

Dystrophin (DMD) - Pipeline Review, H1 2018

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Abstracts

Dystrophin (DMD) - Pipeline Review, H1 2018

SUMMARY

Dystrophin (DMD) pipeline Target constitutes close to 29 molecules. Out of which approximately 25 molecules are developed by companies and remaining by the universities/institutes. The latest report Dystrophin (DMD) - Pipeline Review, H1 2018, 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.

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 molecules developed by companies in Pre-Registration, Phase III, Phase II, Phase I, Preclinical and Discovery stages are 1, 2, 3, 2, 10 and 7 respectively. Similarly, the universities portfolio in Phase II and Preclinical stages comprises 1 and 3 molecules, respectively. Report covers products from therapy areas Genetic Disorders and Musculoskeletal Disorders which include indications Duchenne Muscular Dystrophy and Muscular Dystrophy.

Furthermore, this report also reviews key players involved in Dystrophin (DMD) targeted therapeutics development with respective active and dormant or discontinued projects. Driven by 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

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

Editas Medicine Inc

Genethon SA

NS Pharma Inc

Pfizer Inc

Sarepta Therapeutics Inc

WAVE Life Sciences Ltd

Dystrophin (DMD) - Drug Profiles

Antisense Oligonucleotide 1 to Activate Dystrophin for Duchenne Muscular Dystrophy -
Drug Profile

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Mechanism Of Action

R&D Progress

Antisense Oligonucleotide to Activate Dystrophin for Duchenne Muscular Dystrophy -
Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Antisense Oligonucleotide to Activate Dystrophin for Duchenne Muscular Dystrophy -
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casimersen - Drug Profile

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DS-5141 - Drug Profile

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Mechanism Of Action

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eteplirsen - Drug Profile

Product Description

Mechanism Of Action

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Gene Therapy to Activate DMD for Duchenne Muscular Dystrophy - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate DMD for Duchenne Muscular Dystrophy - Drug Profile

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Product Description

Mechanism Of Action

R&D Progress

golodirsen - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

NS-065 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

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Product Description

Mechanism Of Action

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PF-06939926 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

SGT-001 - Drug Profile

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R&D Progress

SRP-4008 - Drug Profile

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Mechanism Of Action

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SRP-4044 - Drug Profile

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SRP-4050 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

SRP-4052 - Drug Profile

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Mechanism Of Action

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Product Description

Mechanism Of Action

R&D Progress

Dystrophin (DMD) - Dormant Products

Dystrophin (DMD) - Discontinued Products

Dystrophin (DMD) - Product Development Milestones

Featured News & Press Releases

May 18, 2018: Solid Biosciences Announces New Preclinical Data at the American Society of Gene and Cell Therapy Annual Meeting

May 15, 2018: Solid Biosciences Announces Upcoming Preclinical Data Presentations

May 10, 2018: Presentations on NS-065/ NCNP-01 at 2018 New Directions in Biology and Disease of Skeletal Muscle Conference

Apr 25, 2018: Daiichi Sankyo Announces Phase 1/2 Clinical Trial Results for DS-5141 (Therapeutic Agent for Duchenne Muscular Dystrophy) in Japan

Apr 25, 2018: Asklepios BioPharmaceutical Announces Former Portfolio Company's Preclinical Asset for Duchenne Muscular Dystrophy Has Advanced into the Clinic

Apr 19, 2018: Results of Exploratory Investigator-Initiated Clinical Trial of NS-065/NCNP-01 for the Treatment of Duchenne Muscular Dystrophy Published in Science Translational Medicine

Apr 18, 2018: Solid Biosciences Provides Update On SGT-001 Clinical Development Program For Duchenne Muscular Dystrophy

Apr 12, 2018: Pfizer Doses First Patient Using Investigational Mini-Dystrophin Gene Therapy for the Treatment of Duchenne Muscular Dystrophy

Apr 03, 2018: RM LAW Announces Class Action Lawsuit Against Solid Biosciences

Mar 14, 2018: Solid Biosciences Announces Clinical Hold On SGT-001 Phase I/II Clinical Trial For Duchenne Muscular Dystrophy

Mar 12, 2018: Sarepta Therapeutics Announces Plan to Submit a New Drug Application (NDA) for Accelerated Approval of Golodirsen (SRP-4053) in Patients with Duchenne Muscular Dystrophy (DMD) Amenable to Skipping Exon

Jan 17, 2018: First Duchenne Muscular Dystrophy Patient Dosed in Systemic Microdystrophin Gene Therapy

Jan 08, 2018: Sarepta Therapeutics Pre-Announces Fourth Quarter 2017 Revenue and Provides Full-Year 2018 Revenue Guidance for EXONDYS 51 (eteplirsen), Representing Approximately 100 Percent Year-over-Year Growth

Dec 27, 2017: Sarepta Therapeutics Announces Publication of Long-Term Pulmonary Function of Eteplirsen-Treated Patients Compared to Natural History of Duchenne Muscular Dystrophy in The Journal of Neuromuscular Diseases

Nov 30, 2017: Solid Biosciences Initiates Clinical Trial for Gene Transfer Candidate SGT-001 in Patients with Duchenne Muscular Dystrophy

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COMPANIES MENTIONED

Daiichi Sankyo Co Ltd

Editas Medicine Inc

Genethon SA

NS Pharma Inc

Pfizer Inc

Sarepta Therapeutics Inc

WAVE Life Sciences Ltd

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