September 8, 2014

Asfotase Alfa Granted Orphan Drug Designation in Japan

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that Japan's Ministry of Health, Labour and Welfare (MHLW) has granted orphan drug designation (ODD) to asfotase alfa for the treatment of patients with hypophosphatasia (HPP), a genetic, chronic and progressive ultra-rare metabolic disease characterized by defective bone mineralization. Patients with HPP can experience a range of devastating consequences, including the destruction and deformity of bones, profound muscle weakness, seizures, respiratory failure and premature death.[1-5] Asfotase alfa is an investigational, highly innovative, first-in-class targeted enzyme replacement therapy designed to address the underlying cause of HPP.

"HPP is a devastating disease for patients and families and there are currently no approved treatment options," said Martin Mackay, Ph.D., Executive Vice President, Global Head of R&D at Alexion. "The orphan drug designation granted for asfotase alfa undescores the significant need for an effective treatment option for Japanese patients suffering from this severe, ultra-rare disorder."

The Ministry of Health, Labour and Welfare, based on the opinion of the Pharmaceutical Affairs and Food Sanitation Council, grants orphan status to drugs and medical devices that treat serious diseases of high unmet medical need that affect fewer than 50,000 patients in Japan. Orphan drug designation provides drug developers with certain benefits and incentives, including priority review for marketing authorization and a period of 10 years of market exclusivity if regulatory approval is received for the designated indication.

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.[1-5]

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, and pediatric-onset HPP is defined as first symptom 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; mortality in these patients is primarily due to respiratory failure.[1,5] 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.

In April 2014, Alexion initiated the rolling submission of a Biologics License Application (BLA) for asfotase alfa as a treatment for patients with HPP with the FDA. In July 2014, the Marketing Authorization Application (MAA) for asfotase alfa was validated and granted accelerated assessment by the European Medicines Agency (EMA).

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.

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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 June 30, 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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