

# 2020 Spinal Muscular Atrophy (SMA) Drug Pipeline Report- Current Status, Phase, Mechanism, Route of Administration, and Companies, of Pre-Clinical And Clinical Drugs

<https://marketpublishers.com/r/233291A77C2EEN.html>

Date: February 2020

Pages: 116

Price: US\$ 1,999.00 (Single User License)

ID: 233291A77C2EEN

## Abstracts

The 2020 Spinal Muscular Atrophy (SMA) pipeline report presents a comprehensive overview of the research and development of Spinal Muscular Atrophy (SMA) drug candidates. It presents drugs in development that could potentially reach the market in the next 5 to 10 years: Four drugs in Research Phase, 17 drugs in Pre-clinical phase, three drugs in Phase 1, four drugs in Phase 2 and two drugs in Phase 3

As of February 2020, the Spinal Muscular Atrophy (SMA) pipeline remains robust with 30 therapeutic candidates under development. An increasing number of companies are actively participating in the development of Spinal Muscular Atrophy (SMA) treatment. Diverse types of targeted therapies are being explored through clinical trials including Acetylcholine stimulant; activin II receptor antagonist; GABA transaminase inhibitor; MAS receptor agonists; myostatin inhibitor; Nerve tissue protein stimulants; oxygen species (ROS) inhibitor; Potassium channel antagonist; Ryanodine receptor calcium release channel modulators; SMN protein activator; survival motor neuron 2 (SMN2) protein modulator; Troponin stimulants.

The report provides complete details of pipeline drugs including the development phase, mechanism of action, companies involved, clinical trial developments, molecule type, and other details. Further, the report also provides Spinal Muscular Atrophy (SMA) drug development history, latest news, and other developments.

It assists companies, governments, investors and research organizations to understand the current status in 2020 and possible development in the next 10 years. Further, it

enables readers to track new companies in the market and their developments. The product portfolio of different companies and their growth strategies are also detailed in the report.

## **PUBLISHER EXPERTISE**

VPAResearch online databases analyze pipeline drugs and developments for over 2,000 diseases worldwide. All our reports and databases are developed through intensive primary and secondary research methods. The insights and data presented in the databases are validated through industry experts and represent completely unbiased opinions.

## **SCOPE:**

The report scope comprises of both pre-clinical phase and clinical phase development drugs for Spinal Muscular Atrophy (SMA) development

Spinal Muscular Atrophy (SMA) pipeline compounds and molecules under study by both large scale and small companies are included in the report

Spinal Muscular Atrophy (SMA) pipeline across different phases including discovery, research, and pre-clinical stage, phase 1, phase 2, phase 3 and pre-registration phases are covered

Drug profile comprising of current development status, regulatory progress, companies, sponsors, mechanism of action, route of administration, molecule, and discovery details are covered

Further, orphan drug status, fast track designation, different grants awarded and special status for Spinal Muscular Atrophy (SMA) pipeline candidates included

Business overview and snapshot of all companies involved in Spinal Muscular Atrophy (SMA) pipeline are included

Latest market and pipeline developments are provided in the report

Spinal Muscular Atrophy (SMA) pipeline companies included in the report are- Allianz Pharmascience Ltd, Armgo Pharma Inc, AurimMed Pharma Inc, Biogen Inc, BioMarin

Pharmaceutical Inc, Biophytis SA, Cytokinetics Inc, Exicure Inc, Genea Biocells, Genethon SA, GNT Pharma Co Ltd, Kowa Co Ltd, Neurodyn Life Sciences Inc, Neurotune AG, Novartis AG, Ono Pharmaceutical Co Ltd, PTC Therapeutics Inc, Ractigen Therapeutics Inc, Reborna Biosciences Inc, Recursion Pharmaceuticals Inc, Sarepta Therapeutics Inc, Scholar Rock Inc, Skyhawk Therapeutics Inc, Spotlight Innovation Inc, Vybion Inc, Xcelthera INC

Spinal Muscular Atrophy (SMA) pipeline drugs profiled in the report include- ALZ002, ARM210, SMN2 target for Spinal Muscular Atrophy, ALG-802, BIIB110, amifampridine, BIO-103, reldesemtiv (CK-2127107), SMN2 mRNA for Spinal Muscular Atrophy, Small Molecules for Spinal Muscular Atrophy, SMN1 gene therapy for spinal muscular atrophy, AAD-2004 (Crisdesalazine), Sodium valproate (K-828-SP), ND602, NT-1654, branaplam (LMI070), maresin 1, risdiplam, RAG-06, TEC-1, REC-0000716, REC-0001202, PMO25, SRK-105, Small Molecule for Spinal Muscular Atrophy, LDN-5178, STL-182, INT41, Xcel-hNu, Xcel-hNuP

## Contents

### 1. TABLE OF CONTENTS

- 1.1 List of Tables
- 1.2 List of Figures

### 2. EXECUTIVE SUMMARY

- 2.1 Report Scope and Research Methodology
- 2.2 Introduction to Spinal Muscular Atrophy (SMA) Condition
- 2.3 Spinal Muscular Atrophy (SMA) Pipeline Snapshot, 2020
- 2.4 Companies investing in Spinal Muscular Atrophy (SMA) pipeline therapeutics
- 2.5 Phase wise Spinal Muscular Atrophy (SMA) Pipeline Candidates
- 2.6 Most Researched Mechanism of Action of Spinal Muscular Atrophy (SMA) Pipeline Products
- 2.7 Route of Administration of Spinal Muscular Atrophy (SMA) Pipeline Drugs

### 3. COMPANIES ACTIVE IN PIPELINE DEVELOPMENT

- 3.1 Allianz Pharmascience Ltd Overview, Contacts and ASD Pipeline Drugs
- 3.2 Armgo Pharma Inc Overview, Contacts and ASD Pipeline Drugs
- 3.3 AurimMed Pharma Inc Overview, Contacts and ASD Pipeline Drugs
- 3.4 Biogen Inc Overview, Contacts and ASD Pipeline Drugs
- 3.5 BioMarin Pharmaceutical Inc Overview, Contacts and ASD Pipeline Drugs
- 3.6 Biophytis SA Overview, Contacts and ASD Pipeline Drugs
- 3.7 Cytokinetics Inc Overview, Contacts and ASD Pipeline Drugs
- 3.8 Exicure Inc Overview, Contacts and ASD Pipeline Drugs
- 3.9 Genea Biocells Overview, Contacts and ASD Pipeline Drugs
- 3.10 Genethon SA Overview, Contacts and ASD Pipeline Drugs
- 3.11 GNT Pharma Co Ltd Overview, Contacts and ASD Pipeline Drugs
- 3.12 Kowa Co Ltd Overview, Contacts and ASD Pipeline Drugs
- 3.13 Neurodyn Life Sciences Inc Overview, Contacts and ASD Pipeline Drugs
- 3.14 Neurotune AG Overview, Contacts and ASD Pipeline Drugs
- 3.15 Novartis AG Overview, Contacts and ASD Pipeline Drugs
- 3.16 Ono Pharmaceutical Co Ltd Overview, Contacts and ASD Pipeline Drugs
- 3.17 PTC Therapeutics Inc Overview, Contacts and ASD Pipeline Drugs
- 3.18 Ractigen Therapeutics Inc Overview, Contacts and ASD Pipeline Drugs
- 3.19 Reborna Biosciences Inc Overview, Contacts and ASD Pipeline Drugs

- 3.20 Recursion Pharmaceuticals Inc Overview, Contacts and ASD Pipeline Drugs
- 3.21 Sarepta Therapeutics Inc Overview, Contacts and ASD Pipeline Drugs
- 3.22 Scholar Rock Inc Overview, Contacts and ASD Pipeline Drugs
- 3.23 Skyhawk Therapeutics Inc Overview, Contacts and ASD Pipeline Drugs
- 3.24 Spotlight Innovation Inc Overview, Contacts and ASD Pipeline Drugs
- 3.25 Vybion Inc Overview, Contacts and ASD Pipeline Drugs
- 3.26 Xcelthera INC Overview, Contacts and ASD Pipeline Drugs

#### **4. ACTIVE PIPELINE DRUG DETAILS, 2020**

- 4.1 ALZ002 Drug Details
  - 4.1.1 ALZ002 Current Status
  - 4.1.2 ALZ002 Drug Overview
  - 4.1.3 ALZ002 Mechanism of Action
  - 4.1.4 ALZ002 Licensing/Collaboration Companies
  - 4.1.5 ALZ002 Clinical Trials
- 4.2 ARM210 Drug Details
  - 4.2.1 ARM210 Current Status
  - 4.2.2 ARM210 Drug Overview
  - 4.2.3 ARM210 Mechanism of Action
  - 4.2.4 ARM210 Licensing/Collaboration Companies
  - 4.2.5 ARM210 Clinical Trials
- 4.3 SMN2 target for Spinal Muscular Atrophy Drug Details
  - 4.3.1 SMN2 target for Spinal Muscular Atrophy Current Status
  - 4.3.2 SMN2 target for Spinal Muscular Atrophy Drug Overview
  - 4.3.3 SMN2 target for Spinal Muscular Atrophy Mechanism of Action
  - 4.3.4 SMN2 target for Spinal Muscular Atrophy Licensing/Collaboration Companies
  - 4.3.5 SMN2 target for Spinal Muscular Atrophy Clinical Trials
- 4.4 ALG-802 Drug Details
  - 4.4.1 ALG-802 Current Status
  - 4.4.2 ALG-802 Drug Overview
  - 4.4.3 ALG-802 Mechanism of Action
  - 4.4.4 ALG-802 Licensing/Collaboration Companies
  - 4.4.5 ALG-802 Clinical Trials
- 4.5 BIIB110 Drug Details
  - 4.5.1 BIIB110 Current Status
  - 4.5.2 BIIB110 Drug Overview
  - 4.5.3 BIIB110 Mechanism of Action
  - 4.5.4 BIIB110 Licensing/Collaboration Companies

- 4.5.5 BIIB110 Clinical Trials
- 4.6 amifampridine Drug Details
  - 4.6.1 amifampridine Current Status
  - 4.6.2 amifampridine Drug Overview
  - 4.6.3 amifampridine Mechanism of Action
  - 4.6.4 amifampridine Licensing/Collaboration Companies
  - 4.6.5 amifampridine Clinical Trials
- 4.7 BIO-103 Drug Details
  - 4.7.1 BIO-103 Current Status
  - 4.7.2 BIO-103 Drug Overview
  - 4.7.3 BIO-103 Mechanism of Action
  - 4.7.4 BIO-103 Licensing/Collaboration Companies
  - 4.7.5 BIO-103 Clinical Trials
- 4.8 reldesemtiv (CK-2127107) Drug Details
  - 4.8.1 reldesemtiv (CK-2127107) Current Status
  - 4.8.2 reldesemtiv (CK-2127107) Drug Overview
  - 4.8.3 reldesemtiv (CK-2127107) Mechanism of Action
  - 4.8.4 reldesemtiv (CK-2127107) Licensing/Collaboration Companies
  - 4.8.5 reldesemtiv (CK-2127107) Clinical Trials
- 4.9 SMN2 mRNA for Spinal Muscular Atrophy Drug Details
  - 4.9.1 SMN2 mRNA for Spinal Muscular Atrophy Current Status
  - 4.9.2 SMN2 mRNA for Spinal Muscular Atrophy Drug Overview
  - 4.9.3 SMN2 mRNA for Spinal Muscular Atrophy Mechanism of Action
  - 4.9.4 SMN2 mRNA for Spinal Muscular Atrophy Licensing/Collaboration Companies
  - 4.9.5 SMN2 mRNA for Spinal Muscular Atrophy Clinical Trials
- 4.10 Small Molecules for Spinal Muscular Atrophy Drug Details
  - 4.10.1 Small Molecules for Spinal Muscular Atrophy Current Status
  - 4.10.2 Small Molecules for Spinal Muscular Atrophy Drug Overview
  - 4.10.3 Small Molecules for Spinal Muscular Atrophy Mechanism of Action
  - 4.10.4 Small Molecules for Spinal Muscular Atrophy Licensing/Collaboration Companies
  - 4.10.5 Small Molecules for Spinal Muscular Atrophy Clinical Trials
- 4.11 SMN1 gene therapy for spinal muscular atrophy Drug Details
  - 4.11.1 SMN1 gene therapy for spinal muscular atrophy Current Status
  - 4.11.2 SMN1 gene therapy for spinal muscular atrophy Drug Overview
  - 4.11.3 SMN1 gene therapy for spinal muscular atrophy Mechanism of Action
  - 4.11.4 SMN1 gene therapy for spinal muscular atrophy Licensing/Collaboration Companies
  - 4.11.5 SMN1 gene therapy for spinal muscular atrophy Clinical Trials



- 4.12 AAD-2004 (Crisdesalazine) Drug Details
  - 4.12.1 AAD-2004 (Crisdesalazine) Current Status
  - 4.12.2 AAD-2004 (Crisdesalazine) Drug Overview
  - 4.12.3 AAD-2004 (Crisdesalazine) Mechanism of Action
  - 4.12.4 AAD-2004 (Crisdesalazine) Licensing/Collaboration Companies
  - 4.12.5 AAD-2004 (Crisdesalazine) Clinical Trials
- 4.13 Sodium valproate (K-828-SP) Drug Details
  - 4.13.1 Sodium valproate (K-828-SP) Current Status
  - 4.13.2 Sodium valproate (K-828-SP) Drug Overview
  - 4.13.3 Sodium valproate (K-828-SP) Mechanism of Action
  - 4.13.4 Sodium valproate (K-828-SP) Licensing/Collaboration Companies
  - 4.13.5 Sodium valproate (K-828-SP) Clinical Trials
- 4.14 ND602 Drug Details
  - 4.14.1 ND602 Current Status
  - 4.14.2 ND602 Drug Overview
  - 4.14.3 ND602 Mechanism of Action
  - 4.14.4 ND602 Licensing/Collaboration Companies
  - 4.14.5 ND602 Clinical Trials
- 4.15 NT-1654 Drug Details
  - 4.15.1 NT-1654 Current Status
  - 4.15.2 NT-1654 Drug Overview
  - 4.15.3 NT-1654 Mechanism of Action
  - 4.15.4 NT-1654 Licensing/Collaboration Companies
  - 4.15.5 NT-1654 Clinical Trials
- 4.16 branaplam (LMI070) Drug Details
  - 4.16.1 branaplam (LMI070) Current Status
  - 4.16.2 branaplam (LMI070) Drug Overview
  - 4.16.3 branaplam (LMI070) Mechanism of Action
  - 4.16.4 branaplam (LMI070) Licensing/Collaboration Companies
  - 4.16.5 branaplam (LMI070) Clinical Trials
- 4.17 maresin 1 Drug Details
  - 4.17.1 maresin 1 Current Status
  - 4.17.2 maresin 1 Drug Overview
  - 4.17.3 maresin 1 Mechanism of Action
  - 4.17.4 maresin 1 Licensing/Collaboration Companies
  - 4.17.5 maresin 1 Clinical Trials
- 4.18 risdiplam Drug Details
  - 4.18.1 risdiplam Current Status
  - 4.18.2 risdiplam Drug Overview

- 4.18.3 risdiplam Mechanism of Action
- 4.18.4 risdiplam Licensing/Collaboration Companies
- 4.18.5 risdiplam Clinical Trials
- 4.19 RAG-06 Drug Details
  - 4.19.1 RAG-06 Current Status
  - 4.19.2 RAG-06 Drug Overview
  - 4.19.3 RAG-06 Mechanism of Action
  - 4.19.4 RAG-06 Licensing/Collaboration Companies
  - 4.19.5 RAG-06 Clinical Trials
- 4.20 TEC-1 Drug Details
  - 4.20.1 TEC-1 Current Status
  - 4.20.2 TEC-1 Drug Overview
  - 4.20.3 TEC-1 Mechanism of Action
  - 4.20.4 TEC-1 Licensing/Collaboration Companies
  - 4.20.5 TEC-1 Clinical Trials
- 4.21 REC-0000716 Drug Details
  - 4.21.1 REC-0000716 Current Status
  - 4.21.2 REC-0000716 Drug Overview
  - 4.21.3 REC-0000716 Mechanism of Action
  - 4.21.4 REC-0000716 Licensing/Collaboration Companies
  - 4.21.5 REC-0000716 Clinical Trials
- 4.22 REC-0001202 Drug Details
  - 4.22.1 REC-0001202 Current Status
  - 4.22.2 REC-0001202 Drug Overview
  - 4.22.3 REC-0001202 Mechanism of Action
  - 4.22.4 REC-0001202 Licensing/Collaboration Companies
  - 4.22.5 REC-0001202 Clinical Trials
- 4.23 PMO25 Drug Details
  - 4.23.1 PMO25 Current Status
  - 4.23.2 PMO25 Drug Overview
  - 4.23.3 PMO25 Mechanism of Action
  - 4.23.4 PMO25 Licensing/Collaboration Companies
  - 4.23.5 PMO25 Clinical Trials
- 4.24 SRK-105 Drug Details
  - 4.24.1 SRK-105 Current Status
  - 4.24.2 SRK-105 Drug Overview
  - 4.24.3 SRK-105 Mechanism of Action
  - 4.24.4 SRK-105 Licensing/Collaboration Companies
  - 4.24.5 SRK-105 Clinical Trials



- 4.25 Small Molecule for Spinal Muscular Atrophy Drug Details
  - 4.25.1 Small Molecule for Spinal Muscular Atrophy Current Status
  - 4.25.2 Small Molecule for Spinal Muscular Atrophy Drug Overview
  - 4.25.3 Small Molecule for Spinal Muscular Atrophy Mechanism of Action
  - 4.25.4 Small Molecule for Spinal Muscular Atrophy Licensing/Collaboration Companies
  - 4.25.5 Small Molecule for Spinal Muscular Atrophy Clinical Trials
- 4.26 LDN-5178 Drug Details
  - 4.26.1 LDN-5178 Current Status
  - 4.26.2 LDN-5178 Drug Overview
  - 4.26.3 LDN-5178 Mechanism of Action
  - 4.26.4 LDN-5178 Licensing/Collaboration Companies
  - 4.26.5 LDN-5178 Clinical Trials
- 4.27 STL-182 Drug Details
  - 4.27.1 STL-182 Current Status
  - 4.27.2 STL-182 Drug Overview
  - 4.27.3 STL-182 Mechanism of Action
  - 4.27.4 STL-182 Licensing/Collaboration Companies
  - 4.27.5 STL-182 Clinical Trials
- 4.28 INT41 Drug Details
  - 4.28.1 INT41 Current Status
  - 4.28.2 INT41 Drug Overview
  - 4.28.3 INT41 Mechanism of Action
  - 4.28.4 INT41 Licensing/Collaboration Companies
  - 4.28.5 INT41 Clinical Trials
- 4.29 Xcel-hNu Drug Details
  - 4.29.1 Xcel-hNu Current Status
  - 4.29.2 Xcel-hNu Drug Overview
  - 4.29.3 Xcel-hNu Mechanism of Action
  - 4.29.4 Xcel-hNu Licensing/Collaboration Companies
  - 4.29.5 Xcel-hNu Clinical Trials
- 4.30 Xcel-hNuP Drug Details
  - 4.30.1 Xcel-hNuP Current Status
  - 4.30.2 Xcel-hNuP Drug Overview
  - 4.30.3 Xcel-hNuP Mechanism of Action
  - 4.30.4 Xcel-hNuP Licensing/Collaboration Companies
  - 4.30.5 Xcel-hNuP Clinical Trials

## **5. LATEST SPINAL MUSCULAR ATROPHY (SMA) PIPELINE NEWS AND DEALS**

## 6. APPENDIX

6.1 Our Databases and Reports

6.2 Research Methodolgy

## I would like to order

Product name: 2020 Spinal Muscular Atrophy (SMA) Drug Pipeline Report- Current Status, Phase, Mechanism, Route of Administration, and Companies, of Pre-Clinical And Clinical Drugs

Product link: <https://marketpublishers.com/r/233291A77C2EEN.html>

Price: US\$ 1,999.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer Service:

[info@marketpublishers.com](mailto:info@marketpublishers.com)

## Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <https://marketpublishers.com/r/233291A77C2EEN.html>

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name:  
Last name:  
Email:  
Company:  
Address:  
City:  
Zip code:  
Country:  
Tel:  
Fax:  
Your message:

**\*\*All fields are required**

Customer signature \_\_\_\_\_

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at <https://marketpublishers.com/docs/terms.html>

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970

