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28 October 2014 | News | By BioSpectrum Bureau

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Singapore: Alexion pharma has announced the submission of New Drug Application (NDA) to the Japanese Ministry of Health, Labour and Welfare (MHLW), for asfotase alfa, an integrated enzyme therapy for the treatment of hypophosphatasia.

The disease is an ultra-rare metabolic disorder that can lead to destruction and deformity of bones, profound muscle weakness, seizures, respiratory failure and premature death.

Mr Leonard Bell, MD, chairman and chief executive officer, Alexion, said, "Hypophosphatasia is a rare disease in Japan and a devastating, life-threatening disorder with no treatment so far. The NDA submission for asfotase alfa is a critical step in bringing this highly innovative and much-needed potential treatment to the patients in Japan."

He added that upon approval, the drug would be the first therapy to patients suffering from this rare genetic disorder. The MHLW submission includes positive data from 71 patients with the disease in the age group ranging from newborns to 66 years, including Japanese patients, enrolled in three pivotal prospective studies and their extensions, as well as a retrospective natural history study in infants.

The health agency had recently granted orphan drug designation to asfotase alfa. This will fast track drug approval and also earn in many incentives for drug development and marketing. If approved, the drug will enjoy ten years of market exclusivity in Japan.