

# Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) - Pipeline Review, H2 2018

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## Abstracts

Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) - Pipeline Review, H2 2018

### SUMMARY

Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) pipeline Target constitutes close to 15 molecules. Out of which approximately 9 molecules are developed by companies and remaining by the universities/institutes. The latest report Survival Motor Neuron Protein - Pipeline Review, H2 2018, 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.

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 molecules developed by companies in Phase III and Preclinical stages are 3 and 6 respectively. Similarly, the universities portfolio in Preclinical and Discovery stages comprises 4 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.

Furthermore, this report 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. Driven by data and information sourced from 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.

**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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F. Hoffmann-La Roche Ltd

Genethon SA

Genzyme Corp

Ionis Pharmaceuticals Inc

Novartis AG

Sarepta Therapeutics Inc

Spotlight Innovation Inc

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Featured News & Press Releases

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

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

May 08, 2018: Scientists investigate new strategy to treat spinal muscular atrophy in  
infants

Apr 25, 2018: AveXis Announces First Patient Dosed in Phase 3 Trial of AVXS-101 in  
Pre-Symptomatic SMA Types 1, 2 and

Apr 24, 2018: AveXis Presents Initial Data from Pivotal U.S. Trial for SMA Type 1 and  
24-Month Follow-Up Data from Phase 1 Trial of AVXS-101 in SMA Type 1 at the Annual  
Meeting of the American Academy of Neurology

Apr 24, 2018: Preliminary Clinical Data from FIREFISH Trial in Type 1 SMA Patients  
Presented at the American Academy of Neurology Annual Meeting

Apr 19, 2018: AveXis to Present AVXS-101 Data at the Annual Meeting of the American  
Academy of Neurology

Apr 18, 2018: Preliminary Study Suggests Drug May Help Babies with Spinal Muscular  
Atrophy

Apr 15, 2018: Genentech Provided Update on RG-7916

Mar 27, 2018: AveXis Gene Therapy Awarded SAKIGAKE Designation for Spinal  
Muscular Atrophy Type

Mar 15, 2018: FIREFISH Transitions into Pivotal Phase

Mar 13, 2018: Newly Issued U.S. Patent Covers Spotlight Innovations Spinal Muscular Atrophy Drug Candidates

Jan 30, 2018: AveXis to Initiate Screening for Remaining Patients in Pivotal Trial of AVXS-101 for SMA Type 1 Following Review of Preliminary Data from First Three Patients

Jan 27, 2018: Preliminary Data from FIREFISH trial in Type 1 SMA Infants Presented at the International Scientific Congress on Spinal Muscular Atrophy

Jan 23, 2018: Data from RG7916 Programs in SMA to be Presented at the International Scientific Congress on Spinal Muscular Atrophy

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### COMPANIES MENTIONED

AveXis Inc

F. Hoffmann-La Roche Ltd

Genethon SA

Genzyme Corp

Ionis Pharmaceuticals Inc

Novartis AG

Sarepta Therapeutics Inc

Spotlight Innovation Inc

## I would like to order

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