Kanuma approved for rare missing-enzyme disease

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(HealthDay)—Kanuma (sebelipase alfa) has been approved by the U.S. Food and Drug Administration as the first treatment for a rare but often-deadly disease caused by an enzyme deficiency.

People with Lal deficiency, also known as Wolman disease or cholesteryl ester storage disease, produce little or none of the enzyme lysosomal acid lipase (Lal). This causes fat buildup in the body that can lead to life-threatening liver or heart problems. The disease, affecting up to two people per million births, is commonly diagnosed during the first two-to-four months of infancy, the FDA said in a news release.

Life expectancy depends on the disease's severity and its complications, the agency said.

Kanuma is human Lal that's produced by genetically modified chickens in their egg whites. Neither the chicken or its eggs are allowed in the food supply, the FDA said.

The injected drug is given once weekly or once every other week. Of nine infant clinical trial participants treated with Kanuma, six were alive at 12 months of age, compared with no surviving infants among the 21 given a placebo, the FDA said.

The most common human side effects of the drug included diarrhea, vomiting, fever, nasal inflammation, anemia, cough, headache, constipation and nausea. "No adverse outcomes were noted in the

chickens," the agency said.

Kanuma is produced by Cheshire, Conn.-based Alexion Pharmaceuticals.

More information: Learn more about this approval from the FDA.