

Survival Motor Neuron Protein - Pipeline Review, H2 2019

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Abstracts

Survival Motor Neuron Protein - Pipeline Review, H2 2019

SUMMARY

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.

Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) pipeline Target constitutes close to 19 molecules. Out of which approximately 11 molecules are developed by companies and remaining by the universities/institutes. The molecules developed by companies in Pre-Registration, Phase III, Phase II and Preclinical stages are 1, 1, 2 and 7 respectively. Similarly, the universities portfolio in Phase II, Preclinical and Discovery stages comprises 2, 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.

The latest report Survival Motor Neuron Protein - Pipeline Review, H2 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. It also reviews key players involved in Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) targeted therapeutics development with respective active and dormant or discontinued projects.

The report is built using 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 Gems 1 or Gemin 1 or SMN1 or SMN2)

The report reviews Survival Motor Neuron Protein (Component Of Gems 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 Gems 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 Gems 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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AveXis Inc

Biogen Inc

Exicure Inc

F. Hoffmann-La Roche Ltd

Ionis Pharmaceuticals Inc

Novartis AG

Sarepta Therapeutics Inc

Shift Pharmaceuticals

Spotlight Innovation Inc

Survival Motor Neuron Protein (Component Of Gems 1 or Gemin 1 or SMN1 or SMN2) -
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Oligonucleotide to Activate SMN2 for Spinal Muscular Atrophy - Drug Profile

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Product Description

Mechanism Of Action

R&D Progress

Small Molecules for Spinal Muscular Atrophy - Drug Profile

Product Description

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Small Molecules to Activate SMN2 for Spinal Muscular Atrophy - Drug Profile

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Featured News & Press Releases

Nov 12, 2019: Roche and PTC report positive data for risdiplam in SMA

Oct 30, 2019: Novartis' Zolgensma experiences further safety issues

Oct 02, 2019: Risdiplam Spinal Muscular Atrophy data demonstrating continued benefit presented at World Muscle Society Congress

Sep 19, 2019: Novartis presents additional Zolgensma data in children with SMA

Aug 14, 2019: Brother scientists no longer with Novartis after alleged data manipulation

Aug 13, 2019: US Senator seeks records linked to Novartis Zolgensma data issues

Aug 07, 2019: Novartis defends Zolgensma in face of FDA's data concerns

Aug 06, 2019: Statement on data accuracy issues with recently approved gene therapy

Jul 31, 2019: Connecticut Childrens administers first gene therapy for spinal muscular atrophy (SMA) in the state

Jul 25, 2019: AveXis Statement on Access to Zolgensma (onasemnogene abeparvovec-xioi)

Jul 22, 2019: BIA Separations selected for manufacturing process development support for gene therapy pipeline

Jul 16, 2019: Catalent Biologics announces agreement to provide additional production of approved gene therapy treatment for spinal muscular atrophy

Jun 13, 2019: UF Health patient first to receive new gene therapy for neuromuscular disease since FDA approval

Jun 05, 2019: New gene therapy poised to transform care for spinal muscular atrophy

May 30, 2019: Newly approved Spinal Muscular Atrophy Gene Therapy, Zolgensma, validates AskBio Gene Therapy Platform

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COMPANIES MENTIONED

AurimMed Pharma Inc

AveXis Inc

Biogen Inc

Excicure Inc

F. Hoffmann-La Roche Ltd

Ionis Pharmaceuticals Inc

Novartis AG

Sarepta Therapeutics Inc

Shift Pharmaceuticals

Spotlight Innovation Inc

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