September 4, 2015

Alexion Receives Notification of PDUFA Date Extension for Kanuma™ (sebelipase alfa)

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) announced today that the U.S. Food and Drug Administration (FDA) has extended the Prescription Drug User Fee Act (PDUFA) date for its Priority Review of the Company's Biologics License Application (BLA) for Kanuma™ (sebelipase alfa), an investigational enzyme replacement therapy for the treatment of lysosomal acid lipase deficiency (LAL-D). The previously disclosed September 8, 2015 PDUFA date has been extended by the standard extension period of three months.

In response to a recent request from the FDA, Alexion submitted additional Chemistry, Manufacturing and Controls (CMC) information. Due to the timing of this submission, the FDA extended the PDUFA date to allow additional time for review of the new information. The FDA has not asked for additional clinical data.

On September 1, 2015, Alexion announced that the European Commission approved Kanuma for the treatment of patients of all ages with LAL-D. The FDA granted Breakthrough Therapy designation for Kanuma for LAL Deficiency presenting in infants and accepted the Kanuma BLA for Priority Review. In addition, a New Drug Application for Kanuma has been submitted to Japan's Ministry of Health, Labour and Welfare.

Alexion is reiterating its 2015 financial guidance announced on July 30, 2015.

About Lysosomal Acid Lipase Deficiency (LAL-D)

LAL-D is a genetic, chronic and progressive ultra-rare metabolic disease associated with devastating morbidities and premature mortality. In patients with LAL-D, genetic mutations result in decreased activity of the LAL enzyme. This leads to marked accumulation of cholesteryl esters and triglycerides in vital organs, blood vessels, and other tissues, resulting in progressive and multi-organ damage including fibrosis, cirrhosis, liver failure, accelerated atherosclerosis, cardiovascular disease, and other devastating consequences.[1,2]

LAL-D affects patients of all ages with clinical manifestations from infancy through adulthood and may have sudden and unpredictable clinical complications. Infants experience profound growth failure, liver fibrosis, and cirrhosis with a median age of death at 3.7 months.[3] In an observational study, approximately 50% of children and adults with LAL-D progressed to fibrosis, cirrhosis, or liver transplant in 3 years.[4] The median age of onset of LAL-D is 5.8 years and the disease can be diagnosed with a simple blood test.[5,6]

About Kanuma™ (sebelipase alfa)

Kanuma™ (sebelipase alfa) is an innovative enzyme replacement therapy designed to address the underlying cause of lysosomal acid lipase deficiency (LAL-D) by aiming to reduce substrate accumulation in the lysosomes of cells throughout the body, including the liver, to prevent vital organ damage and premature death.

About Alexion

Alexion is a global biopharmaceutical company focused on developing and delivering life-transforming therapies for patients with devastating and rare disorders. Alexion developed and commercializes Soliris® (eculizumab), the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), two life-threatening ultra-rare disorders. Alexion is also establishing a premier global metabolic rare disease franchise, which includes Kanuma™ (sebelipase alfa) for patients with lysosomal acid lipase deficiency (LAL-D), and Strensiq™ (asfotase alfa) for patients with hypophosphatasia (HPP). In addition, Alexion is advancing the most robust rare disease pipeline in the biotech industry, with highly innovative product candidates in multiple therapeutic areas. As the global leader in complement inhibition, the Company is strengthening and broadening its portfolio of complement inhibitors across diverse platforms, including evaluating potential indications for Soliris in additional severe and ultra-rare disorders. This press release and further information about Alexion can be found at: www.alexion.com.

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Forward-Looking Statements

This news release contains forward-looking statements, including statements related to potential medical benefits of Kanuma™ (sebelipase alfa) for lysosomal acid lipase deficiency (LAL-D, expectations regarding the FDA regulatory process, the outcome of the FDA's review, and guidance regarding anticipated financial results for 2015. 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 Kanuma for LAL-D, delays in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure for Kanuma for LAL-D, the possibility that results of clinical trials are not predictive of safety and efficacy results of Kanuma in broader or different patient populations, the risk that third party payors (including governmental agencies) will not reimburse for the use of Kanuma at acceptable rates or at all, the risk that estimates regarding the number of patients with Kanuma and observations regarding the natural history of patients with Kanuma 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, 2015. 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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2. Reiner Z, et al. Lysosomal acid lipase deficiency - an under-recognized cause of dyslipidemia and liver dysfunction. Atherosclerosis. 2014;235:21-30. doi:10.1016/j.atherosclerosis.2014.04.003.
3. Jones S, et al. Severe and rapid disease course in the natural history of infants with lysosomal acid lipase deficiency. Mol Genet Metab. 2014 Feb;111(2):S57-58.
4. Data on file. Based on modelling using the subset of 31 patients (≥ 5 years) in Natural History Study LAL2-NH01 who had a liver biopsy performed during their medical care plus 1 patient without a biopsy who received a liver transplant; An important source of selection bias in this analysis is that patients who were selected by their clinician for liver biopsy would be expected to have more evidence of disease progression than the overall population of patients with CESD.
5. Burton B, et al. Clinical Features of Lysosomal Acid Lipase Deficiency - a Longitudinal Assessment of 48 Children and Adults. J Pediatr Gastroenterol Nutr. 2015 August 6. doi: 10.1097/MPG.0000000000000935
6. Hamilton J, et al. A new method for the measurement of lysosomal acid lipase in dried blood spots using the inhibitor Lalistat 2. Clin Chim Acta. 2012;413:1207-10. doi:10.1016/j.cca.2012.03.019.

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