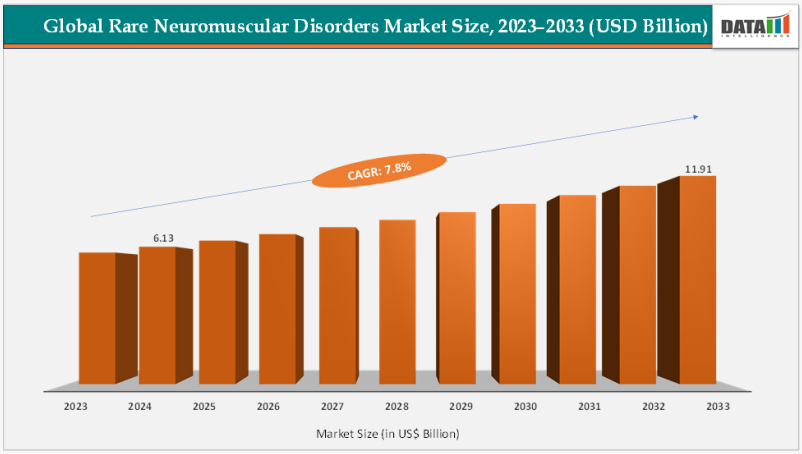
Rare Neuromuscular Disorders Market to Reach USD 11.91 Billion by 2033 at 7.8% CAGR | DataM Intelligence

Global rare neuromuscular disorders market to hit USD 11.91B by 2033 at 7.8% CAGR, driven by gene therapies, RNA innovations, and rising diagnostic rates.



Rare Neuromuscular Disorders Market

LOS ANGELES, CA, UNITED STATES, November 14, 2025 / EINPresswire.com/ -- Global rare neuromuscular disorders market size reached US$ 6.13 Billion in 2024 from US$ 5.72 Billion in 2023 and is expected to reach US$ 11.91 Billion by 2033, growing at a CAGR of 7.8% during the forecast period 2025-2033

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> Breakthroughs in gene and RNA therapies are rapidly transforming outcomes for patients with rare neuromuscular diseases, fueling strong market expansion worldwide."
>
> *DataM Intelligence*

Industry Latest News 2025:

 2025-10-26 — Novartis agrees to acquire Avidity Biosciences (RNA-therapeutics deal to bolster neuromuscular pipeline).

 2025-05-13 — ELEVIDYS (delandistrogene moxeparvovec) approved in Japan for specified pediatric Duchenne patients (market entry).

 2025-07-24 — EMA/CHMP issues a negative opinion on ELEVIDYS' conditional marketing authorisation in the EU (regulatory setback).

 2025-09-23 — U.S. FDA declines approval of a higher-dose submission for Biogen's nusinersen

(Spinraza) high-dose regimen for SMA.

 2025-02-21 — South Korea implements a Regenerative Medicine Law/advanced-therapy pathway to expand patient access to cell & gene therapies for severe/rare diseases.

 2025-09-02 — Arrowhead Pharmaceuticals signs a licensing deal with Novartis (up to $2B) for an RNA-targeting neuromuscular therapy program.

 2025-09-04 — Japanese firm Chugai/Roche implements safety measures and regulatory discussions after reports of acute liver failure in some ELEVIDYS-treated non-ambulatory patients.

Market Geographical Share:

North America holds a significant share of the rare neuromuscular disorders market, driven by strong diagnostic capabilities, high adoption of advanced gene therapies, and growing patient enrollment in clinical trials. The U.S. leads the region with substantial investments in genetic research, newborn screening expansion, and favorable reimbursement pathways for high-cost orphan drugs.

Europe accounts for a major portion of global revenue due to robust regulatory incentives for orphan drug development, well-established rare disease networks, and increased government funding. Countries such as Germany, France, and the U.K. show high therapy uptake supported by structured national rare disease strategies and early-access programs.

Asia-Pacific is emerging as the fastest-growing region, supported by rising disease awareness, improved healthcare access, and growing clinical research in countries like Japan, China, and South Korea. Increasing investment in genetic testing infrastructure and the expansion of local biotech capabilities further accelerate market growth.

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Key Market Drivers:

 Increasing Prevalence of Genetic Mutations & Improved Diagnosis

Advancements in genomic sequencing, biomarker-based testing, and newborn screening programs are enabling earlier and more accurate detection of rare neuromuscular disorders such as SMA, Duchenne muscular dystrophy, and Pompe disease significantly increasing treatment demand.

 Expansion of Gene and Cell Therapies

Innovative therapeutic approaches, including gene replacement therapy, antisense oligonucleotides, and RNA-targeted treatments, are transforming disease management. The approval of novel one-time gene therapies has reshaped the market landscape and encouraged more R&D investment.

 Strong Regulatory Support for Orphan Drugs

Governments worldwide are offering incentives—priority review, tax credits, fast-track approvals, and extended market exclusivity—to encourage the development of treatments for rare neuromuscular diseases. These policies reduce R&D burdens and support pipeline growth.

 Rising Awareness Through Patient Advocacy Organizations

Global patient groups and non-profit foundations are increasing awareness, supporting diagnostic access, and funding clinical research, collectively accelerating therapy uptake and early intervention.

 Growing Investment in Precision Medicine and Biotech R&D

The shift toward personalized therapies, coupled with increased venture capital inflow into neuromuscular disorder research, drives innovation. Advances in CRISPR, mRNA platforms, and viral vector technologies are contributing to a robust therapeutic pipeline.

 Increasing Healthcare Expenditure & Access to Advanced Treatments

Improved insurance coverage, expanded public healthcare programs, and greater availability of neuromuscular specialists are supporting treatment adoption, especially in developed markets.

Segments Covered in the Rare Neuromuscular Disorders Market:

By Disease Type (Spinal Muscular Atrophy (SMA), Duchenne Muscular Dystrophy (DMD), Myasthenia Gravis, Charcot-Marie-Tooth (CMT), and Others)

By Therapeutic Type (Gene Therapy, Antisense Oligonucleotides, Cell Therapy, Disease-Modifying Agents, RNA Therapeutics, and Others)

By Distribution Channel (Hospital Pharmacies, Retail Pharmacies, and Online Pharmacies)

Regional Analysis for Rare Neuromuscular Disorders Market:

 North America (U.S., Canada, Mexico)

 Europe (U.K., Italy, Germany, Russia, France, Spain, The Netherlands and Rest of Europe)

 Asia-Pacific (India, Japan, China, South Korea, Australia, Indonesia Rest of Asia Pacific)

 South America (Colombia, Brazil, Argentina, Rest of South America)

 Middle East & Africa (Saudi Arabia, U.A.E., South Africa, Rest of Middle East & Africa)

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Major Key Players: Biogen, Genentech USA, Inc., Novartis AG, Sarepta Therapeutics, Inc., PTC Therapeutics, Inc., Catalyst Pharmaceuticals, Inc., NMD PHARMA A/S, and ActioBio.

 Biogen — Still a major commercial player via Spinraza (nusinersen); Spinraza generated roughly $1.57 billion in global sales in 2024, but Biogen faces growing competition and regulatory/CMC reviews for higher-dose filings.

 Genentech (Roche) — Owner/developer of Evrysdi (risdiplam) (oral SMA therapy) and an active neuromuscular R&D portfolio; Evrysdi's regulatory approvals and Roche's broad neuromuscular data make Genentech a top competitor in the SMA segment.

 Novartis — A lead commercial gene-therapy player through Zolgensma (onasemnogene abeparvovec) (reported product sales ~1,214 on Novartis product sales table for 2024), and Novartis has amplified its rare-muscle pipeline with large 2025 deals (e.g., Avidity acquisition) — positioning it for substantial share in gene-therapy-driven growth.

 Sarepta Therapeutics — Focused on Duchenne muscular dystrophy (DMD); regulatory wins and expanded approvals for Elevidys have materially increased the company's addressable market and analyst revenue scenarios (Elevidys expansion cited as a potential ~$1B-level opportunity).

 PTC Therapeutics — A meaningful DMD/rare-disease commercial presence via Translarna (ataluren) and related DMD franchise products — Translarna net product revenue reported at ~$340 million in 2024 (DMD franchise ~$547M unaudited for 2024), giving PTC a strong niche share in DMD segments.

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