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U.S. Lysosomal Storage Disease Treatment Market Size, Share & Trends Analysis Report By Type (Enzyme Replacement Therapy (ERT)), By Disease Type, By Country, And Segment Forecasts, 2025 - 2033

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Abstracts

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U.S. Lysosomal Storage Disease Treatment Market Summary

The U.S. lysosomal storage disease treatment market size was estimated at USD 1.41 billion in 2024 and is projected to reach USD 1.99 billion by 2033, growing at a CAGR of 4.10% from 2025 to 2033. The rising prevalence of rare genetic disorders, better diagnostic capabilities, and broader access to enzyme replacement and gene-based therapies drive this growth. In May 2025, the FDA accepted REGENXBIO's BLA for RGX-121, a gene therapy for MPS II, showing 85% CSF heparan sulfate reduction and potential to replace long-term ERT. Increased awareness has led to earlier intervention and improved outcomes. Advancements in therapy design and strong industry-academic collaborations continue to drive innovation, supported by FDA incentives for orphan drug development.

One of the key drivers is the rapid evolution of gene therapy as a transformative approach for treating LSDs. Unlike enzyme replacement therapies, gene therapies target the root genetic cause, offering potential long-term or permanent benefits. In the U.S., several candidates for conditions such as MLD, Sanfilippo syndrome, and Gaucher disease are advancing through clinical stages. In March 2024, the FDA approved Lenmeldy (atidarsagene autotemcel), the first gene therapy in the U.S. for early-stage MLD, showing 100% survival at age 6 in treated presymptomatic patients

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versus 58% in untreated cases. Such milestones are increasing investment, accelerating adoption, and reshaping long-term LSD management.

Another major driver is the advancement of newborn screening programs across the U.S., which has led to earlier diagnosis of various LSDs. States are progressively expanding their recommended panels under the RUSP to include conditions such as Pompe disease and MPS I. Early detection enables the timely initiation of treatment, significantly improving long-term disease outcomes. For instance, in July 2024, Florida Newborn Screening added MPS II to its statewide panel using tandem mass spectrometry, enabling early diagnosis and clinical referral. As more states implement such measures, diagnosed cases are expected to rise. This trend supports proactive management and aligns industry strategies with early therapeutic intervention.

U.S. Lysosomal Storage Disease (LSDs) Treatment Market Report Segmentation

This report forecasts revenue growth at regional levels and provides an analysis of the latest industry trends in each of the sub-segments from 2021 to 2033. For this study, Grand View Research has segmented the U.S. lysosomal storage disease treatment market report based on type and disease type:

Type Outlook (Revenue, USD Billion, 2021 - 2033)

Enzyme Replacement Therapy (ERT)

Imiglucerase (Cerezyme)

Alglucosidase alfa (Myozyme/Lumizyme)

Idursulfase (Elaprase)

Velaglucerase alfa

Others

Substrate Reduction Therapy (SRT)

Other Types

Disease Type Outlook (Revenue, USD Billion, 2021 - 2033)

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Gaucher Disease

Fabry Disease

Pompe Disease

Mucopolysaccharidoses (MPS)

Others

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CHAPTER 6. COMPETITIVE LANDSCAPE

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