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FDA Grants Breakthrough Therapy Designation to Asfotase Alfa for Perinatal-, Infantile- and Juvenile-Onset Hypophosphatasia (HPP)

LAUSANNE, Switzerland--(BUSINESS WIRE)-- Alexion Pharma International Sàrl, a subsidiary of Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN), today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to asfotase alfa for the treatment of patients with hypophosphatasia (HPP) whose first signs or symptoms occurred prior to 18 years of age, including perinatal-, infantile-, and juvenile-onset forms of the disease. HPP is an inherited, life-threatening, ultra-rare metabolic disorder that leads to progressive damage to multiple vital organs, including destruction and deformity of bones.

The FDA also confirmed that adult-onset HPP is "a serious and life threatening disease or condition" and that Breakthrough Therapy designation could be obtained for this aspect of the disease with additional clinical information.

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.[1]

"The FDA's Breakthrough Therapy designation for perinatal-, infantile- and juvenile-onset HPP recognizes the severe, debilitating and life-threatening nature of the disease, the clear unmet medical need of patients, and the clinical evidence collected to date on asfotase alfa," said Martin Mackay, Ph.D., Executive Vice President, Global Head of R&D at Alexion. "Asfotase alfa is a highly innovative therapeutic candidate with the potential to transform the lives of patients with HPP who currently have no treatment options and often receive only palliative care for this life-threatening disease."

Alexion looks forward to working closely with the FDA and obtaining FDA guidance on the subsequent development of asfotase alfa for the treatment of HPP, including obtaining advice on generating evidence needed to support approval of the drug in an efficient manner. Clinical studies of asfotase alfa are ongoing for patients with HPP whose first signs or symptoms occurred prior to 18 years of age, including perinatal-, infantile- and juvenile-onset forms of HPP.

About Hypophosphatasia (HPP)

HPP is a life-threatening, genetic, and ultra-rare metabolic disease characterized by defective bone mineralization and impaired phosphate and calcium regulation that can lead to progressive damage to multiple vital organs including destruction and deformity of bones, profound muscle weakness, seizures, impaired renal function, and respiratory failure.[2-5]

HPP is caused by a genetic deficiency of an enzyme known as tissue non-specific alkaline phosphatase (TNSALP), which causes life-long abnormalities in metabolism of two minerals, calcium and phosphate, leading directly to the debilitating morbidities and premature mortality of the disease.[2]

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 the disease. Patients with perinatal-onset HPP manifest their first signs of disease in utero or at birth. This form of the disease is usually lethal and often leads to death in-utero. Those patients who survive birth often have severely compromised respiratory function. [6]

Patients with infantile-onset HPP develop their first signs or symptoms of HPP before 6 months of age. Individuals with this form of disease develop skeletal abnormalities and may present with failure to thrive and respiratory failure within the first 6 months of post-natal life. The prognosis of these patients is very poor with mortality estimated at 50%.[2]

Patients with juvenile-onset HPP exhibit their first signs or symptoms of HPP after 6 months of age and before 18 years of age. Individuals with this form of the disease are at risk for respiratory complications, painful fractures and can have delayed acquisition of age-appropriate motor skills due to hypo-mineralization and muscle weakness leading to need for walking assistance; some may never walk.[2]

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 life-threatening complications of life-long dysregulated mineral metabolism.

About Alexion

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company focused on serving patients with severe and ultra-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 more than 35 countries for the treatment of PNH, and in the United States and the European Union for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris and is developing four other highly innovative biotechnology product candidates, including asfotase alfa. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.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 Quarterly Report on Form 10-Q for the period ended March 31, 2013. 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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