
# Market Analysis of Duchenne Muscular Dystrophy-2022

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# Organizational Profile

Foundation of Healthcare Technologies Society
New Blessing Through Technology Empowered Reforms

FHTS is a research organization generating new knowledge using community data to design, develop, implement and evaluate human-cantered Population Health Informatics innovations, interventions, strategic solutions to population health issues, programs and, policies to address social-economic inequalities for overall good health and well-being of individuals, communities and the families they live in. FHTS is established using the principles of SMAART Informatics framework. The organization was founded in March 2012. The foundation aims to establish a centre of excellence in developing a skilled workforce in the area of informatics through human resource development, capacity building, innovative research, translation and dissemination of research findings into practice, and health systems strengthening.

**Our Mission**

To translate research into practice through use of technology based solutions that can empower individuals and the environment where they live in for the well being of themselves, their families and the communities.

**Our Vision**

To improve population outcomes through innovative multispectral and evidence based practices using informatics applications that can empower individuals, communities and institutions for their overall wellbeing.

# Characteristics of the Study

## Rationale of the Study

- The rising disease burden of Duchenne muscular dystrophy (DMD), increased expenditures in biopharmaceutical R&D to produce innovative disease therapeutics, and increased awareness campaigns for DMD are all driving the growth of the DMD treatment market. Though, the high cost of therapies continues to be a barrier to the expansion of the DMD medication landscape. With indigenous innovation and expert training, it is envisaged that cost would decline, enabling the potential to expand. The following topics are covered in the report:

  - ✓ Current status of DMD therapeutic market-2022
  - ✓ Competitive landscape associated with Duchenne muscular dystrophy treatment across US, EU5 and Japan
  - ✓ Regulatory considerations for the treatment of Duchenne muscular dystrophy
  - ✓ Factors driving the growth of DMD treatment market

## Research Question

What are the key market trends for Duchenne muscular dystrophy in the second quarter of 2022, and how will the competition environment shift?

## Research Objectives

- To analyze the current market scenario through reports in the second quarter of 2022 of the Duchenne muscular dystrophy global market.
- To understand the competitive landscape associated with Duchenne muscular dystrophy treatment.
- To comprehend the developments and regulatory considerations for the treatment of Duchenne muscular dystrophy
- To learn about market drivers and barriers.

## Research Methodology

Descriptive study

A non-systematic random, virtual, digital study

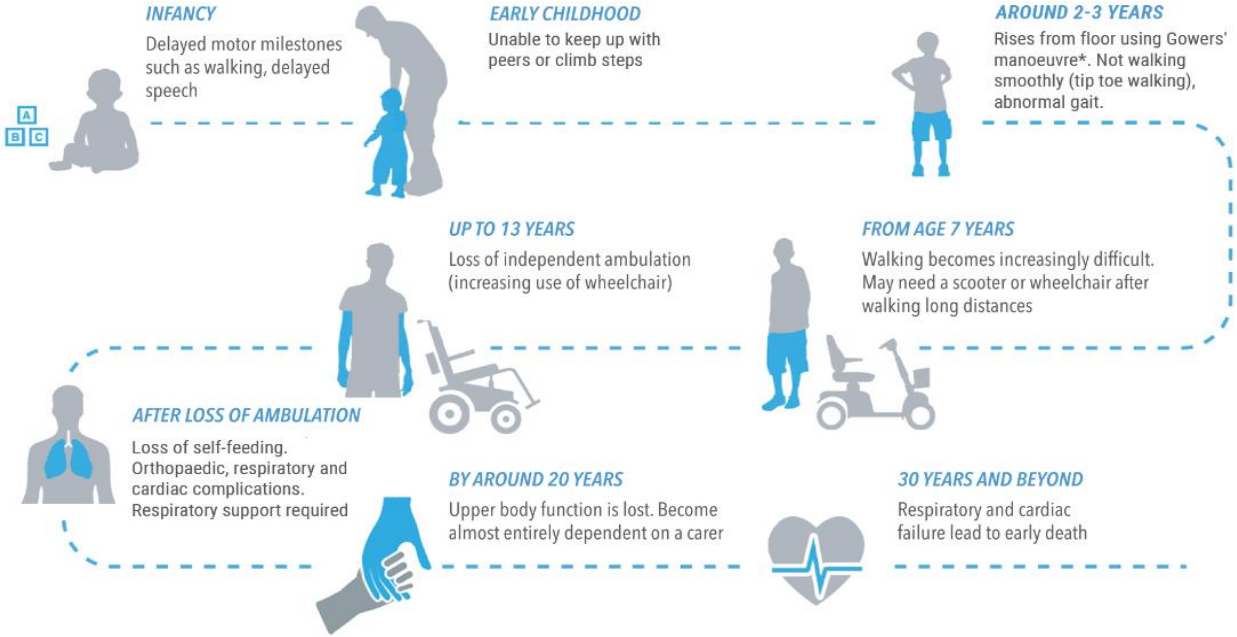
March to June 2022

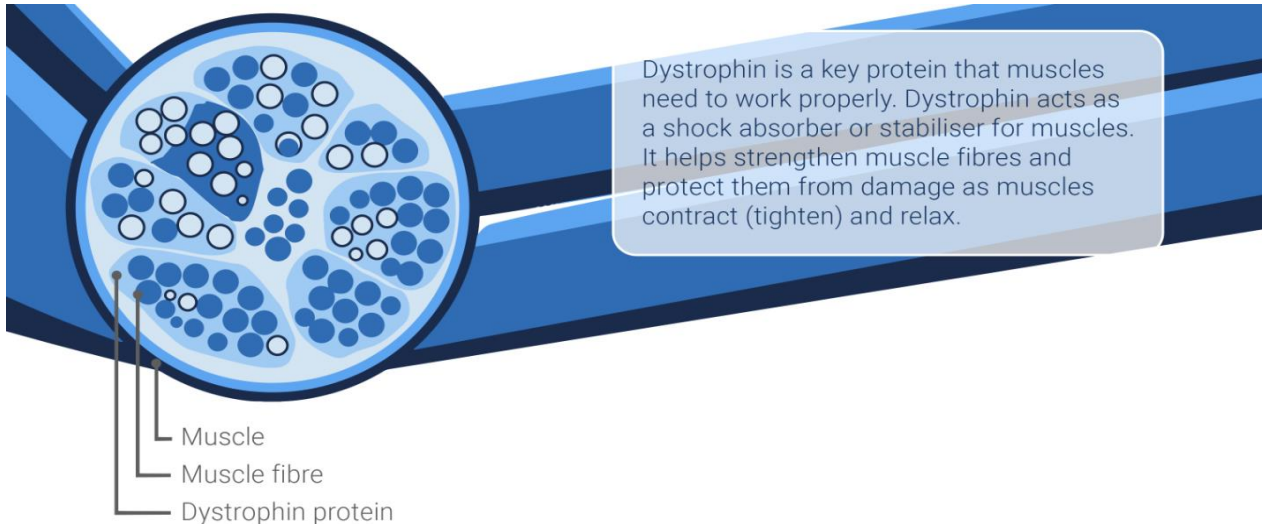
Survey, annual reports, company websites, articles

Search engine Keywords and Booleans

# Disease Overview (1/2)

Duchenne muscular dystrophy (DMD) is a rare muscle disorder but it is one of the most frequent genetic conditions affecting approximately 1 in 3,500 male births worldwide. It is usually recognized between three and six years of age. DMD is characterized by weakness and wasting (atrophy) of the muscles of the pelvic area followed by the involvement of the shoulder muscles. As the disease progresses, muscle weakness and atrophy spread to affect the trunk and forearms and gradually progress to involve additional muscles of the body. In addition, the calves appear enlarged in most patients. The disease is progressive and most affected individuals require a wheelchair by the teenage years. Serious life-threatening complications may ultimately develop including disease of the heart muscle (cardiomyopathy) and breathing (respiratory) difficulties. It is caused by a lack of dystrophin.

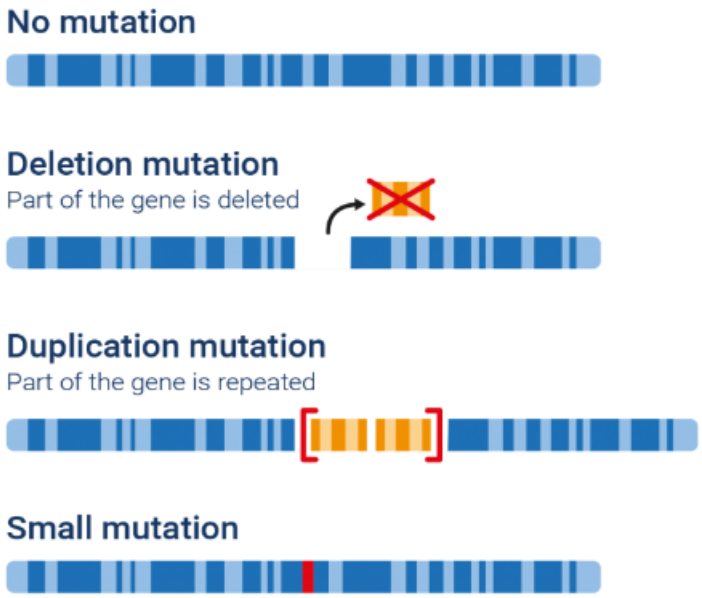


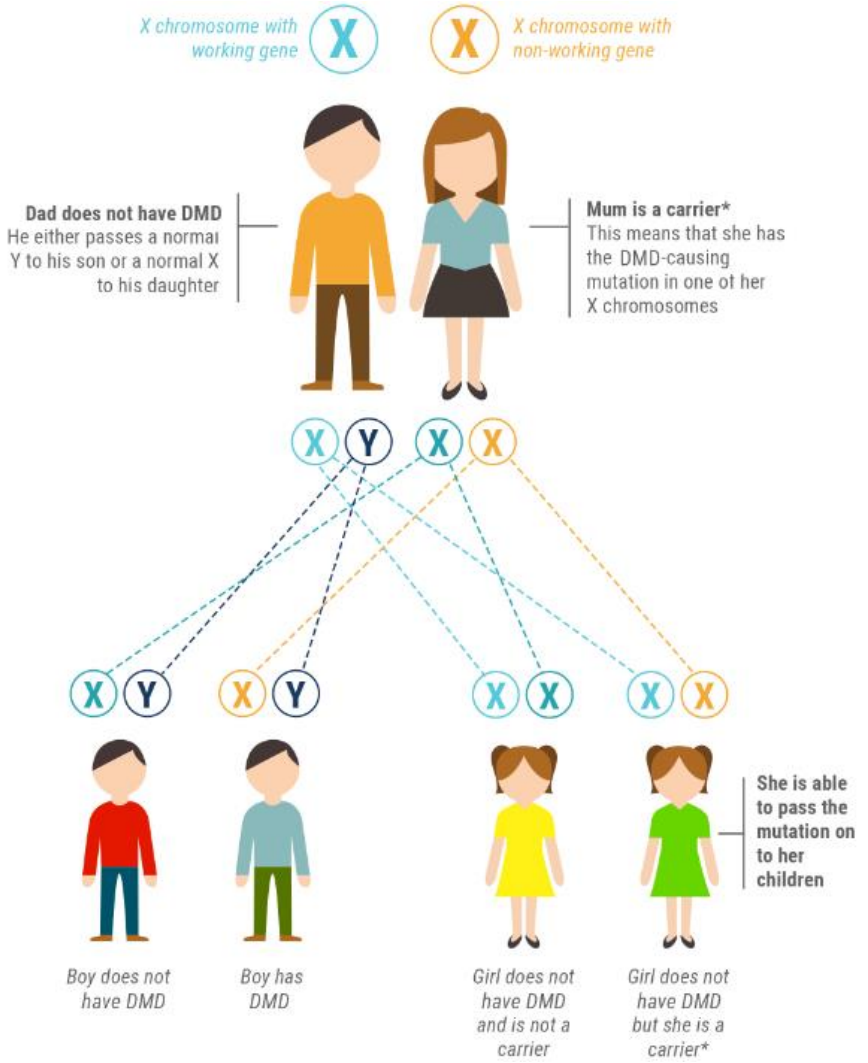
# Disease Overview (2/2)

## DMD can be caused by different types of mutations

Most cases of DMD are caused when large pieces of the dystrophin gene are lost or deleted (‘deletions’). Less often, large pieces of the dystrophin gene are copied or duplicated (‘duplications’). DMD can also be caused by smaller changes in the DNA code.




## How is Duchenne muscular dystrophy inherited?



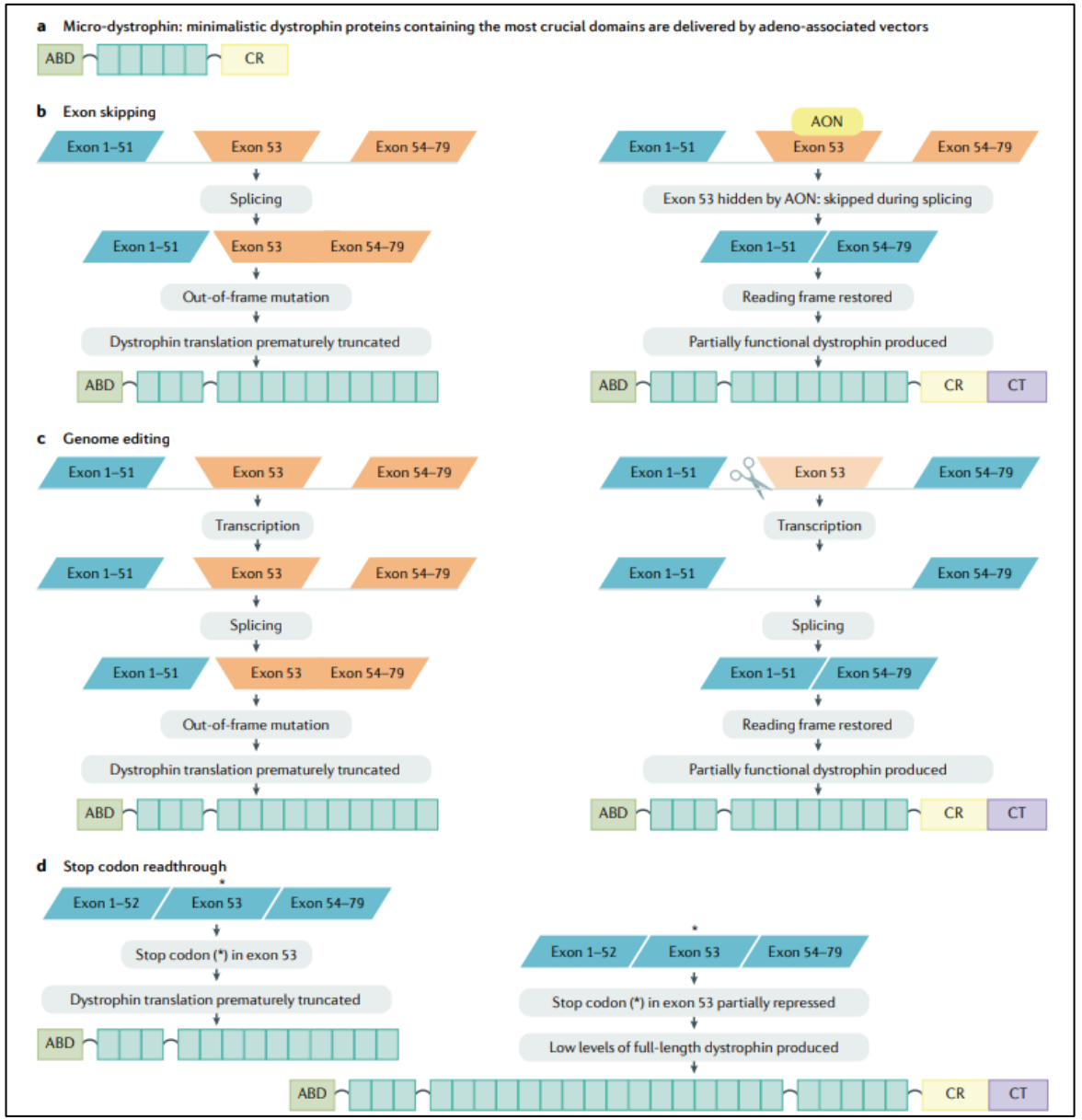
# Dystrophin-restoring Approaches

**Micro-dystrophin gene therapy**: To accommodate the limited capacity of adeno-associated viral vectors, cDNA constructs encoding only the most crucial parts of dystrophin have been generated to enable the expression of micro-dystrophins

**Exon skipping**: Antisense oligonucleotides (AONs) are used to target a specific exon during pre-mRNA splicing. The AON hides the targeted exon from the splicing machinery causing it to be skipped, thus restoring the reading frame, allowing the production of a Becker-like dystrophin.

**Genome editing**: Guide RNAs are used to direct Cas9 to target regions in the DNA to delete an exon from the gene, thereby restoring the reading frame of mRNA produced from this gene, allowing the production of Becker-like dystrophin.

**Stop codon readthrough**: For patients with nonsense mutations (indicated by an asterisk), compounds can suppress the premature stop codon use, facilitating instead the inclusion of an amino acid. Thus, a full-length dystrophin can be produced. ABD, actin-binding domain; CR, cysteine-rich domain; CT, C-terminal domain.



# Approved Therapies for DMD

| Company | Product | Approval | Age-group | ROA | MOA | Molecule Type |
|---|---|---|---|---|---|---|
| SAREPTA THERAPEUTICS | Amondys 45 | US: 2021 | Child: 7 years to 13 Years | Intravenous | Exon 45 skipping | Antisense oligonucleotide |
| | Vyondys 53 | US: 2019 | Child: 6 years to 11 years | Intravenous | Exon 53 skipping | Antisense oligonucleotide |
| | Exondys 51 | US: 2016 | Child: 2 years to 5 years | Intravenous | Exon 51 skipping | Antisense oligonucleotide |
| NIPPON SHINYAKU CO., LTD. | Viltepso | US: 2020 Japan: 2020 | Child: 4 years to 9 years | Intravenous | Exon 53 skipping | Antisense oligonucleotide |
| PTC THERAPEUTICS | Emflaza | US: 2017 | | Oral | Steroid receptor agonists | Small molecule |
| | Translarna | EU: 2014 | Child, Adult Older Adult: 5 years and older | Oral | Protein synthesis stimulants | Small molecule |

It's worth mentioning that all of the approved drugs were approved quickly, and further proof is required to keep all of the therapies on the market. Furthermore, based on DMD's historical history, the chances of entering the market vary between country to country. Surprisingly, in terms of regulatory approval (i.e., EMA vs. FDA), the DMD market is regionally varied, with several cases of medications being approved in one jurisdiction but refused in the other based on the same clinical data. As a result, current medications confront a considerable challenge in securing approval in locations other than those where they have been pre-approved

# Scope of Upcoming Therapies

The current scenario is anticipated to shift toward the upcoming therapies with fair competition between the approved and emerging therapies, as some of the emerging therapies are targeting the patient pool captured by the already existing drugs. Here is a list of major key players and competitors

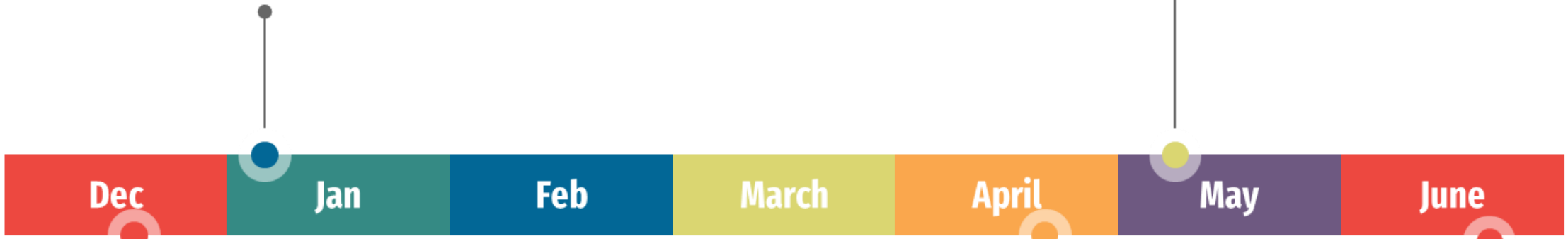
| Upcoming therapies | Emerging key players and major competitors |
|---|---|
| **SRP-5051 (Sarepta)** | Sarepta's SRP-5051 is a potentially more effective therapy, we expect it to replace Exondys 51 once it gets launched at less frequent doses by the year 2023, as per estimates. |
| **DS-5141b (Daiichi Sankyo)** | It is anticipate that the drug may enter the Japanese market by 2024. Daiichi's drug is expected to be a major competitor to Sarepta's Amondys 45 in terms of the same target patient pool. |
| **PF-06939926 (Pfizer), SRP-9001 (Sarepta), SGT-001 (Solid Biosciences)** | After almost 15 years since the first gene therapy trial for DMD began, the dream of a DMD gene therapy might turn into a reality, though with a lot of hurdles. There are currently three companies with competitive trials in the US. |
| **Vamorolone (Reveragen + Santhera)** | Vamorolone, which is being developed for 4–7 year's ambulatory patients in a Phase II trial, will capture a major chunk of the market during the forecast period, considering the large patient pool it is targeting. The drug is a dissociative steroid that works similar to other corticosteroids. |
| **Givinostat (Italfarmaco), TAS-205 (Taiho Pharma)** | Both Givinostat and TAS-205, are being evaluated in Ambulatory DMD Patients. |
| **Pamrevlumab (Fibrogen)** | The major driving force for Pamrevlumab is the increase in cardiac function, grip strength, and preservation of muscle function offered by the drug. Therefore, with the additional benefit of increasing these key problems faced by DMD patients, the drug is believed to be a potential market player. |
| **CAP-1002 (Capricor),ATL1102 (Antisense)** | It is believed that the drug has a great growth opportunity to capture a large patient share of the non-ambulant DMD market. Furthermore, it is anticipated that the drug will launch in EU5 prior to its launch in the US, given the feedback on the Paediatric Investigation Plan (PIP) from the PDCO of the EMA. |

# NGO Initiatives

| | |
|---|---|
| **DMD Care Considerations Working Group** | • The DMD Care Considerations Working Group establishes standards for DMD patients' care |
| **Muscular Dystrophy Association** | • MDA is a non-profit health organization devoted to sponsoring global research to cure muscular dystrophy, ALS, and associated disorders |
| **Parent Project Muscular Dystrophy** | • PPMD creates clinical trial procedures and technologies to solve present hurdles in the development of Duchenne drugs |
| **Muscular Dystrophy UK** | • Muscular Dystrophy UK is a charity that brings together individuals, families, and professionals to fight muscle-wasting diseases |
| **TREAT-NMD** | • TREAT-NMD is a neuromuscular network that offers an infrastructure to guarantee that the most promising new medicines go to patients |
| **World Duchenne Organization** | • WDO is a worldwide umbrella organization that brings together national patient organizations from all over the world. Its mission is to develop a cure and effective therapies for DMD |
| **Action Duchenne** | • Action Duchenne aim to develop effective treatments for all by funding research, supporting clinical trials, campaigning for access, and supporting families, educating about Duchenne |
| **Japan Muscular Dystrophy Association** | • The Japan Muscular Dystrophy Association (Corporation) tasks are to support research into the pathophysiology of muscular dystrophy and identify appropriate treatments for it |

# Recent Developmental Activities

- Phase III trial of CAP-1002 therapy launching in the US
- Sarepta's gene therapy trial shows improved motor abilities
- SOLID gene therapy pipeline community update
- Dystrogen Therapeutics announces first patient dosing of Chimeric Cell Therapy for DMD and reports on 6-weeks outcome
- RegenXBio receives FDA approval for a gene therapy trial




- Antisense Therapeutics received positive PIP decision from UK MHRA
- Myogene Bio award to accelerate novel gene-editing therapy to the clinic

- Vamorolone Filing for FDA Approval for DMD Expected by June
- Three DMD Gene Therapy Programs Terminated by Astellas, Citing Data

- Phase III Trial of Pfizer's Gene Therapy Expected to Open in the US in June

- FibroGen Completes Enrollment for Second Phase III Trial of Pamrevlumab

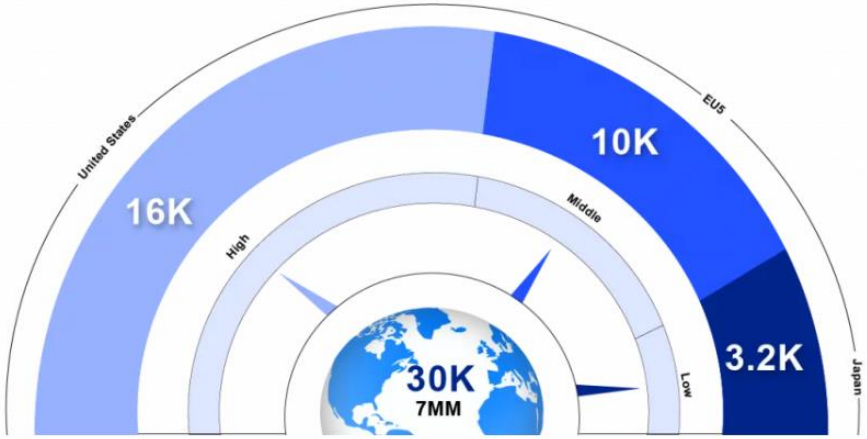
# Reimbursement and Market Access

Patients with DMD face a severe challenge in combating the **high price** associated with the approved therapies. In 2017, Marathon Pharmaceuticals agreed to sell its newly approved steroidal treatment– Emflaza, to PTC, offloading the drug after backlash over initial plans to charge a list price of USD 89,000 per year. PTC cut down to around USD 35,000 per year for a child weighing 25 kg but later increased the drug's list price by about 9%, to more than USD 65,000 annually. This high price makes this old-turned-new drug a significant cost burden for DMD patients. Through the **PTC Care Assistance Program**, PTC therapeutics provides financial assistance for DMD patients who are prescribed Emflaza. Moreover, for patients who have commercial insurance and have out-of-pocket costs associated with Emflaza and who qualify for assistance, PTC Cares offers a **co-pay assistance program**. Additionally, the **Bridge Program** allows patients currently taking Emflaza to continue receiving it free of charge while waiting for insurance verification of coverage.

To make the drugs (Exondys 51, Vyondys 53, and Amondys 45) easily available and affordable to the patients, the company (Sarepta) has a patient support program called **SareptAssist**, designed to help patients navigate the process of starting and staying on these drugs. The company also has a **Sarepta Patient Co-Pay Assistance Program** which was created for eligible individuals with commercial health insurance in the US who are prescribed these therapies. This program may help with some out-of-pocket costs related to receiving treatment, such as co-pays, co-insurance, and deductibles. Similarly, the only DMD drug approved in Japan – Nippon Shinyaku's Viltepso-offers services and resources to patients through the **NS Support program**.

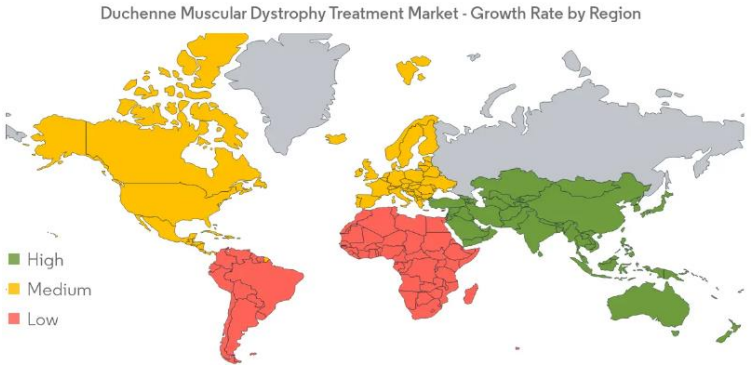
# Market Outlook

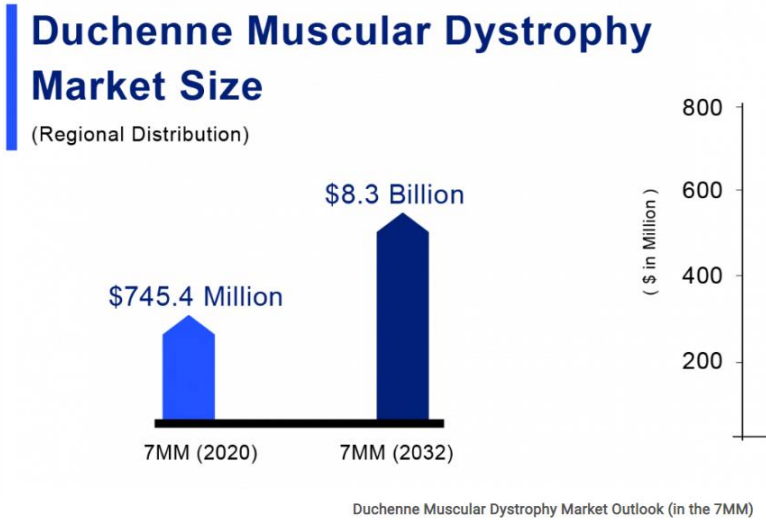
**DMD Epidemiological Insight**




Duchenne Muscular Dystrophy Epidemiology Analysis (For the 7MM)

**North American Region holds the Largest Market Share and is Believed to Follow the Same Trend**




Duchenne Muscular Dystrophy Market Outlook (in the 7MM)

**The market is classified into three categories based on the treatment approach: mutation suppression, exon skipping, and steroid therapy. Exon-skipping therapy is expected to increase at the fastest rate of all treatment approaches.**

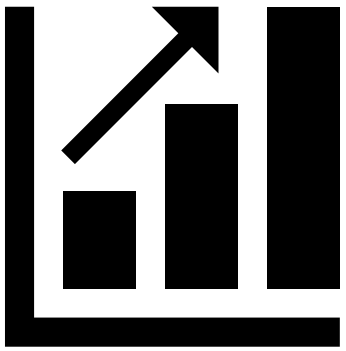
**Key Competitors**


Prominent Players in the Duchenne Muscular Dystrophy Market

**Covid 19 Analysis**


**The COVID-19 epidemic has had an impact on a variety of businesses throughout the world. The economic crisis that followed the epidemic might cause a major delay in the healthcare industry's commercialization. Small and medium-sized companies, which are the backbone of the pharmaceutical industry, have seen a sharp reduction in income since the pandemic began in 2020. As a result, market participants faced several hurdles as supply chain interruptions were noticed. However, when additional supplies become available in the second half of 2022, things will improve**

# Market Drivers and Barriers

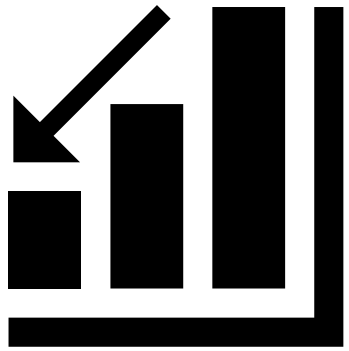


## Market Drivers

- Increasing Awareness Program
- Upcoming Launches and Approval
- New-born Screening of DMD

## Market Barriers

- Lack of treatment options for non-ambulant DMD patients
- Multidisciplinary Care
- High Treatment Cost



# Unmet Needs

**Challenges in the management of DMD**

Reduced inflammation, which exacerbates muscle fiber destruction, is a major problem in the treatment of DMD patients. Because the standard of therapy for DMD is dominated by corticosteroids, there is an unmet medical need. Corticosteroids are ineffective owing to several side effects that must be handled

**Lack of approved therapies in Europe**

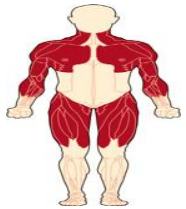
In Europe, Translarna is now the only approved therapy for DMD. This indicates that there is an unmet need for effective medicines to address the rising desire for better and more treatment alternatives among DMD patients in Europe

**High requirement for parallel interventions**

Patients with DMD have gradual muscle degeneration and weakening, which leads to a loss of muscular strength in all of the body's organs. This increases the risk of cardiac, pulmonary, and some bone-related problems. As a result, DMD patients require many procedures for full care and management of bone therapy, pulmonary difficulties, and cardiology-related disorders, all of which are not well established

**Coordination of clinical care**

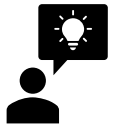
DMD is linked to a slew of additional symptoms, including ADHD, autism spectrum disorder, cardiomyopathy, depression, dysphagia, and a variety of other conditions. This necessitates treating these symptoms alongside the major indications, which are sometimes overlooked during DMD treatment and care



# SWOT Analysis

## Strengths

- DMD's pipeline is strong, with several novel treatments in late-stage and early-stage clinical trials.
- Several exons skipping therapies have been approved in the United States for the treatment of DMD, and Viltepso has just been launched in Japan.
- Several organizations, including Muscular Dystrophy UK, Muscular Dystrophy Association, Parent Project Muscular Dystrophy, World Duchenne Organization, and others, are trying to address the issues that DMD sufferers' encounter.
- Increased neonatal screening in the United States will aid in early therapy, preventing ambulation loss shortly.



## Weaknesses

- Glucocorticoids have a variety of side effects in the major therapy choices, although newer glucocorticoids, such as Emflaza, are not cost-effective in the United States.
- There is no cure or reversible therapy for non-ambulant DMD patients.
- Exon-skipping therapies have been denied approval in Europe for a variety of reasons.
- According to the most recent findings, none of the gene treatments are producing adequate outcomes to go to the next stage. It seems unlikely that a gene treatment for DMD will be approved anytime soon.

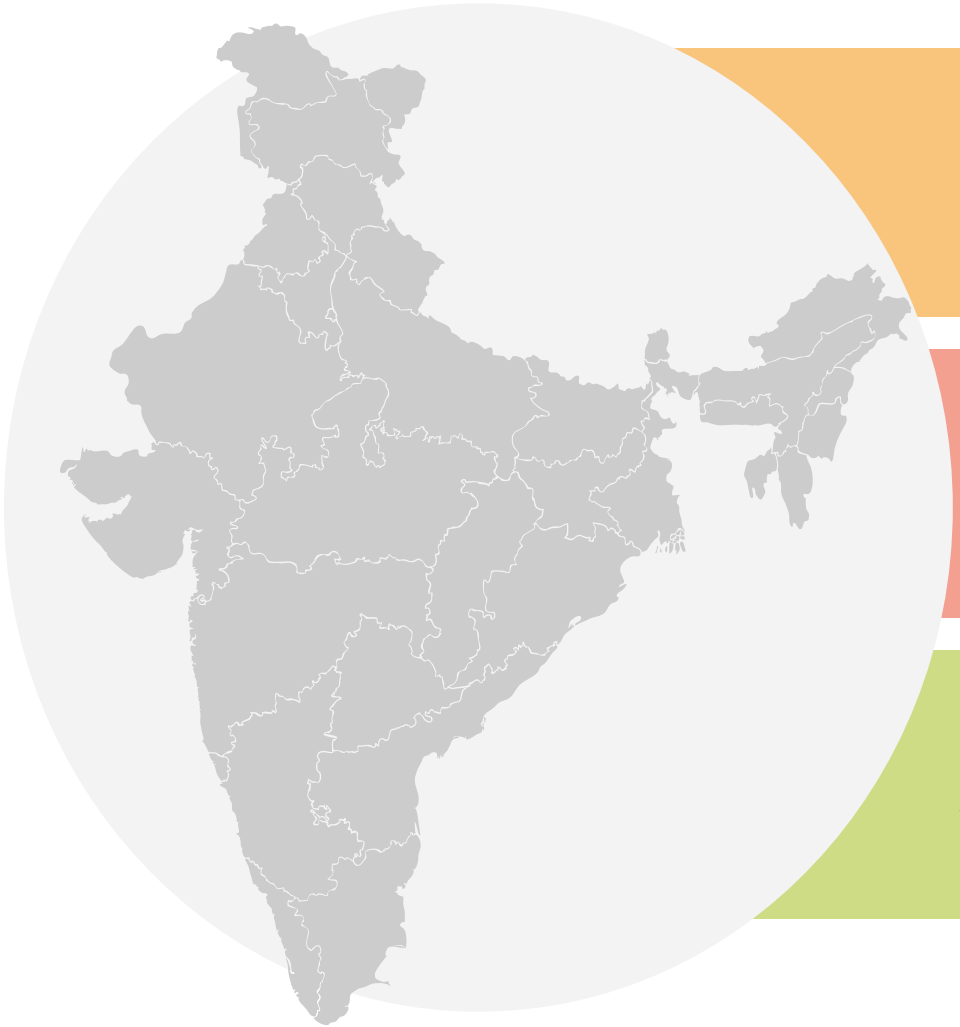
## Opportunities

- Over the last several years, the diagnosed prevalence of DMD patients has increased significantly, creating a big window of opportunity for innovative therapies.
- Possibility for Pfizer's gene therapy, which has a competitive advantage over Sarepta's gene therapy.
- Greater commercial potential for therapies like Capricor's CAP-1002 and Antisense's ATL1102, which target a considerably bigger patient segment than Exon-Skipping therapies and are explicitly aimed at enhancing upper limb capabilities in DMD patients.

## Threats

- Existing treatments are expensive.
- Insufficient participant availability for trial execution. This is because DMD is a rare childhood condition, and the number of people who volunteer for clinical trials of new medications for DMD is quite low compared to other indications.
- Rapid illness progression.
- High pricing creates reimbursement and insurance issues.
- To gain regulatory approval, increased effectiveness must be demonstrated.

# DMD Scenario in India



The **Ministry of Health** notified the Delhi High Court in February 2021 that there are over five lakh individuals in the country suffering from the uncommon ailment Duchenne Muscular Dystrophy, and that genetic treatment is essential for them. The present cost of such therapy per child is around **Rs 6 crore** per year.

Drugs are generally imported from other countries, which drives up the cost of dosage and puts them beyond reach for most families. Many people in India take the help of **crowdfunding** services like Impact Guru, to collect money for treatment of rare illnesses and disorders like SMA and DMD.

**IIT Jodhpur** has established a **research center for DMD** in collaboration with **Dystrophy Annihilation Research Trust**, **Bengaluru** and **AIIMS, Jodhpur**. The research center's key goals include developing an inexpensive and effective therapy for DMD patients using a bioengineered approach as part of the Make in India drive, as well as perform social outreach programs for DMD patients and their parents

**In India, increased awareness is needed about DMD so that young children can receive early diagnosis and, as a result, early treatment**

# Recommendations

The DMD therapy industry has grown dramatically over the years , but there is still a long way to go before these therapeutic techniques may fully cure—rather than just modify—the pathology and phenotype of DMD patients

Increased campaigns and initiatives for awareness of DMD

Increased newborn screening and early diagnosis of genetic conditions to alter the course of disease

Unique payor models to support access to the treatment

Develop combined therapy options to treat the secondary consequences of DMD that decrease the efficiency of single therapies

Rational and rigorous application of appropriate design principles to convincingly establish safety and efficacy in bringing treatments to patients

Current infrastructure needs to be strengthened

Current health personnel needs to be trained and adequately incentivized

# Conclusion

- In the coming years, the market is expected to develop at a rapid pace. The advent of mutation-specific medicines, increased prevalence of the disease, and enhanced diagnostics are all contributing to the rise. The market is likely to increase as a result of government initiatives encouraging target-specific therapies and attractive reimbursement rules. Furthermore, the increasing acceptability of targeted medicines like Exondys51, Translarna, and Emflaza is expected to expand the therapeutic area.
- To restore dystrophin function in DMD muscles, several distinct and new techniques (gene-based, cell-based, and pharmacological) have been explored. Gene therapy to restore dystrophin expression using a safe, non-pathogenic viral vector termed adeno-associated viral (AAV) vector is a potential strategy for treating this life-threatening condition. It is becoming increasingly clear that future therapeutic methods should comprise a combination of therapies, taking into account the health of the muscle as well as the downstream consequences of dystrophin deficiency.
- The high cost of therapeutics remains an impediment to the growth of the DMD drugs landscape. However, affordable healthcare measures are being adopted by governments across all major regions, impacting the pricing strategies of companies as well as reimbursement scenario. Rising cost consciousness is poised to hinder premium pricing opportunities for upcoming DMD drugs.
- Emerging therapies for rare diseases are shifting the paradigms for how to conduct clinical programs under challenging conditions including variable disease course, evaluation of historical controls, and risk of underdosing.

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