolic disease in which patients experience devastating, progressive effects on multiple organs of the body, leading to debilitating morbidities or pre-mature mortality.

"Strensiq is critical and pivotal for the treatment of patients with HPP who to date have not had an effective therapy. HPP is a rare and extremely debilitating disease. It has a very high mortality rate in the most severe paediatric cases," said Dr. Cheryl Rockman-Greenberg, Distinguished Professor, Department of Pediatrics and Child Health, University Of Manitoba and Clinician Scientist in the Children's Hospital Research Institute of Manitoba, and lead Canadian investigator in the HPP trials. "In clinical studies to date, Strensiq has greatly reduced mortality in affected infants with HPP with 89 per cent overall survival after three years of treatment compared with almost 100 per cent mortality documented in the past. As well, in clinical studies to date, Strensiq has greatly reduced pain associated with HPP and improved physical functioning of patients, including healing of the 'rickets' and improvement in mobility such as increased speed of walking, jumping, endurance and balance. It is very exciting to now have an approved therapy for patients who previously only faced early death, or had to live with severe disability and pain."

The approval of Strensiq for patients with paediatric-onset HPP results from a series of medical advances in Canada. "HPP was first correctly diagnosed in Canada, the asfotase alfa molecule was invented here, and the first patient to ever receive asfotase alfa was treated at the University of Manitoba in Winnipeg. In addition, although HPP is very rare, we recognize that the incidence of the severe form of the disease in the Mennonite population in Manitoba is higher than in other studied populations," said Dr. Philippe Crine, Ph.D. and inventor of asfotase alfa. "It is very gratifying that this important medical breakthrough can now benefit patients and families suffering with HPP in Canada."

The marketing of Strensiq was authorized under Health Canada's Notice of Compliance with Conditions (NOC/c) policy. Approvals under the NOC/c policy are granted to products that, among other factors, are intended for the treatment of a life-threatening or severely debilitating illness, have promising evidence of clinical effectiveness and an acceptable safety profile, and respond to a serious unmet

medical need in Canada. Under this NOC/c approval, Alexion will complete and submit additional data from existing trials and one additional study with Strensiq.

"Health Canada's approval of Strensiq represents a very important milestone in Alexion's mission to provide transformative therapies for patients with severe and rare diseases," said John Haslam, General Manager of Alexion Pharma Canada. "However, the approval is not the final step required in ensuring that patients can receive Strensiq. We look forward to the Provinces and private insurers in Canada providing access for the very few patients with HPP as rapidly as possible."

Strensiq recently received marketing approval for patients with HPP in Japan from the Ministry of Health, Labour and Welfare (MHLW). Alexion has submitted a Biologics License Application for Strensiq with the U.S. Food and Drug Administration (FDA), which was accepted for priority review, and received a positive CHMP opinion recommending marketing authorization by the European Commission for Strensiq for patients with paediatric-onset HPP.

Clinical Data

Health Canada's review and approval of Strensiq is supported by clinical data from four clinical studies and their extensions, as well as a retrospective natural history study in infants. The studies comprised 71 patients, including nearly 20 patients from Canada. Study results showed that patients with infantile-onset HPP (ages ≤5 years at enrollment) 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 with juvenile-onset HPP treated with Strensiq demonstrated superior improvements in bone health compared to a control group of HPP patients selected from a natural history database, as well as improvements in ambulation, physical function and growth. The most common adverse reactions observed in studies were injection site reactions and injection-associated adverse reactions. Most of these reactions were non-serious and mild to moderate in intensity.

About 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.[1-5]

HPP is caused by mutations in the gene encoding an enzyme known as tissue non-specific alkaline phosphatase (TNSALP).[1,2] 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, 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.[1] In a natural history study, infants who had their first symptom of HPP within the first six months of life had high mortality, with an overall mortality rate of 73 per cent at five years.[6] In these patients, mortality is primarily due to respiratory failure.[1,5,7] 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.[1,4]

About Strensiq™ (asfotase alfa)

Strensiq™ (asfotase alfa) is a first-in-class bone-targeted 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 patient morbidity and premature death.

About Alexion Pharmaceuticals, Inc.

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 with the development of Strensiq™ (asfotase alfa) for hypophosphatasia (HPP) and an additional therapy for 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. As the global leader in complement inhibition, Alexion 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.

Safe Harbor 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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