

# **Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) - Pipeline Review, H1 2019**

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## **Abstracts**

Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) - Pipeline Review, H1 2019

### **SUMMARY**

According to the recently published report 'Survival Motor Neuron Protein - Pipeline Review, H1 2019'; Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) pipeline Target constitutes close to 18 molecules. Out of which approximately 11 molecules are developed by companies and remaining by the universities/institutes.

Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) - Survival of motor neuron or survival motor neuron (SMN) is a protein encoded by the SMN1 and SMN2 genes. The SMN complex plays a catalyst role in the assembly of small nuclear ribonucleoproteins (snRNPs). They play an important role in the splicing of cellular pre-mRNAs. They ensure the correct splicing of U12 intron-containing genes that is important for normal motor and proprioceptive neurons development. They are required for resolving RNA-DNA hybrids created by RNA polymerase II. They play a role in the metabolism of small nucleolar ribonucleoprotein.

The report 'Survival Motor Neuron Protein - Pipeline Review, H1 2019' outlays comprehensive information on the Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type; that are being developed by Companies/Universities.

It also reviews key players involved in Survival Motor Neuron Protein (Component Of Gens 1 or Gemin 1 or SMN1 or SMN2) targeted therapeutics development with respective active and dormant or discontinued projects. Currently, The molecules developed by companies in Pre-Registration, Phase III, Phase II, Phase I and Preclinical stages are 1, 1, 1, 1 and 7 respectively. Similarly, the universities portfolio in Preclinical and Discovery stages comprises 5 and 2 molecules, respectively. Report covers products from therapy areas Central Nervous System and Respiratory which include indications Spinal Muscular Atrophy (SMA) and Cystic Fibrosis.

**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 Survival Motor Neuron Protein (Component Of Gens 1 or Gemin 1 or SMN1 or SMN2)

The report reviews Survival Motor Neuron Protein (Component Of Gens 1 or Gemin 1 or SMN1 or SMN2) 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 Survival Motor Neuron Protein (Component Of Gens 1 or Gemin 1 or SMN1 or SMN2) targeted therapeutics and enlists all their major and minor projects

The report assesses Survival Motor Neuron Protein (Component Of Gens 1 or Gemin 1 or SMN1 or SMN2) 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 Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) 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 Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2)

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 Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) 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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Excicure Inc

F. Hoffmann-La Roche Ltd

Ionis Pharmaceuticals Inc

Novartis AG

Sarepta Therapeutics Inc

Spotlight Innovation Inc

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Featured News & Press Releases

Apr 03, 2019: ICER Issues Final Report on Zolgensma, provides Policy  
recommendations related to Pricing and Coverage of treatments for Spinal Muscular  
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Apr 01, 2019: AveXis expands world-leading gene therapy manufacturing capacity with  
purchase of advanced biologics therapy manufacturing campus in Longmont, Colorado

Mar 27, 2019: Chugai receives Orphan Drug Designation for Risdiplam in Spinal  
Muscular Atrophy

Feb 18, 2019: Gene Therapy Firm AveXis to expand in Durham County adding 200 jobs

and \$60 Million in investment

Feb 01, 2019: First phase 3 gene therapy trial in spinal muscular atrophy at The John Walton Muscular Dystrophy Centre

Jan 08, 2019: Genentech, Roche and PTC Therapeutics provide update on Regulatory Filing for Risdiplam

Dec 19, 2018: Roche gets PRIME designation for SMA drug in Europe

Dec 03, 2018: Novartis announces FDA filing acceptance and Priority Review of AVXS-101, a one-time treatment designed to address the genetic root cause of SMA Type

Nov 05, 2018: Novartis provides update on AVXS101

Oct 03, 2018: Roche announces new data for Risdiplam in spinal muscular atrophy(SMA) at the World Muscle Society Congress

Oct 03, 2018: Risdiplam Demonstrates Preliminary Evidence of Clinical Benefit in Type 1, 2, & 3 Spinal Muscular Atrophy Patients

Jul 24, 2018: Spotlight Innovation spinal muscular atrophy research collaborator professor Kevin Hodgetts receives \$300,000 grant from Cure SMA

Jun 16, 2018: Updated Preliminary Data from SMA FIREFISH Program in Type 1 Babies Presented at the CureSMA Conference

Jun 14, 2018: Exicure to Present Data at the Cure Spinal Muscular Atrophy Annual Conference in Dallas

May 08, 2018: AveXis Issues Community Statement on the SPR1NT Trial

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### COMPANIES MENTIONED

AveXis Inc

Biogen Inc

Exicure Inc

F. Hoffmann-La Roche Ltd

Ionis Pharmaceuticals Inc

Novartis AG

Sarepta Therapeutics Inc

Spotlight Innovation Inc

## I would like to order

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