December 8, 2015

Kanuma Approved for Lysosomal Acid Lipase Deficiency

On Dec. 8, 2015, Alexion Pharmaceuticals received approval from the U.S. Food and Drug Administration (FDA) for Kanuma™ (sebelipase alfa) for the treatment of patients with lysosomal acid lipase (LAL) deficiency. Kanuma is administered as a once-weekly intravenous infusion in patients with rapidly progressing LAL deficiency presenting within the first six months of life, and once every other week in all other patients. Alexion plans on launching Kanuma the first week of January 2016. It will be available through a limited network of specialty pharmacies that does not include Accredo. Full prescribing information can be found at: http://alexion.com/Documents/Kanuma-USPI

At a Glance

- **Brand (Generic) Name:** Kanuma™ (sebelipase alfa)
- **Manufacturer:** Alexion Pharmaceuticals
- **Date Approved:** Dec. 8, 2015
- **Indication:** Treatment of patients with LAL deficiency
- **Dosage Forms Available:** 20mg/10mL solution in single-dose vials
- **Launch Date:** First week of January 2016
- **Estimated Annual Cost:** Pricing information is not yet available.
- **Specialty Status:** Kanuma will be added to Express Scripts’ specialty drug list.
- LAL deficiency is a rare, progressive metabolic disorder that is caused from a genetic mutation that results in little or no LAL enzyme activity. It can lead to premature death and multi-organ damage including liver failure and cardiovascular disease. It affects less than 20 patients per one million of the general population.
- Kanuma is a hydrolytic lysosomal cholesteryl ester and triacylglycerol-specific enzyme replacement therapy. It is the first drug approved to treat LAL deficiency.
- Kanuma was approved under FDA’s breakthrough therapy, orphan drug and priority review programs. In clinical studies, Kanuma improved survival in infants with LAL deficiency and improved several markers of liver and lipid abnormalities in children and adults with LAL deficiency.