

Treatment for rare metabolic disorder approved in Japan

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NASDAQ-listed Alexion Pharmaceuticals Inc. has announced that Japan's Ministry of Health, Labour and Welfare (MHLW) approved the company's New Drug Application (NDA) for the use of Strensiq (asfotase alfa) as a treatment for patients in Japan with hypophosphatasia (HPP), a life-threatening, ultra-rare metabolic disorder.

Strensiq, a bone-targeted enzyme replacement therapy, is said to be the first therapy approved in Japan for the treatment of patients with HPP. Alexion expects that initial patients with HPP in Japan will start commercial treatment with Strensiq by late Q3 2015.

HPP is a genetic, progressive, ultra-rare metabolic disease in which patients experience devastating effects on multiple systems of the body, leading to debilitating or life-threatening complications. It is characterized by defective bone mineralization that can lead to deformity of bones and other skeletal abnormalities, as well as systemic complications such as profound muscle weakness, seizures, pain, and respiratory failure leading to premature death in infants. As reflected in the prescribing information in Japan, infants with HPP treated with Strensiq had 84% overall survival, as estimated by Kaplan-Meier analysis, at 168 weeks.

Strensiq has been granted orphan drug designation by the U.S. Food and Drug Administration (FDA), the European Medicines Agency (EMA), and the Japanese Ministry of Health, Labour and Welfare (MHLW). Alexion has submitted a Biologics License Application for Strensiq with the U.S. Food and Drug Administration, which was accepted for priority review,

and a Marketing Authorization Application for Strensiq in Europe is under review.