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Dystrophin (DMD) Drugs in Development by Therapy Areas and Indications, Stages, MoA, RoA, Molecule Type and Key Players, 2022 Update

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Abstracts

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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 acts 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 67 molecules. Out of which approximately 60 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 The molecules developed by companies in Pre-Registration, Filing rejected/Withdrawn, Phase III, Phase II, Phase I, IND/CTA Filed, Preclinical and Discovery stages are 1, 1, 3, 6, 2, 1, 31 and 15 respectively. Similarly, the universities portfolio in Phase I and Preclinical stages comprises 1 and 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.

The latest report Dystrophin - Drugs In Development, 2022, 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)

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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.

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

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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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Apr 06, 2022: PepGen announces first participant dosed in a phase 1 clinical trial of PGN-EDO51 for the treatment of Duchenne Muscular Dystrophy
Mar 18, 2022: Exon 44-targeted DMD drug hits goals in PI/II: NCNP/Nippon Shinyaku
Mar 17, 2022: The result of an investigator-initiated clinical trial (First In Human trial) of NS-089/NCNP-02 for the treatment of Duchenne muscular dystrophy
Mar 15, 2022: PepGen announces approval by Health Canada of CTA to begin first in human trials of PGN-EDO51 to treat Duchenne Muscular Dystrophy
Mar 11, 2022: The result of an investigator-initiated clinical trial (First In Human trial) of NS-089/NCNP-02 for the treatment of Duchenne muscular dystrophy
Mar 08, 2022: Roche to present new SRP-9001 data at MDA 2022 and highlight expanding neuromuscular disease portfolio
Jan 10, 2022: Sarepta Therapeutics' Gene Therapy SRP-9001 shows statistically significant functional improvements compared to pre-specified matched external control in Part 2 of study SRP-9001-102 for the treatment of Duchenne Muscular Dystrophy
Jan 07, 2022: FDA grants IND clearance for REGENXBIO's gene therapy trial for

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Duchenne
Jan 06, 2022: Sarepta Therapeutics to present at the 40th Annual J.P. Morgan Healthcare Conference
Nov 29, 2021: Nippon Shinyaku implements VILTEPSO managed access program
Nov 25, 2021: Addition of Viltepso to the list of Injection Drugs that physicians providing health insurance treatment can administer
Nov 22, 2021: REGENXBIO announces Orphan Drug Designation granted to RGX-202, a novel gene therapy candidate for the treatment of Duchenne Muscular Dystrophy
Oct 11, 2021: Sarepta Therapeutics' SRP-9001 shows sustained functional improvements in multiple studies of patients with Duchenne
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