

Spinal Muscular Atrophy (SMA) Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

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Abstracts

Spinal Muscular Atrophy (SMA) Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Spinal Muscular Atrophy (SMA) - Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update, provides an overview of the Spinal Muscular Atrophy (SMA) (Central Nervous System) pipeline landscape.

Spinal muscular atrophy (SMA) is a genetic disease that attacks nerve cells, called motor neurons, in the spinal cord. These critically important cells are responsible for supplying electrical and chemical messages to muscle cells. Without the proper input from the motor neurons, muscle cells cannot function properly. The muscle cells will, therefore, become much smaller (atrophy) and will produce symptoms of muscle weakness. This can affect walking, crawling, breathing, swallowing, and head and neck control.

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Spinal Muscular Atrophy (SMA) - Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update, provides comprehensive information on the therapeutics under development for Spinal Muscular Atrophy (SMA) (Central Nervous System), complete with analysis by stage of development, drug target,

Spinal Muscular Atrophy (SMA) Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players,...

mechanism of action (MoA), route of administration (RoA) and molecule type. The guide covers the descriptive pharmacological action of the therapeutics, its complete research and development history and latest news and press releases.

The Spinal Muscular Atrophy (SMA) (Central Nervous System) pipeline guide also reviews of key players involved in therapeutic development for Spinal Muscular Atrophy (SMA) and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies /Universities /Institutes, the molecules developed by Companies in Pre-Registration, Phase III, Phase II, Phase I, IND/CTA Filed, Preclinical, Discovery and Unknown stages are 2, 1, 5, 5, 1, 23, 9 and 1 respectively. Similarly, the Universities portfolio in Phase II, Preclinical and Discovery stages comprises 2, 8 and 2 molecules, respectively.

Spinal Muscular Atrophy (SMA) (Central Nervous System) pipeline guide helps in identifying and tracking emerging players in the market and their portfolios, enhances decision making capabilities and helps to create effective counter strategies to gain competitive advantage. The guide is built using data and information sourced from Global Markets Direct's 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. Additionally, various dynamic tracking processes ensure that the most recent developments are captured on a real time basis.

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

SCOPE

The pipeline guide provides a snapshot of the global therapeutic landscape of Spinal Muscular Atrophy (SMA) (Central Nervous System).

The pipeline guide reviews pipeline therapeutics for Spinal Muscular Atrophy (SMA) (Central Nervous System) by companies and universities/research institutes based on information derived from company and industry-specific sources.

The pipeline guide covers pipeline products based on several stages of development ranging from pre-registration till discovery and undisclosed stages.

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

The pipeline guide reviews key companies involved in Spinal Muscular Atrophy (SMA) (Central Nervous System) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Spinal Muscular Atrophy (SMA) (Central Nervous System) therapeutics based on mechanism of action (MoA), drug target, route of administration (RoA) and molecule type.

The pipeline guide encapsulates all the dormant and discontinued pipeline projects.

The pipeline guide reviews latest news related to pipeline therapeutics for Spinal Muscular Atrophy (SMA) (Central Nervous System)

REASONS TO BUY

Procure strategically important competitor information, analysis, and insights to formulate effective R&D strategies.

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

Find and recognize significant and varied types of therapeutics under development for Spinal Muscular Atrophy (SMA) (Central Nervous System).

Classify potential new clients or partners in the target demographic.

Develop tactical initiatives by understanding the focus areas of leading companies.

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

Formulate corrective measures for pipeline projects by understanding Spinal

Muscular Atrophy (SMA) (Central Nervous System) pipeline depth and focus of Indication therapeutics.

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.

Adjust the therapeutic portfolio by recognizing discontinued projects and understand from the know-how what drove them from pipeline.

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Apteeus SAS

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Beijing GeneCradle Technology Co Ltd

Biocad

Biogen Inc

Biohaven Pharmaceutical Holding Company Ltd

Bioleaders Corp

BioMarin Pharmaceutical Inc

Biophytis SA

Cell Tech Pharmed Co

Chugai Pharmaceutical Co Ltd

Enzerna Biosciences LLC

Exegensis Bio Inc

F. Hoffmann-La Roche Ltd

Genentech USA Inc

GNT Pharma Co Ltd

Huida (Shanghai) Biotechnology Co Ltd

Imago Pharmaceuticals Inc
Myoceia Inc
Neurotune AG
NMD Pharma AS
Novartis AG
Novartis Gene Therapies
Oncternal Therapeutics
Ono Pharmaceutical Co Ltd
PTC Therapeutics Inc
Ractigen Therapeutics Inc
Reborna Biosciences Inc
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Featured News & Press Releases

Mar 22, 2022: Scholar Rock presents data analysis of multiple efficacy endpoints from the apitegromab TOPAZ phase 2 trial at the American Academy of Neurology 2022 Annual Meeting

Mar 16, 2022: New data for Roche's Evrysdi (risdiplam) demonstrate long-term efficacy and safety in a broad population of people with spinal muscular atrophy (SMA)

Mar 16, 2022: New data for Genentech's Evrysdi (risdiplam) demonstrate long-term efficacy and safety in a broad population of people with Spinal Muscular Atrophy (SMA)

Mar 13, 2022: Scholar Rock to present data from TOPAZ Ambulatory Cohort Analysis at the 2022 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference

Mar 13, 2022: Novartis data again demonstrate age-appropriate development when Zolgensma is used presymptomatically, and post-hoc data reveal SMA Type 1 patients could speak, swallow and maintain airway protection

Mar 08, 2022: Roche to present new Evrysdi data at MDA 2022 and highlight expanding neuromuscular disease portfolio

Feb 03, 2022: Roche Canada and the pan-Canadian Pharmaceutical Alliance (pCPA) successfully complete negotiations for EVRYSDI (risdiplam) for the treatment of adults and children with spinal muscular atrophy (SMA)

Jan 31, 2022: Cost-effectiveness analysis temporarily halted for Zolgensma to collect long-term data

Jan 25, 2022: Roche's Evrysdi (risdiplam) granted FDA priority review for treatment of pre-symptomatic babies under 2 months of age with spinal muscular atrophy (SMA)

Nov 30, 2021: Scholar Rock announces design of phase 3 SAPPHIRE clinical trial evaluating apitegromab in non-ambulatory patients with Type 2 and Type 3 spinal muscular atrophy (SMA)

Nov 19, 2021: NICE draft guidance recommends new treatment for spinal muscular atrophy as part of a managed access agreement

Oct 19, 2021: Novartis applauds Quebec, the first province to list Zolgensma for the treatment of pediatric patients with spinal muscular atrophy (SMA)

Oct 13, 2021: Novartis and the pan-Canadian Pharmaceutical Alliance (pCPA) complete negotiations for Zolgensma for the treatment of pediatric patients with spinal muscular atrophy (SMA)

Oct 03, 2021: Scholar Rock to present Apitegromab TOPAZ phase 2 pharmacologic data at the 2021 World Congress of Neurology

Sep 30, 2021: Scholar Rock presents exploratory responder analysis on efficacy data from the apitegromab TOPAZ phase 2 trial at the Child Neurology Society Annual Meeting

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