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Duchenne muscular dystrophy: market and therapeutic analysis

Abstract

Duchenne muscular dystrophy (DMD) is a severe, hereditary neuromuscular disorder characterized by the absence of the functional dystrophin protein. This deficiency leaves muscle fibers highly vulnerable to contraction-induced damage, triggering a cycle of chronic inflammation and the unregulated deposition of extracellular matrix (ECM). As the disease progresses, healthy tissue is replaced by dense fibrotic scarring, which leads to a loss of mobility and fatal respiratory or cardiac complications. Driven by the critical need to address this pathology, the global DMD treatment market is expanding rapidly, transitioning from traditional symptom-management strategies, such as corticosteroids and physical rehabilitation, to targeted, molecular-based interventions like exon-skipping and gene replacement therapies. Ultimately, integrating advanced biotechnologies, localized delivery mechanisms, and ongoing supportive care will be vital to actively regenerate functional skeletal muscle and extend the quality of life for patients living with DMD.

Keywords: Duchenne muscular dystrophy, dystrophin restoration, cell-based therapies, biomaterial scaffolds, tissue engineering, regenerative medicine

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Abbreviations: DMD, Duchenne muscular dystrophy; ECM, extracellular matrix; ATP, adenosine triphosphate; TGF-β, transforming growth factor-beta; CTGF, connective tissue growth factor; MMPs, matrix metalloproteinases; HUI, Health Utilities Index; CAGR, compound annual growth rate; CRISPR, clustered regularly interspaced short palindromic repeats; EMA, European Medicines Agency; BMD, Becker muscular dystrophy; FDA, Food and Drug Administration; HDAC, histone deacetylase; NSAIDs, nonsteroidal anti-inflammatory drugs; GR, glucocorticoid receptor; MR, mineralocorticoid receptor; DNA, deoxyribonucleic acid; AAV, adeno-associated virus; UC-MSC, umbilical cord mesenchymal stem cell; MSC, mesenchymal stem cell; ADSCs, adipose-derived stem cells; dECM, decellularized extracellular matrix; 3D, three-dimensional; PEO, polyethylene oxide; PCL, polycaprolactone; HUVEC, human umbilical vein endothelial cells; VML, volumetric muscle loss; ACE, angiotensin-converting enzyme; COVID-19, coronavirus disease 2019; DEC, dystrophin-expressing chimeric; PEG, polyethylene glycol

Introduction

Duchenne muscular dystrophy (DMD) is recognized as the most widespread and one of the most severe hereditary neuromuscular diseases, affecting individuals without any predilection for a specific race or ethnic group (Venugopal & Pavlakis, 2023). As an X-linked recessive disorder, it primarily impacts males, with a global prevalence estimated at 4.8 per 100,000 people.[1,2] In the United States specifically, DMD has an estimated incidence of 1 in 3,600 male live-born infants and a prevalence of approximately 2 per 10,000.[1] The disease is characterized by a debilitating clinical progression where patients typically lose the ability to walk by age 12, often leading to fatal respiratory or cardiac complications by their teens or twenties.

At the cellular level, this progression is driven by a lack of the protein dystrophin, which leaves muscle fibers vulnerable to contraction-induced damage, chronic inflammation, and severe fibrosis caused by the unregulated deposition of the extracellular matrix (ECM).[3,4] This progression imposes a staggering economic and societal burden, with total annual costs reaching as high as $120,910 per patient as the disease advances.[5] Driven by a critical need for effective interventions, the global DMD treatment market is experiencing rapid expansion and is projected to reach between $9.59 billion and $27.4 billion by the early 2030s.[6] This growth is further fueled by advancements in molecular-based therapies, which account for over 60% of the market share, and an expansion in treatment windows as patient survival rates climb.

This paper provides a comprehensive analysis of the current market dynamics and the evolving therapeutic landscape, which now encompasses traditional corticosteroids, mutation-specific gene therapies, and emerging tissue engineering approaches.[7-9]

Healthy muscle tissue

There are three types of muscles in the human body: skeletal, cardiac, and smooth muscle. Most of the muscles in the body are skeletal muscles. In a healthy person, skeletal muscle comprises 30-40% of total body mass and is responsible for voluntary movements governed by the somatic nervous system. These striated muscles connect to the skeletal system via tendons and are composed of thousands of flexible muscle fibers. Cardiac and smooth muscle tissues function involuntarily under the regulation of the autonomic nervous system.[10]

To maintain structural integrity and function, muscle fibers are encased in three layers of protective coverings: the epimysium on the outside, the perimysium in the middle, and the endomysium surrounding individual fibers (Figure 1). These fibers are 1-3 inches in diameter and typically stretch across the entire length of the muscle.

When functioning normally, the controlled contraction of these fibers performs vital roles ranging from moving bones and protecting joints to maintaining posture, storing nutrients, sustaining body temperature, and expanding the chest cavity for respiration.

Dystrophin connects the intracellular cytoskeleton of a muscle cell to the surrounding cell membrane and ECM, as seen in Figure 2. It therefore protects the muscle cell from damage that may be caused by muscle contraction.[3]

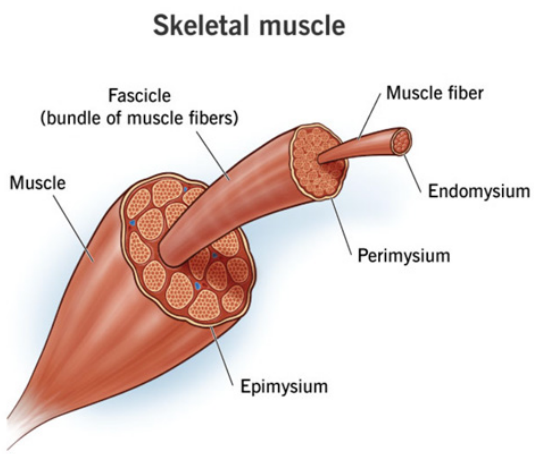

Figure 1 Schematic diagram of healthy skeletal muscle tissue structure.

The image shows the three layers of protective connective tissue coverings, the organization of individual muscle fibers into a fascicle, and the cross-sectional anatomy of a single muscle fiber.

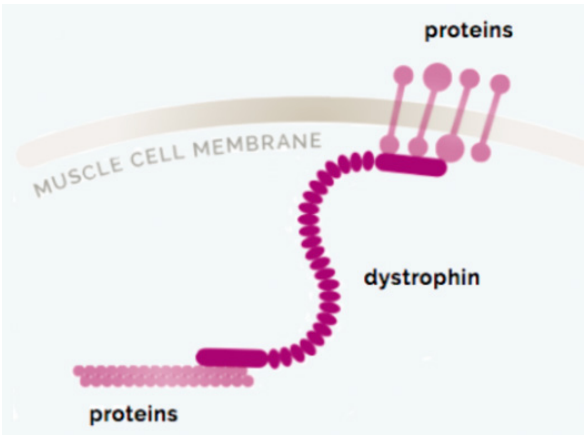

Figure 2 Schematic diagram of dystrophin structural linkage between intracellular and extracellular proteins.

The image shows the dystrophin protein anchoring the intracellular cytoskeleton of a muscle cell to the surrounding cell membrane and ECM, highlighting its role in protecting the muscle cell during contraction.

At the cellular level, the fundamental unit of healthy muscle contraction is the sarcomere, which forms long chains within the myofibrils of each individual muscle fiber. Myofibrils consist of two essential contractile proteins: thick myosin filaments (15 nm in diameter) and thin actin filaments (7 nm in diameter). Healthy muscle contraction is triggered by nerve impulses that release calcium ions from the cell's sarcoplasmic reticulum. This calcium binds to troponin, a protein complex that shifts the position of tropomyosin, unblocking the necessary binding sites between the actin and myosin. Once these sites are exposed, the globular heads of the myosin molecules bind to the actin filaments. Using energy from the hydrolysis of adenosine triphosphate (ATP), the myosin heads undergo a conformational change that pulls the actin filaments toward the center of the sarcomere. This slides the actin and myosin filaments past one another, shortening the sarcomere and resulting in overall muscle contraction without altering the actual length of the individual filaments.[11]

When healthy skeletal muscle sustains damage, it initiates a coordinated regenerative process that occurs in five phases: degeneration, inflammation, regeneration, tissue remodeling, and functional recovery. The process begins when injured myofibers undergo necrosis, releasing intracellular components that act as damage signals to trigger an inflammatory response. During this inflammatory phase, specialized immune cells such as neutrophils and macrophages arrive to the damaged area to clear cellular debris and secrete regulatory cytokines. These signals subsequently activate the regenerative phase, which is driven by muscle stem cells known as satellite cells. These cells are stimulated to multiply and differentiate into myoblasts, aided by supportive signals from other cells like fibro-adipogenic progenitors. As these myoblasts repair the muscle, the tissue enters a remodeling and maturation phase where an ECM scaffold is deposited to guide the formation and fusion of new muscle fibers. The healing process concludes with functional recovery, which ensures the complete re-innervation of the newly formed myofibers and the restoration of their original contractile strength.[12]

Diseased Duchenne muscular dystrophy tissue

Duchenne muscular dystrophy is a genetic disorder where a mutation stops the body from making enough of the structural protein dystrophin, leaving muscle membranes fragile.[4] Most often, sections of the dystrophin gene are missing, causing a mismatch where the left-behind pieces no longer fit together.[3]

A lack of dystrophin leads to constant muscle fiber breakdown and eventual replacement by non-functional fibrous and fatty tissue (Figure 3). DMD pathogenesis involves more than just dystrophin deficiency; it's driven by a dysfunctional cellular environment. Chronic inflammation and fibroblast activity create a cycle that blocks stem cell repair and promotes scarring.

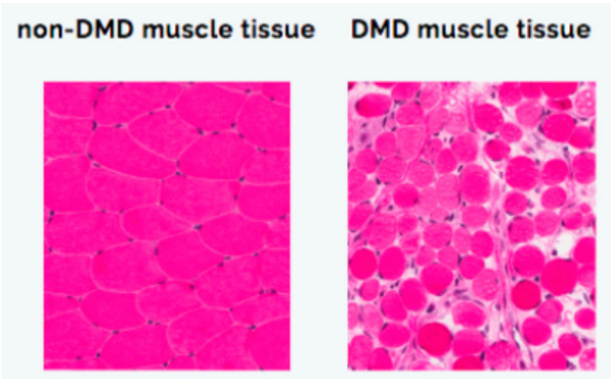

Figure 3 Histological comparison of healthy non-DMD and degenerated DMD muscle tissue.

The image shows the structural differences between normal muscle fiber organization and the pathological changes in Duchenne muscular dystrophy caused by a lack of dystrophin protein, including muscle cell degeneration and replacement by non-functional fibrous and fatty tissue.

Fibrosis is the buildup of excess connective tissue that replaces healthy muscle. While ECM is normally a helpful scaffold for repair, in DMD it becomes unregulated, leading to lost muscle function and shrinking the total volume of viable muscle tissue that can respond to medical intervention. This unregulated ECM buildup is driven by an overproduction of growth factors, particularly transforming growth factor-beta (TGFβ) and Connective Tissue Growth Factor (CTGF), which stimulate cells to produce scar tissue and inhibit myogenesis in DMD.[4]

At the cellular level, specific mesenchymal stem cells (fibro-adipogenic progenitors) hyperproliferate and transform into the fibrotic tissue that replaces healthy muscle. Chronic inflammation brings in M2 macrophages, which actively produce proline—an

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amino acid required to build collagen. Furthermore, the diseased muscle suffers from a severe imbalance in matrix metalloproteinases (MMPs), which facilitate the migration of myogenic cells to damaged tissue by degrading the ECM, and their inhibitors. As a result, the dystrophic muscle is forced to overproduce the fibrous ECM scaffold scaffold while simultaneously losing the ability to properly degrade and clear it away.

Market size

Duchenne muscular dystrophy is the most widespread and one of the most severe hereditary neuromuscular diseases, affecting individuals without any predilection for a specific race or ethnic group.[1] Globally, the prevalence of DMD is estimated to be 4.8 per 100,000 people. Since it is an X-linked recessive disorder, males are much more frequently affected than females.[1,13] In the United States specifically, DMD has an estimated incidence of 1 in 3,600 male live-born infants and a prevalence of approximately 2 per 10,000. Patients typically lose the ability to walk by age 12, and the prognosis is poor, with death usually occurring in the teens or twenties due to respiratory or cardiac complications.

A study by Landfeldt et al. estimated the average annual direct cost per patient for DMD to be between $23,920 and $54,270 (in 2012 international dollars).[5] These numbers represent a seven- to sixteen-fold increase over the mean per-capita health expenditure in the countries studied. The components of the annual cost of DMD are shown in Figure 4. The overall societal burden of DMD reached as high as $120,910 per patient per year, escalating alongside disease progression as seen in Figure 5. Within this total, household-specific expenses were valued between $58,440 and $71,900.

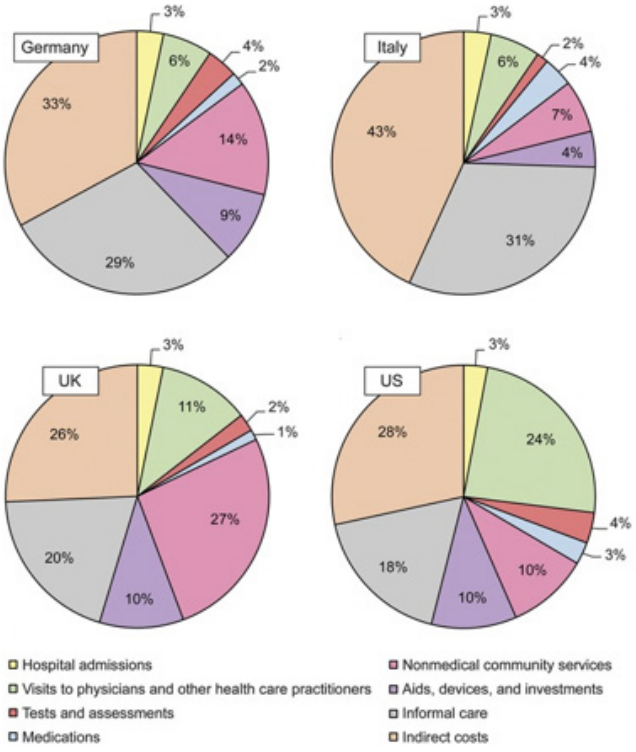

Figure 4 Graphical breakdown of the composition of annual DMD-related expenses across multiple countries.

The image shows the percentage allocation of direct and indirect healthcare costs for patients in Germany, Italy, the UK, and the US.

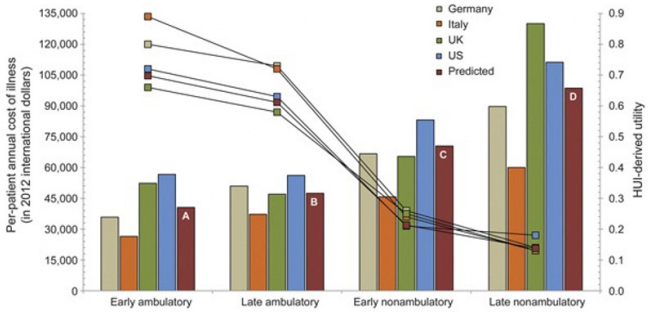

Figure 5 Graphical representation of the correlation between increasing annual care costs and declining patient utility in DMD.

The image shows how per-patient annual cost of illness escalates across four distinct disease progression stages, contrasted against a corresponding decline in the Health Utilities Index (HUI)-derived utility across the studied nations.

Global Duchenne muscular dystrophy market size

The global market for DMD treatments is experiencing rapid expansion (Mordor Intelligence, 2026). Driven primarily by advancements in molecular-based therapies—which account for over 60% of the market share—Mordor Intelligence estimates the 2026 market size at $4.06 billion. The industry is projected to reach $9.59 billion by 2031, representing a compound annual growth rate (CAGR) of 18.76% (Figure 6).

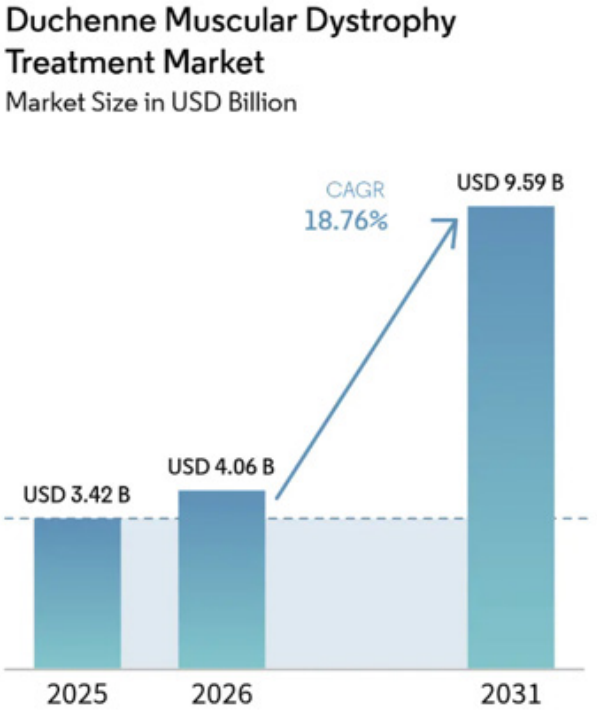

Figure 6 Bar chart tracking the projected growth of the global DMD treatment market.

The image shows the market valuation expanding from 2025 through 2026 to a projected value of $9.59 billion by 2031, highlighting a CAGR of 18.76% over the forecast period.

As survival rates for DMD patients have climbed, the resulting expansion in treatment windows has significantly driven market demand.

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In 2025, North America dominated the global DMD market, accounting for 41.11% of the revenue with a valuation exceeding $1.41 billion (Mordor Intelligence, 2026). This leading position is supported by robust private insurance coverage, extensive venture funding, and established orphan-drug frameworks, alongside expanding newborn screening programs and harmonized cross-border policies in Canada and Mexico. The Asia-Pacific region is projected to be the fastest-growing market, with an anticipated 19.61% CAGR through 2031. This rapid expansion is fueled by accelerated regulatory approvals in Japan and China, government-backed clustered regularly interspaced short palindromic repeats (CRISPR) initiatives, and increasing clinical trial participation across India, South Korea, and Australia (Mordor Intelligence, 2026). Europe also remains a crucial market due to its extensive academic-medical networks and coordinated regulatory accelerators. Germany and France are early adopters due to statutory insurance, while recent conditional drug approvals by the European Medicines Agency (EMA) and emerging reimbursement pilot projects in Eastern Europe further strengthen the region's position. Figure 7 illustrates the global DMD treatment market growth rates by region (Mordor Intelligence, 2026). Industry leaders like Sarepta Therapeutics, PTC Therapeutics, and Nippon Shinyaku at the forefront of the DMD market.

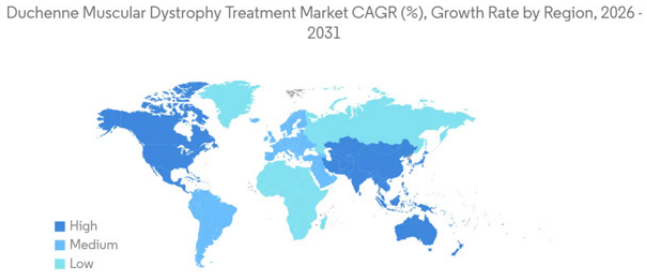

Figure 7 Schematic choropleth map illustrating the global DMD treatment market growth rates by region forecasted from 2026 to 2031.

The image shows the geographic distribution of market expansion classified into high, medium, and low growth tiers across different global territories.

Estimates vary across industry analysts, primarily due to emerging gene therapies. ResearchAndMarkets.com forecasts an aggressive climb in the global market for DMD drugs, rising from $2 billion in 2022 to $27.4 billion by 2030, with a CAGR of 39.1% from 2022 to 2030. Within this, China is poised to reach a market size of $2.4 billion by 2030, with a CAGR of 46%, Japan and Canada are expected to grow at rates of 32.6% and 31.7% respectively from 2022 to 2030, and Europe is predicted to grow at 34.8%.[6]

Duchenne muscular dystrophy market segments and trends

DMD medications can be primarily divided into corticosteroids, gene therapy, exon skipping drugs, and therapies that promote growth and regeneration. These treatments can prolong life and function.[8]

Corticosteroids, such as prednisone, deflazacort, and vamorolone, attenuate the progression of muscle weakness and extend the period of independent ambulation.

Gene therapies aim to transform the aggressive DMD disease course into a more manageable Becker muscular dystrophy (BMD)-like condition. In some DMD gene therapies, miniaturized dystrophin genes are delivered into cells via viral vectors, which can lead to the restoration of dystrophin function. Elevidys is the first gene therapy approved for DMD. It is a single-dose therapy that is indicated for ambulatory patients four years of age and older who have a documented DMD gene mutation.[14]

Exon skipping therapies use antisense oligonucleotides (ASOs) to skip over mutated genetic sections, enabling the production of a partially functional dystrophin.

Givinostat was the first non-steroidal therapy approved by the Food and Drug Administration (FDA) for the treatment of DMD regardless of the patient's specific genetic mutation.[15] Givinostat is a histone deacetylase (HDAC) inhibitor approved for DMD patients six years of age and older. By mitigating inflammation and fibrosis, Givinostat helps preserve muscular integrity and reduce tissue necrosis, ultimately slowing disease progression.[8]

The global DMD drug market size was estimated by Grand View Research at $3.47 billion in 2023 and is anticipated to climb to $9.91 billion by 2030, with a CAGR of 16.8% from 2024 to 2030. This market expansion is driven by the rising prevalence of DMD and a surge in research and development funding, particularly within the field of genomic research.

In 2023, molecular-based therapies had a leading market share of 42.0%, driven by a growing clinical preference for treatments that target the genetic root of DMD and provide a personalized approach by addressing specific mutations (Figure 8) (Grand View Research, 2024). The steroid-based segment is projected to grow at a CAGR of 16.2%. Corticosteroids remain a standard of care due to their proven ability to delay functional decline and preserve patient mobility.

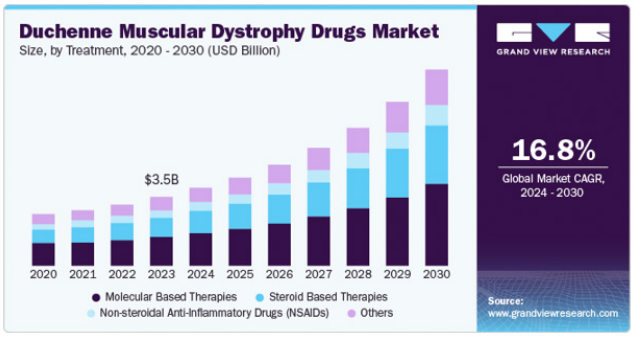

Figure 8 Stacked bar chart tracking the projected DMD drugs market size and therapeutic segment breakdown from 2020 to 2030.

The image shows the growth of the global market valuation over a decade, color-coded by specific drug categories including molecular-based therapies, steroid-based therapies, non-steroidal anti-inflammatory drugs (NSAIDs), and others, alongside a highlighted CAGR of 16.8% from 2024 to 2030.

In 2025, hospital pharmacies controlled the majority of the DMD market (67.05%), largely due to the specialized requirements of gene therapy, such as precise companion diagnostics and intricate reimbursement procedures (Figure 9). However, online specialty pharmacies are projected to expand at 19.55% annually. This growth is driven by integrated patient-support portals that centralize the management of insurance approvals, cold-chain requirements, and real-time clinical monitoring. By 2031, the market share for digital distribution could double, as a result of insurer acceptance of mail-order gene therapies when paired with telehealth-enabled specialist oversight.

Products, therapies, and treatments

Existing Duchenne muscular dystrophy products and products in clinical trials

Therapeutic options for DMD have expanded extensively over the past decade, shifting from purely symptom-management strategies to highly targeted, disease-altering medical interventions including corticosteroids, mutation-specific therapies, small molecule

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approaches, and gene replacement therapies (Figure 10).[7] Though there is currently no available therapy that is able to completely halt disease progression or fully restore dystrophin, disease management focuses on converting the severe Duchenne phenotype into a milder condition.

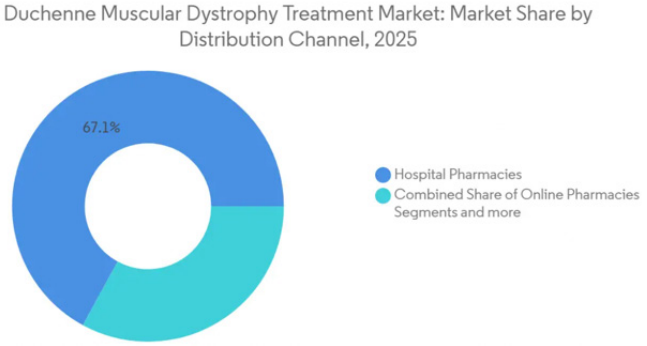

Figure 9 Donut chart illustrating the DMD treatment market share by distribution channel in 2025.

The image shows the percentage breakdown of the market divided between hospital pharmacies and the combined share of online pharmacies segments and more, highlighting that hospital pharmacies accounted for a 67.1% majority share.

Figure 10 Examples of existing therapeutic options for DMD management.

The image shows three distinct pharmaceutical modalities, including Prednisone tablets (a traditional corticosteroid), Duvyzat oral suspension (a histone deacetylase inhibitor), and a Vyondys 53 (golodirsen) single-dose vial for intravenous infusion (an exon-skipping antisense oligonucleotide).

The standard therapy for DMD is glucocorticoids (Table 1). Glucocorticoids are prescribed to help preserve mobility, lower the rate of surgical correction for spinal deformities, and protect breathing functions for as long as possible. While highly effective at delaying disease progression, long-term use requires careful management due to a wide range of hormonal and systemic side effects. Recently, newer dissociative steroids have been introduced to offer similar benefits with potentially fewer severe toxicities. Vamorolone, an oral selective dissociative corticosteroid for patients aged 2 years and older, was FDA approved in 2023. While it targets the same receptors as traditional corticosteroids, its unique profile—acting as a glucocorticoid receptor (GR) agonist and a mineralocorticoid receptor (MR) antagonist—may mitigate common steroid-associated toxicities.

Table 1 Traditional and selective dissociative corticosteroids for Duchenne muscular dystrophy management

Treatment	Examples	Company	Advantages	Limitations	General stage
Traditional Corticosteroids	Prednisone, Deflazacort (oral)	Pfizer, PTC Therapeutics	Delays the loss of walking ability, protects upper limb and breathing functions, and reduces the likelihood of needing spinal surgery.	Causes stunted linear growth, bone density loss, adrenal suppression, immunosuppress-ion, delayed puberty, and behavioral changes.	FDA Approved/ Standard of Care
Selective Dissociative Corticosteroids	Vamorolone (oral)	Revera Gen BioPharma	Improves functional mobility test scores and aims to reduce traditional steroid side effects by selectively binding to specific receptors.	Long-term exposure poses risks of immune suppression, bone density loss, secondary adrenal insufficiency, Cushing's syndrome, hyperglycemia, impaired growth, and mood disturbances; cannot be used as a stress dose steroid.	FDA Approved

Rather than trying to replace the missing dystrophin protein, non-steroidal disease-modifying therapies target the secondary damage caused by DMD, such as ongoing inflammation and the buildup of scar tissue inside the muscles, in order to slow muscle degeneration (Table 2).

Table 2 Non-steroidal disease-modifying therapies (Histone deacetylase inhibitors) for Duchenne muscular dystrophy

Treatment	Examples	Company	Advantages	Limitations	General Stage
Histone Deacetylase Inhibitor	Givinostat (Duvyzat) (oral)	ITF Therapeutics, LLC	Slows down muscle deterioration and functional decline regardless of the patient's specific genetic mutation.	Frequently causes gastrointestinal issues (nausea, vomiting, diarrhea, abdominal pain), thrombocytopeni-a, and hypertriglyceride-mia.	FDA Approved

Exon-skipping therapies use specialized molecules to skip over faulty sections of the patient's genetic code, allowing the body to produce a shorter, but partially functioning, dystrophin protein (Table 3).

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Table 3 Exon-skipping antisense oligonucleotide therapies for Duchenne muscular dystrophy

Treatment	Examples	Company	Advantages	Limitations	General Stage
Exon-Skipping Antisense Oligonucleotid-es	Eteplirsen, Golodirsen, Viltolarsen, Casimersen (weekly intravenous infusions)	Sarepta Therapeuti-cs, Nippon Shinyaku	Enables the creation of a truncated dystrophin protein, theoretically converting severe DMD into a milder form of muscular dystrophy.	Only works for specific mutations (treating ~30% of patients), yields extremely low levels of dystrophin (usually 1-6%), and is cleared too quickly by the kidneys to be highly effective in muscle tissues.	FDA Approved

Mutation-agnostic and readthrough therapies attempt to tackle the disease from different angles, such as protecting muscle fibers from movement-related tearing, boosting muscle stem cell recovery, or helping the body read through specific stop signals in the genetic code (Table 4).

Table 4 Mutation-agnostic and translational readthrough therapies for Duchenne muscular dystrophy

Treatment	Examples	Company	Advantages	Limitations	General Stage
Selective Fast Skeletal Muscle Myosin Inhibitors	Sevasemten (EDG-5506) (oral)	Edgewise Therapeuti-cs	Reduces the mechanical stress on fast-twitch muscle fibers, which prevents contraction-induced muscle tearing across all mutation types; resulted in significant decreases in serum biomarkers of muscle injury.	Does not restore the missing dystrophin protein; optimal dosing is still unknown.	Phase 2 Clinical Trials
Satellite Cell-Modulating Therapy	SAT-3247 (oral)	Satellos Bioscience, Inc.	Restores the muscle's natural ability to repair itself by fixing defective stem cell division, boosting the generation of myogenic progenitors and thereby facilitating muscle repair.	Safety and long-term effectiveness in humans are not yet established.	Early Clinical Development
Translational Readthrough Agents	Ataluren (oral)	PTC Therapeuti-cs	Instructs ribosomes to bypass nonsense stop-codon mutations, allowing the body to naturally produce full-length/near-full-length dystrophin without permanently altering deoxyribonucleic acid (DNA); is generally well tolerated.	Only applies to 10-15% of patients (nonsense mutations); can cause gastrointestinal symptoms and headache; produces variable clinical benefits; faces major regulatory challenges.	FDA Resubmission under review (Withdrawn in Europe)

Gene therapies use engineered viruses or donor cells to deliver functional dystrophin genes directly into the patient's muscles, offering the most direct approach to replacing the missing protein (Table 5).[7,16,17]

Table 5 Approved and investigational gene therapies and cellular replacement strategies for Duchenne muscular dystrophy

Treatment	Examples	Company	Advantages	Limitations	General Stage
Micro-dystroph-in Adeno-Associated Virus (AAV) Gene Therapy	Delandistro-gene moxeparvo-vec-rokl (Elevidys) (single-dose intravenous infusion)	Sarepta Therapeut-ics, Inc.	Delivers a miniaturized functional dystrophin gene that can stabilize motor function and convert the disease to a milder state.	Patients with existing AAV antibodies cannot receive it; vomiting, nausea, fever, and thrombocytopenia can occur; serious adverse events like acute liver injury, myocarditis, and immune-mediated myositis can occur.	FDA Approved

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Table 5 Continued...

Investigational Micro-dystroph-in Therapies Gene Therapy	RGX-202 (single intravenous infusion), SGT-003 (single intravenous infusion), INS1201 (single intrathecal administrati-on)	REGENX-BIO, Solid Bioscienc-es, Insmed	Testing improved delivery vectors, novel transgene designs, or different administration routes (like intrathecal injections) to bypass existing immunities.	Requires intense immune suppression regimens; exact durability and long-term safety remain uncertain.	Phase 1, 2, and 3 Clinical Trials
Allogeneic Myoblast Transplantation (Allogeneic) Gene Therapy	Normal Donor Myoblasts (intramuscu-lar injection)	Dystrogen Therapeut-ics	Plants healthy donor cells directly into target muscles to restore dystrophin locally.	Highly localized (only helps the injected muscle) and requires ongoing immunosuppressi-on to prevent cell rejection.	Phase 1/2 Clinical Trials
Umbilical Cord Mesenchymal Stem Cell (UC-MSC) Transplantation (Allogeneic) (Allergy and Asthma Consultants, 2019)	UC-MSCs	Allergy and Asthma Consultan-ts	Possesses potential muscle regenerative and anti-inflammatory properties that could benefit patients.	Efficacy and broader safety are yet to be established and results are not posted; this was a single-group open-label study restricted to a single adult male patient (28-31 years old).	Phase 1 Clinical Trial (Complete-d)
Bone Marrow-Derived Autologous Stem Cell Transplantation (Autologous)	Bone Marrow-De-rived Autologous Stem Cells	Stem Cells Arabia	Uses the patient's own cells, which can potentially be genetically altered outside the body to restore functional dystrophin expression before being transplanted back into the patient.	Efficacy and broader safety are yet to be established as the current trial status is unknown and results are not posted; trial eligibility restricts this treatment to patients aged 3-25 without respiratory distress or acute infections.	Phase 1 / Phase 2 Clinical Trial

Preclinical tissue engineering approaches for Duchenne muscular dystrophy

To address the severe muscle loss that is characteristic muscular dystrophy, researchers are turning to regenerative medicine and tissue engineering therapies (Park et al., 2024). Rather than just managing symptoms, these approaches aim to actively restore damaged muscle by utilizing therapeutic stem cells, biomaterials, and targeted biological factors.[9]

For DMD specifically, clinical trials are currently evaluating the use of cell therapy—specifically the infusion of mesenchymal stem cells (MSCs). When introduced into the patient, these MSCs can evade immune rejection and secrete specific biological factors that alter the highly inflamed muscle environment. According to recent clinical studies on DMD patients, these stem cell treatments have proven safe across varying doses and have successfully helped to prevent the death of muscle cells, reduce the buildup of fibrotic scar tissue, and stimulate the active regeneration of new muscle fibers. While MSC infusion is currently being trialed for DMD, other tissue engineering approaches have demonstrated success in broader severe muscle and soft tissue injuries (Table 6).[9]

Table 6 Tissue engineering approaches in clinical trials for musculoskeletal repair

Treatment approach	Materials	Target condition	Methodology	Clinical trial outcomes & status
Stem Cell Therapy for Genetic Myopathy	MSCs	Duchenne Muscular Dystrophy	Injected stem cells release immunomodulatory factors that reduce inflammation and create a favorable environment for the patient's native muscle stem cells to activate and repair tissue.	Clinical trials demonstrated no toxicity limits at low or high doses. The therapy successfully inhibited muscle cell death, reduced fibrosis, and promoted active muscle regeneration.
Acellular Biologic Scaffolds	Small intestinal submucosa, urinary bladder matrix, or dermal ECM scaffolds	Severe Muscle & Soft Tissue Trauma	When appropriately selected for the specific tissue—such as porcine urinary bladder ECM scaffolds—a cell-free, structural biomaterial implanted directly into a massive muscle wound provides a supportive microenvironment. This physical framework facilitates the mobilization and accumulation of the patient's own perivascular stem cells, altering the default healing response to build new skeletal muscle fibers rather than non-functional scar tissue.	Implantation in 13 patients successfully generated new soft tissue and innervated skeletal muscle fibers within 6 months of the procedure.

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Table 6 Continued...

Adipose-Deriv-ed Cell Therapy	Adipose-Derived Stem Cells (ADSCs)	Lateral Epicondyl-itis	Stem cells harvested from the patient's own fat tissue are utilized for their high capacity to differentiate into various musculoskeletal cell types and rapidly repair damaged structural tissue.	Patients experienced a substantial reduction in the size of the targeted defect after 12 weeks.

Currently, muscular dystrophy lacks a cure, and developing new treatments is a highly expensive and time-consuming process with a 90% clinical failure rate, likely due to high toxicities and inadequate preclinical testing models. Because the disease affects patients differently, there is also a critical need for customized treatments. To accelerate drug discovery, reduce costs, and enable personalized medicine, researchers are utilizing bioengineered in vitro human skeletal muscles. This approach also provides a much more accurate and predictive platform for preclinical drug testing.[18]

Using three-dimensional (3D) tissue engineering techniques, such as bioprinting or micromolding, researchers encapsulate cells within biocompatible scaffolds like fibrin,[19] collagen,[20] or decellularized extracellular matrix (dECM) hydrogels[21] (Table 7).[21-23]

Table 7 Bioengineered in vitro human skeletal muscles

Scaffold material	Cell type	Biofabrication technique	Advantages	Limitations
Alginate—PEO (Polyethylene oxide) bioink with PCL (Polycaprolact-one)/ collagen struts	C2C12 myoblasts and human umbilical vein endothelial cells (HUVECs)	3D printing and electrospinning	Provides homogeneous cell distribution and high cell viability (~90%), induces successful HUVEC elongation, and enhances myogenesis/vascula-rization.	The cell-laden alginate-PEO bioink possesses low mechanical properties and cannot form a 3D structure on its own, requiring the use of modified fabrication techniques to integrate it with rigid mechanical supporters.
Fibrin	Primary human myoblasts	Hydrogel molding of bundles within nylon frame	Fibrin is remodeled by cells and induces native ECM synthesis, has tunable mechanical properties, allows for long-term cell cultures, and engineered muscle using this scaffold has recorded the highest specific forces to date.	Both the size of the myofibers and their capacity for isometric contraction remain below the levels observed in native adult muscle, indicating incomplete maturation; the electrical stimulation required to enhance the tissue's force and hypertrophy causes a decrease in the tissue's fatigue resistance.
Collagen	C2C12 myoblasts and human microvascular endothelial cells	Extrusion technique	Successful vascular/structural integration in live mice, aligns tissue to respond to electrical stimulation with highly synchronized movement, and exhibits greater contractile magnitudes.	Collagen is naturally degradable by mammalian cells, function in vivo requires further evaluation, and this method requires integration of additional supportive cells to achieve complete muscle functionality.
Gelatin	C2C12 myoblasts	Micropatterning	Simple and affordable, accelerates muscle maturation, improves sarcomere alignment, increases contractile protein content, and upregulates genes related to in vivo muscle development.	Produces tissues that lack developmental maturity of primary cells and requires further research to fully validate the in vitro findings against in vivo skeletal muscle.
dECM	Primary human myoblasts and HUVEC endothelial cells	3D bioprinting	Muscle constructs with high viability, contractility, differentiation, maturation, and myotube formation; excellent regenerative ability; heightened *de novo* muscle formation in a volumetric muscle loss (VML) rat model.	dECM bioinks possess inherently weak mechanical integrity and low shape fidelity, making it difficult to construct large, stable 3D tissues without a specialized support system.

Supportive treatments for Duchenne muscular dystrophy

Because there is currently no cure for DMD, supportive treatments are essential for helping patients maintain their muscle strength, prevent severe complications, and stay mobile and independent for as long as possible. These treatments include cardiovascular care, physical rehabilitation, respiratory support, and surgical corrections (Table 8) (Figure 11).[24]

Table 8 Supportive therapies and interventions for managing Duchenne muscular dystrophy

Treatment category	Examples/ interventions	Purpose/Advantages	Important considerations
Heart Medicines	Angiotensin-converti-ng enzyme (ACE) inhibitors, Beta blockers	Prescribed to treat and manage symptoms when muscular dystrophy damages the heart muscle.	Must be overseen by a cardiologist.
Physical Therapy & Exercise	Range-of-motion exercises, stretching, low-impact aerobics (walking, swimming)	Helps keep joints as flexible and mobile as possible, improves overall strength, and supports general health.	Patients must consult a healthcare professional before starting exercise, as certain types of workouts can be harmful or unsafe for DMD.
Orthotics & Mobility Aids	Braces, canes, walkers, wheelchairs	Braces support weak muscles, keep tendons stretched, and slow the worsening of contractures; mobility aids allow patients to move around and maintain independence.	Needs continuous monitoring to adapt the equipment over time.
Respiratory Support & Prevention	Deep breathing/coughing exercises, face mask breathing devices during sleep, ventilators, vaccinations (pneumonia, flu, Coronavirus Disease 2019 (COVID-19))	Assists weakened breathing muscles, forces air in and out of the lungs when necessary, and protects vulnerable patients from respiratory infections.	Often requires coordination with a pulmonologist and respiratory therapist. Staying away from sick individuals is highly recommended.
Surgical Interventions	Contracture release surgery, spinal surgery, pacemaker implantation	Fixes severe joint contractures, corrects spinal curves that could otherwise make breathing harder over time, and improves heart function.	Performed by specialized orthopedic surgeons or cardiologists depending on the complication.

Figure 11 Examples of medical devices utilized for supportive treatment interventions in DMD management.

The image shows clinical modalities for multidisciplinary patient care, including a manual wheelchair for mobility assistance, a mechanical ventilator station for respiratory support, and cardiac pacing devices (a traditional transvenous pacemaker and a leadless pacemaker) for cardiovascular management.

Dystrophin expressing chimeric cell therapy for Duchenne muscular dystrophy

Dystrophin Expressing Chimeric (DEC) cell therapy is an emerging, mutation-agnostic cellular approach that restores functional dystrophin expression.[25] The therapy is created by fusing a DMD patient's autologous myoblasts with myoblasts from a normal, healthy donor ex vivo using polyethylene glycol.

The mechanism for this therapy is presented in Figure 12. This fusion creates a chimeric cell capable of producing dystrophin without the need for viral vectors or genetic manipulation, eliminating the risk of off target mutations. Chimeric cells maintain the ability to express wild-type dystrophin, expand the native myoblast pool, attenuate chronic inflammation, and reverse tissue fibrosis.[26-30] Because the host immune system recognizes DEC cells as "self," the therapy minimizes immune response and avoids immunosuppression, preserving mobility and extending survival by targeting the underlying pathology of DMD. This therapy is administered via a localized intraosseous injection into the bone marrow cavity of the iliac crest, and has been shown to improve motor function in both ambulatory and non-ambulatory patients.[31-35]

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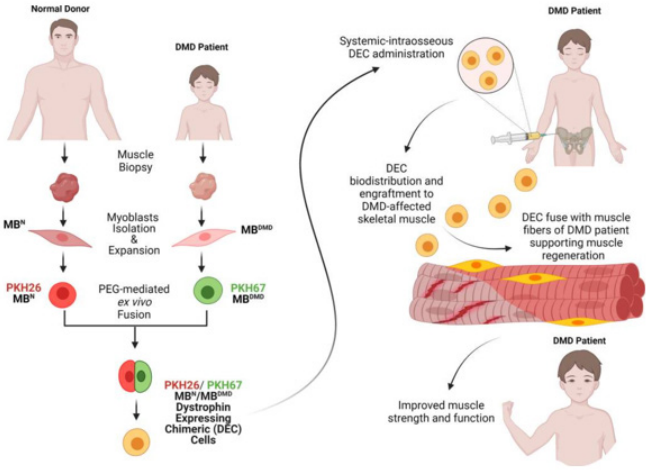

Figure 12 Schematic workflow diagram illustrating the mechanism of Dystrophin Expressing Chimeric (DEC) cell therapy manufacturing and administration.

The image shows the ex vivo isolation, expansion, and PEG (polyethylene glycol)-mediated fusion of myoblasts obtained via muscle biopsies from a normal donor and a DMD patient, followed by the systemic-intraosseous delivery of the resulting DEC cells into the patient's iliac crest, their subsequent biodistribution, and fusion with affected skeletal muscle fibers to improve muscle strength and function.

Conclusion and future considerations

The therapeutic approach to Duchenne muscular dystrophy is currently shifting from purely symptomatic management to targeted, disease-altering interventions. While traditional corticosteroids remain the standard of care for delaying functional decline, the approval of molecular-based therapies like Elevidys and non-steroidal options like Givinostat highlights a major step toward addressing the genetic basis of the disease. Despite these advancements, the path to a cure faces a 90% clinical failure rate in drug development, largely due to high toxicities and the limitations of current preclinical models.[18,36]

To overcome these challenges, researchers are turning to 3D bioengineered human skeletal muscle and regenerative medicine using mesenchymal stem cells to enhance drug discovery and restore damaged tissue. As the market continues to expand, the integration of specialized distribution channels and advanced biotechnologies will be indispensable for improving patient outcomes. Ultimately, these integrated medical and supportive strategies are essential for extending the functional life and improving the quality of life for those living with DMD.

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Conflict of interest

The authors declare that there are no conflicts of interest.

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