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North America Duchenne Muscular Dystrophy Treatment Market - 2025-2033

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Abstracts

Overview

The North America duchenne muscular dystrophy treatment market size reached US$ 2.06 billion in 2024 and is expected to reach US$ 8.84 billion by 2033, growing at a CAGR of 20.2% during the forecast period 2025-2033.

Duchenne Muscular Dystrophy (DMD) is a rare, progressive genetic disorder characterized by the degeneration and weakening of muscles over time. It is one of the most common and severe forms of muscular dystrophy. DMD primarily affects young male and is caused by mutations in the dystrophin gene, which is responsible for producing dystrophin, a protein that plays a crucial role in maintaining the integrity of muscle fibers.

North America continues to lead the global DMD drugs market, driven by several key factors. The rising prevalence of DMD and the recently approved new treatments. A well-developed healthcare infrastructure, a strong presence of major pharmaceutical companies, and widespread availability of brand drugs in the US support this demand.

Additionally, the region benefits from high prescription rates and easy access to top-selling drugs, making treatment more accessible to patients. Innovation also plays a crucial role, with the ongoing development of novel drugs offering improved safety and efficacy. There are strong growth opportunities in the development of gene therapies, driven by the urgent need to restore the function of the dystrophin gene. These combined factors firmly position North America at the forefront of the evolving DMD drugs landscape.

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Market Dynamics: Drivers & Restraints

Rising FDA drug approvals are significantly driving the Duchenne muscular dystrophy treatment market growth.

The FDA's Accelerated Approval and Orphan Drug Designation have expedited the availability of life-changing treatments for DMD patients in North America. These regulatory incentives have shortened the approval timelines, allowing new therapies to reach the market more quickly. The rising FDA drug approvals by major market players are driving the growth of the market.

For instance, in March 2024, Italfarmaco S.p.A. announced the U.S. Food and Drug Administration (FDA) approval of Duvyzat (givinostat), a novel histone deacetylase (HDAC) inhibitor, for the treatment of patients 6 years or older with Duchenne muscular dystrophy (DMD), a rare X-linked progressive and life-limiting neuromuscular condition with symptoms from early childhood.

Additionally, in June 2024, Sarepta Therapeutics, Inc. announced U.S. Food and Drug Administration (FDA) approval of an expansion to the labeled indication for ELEVIDYS (delandistrogene moxeparvovec-rokl) to include individuals with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene who are at least 4 years of age. Confirming the functional benefits, the FDA granted traditional approval for ambulatory patients. The FDA granted accelerated approval for non-ambulatory patients. Continued approval for non-ambulatory Duchenne patients may be contingent upon verification of clinical benefit in a confirmatory trial. ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the DMD gene.

In North America, the rising FDA drug approvals are playing a pivotal role in driving the Duchenne Muscular Dystrophy (DMD) treatment market growth. Accelerated approval pathways, the introduction of gene and exon-skipping therapies, and increasing investment in research are significantly expanding treatment options and improving patient outcomes. With the FDA’s continued support for breakthrough therapies and growing access to these treatments, the market is poised for further growth, offering hope for a better quality of life for DMD patients in North America.

High drug costs for DMD treatment are hampering the market growth.

One of the most significant barriers to the growth of the Duchenne muscular dystrophy (DMD) treatment market is the high cost of therapies, particularly in advanced markets

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like North America. While recent innovations, such as gene therapies and targeted exon-skipping treatments, offer hope for improved outcomes, their price tags pose serious challenges for widespread access.

For instance, the FDA-approved gene therapy Elevidys carries a list price of approximately $3.2 million per patient, making it one of the most expensive treatments ever developed. Other DMD drugs, such as Exondys 51 and Vyondys 53, also come with annual costs exceeding $300,000, even though their efficacy may vary depending on the patient's specific genetic mutation.

These high costs strain both public and private healthcare systems and often lead to limited insurance coverage or reimbursement delays, especially for therapies considered to have marginal or uncertain clinical benefits. Many families face substantial out-of-pocket expenses, which can delay or completely prevent treatment initiation. While the DMD treatment landscape is advancing rapidly, the high cost of therapies remains a key market restraint, limiting patient access and posing economic challenges for healthcare systems and families alike.

Segment Analysis

The North America Duchenne muscular dystrophy treatment market is segmented based on treatment type and route of administration.

Treatment Type:

The exon-skipping therapies segment is expected to dominate the Duchenne muscular dystrophy treatment market with the highest market share.

Exon-skipping therapies play a critical role in the treatment of Duchenne Muscular Dystrophy (DMD) by targeting the underlying genetic mutation responsible for the disease. These therapies work by "skipping" over faulty exons in the dystrophin gene, enabling the production of a shorter but functional version of the dystrophin protein. Dystrophin is essential for maintaining the integrity of muscle cells, and its absence or deficiency leads to muscle degeneration in DMD patients. Exon-skipping therapies are revolutionizing DMD treatment by offering a way to correct these genetic mutations at the molecular level.

DMD is caused by mutations in the dystrophin gene, and the mutation type varies among patients. Exon-skipping therapies are designed to target specific exons in the

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gene, depending on the mutation. By skipping over these faulty exons, these therapies allow for the production of a truncated, yet functional, form of dystrophin that helps stabilize muscle cells.

For instance, Eteplirsen (Exondys 51) targets exon 51 of the dystrophin gene and was the first exon-skipping drug approved by the FDA in 2016. This drug helps patients with a specific mutation (deletion of exon 51) produce a shortened version of dystrophin. Golodirsen (Vyondys 53) targets exon 53, and Viltolarsen (Viltepso) targets exon 53 as well, providing options for patients with different mutations in the dystrophin gene.

The approval of several exon-skipping therapies has dramatically expanded the treatment landscape for DMD. Previously, treatment options for DMD were limited primarily to corticosteroids and supportive therapies. Now, exon-skipping therapies provide a more targeted approach to address the root cause of the disease. For instance, Vyondys 53 (Golodirsen), approved in 2019, and Viltepso (Viltolarsen), approved in 2020, offer alternative treatment options for patients with exon 53 mutations, complementing Exondys 51 (Eteplirsen), which targets exon 51.

Competitive Landscape

Top companies in the Duchenne muscular dystrophy treatment market include Sarepta Therapeutics, Inc., ITF Therapeutics LLC, NS Pharma, Inc., Catalyst Pharmaceuticals, Inc., and PTC Therapeutics. Emerging players in the market include F. Hoffmann-La Roche Ltd, Capricor Therapeutics, Inc., REGENXBIO Inc., Solid Biosciences Inc., Wave Life Sciences, Genethon, and among others.

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The North America Duchenne Muscular Dystrophy Treatment market report delivers a detailed analysis with 39 key tables, more than 22 visually impactful figures, and 168 pages of expert insights, providing a complete view of the market landscape.

Target Audience 2024

Manufacturers: Pharmaceutical, Medical Device, Biotech Companies, Contract Manufacturers, Distributors, Hospitals.

Regulatory & Policy: Compliance Officers, Government, Health Economists, Market Access Specialists.

Technology & Innovation: AI/Robotics Providers, R&D Professionals, Clinical

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