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FDA Grants Priority Review for Asfotase Alfa as a Treatment for Patients with Hypophosphatasia

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq:ALXN) announced today that the U.S. Food and Drug Administration (FDA) has accepted for Priority Review the Company's Biologics License Application (BLA) for asfotase alfa, an investigational, first-in-class enzyme replacement therapy for treatment of patients with infantile- and juvenile-onset hypophosphatasia (HPP). The BLA submission is supported by data from 71 treated patients with HPP enrolled in three prospective studies and their extensions, as well as two retrospective natural history studies.

"If approved, asfotase alfa will be the first treatment for patients with HPP, a devastating disease that can result in impaired respiratory function, severe disability and premature death for some patients," said Leonard Bell, M.D., Chairman and Chief Executive Officer of Alexion. "The FDA's acceptance of our BLA for Priority Review is a significant step toward bringing this highly innovative and much-needed potential treatment to patients in the United States suffering from HPP."

In May 2013, the FDA granted Breakthrough Therapy designation for asfotase alfa and in April 2014, Alexion initiated the rolling submission of the BLA. 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. Priority Review designation is given to drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. Alexion has also submitted a Marketing Authorization Application for asfotase alfa with the European Medicines Agency and has submitted a New Drug Application for asfotase alfa to Japan's Ministry of Health, Labour and Welfare.

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.[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 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 6 months of life had high mortality, with an overall mortality rate of 73% at 5 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 Asfotase Alfa

Asfotase alfa is an investigational, highly innovative, first-in-class enzyme replacement therapy. Asfotase alfa is designed to address the underlying cause of HPP by aiming to restore the genetically defective metabolic process, thereby preventing or reversing the severe and potentially life-threatening complications of life-long dysregulated mineral metabolism.

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.alexion.com.

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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 Annual Report on Form 10-K for the period ended December 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

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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. 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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