

Spinal muscular atrophy - Pipeline Insight, 2021

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Abstracts

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DelveInsight's, "Spinal muscular atrophy - Pipeline Insight, 2021," report provides comprehensive insights about 18+ companies and 18+ pipeline drugs in Spinal muscular atrophy pipeline landscape. It covers the pipeline drug profiles, including clinical and nonclinical stage products. It also covers the therapeutics assessment by product type, stage, route of administration, and molecule type. It further highlights the inactive pipeline products in this space.

Geography Covered

Global coverage

Spinal muscular atrophy Understanding

Spinal muscular atrophy: Overview

Spinal muscular atrophy (SMA) is a group of inherited disorders characterized by a loss of certain nerve cells in the spinal cord called motor neurons or anterior horn cells. Motor neurons receive the nerve impulses transmitted from the brain to the spinal cord (brainstem) and, in turn, transmit the impulses to the muscle via the peripheral nerves. The loss of motor neurons leads to progressive muscle weakness and muscle wasting (atrophy) in muscles closest to the trunk of the body (proximal muscles) such as the shoulders, hips and back. These muscles are necessary for crawling, walking, sitting up and head control. The more severe types of SMA can affect muscles involved in feeding, swallowing and breathing.

'Spinal muscular atrophy - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Spinal muscular atrophy pipeline landscape is provided which includes the disease overview and Spinal muscular atrophy treatment guidelines. The assessment part of the report embraces, in depth Spinal muscular atrophy commercial assessment and clinical assessment of the pipeline products under development. In the report, detailed description of the drug is given which includes mechanism of action of the drug, clinical studies, NDA approvals (if any), and product development activities comprising the technology, Spinal muscular atrophy collaborations, licensing, mergers and acquisition, funding, designations and other product related details.

Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Spinal muscular atrophy R&D. The therapies under development are focused on novel approaches to treat/improve Spinal muscular atrophy.

Spinal muscular atrophy Emerging Drugs Chapters

This segment of the Spinal muscular atrophy report encloses its detailed analysis of various drugs in different stages of clinical development, including phase II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological action, agreements and collaborations, and the latest news and press releases.

Spinal muscular atrophy Emerging Drugs

Apitegromab: Scholar Rock

Apitegromab is a selective inhibitor of the activation of myostatin and is an investigational product candidate for the treatment of patients with spinal muscular atrophy (SMA). Myostatin, a member of the TGF β superfamily of growth factors, is expressed primarily by skeletal muscle cells, and the absence of its gene is associated with an increase in muscle mass and strength in multiple animal species, including humans. Scholar Rock believes that inhibiting myostatin activation with apitegromab may promote a clinically meaningful improvement in motor function in patients with

SMA. The U.S. Food and Drug Administration (FDA) has granted Fast Track (FTD), Orphan Drug (ODD) and Rare Pediatric Disease (RPD) designations, and the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) and Orphan Medicinal Product designations, to apitegromab for the treatment of SMA. Currently, it is in phase II stage of development.

BIIB110: Biogen

BIIB110 is a hybrid activin II receptor (ACTIIR) ligand trap that sequesters both myostatin and activins while sparing the related ligand bone morphogen protein 9 (BMP9). This targeted mechanism of action may result in greater muscle mass, function and improved safety compared to other myostatin inhibition approaches. It is currently in phase I stage of development.

Further product details are provided in the report.....

Spinal muscular atrophy: Therapeutic Assessment

This segment of the report provides insights about the different Spinal muscular atrophy drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Spinal muscular atrophy

There are approx. 18+ key companies which are developing the therapies for Spinal muscular atrophy. The companies which have their Spinal muscular atrophy drug candidates in the most advanced stage, i.e. phase II include, Scholar Rock.

Phases

DelveInsight's report covers around 18+ products under different phases of clinical development like

Late stage products (Phase III)

Mid-stage products (Phase II)

Early-stage product (Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

Spinal muscular atrophy pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

Oral

Parenteral

intravenous

Subcutaneous

Topical.

Molecule Type

Products have been categorized under various Molecule types such as

Monoclonal Antibody

Peptides

Polymer

Small molecule

Gene therapy

Product Type

Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.

Spinal muscular atrophy: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Spinal muscular atrophy therapeutic drugs key players involved in developing key drugs.

Pipeline Development Activities

The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Spinal muscular atrophy drugs.

Spinal muscular atrophy Report Insights

Spinal muscular atrophy Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Spinal muscular atrophy Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

Unmet Needs

Key Questions

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Spinal muscular atrophy drugs?

How many Spinal muscular atrophy drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Spinal muscular atrophy?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Spinal muscular atrophy therapeutics?

What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Spinal muscular atrophy and their status?

What are the key designations that have been granted to the emerging drugs?

Key Players

Scholar Rock

Biogen

Astellas Pharma

AurimMed Pharma

Alcyone Therapeutics

Kowa

Helixmith

Novartis

PTC Therapeutics

AndroScience Corporation

Catalyst Pharmaceuticals

Key Products

Apitegromab

BIIB 110

Reldesemtiv

ACTX 401

Sodium valproate

VM 101

BVS 857

NVS-SM1

SMN-C1

ASCJ 9

Contents

- Introduction
- Executive Summary
- Spinal muscular atrophy: Overview
 - Causes
 - Mechanism of Action
 - Signs and Symptoms
 - Diagnosis
 - Disease Management
- Pipeline Therapeutics
 - Comparative Analysis
- Therapeutic Assessment
 - Assessment by Product Type
 - Assessment by Stage and Product Type
 - Assessment by Route of Administration
 - Assessment by Stage and Route of Administration
 - Assessment by Molecule Type
 - Assessment by Stage and Molecule Type
- Late Stage Products (Phase III)
 - Comparative Analysis
- Sodium valproate: Kowa
 - Product Description
 - Research and Development
 - Product Development Activities
- Drug profiles in the detailed report.....
- Mid Stage Products (Phase II)
 - Comparative Analysis
- Apitegromab: Scholar Rock
 - Product Description
 - Research and Development
 - Product Development Activities
- Drug profiles in the detailed report.....

- Early Stage Products (Phase I)
 - Comparative Analysis
- BIIB 110: Biogen
 - Product Description
 - Research and Development

Product Development Activities
Drug profiles in the detailed report.....
Preclinical and Discovery Stage Products
 Comparative Analysis
SMN-C1: PTC Therapeutics
 Product Description
 Research and Development
 Product Development Activities
Drug profiles in the detailed report.....

Inactive Products
 Comparative Analysis
Spinal muscular atrophy Key Companies
Spinal muscular atrophy Key Products
Spinal muscular atrophy- Unmet Needs
Spinal muscular atrophy- Market Drivers and Barriers
Spinal muscular atrophy- Future Perspectives and Conclusion
Spinal muscular atrophy Analyst Views
Spinal muscular atrophy Key Companies
Appendix

List Of Tables

LIST OF TABLES

- Table 1 Total Products for Spinal muscular atrophy
- Table 2 Late Stage Products
- Table 3 Mid Stage Products
- Table 4 Early Stage Products
- Table 5 Pre-clinical & Discovery Stage Products
- Table 6 Assessment by Product Type
- Table 7 Assessment by Stage and Product Type
- Table 8 Assessment by Route of Administration
- Table 9 Assessment by Stage and Route of Administration
- Table 10 Assessment by Molecule Type
- Table 11 Assessment by Stage and Molecule Type
- Table 12 Inactive Products

List Of Figures

LIST OF FIGURES

- Figure 1 Total Products for Spinal muscular atrophy
- Figure 2 Late Stage Products
- Figure 3 Mid Stage Products
- Figure 4 Early Stage Products
- Figure 5 Preclinical and Discovery Stage Products
- Figure 6 Assessment by Product Type
- Figure 7 Assessment by Stage and Product Type
- Figure 8 Assessment by Route of Administration
- Figure 9 Assessment by Stage and Route of Administration
- Figure 10 Assessment by Molecule Type
- Figure 11 Assessment by Stage and Molecule Type
- Figure 12 Inactive Products

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