July 24, 2014

European Medicines Agency Accepts Marketing Authorization Application for Asfotase Alfa as a Treatment for Patients with Hypophosphatasia

-- Application designated for review under accelerated assessment process --

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that the Marketing Authorization Application (MAA) for asfotase alfa, an investigational, first-in-class targeted enzyme replacement therapy for the treatment of hypophosphatasia (HPP), has been validated and granted accelerated assessment by the European Medicines Agency (EMA). The acceptance of this MAA marks the beginning of the review process in the European Union (EU) for this potential new treatment.

"HPP is a devastating disease for patients and their families due to progressive deterioration of bones and muscle weakness, which can result in impaired respiratory function, severe disability and death," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "If approved, asfotase alfa would be the first therapy for patients with this life-threatening disorder."

The EU filing includes positive data from 68 patients with pediatric-onset HPP (ranging from newborns to 66 years of age) enrolled in three pivotal prospective studies and their extensions, as well as a retrospective natural history study in infants.

In April, Alexion initiated the rolling submission of a Biologics License Application (BLA) for asfotase alfa as a treatment for patients with HPP with the U.S. Food and Drug Administration (FDA).

About Hypophosphatasia (HPP)

Hypophosphatasia (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.[1-6]

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 classified by the age of the patient at the onset of symptoms of the disease, and pediatric-onset HPP is defined as patients whose first signs or symptoms of HPP occurred prior to 18 years of age.[1]

HPP can have devastating consequences for patients at any stage of life.[1] Pediatric patients with HPP have a high mortality rate, with 73% mortality reported in a natural history study at 5 years.[6] In these patients, mortality is primarily due to respiratory failure.[1,5,6] In patients surviving to adolescence and adulthood, long-term clinical sequelae include recurrent and non-healing fractures, debilitating weakness, severe pain, and the requirement for ambulatory assistive devices such as wheelchairs, wheeled walkers and canes.[1,4]

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.

In 2013, the U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy designation for asfotase alfa. 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.[7]

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 paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (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 nearly 40 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, including asfotase alfa, across multiple therapeutic areas. This press release and further information about Alexion can be found at: www.alexionpharma.com.

[ALXN-G]

Safe Harbor Statement

This news release contains forward-looking statements, including statements related to potential medical benefits of 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 asfotase alfa for HPP, delays in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure for asfotase alfa for HPP, the possibility that results of clinical trials are not predictive of safety and efficacy results of asfotase alfa in broader or different patient populations, the risk that third party payors (including governmental agencies) will not reimburse for the use of asfotase alfa (if approved) at acceptable rates or at all, the risk that estimates regarding the number of patients with asfotase alfa and observations regarding the natural history of patients with asfotase alfa 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 March 31, 2014. 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

1. Rockman-Greenberg C. Hypophosphatasia. *Pediatr Endocrinol Rev*. 2013; 10(suppl 2):380-388.

2. 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.

3. Whyte MP, Greenberg CR, Salman N, et al. Enzyme-replacement therapy in life-threatening hypophosphatasia. *N Engl J Med*. 2012; 366(10):904-913.

4. Seshia SS, Derbyshire G, Haworth JC, Hoogstraten J. Myopathy with hypophosphatasia. *Arch Dis Child*. 1990; 65(1):130-131.

5. 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.

6. 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.

7. Public Law 112-144. U.S. Government Printing Office, July 9, 2012. http://www.gpo.gov/fdsys/pkg/PLAW-112publ144/pdf/PLAW-112publ144.pdf.

Alexion Pharmaceuticals:
Media
Irving Adler, 203-271-8210
Executive Director, Corporate Communications
or
Kim Diamond, 203-439-9600
Senior Director, Corporate Communications
or
Investors
Elena Ridloff, 203-699-7722
Executive Director, Investor Relations

Source: Alexion Pharmaceuticals, Inc.