

# Duchenne Muscular Dystrophy (DMD) Drugs Market Size, Share & Trends Analysis Report By Therapeutic Approach (Mutation Suppression, Exon Skipping, Steroid Therapy) And Segment Forecasts, 2018 - 2023

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## Abstracts

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The global duchenne muscular dystrophy (DMD) drugs market size is expected to reach USD 4.11 billion by 2023, according to a new report by Grand View Research, Inc., expanding at a CAGR of 41.3% during the forecast period. Several factors such as emergence of mutation-specific therapies, growing target population, and favorable government initiatives are driving the market.

The DMD therapeutics market has only three approved products - Exondys51, Translarna, and Emflaza. Increasing uptake of mutation-targeted therapies is likely to boost the sales of branded drugs. However, stringent regulatory procedures and lack of standardized protocol for determination of clinical efficacy are key challenges for the market.

Exon-skipping platform is estimated to hold about 45.0% of the market share by 2023, driven by increasing adoption of Exondys51 and impending approval of golodirsen and casimersen. Mutation-specific therapies, such as Translarna, are anticipated to face limited adoption due to premium pricing. Associated adverse effects of steroid therapy make them a less favored treatment option.

The DMD drugs market is projected to become intensely competitive in medium- to long-term. The sector has witnessed delays and denials of several key products by the U.S. FDA due to insufficient trials. Raxone (by Santhera) and Givinostat (by Italfarmaco)

remain the most strategically significant R&D pipeline assets for the DMD market, with good possibility of regulatory approval during the forecast period.

Launch of late-stage pipeline products is poised to alter the DMD therapeutic landscape in the near future. Several novel mechanisms of action, such as NF- $\kappa$ B inhibition, myostatin inhibition, and gene therapy are under investigation. Other drug classes, such as exon-skipping and mutation-suppression, are expected to gain traction over the forecast period, supported by rising adoption of these therapeutics.

Milo Biotechnology is one of the first companies to venture into gene therapy for DMD. Company's investigational therapy uses an adeno-associated virus as a myostatin inhibitor and is in early stages of development. Wave Life Science's pipeline product is likely to perform 25 times better in terms of clinical efficacy than the existing exon-skipping drugs.

Further key findings from the report suggest:

Exon-skipping platform dominated the therapeutic approaches in 2017, capturing nearly 40.0% of the market. Exondys51 is estimated to be the leading drug for DMD by 2023

The U.S. dominated the regional market share and is projected to maintain its lead through 2023, owing to rising disease incidence and anticipated launch of promising pipeline candidates

Mutation-suppressive therapeutics and steroids are projected to witness healthy growth due to increasing adoption of Translarna and Emflaza

Translarna received conditional approval in Europe in 2014 for treatment of nonsense mutation DMD patients who are in ambulatory state and are 5 years or older. Exondys51 and Emflaza have not been approved for DMD treatment in Europe

Sarepta, PTC, Santhera, Italfarmaco, and Catabasis are some of the key players operating in this arena

Sarepta is poised to lead the market by 2023 due to higher adoption of Exondys51 as well as anticipated launch of pipeline candidates

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