

# Dystrophin (DMD) - Pipeline Review, H2 2019

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## Abstracts

Dystrophin (DMD) - Pipeline Review, H2 2019

### SUMMARY

Dystrophin (DMD) - Dystrophin is a cytoplasmic protein. It anchors the extracellular matrix to the cytoskeleton via F-actin. It acts as ligand for dystroglycan. It act as component of the dystrophin-associated glycoprotein complex which accumulates at the neuromuscular junction and at a variety of synapses in the peripheral and central nervous systems and has a structural function in stabilizing the sarcolemma.

Dystrophin (DMD) pipeline Target constitutes close to 48 molecules. Out of which approximately 44 molecules are developed by companies and remaining by the universities/institutes. The molecules developed by companies in Pre-Registration, Filing rejected/Withdrawn, Phase III, Phase II, Phase I, Preclinical and Discovery stages are 1, 1, 2, 4, 1, 23 and 12 respectively. Similarly, the universities portfolio in Preclinical stages comprises 4 molecules, respectively. Report covers products from therapy areas Genetic Disorders and Musculoskeletal Disorders which include indications Duchenne Muscular Dystrophy and Muscular Dystrophy.

The latest report Dystrophin (DMD) - Pipeline Review, H2 2019, outlays comprehensive information on the Dystrophin (DMD) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type. It also reviews key players involved in Dystrophin (DMD) targeted therapeutics development with respective active and dormant or discontinued projects.

The report is built using data and information sourced from proprietary databases, company/university websites, clinical trial registries, conferences, SEC filings, investor

presentations and featured press releases from company/university sites and industry-specific third party sources.

## **SCOPE**

The report provides a snapshot of the global therapeutic landscape for Dystrophin (DMD)

The report reviews Dystrophin (DMD) targeted therapeutics under development by companies and universities/research institutes based on information derived from company and industry-specific sources

The report covers pipeline products based on various stages of development ranging from pre-registration till discovery and undisclosed stages

The report features descriptive drug profiles for the pipeline products which includes, product description, descriptive MoA, R&D brief, licensing and collaboration details & other developmental activities

The report reviews key players involved in Dystrophin (DMD) targeted therapeutics and enlists all their major and minor projects

The report assesses Dystrophin (DMD) targeted therapeutics based on mechanism of action (MoA), route of administration (RoA) and molecule type

The report summarizes all the dormant and discontinued pipeline projects

The report reviews latest news and deals related to Dystrophin (DMD) targeted therapeutics

## **REASONS TO BUY**

Gain strategically significant competitor information, analysis, and insights to formulate effective R&D strategies

Identify emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage

Identify and understand the targeted therapy areas and indications for Dystrophin (DMD)

Identify the use of drugs for target identification and drug repurposing

Identify potential new clients or partners in the target demographic

Develop strategic initiatives by understanding the focus areas of leading companies

Plan mergers and acquisitions effectively by identifying key players and it's most promising pipeline therapeutics

Devise corrective measures for pipeline projects by understanding Dystrophin (DMD) development landscape

Develop and design in-licensing and out-licensing strategies by identifying prospective partners with the most attractive projects to enhance and expand business potential and scope

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Daiichi Sankyo Co Ltd

Dystrogen Therapeutics SA

Editas Medicine Inc

Evox Therapeutics Ltd

MyoGene Bio LLC

Nippon Shinyaku Co Ltd

NS Pharma Inc

OliPass Corporation

Pepgen Ltd

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Sarepta Therapeutics Inc

Spinalcyte Llc

Sutura Therapeutics Ltd

Tolerion Inc

Vertex Pharmaceuticals Inc

Wave Life Sciences Ltd

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Mechanism Of Action

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Featured News & Press Releases

Dec 13, 2019: Sarepta's DMD therapy Vyondys 53 secures FDA accelerated approval

Nov 05, 2019: Dystrogen Therapeutics announces that treatment with Dystrophin expressing Chimeric (DEC) Cells improves cardiac function in Preclinical Duchenne's Study

Aug 21, 2019: Sarepta fails to receive FDA approval for DMD drug golodirsen

Aug 08, 2019: Sarepta Therapeutics comments on erroneous submission to US FDA Adverse Event Reporting System (FAERS)

Jul 09, 2019: New analysis shows drug slows down respiratory decline

Jul 01, 2019: AskBio's Perspective on Pfizer's 1b clinical trial results on Duchenne MD Gene

Jun 28, 2019: Pfizer presents initial clinical data on phase 1b gene therapy study for Duchenne Muscular Dystrophy (DMD)

Apr 18, 2019: Parent Project Muscular Dystrophy awards \$100,000 grant to Nationwide Children's Hospital to further explore GALGT2 gene therapy in duchenne muscular dystrophy

Mar 28, 2019: Sarepta Therapeutics announces positive expression results from the Casimersen (SRP-4045) arm of the ESSENCE study

Mar 25, 2019: Sarepta Therapeutics to provide update on Duchenne Muscular Dystrophy gene therapy program

Feb 15, 2019: FDA accepts Sarepta's NDA for precision medicine Golodirsen

Feb 06, 2019: NS Pharma begins rolling NDA submission to FDA for Viltolarsen (NS-065/NCNP-01)

Dec 20, 2018: Sarepta Therapeutics completes Submission of New Drug Application Seeking approval of Golodirsen (SRP-4053) in patients with Duchenne Muscular Dystrophy Amenable to Skipping Exon 53

Nov 13, 2018: Evox Therapeutics wins funding from Duchenne UK to explore exosome-mediated delivery of dystrophin

Oct 03, 2018: NS-065/NCNP-01 (Viltolarsen) of Nippon Shinyaku' in-house product presentation on results of Phase I/II study in Japan

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Editas Medicine Inc

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MyoGene Bio LLC

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OliPass Corporation

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