

# Dystrophin - Pipeline Review, H2 2020

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## Abstracts

Dystrophin - Pipeline Review, H2 2020

### Summary

According to the recently published report 'Dystrophin - Pipeline Review, H2 2020'; Dystrophin (DMD) pipeline Target constitutes close to 50 molecules. Out of which approximately 44 molecules are developed by companies and remaining by the universities/institutes.

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.

The report 'Dystrophin - Pipeline Review, H2 2020' 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; that are being developed by Companies/Universities.

It also reviews key players involved in Dystrophin (DMD) targeted therapeutics development with respective active and dormant or discontinued projects. Currently, The molecules developed by companies in Pre-Registration, Filing rejected/Withdrawn, Phase III, Phase II, Phase I, Preclinical and Discovery stages are 1, 1, 2, 5, 1, 24 and 10 respectively. Similarly, the universities portfolio in Preclinical stages comprises 6 molecules, respectively. Report covers products from therapy areas Genetic Disorders and Musculoskeletal Disorders which include indications Duchenne Muscular Dystrophy, Alport Syndrome and Muscular Dystrophy.

**Note:** Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

## 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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AstraZeneca Plc

Avalyn Pharma Inc

Boehringer Ingelheim International GmbH

Fratagene Therapeutics Srl

Lead Discovery Center GmbH

Prous Institute for Biomedical Research SA

TopiVert Ltd

Turning Point Therapeutics Inc

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### COMPANIES MENTIONED

Alpha Anomeric

Audentes Therapeutics Inc

Daiichi Sankyo Co Ltd

Dystrogen Therapeutics SA

Editas Medicine Inc

Evox Therapeutics Ltd

FibroGenesis LLC

MyoGene Bio LLC

Myosana Therapeutics Inc

Nippon Shinyaku Co Ltd

OliPass Corporation

Pepgen Ltd

Pfizer Inc

Sarepta Therapeutics Inc

Sutura Therapeutics Ltd

Tolerion Inc

Vertex Pharmaceuticals Inc

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