

# Spinal Muscular Atrophy (SMA) - Pipeline Review, H2 2020

<https://marketpublishers.com/r/S133834AAAFEN.html>

Date: August 2020

Pages: 164

Price: US\$ 2,000.00 (Single User License)

ID: S133834AAAFEN

## Abstracts

Spinal Muscular Atrophy (SMA) - Pipeline Review, H2 2020

### Summary

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Spinal Muscular Atrophy - Pipeline Review, H2 2020, provides an overview of the Spinal Muscular Atrophy (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 - Pipeline Review, H2 2020, provides comprehensive information on the therapeutics under development for Spinal Muscular Atrophy (Central Nervous System), complete with analysis by stage of development, drug target, 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 (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, Preclinical and Discovery stages are 1, 2, 4, 3, 18 and 5 respectively. Similarly, the Universities portfolio in Phase II, Preclinical and Discovery stages comprises 2, 8 and 2 molecules, respectively.

Spinal Muscular Atrophy (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 (Central Nervous System).

The pipeline guide reviews pipeline therapeutics for Spinal Muscular Atrophy (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 (Central Nervous System) therapeutics and enlists all their major and minor

projects.

The pipeline guide evaluates Spinal Muscular Atrophy (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 (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 (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 (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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### COMPANIES MENTIONED

Allianz Pharmascience Ltd  
AlphaCognition Inc  
Anima Biotech Inc  
Armgo Pharma Inc  
AveXis Inc  
Biogen Inc  
Bioleaders Corp  
BioMarin Pharmaceutical Inc  
Biophytis SA  
Cell Tech Pharmed Co  
Enzerna Biosciences LLC  
Exicure Inc  
F. Hoffmann-La Roche Ltd  
Genea Biocells  
GNT Pharma Co Ltd  
Imago Pharmaceuticals Inc  
Kowa Co Ltd  
Neurotune AG  
Novartis AG  
Ono Pharmaceutical Co Ltd  
Ractigen Therapeutics Inc

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Sarepta Therapeutics Inc  
Scholar Rock Inc  
Shift Pharmaceuticals  
Spotlight Innovation Inc  
Vybion Inc  
Xcelthera INC



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