

Lysosomal Acid Lipase Deficiency Treatment Market Size, Share & Trends Analysis Report By Indication (Wolman Disease, Cholesteryl Ester Storage Disease), By Treatment, By Region, And Segment Forecasts, 2025 - 2030

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Abstracts

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Lysosomal Acid Lipase Deficiency Treatment Market Growth & Trends

The global lysosomal acid lipase deficiency treatment market size is expected to reach USD 433.2 million by 2030, registering a CAGR of 7.2% from 2025 to 2030, according to a new report by Grand View Research, Inc. High unmet needs, rising prevalence, and increasing uptake of enzyme replacement therapy are some of the primary factors driving the market.

LAL deficiency (LAL-D) is a rare type of lysosomal storage disorder in which patients are unable to breakdown cholesteryl esters and triglycerides due to a mutation in the LAL gene. As a result, there is an accumulation of lipids in the liver, spleen, and walls of blood vessels. LAL deficiency is a progressive disease with multi-systemic clinical manifestations, significant medical complications, and early mortality due to liver failure, especially among infants.

Until 2015, the global LAL deficiency treatment market witnessed modest growth due to a lack of approved treatments for the disease. Medical care was limited to supportive care, with surgery and lipid-modifying drugs such as statins remaining the mainstay for treatment. In 2015, approval of Alexion Pharma's Kanuma (sebelipase alfa) marked the entry of enzyme replacement therapy (ERT) in the LAL-D space. Kanuma is a hydrolytic

lysosomal cholesterol ester and triacylglycerol specific enzyme, administered intravenously in patients with LAL deficiency. Currently, Kanuma is the only drug approved for both Wolman disease (WD) and Cholesteryl Ester Storage Disease (CESD) in the key markets.

Based on treatment, supportive care was the leading revenue contributor in the market in 2017. However, the ERT segment is expected to register the fastest growth during the forecast period, supported by continued uptake of Kanuma. Disease prognosis remains poor, as a majority of patients are either undiagnosed or misdiagnosed. Thus, there remains a strong need for extensive research to understand disease biology and develop effective therapeutic options with the goal to overcome resistance and minimize toxicity.

Lysosomal Acid Lipase Deficiency Treatment Market Report Highlights

CESD manifests later in life and is more prevalent among Caucasians and Hispanic populations, affecting approximately 1 in 300,000 individuals.

Germany is at the forefront of the Europe market and is driven by high disease prevalence and availability of drugs in the ERT class.

Approval of Kanuma in ERT class highlights a fundamental shift in treatment approach for LAL-D deficiency.

The ERT segment is estimated to be the fastest-growing during the forecast period, owing to Kanuma's first-mover advantage as the only long-term treatment in the untapped LAL-D patient population pool.

Statins are anticipated to lose market share due to rising competition from generics within the class and increasing uptake of Alexion's Kanuma.

Rising eligible patient population and limited therapeutic intervention for LAL deficiency offer a strong commercial opportunity for players to invest in developing novel treatments in this market.

Lack of awareness in disease diagnosis and delayed access to medications due to poor prognosis may restrain the growth of the market.

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