

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- κ 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

Contents

CHAPTER 1 RESEARCH METHODOLOGY

- 1.1 Information Procurement
- 1.2 Information or Data Analysis
 - 1.2.1 Market formulation & validation

CHAPTER 2 EXECUTIVE SUMMARY

CHAPTER 3 DISEASE PRIMER AND EPIDEMIOLOGY

- 3.1 Disease Primer
 - 3.1.1 Symptoms
 - 3.1.2 Progression of Disease
- 3.2 Epidemiology
- 3.3 Current Prevalence and Incidence for Seven Major Markets (U.S., Japan, EU5)
- 3.4 Forecast Prevalence and Incidence for Seven Major Markets (U.S., Japan, EU5)

CHAPTER 4 GLOBAL DUCHENNE MUSCULAR DYSTROPHY DRUGS MARKET OVERVIEW

- 4.1 Introduction and Market Overview
 - 4.1.1 Segmentation, by therapeutic approach
 - 4.1.1.1 Mutation suppression
 - 4.1.1.2 Exon skipping
 - 4.1.1.3 Steroid therapy
 - 4.1.2 Segmentation, by major markets
 - 4.1.2.1 U.S.
 - 4.1.2.2 EU5
 - 4.1.2.3 Japan
 - 4.1.3 Market size and forecast
 - 4.1.4 Sales performance
 - 4.1.5 Market share distribution
 - 4.1.6 Market dynamics among leading brands
- 4.2 Drivers and Challenges
- 4.3 Deals Landscape (2013-2018)
- 4.4 Pricing and Reimbursement
- 4.5 SWOT Analysis

CHAPTER 5 DUCHENNE MUSCULAR DYSTROPHY DRUGS MARKET: PIPELINE INTELLIGENCE

5.1 Pipeline Landscape

5.1.1 Drugs in development

5.1.2 Key R&D trends

5.1.2.1 Corticosteroids

5.1.2.2 NF- κ B Inhibitor

5.1.2.3 Anti-myostatin

5.1.2.4 Gene Therapy

5.1.2.5 Exon Skipping Platform

5.2 Late-Stage Pipeline

5.3 Profile of Disruptive Drugs

5.3.1 Vamorolone (VBP15)

5.3.2 SMT-C1100 (ezutromid)

5.3.3 CAT-1004 (Edasalonexent)

5.3.4 WVE-210201

5.3.5 AAV1-FS344

CHAPTER 6 COMPANY PROFILES

6.1 Sarepta Therapeutics

6.1.1 Company overview

6.1.2 Current product portfolio

6.1.3 Product forecast sales up to 2023

6.1.4 Company - key news flow

6.1.5 Pipeline view

6.1.6 Pipeline forecast

6.1.7 Catalysts and event calendar

6.1.8 SWOT analysis

6.2 PTC Therapeutics

6.2.1 Company overview

6.2.2 Current product portfolio

6.2.3 Product forecast sales up to 2023

6.2.4 Company - key news flow

6.2.5 Pipeline view

6.2.6 Pipeline forecast

6.2.7 Catalysts and event calendar

6.2.8 SWOT Analysis

6.3 Santhera Pharmaceuticals

6.3.1 Company overview

6.3.2 Current product portfolio

6.3.3 Product forecast sales up to 2023

6.3.4 Company - key news flow

6.3.5 Pipeline view

6.3.6 Pipeline forecast

6.3.7 Catalysts and event calendar

6.3.8 SWOT analysis

6.4 Italfarmaco

6.4.1 Company overview

6.4.2 Current product portfolio

6.4.3 Company - key news flow

6.4.4 Pipeline view

6.4.5 Pipeline forecast

6.4.6 Catalysts and event calendar

6.4.7 SWOT analysis

6.5 Catabasis

6.5.1 Company overview

6.5.2 Current product portfolio

6.5.3 Company - key news flow

6.5.4 Pipeline view

6.5.5 Pipeline forecast

6.5.6 Catalysts and event calendar

6.5.7 SWOT analysis

CHAPTER 7 MARKET OUTLOOK

7.1 What the Future Holds

7.2 Winners and Losers

7.3 Emerging Companies

7.4 The Road Ahead

List Of Tables

LIST OF TABLES

Table 1	Duchenne Muscular Dystrophy Prevalence and Incidence - 2017
Table 2	Forecast Duchenne Muscular Dystrophy Prevalence and Incidence, 2017 - 2023
Table 3	Duchenne Muscular Dystrophy Drugs Market Size and Forecast (in USD million)
Table 4	Geographic Sales Performance, by Seven Major Markets (in USD million)
Table 5	Duchenne Muscular Dystrophy Drugs Market, by Therapeutic Approach (in USD million)
Table 6	Sales Forecast of Approved Treatments (in USD million)
Table 7	Duchenne Muscular Drugs Sales Forecast, by Company (in USD million)
Table 8	Duchenne Muscular Dystrophy Drugs Market Share, by Company (in USD million)
Table 9	Duchenne Muscular Dystrophy Deals Landscape (2013 - 2018)
Table 10	Duchenne Muscular Dystrophy Drugs in Development
Table 11	Late-Stage Duchenne Muscular Dystrophy Pipeline
Table 12	Pipeline Forecast - Global Duchenne Muscular Drugs Market
Table 13	Profile of Disruptive Drug: Vamorolone (VBP15)
Table 14	Profile of Disruptive Drug: SMT-C1100 (ezutromid)
Table 15	Profile of Disruptive Drug: CAT-1004 (edasalonexent)
Table 16	Profile of Disruptive Drug: WVE-210201
Table 17	Profile of Disruptive Drug: AAV1-FS344
Table 18	Product Portfolio: Exondys
Table 19	Exondys 51 Sales Forecast through 2023
Table 20	Sarepta Pipeline View
Table 21	Sarepta - Upcoming Events and Catalysts
Table 22	Product Portfolio: Translarna
Table 23	Product Portfolio: Emflaza
Table 24	Translarna Sales Forecast through 2023
Table 25	Emflaza Sales Forecast through 2023
Table 26	PTC Pipeline View
Table 27	PTC - Upcoming Events and Catalysts
Table 28	Product Portfolio: Raxone
Table 29	Raxone Sales Forecast through 2023
Table 30	Santhera Pipeline View
Table 31	Santhera - Upcoming Events and Catalysts

Table 32 Product Portfolio: Givinostat

Table 33 Italfarmaco Pipeline View

Table 34 Italfarmaco - Upcoming Events and Catalysts

Table 35 Product Portfolio: CAT-1004

Table 36 Catabasis Pipeline View

Table 37 Catabasis - Upcoming Events and Catalysts

List Of Figures

LIST OF FIGURES

- Fig. 1 Market Research Process
- Fig. 2 Information Procurement
- Fig. 3 Primary Research Pattern
- Fig. 4 Market Research Approaches
- Fig. 5 Value Chain Based Sizing & Forecasting
- Fig. 6 QFD Modelling for Market Share Assessment
- Fig. 7 Progression of Duchenne Muscular Dystrophy
- Fig. 8 Current Prevalence Across Seven Major Markets - 2017
- Fig. 9 Current Incidence Across Seven Major Markets - 2017
- Fig. 10 Market segmentation & scope
- Fig. 11 Geographic Sales Performance, by Seven Major Markets 2017
- Fig. 12 Duchenne Muscular Dystrophy Drugs Market, by Therapeutic Approach
- Fig. 13 Duchenne Muscular Dystrophy Sales Trend, by Therapeutic Approach
- Fig. 14 Duchenne Muscular Dystrophy Drugs Market Shares, by Drug Class (2017A-2023E)
- Fig. 15 U.S. Duchenne Muscular Dystrophy Drugs Market, by Therapeutic Approach
- Fig. 16 EU5 Duchenne Muscular Dystrophy Drugs Market, by Therapeutic Approach
- Fig. 17 Japan Duchenne Muscular Dystrophy Drugs Market, by Therapeutic Approach
- Fig. 18 Duchenne Muscular Dystrophy Drugs Market Share, by Company (2017A-2023E)
- Fig. 19 Market Trends & Outlook
- Fig. 20 Market Driver Relevance Analysis (Current & Future Impact)
- Fig. 21 Market Restraint Relevance Analysis (Current & Future Impact)
- Fig. 22 SWOT Analysis (Duchenne Muscular Dystrophy Drugs Market)
- Fig. 23 Pipeline Assets by Phase
- Fig. 24 Exon Skipping and Estimated DMD Risk
- Fig. 25 SWOT Analysis (Sarepta Therapeutics)
- Fig. 26 SWOT Analysis (PTC Therapeutics)
- Fig. 27 SWOT Analysis (Santhera Therapeutics)
- Fig. 28 SWOT Analysis (Italfarmaco)
- Fig. 29 SWOT Analysis (Catabasis)

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