

Spinocerebellar Ataxias - Pipeline Insight, 2020

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Abstracts

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DelveInsight's, "Spinocerebellar Ataxias – Pipeline Insight, 2020," report provides comprehensive insights about 3+ companies and 3+ pipeline drugs in Spinocerebellar Ataxias 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

Spinocerebellar Ataxias Understanding

Spinocerebellar Ataxias: Overview

The Spinocerebellar ataxias (SCA) are a subset of hereditary cerebellar ataxias that are autosomal dominantly transmitted. They are progressive neurodegenerative diseases that share the clinical features of ataxia, which arise from the progressive degeneration of the cerebellum but can also affect other connected regions, including the brain stem. They are a highly heterogenous group of disorders with a complex genotype–phenotype spectrum; many SCAs are caused by CAG nucleotide repeat expansions that encode polyglutamine, and therefore, involve the toxic polyglutamine protein (polyQ).

The spinocerebellar ataxias (SCAs) are a large complex group of inherited neurodegenerative disorders characterized by progressive cerebellar ataxia, ocular

motor abnormalities, and a range of other variable neurologic features, including retinopathy, optic atrophy, peripheral neuropathy, extrapyramidal symptoms, and cognitive dysfunction.

Symptoms

Gait ataxia and incoordination

Nystagmus/visual problems

Dysarthria

Extrapyramidal signs

Ophthalmoplegia

Cognitive impairment in specific SCAs

Diagnosis

A phenotype-first approach remains pertinent in molecular diagnosis of rare genetic disorders. Clinicians should also consider genetic testing for primary episodic ataxias (EA). Advances in next-generation sequencing (NGS) have facilitated further insights into the molecular causes of SCA. Exome sequencing identifies less classical phenotype–genotype correlations and detects new mutations in known cerebellar genes.

Treatment

Advancements in the understanding of pathophysiologic mechanisms facilitate the potential to find new therapeutic targets. Riluzole, a drug licensed for the treatment of ALS, was reconsidered for the treatment of patients with ataxia owing to its ability to inhibit presynaptic glutamate release and activate calcium-activated potassium channels. The anticonvulsant valproic acid has been proposed as a pharmacological treatment in SCA3/MJD for its neuroprotective properties as a pan-histone deacetylase inhibitor. Lithium carbonate was evaluated in 62 ambulatory patients with SCA3/MJD for 48 weeks, but no difference was seen in mean scores of the neurological scale for ataxia. Varenicline is a partial agonist of the $\alpha 4\beta 2$ neuronal nicotinic acetylcholine

receptor that is used to aid the cessation of smoking. Varenicline was anecdotally reported to have beneficial effects in patients with ataxia that were taking it as part of a smoking cessation programme. General supportive management options include physiotherapy, occupational therapy and speech therapy. Physiotherapy focused on improving gait, balance, coordination, posture and muscle strength is often recommended.

Spinocerebellar Ataxias Emerging Drugs Chapters

This segment of the Spinocerebellar Ataxias 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.

Spinocerebellar Ataxias Emerging Drugs

KPS-0373: Kissei Pharmaceutical

KPS-0373 is an I drug that is a TRH receptor agonist. It has recently completed Phase III trial of clinical study. It was originally developed by Kissei Pharmaceutical but now licensed to Shinogi.

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

Spinocerebellar Ataxias: Therapeutic Assessment

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

Major Players in Spinocerebellar Ataxias

There are approx. 3+ key companies which are developing the therapies for Spinocerebellar Ataxias. The companies which have their Spinocerebellar Ataxias drug candidates in the most advanced stage, i.e. phase III include, Kissei Pharmaceutical.

Phases

DelveInsight's report covers around 3+ 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

Spinocerebellar Ataxias 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

intravitreal

Subretinal

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.

Spinocerebellar Ataxias: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Spinocerebellar Ataxias 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 Spinocerebellar Ataxias drugs.

Report Highlights

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

Spinocerebellar Ataxias Report Insights

Spinocerebellar Ataxias Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Spinocerebellar Ataxias 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 Spinocerebellar Ataxias drugs?

How many Spinocerebellar Ataxias drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Spinocerebellar Ataxias?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Spinocerebellar Ataxias 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 Spinocerebellar Ataxias and their

status?

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

Key Players

Kissei Pharmaceutical

Key Products

KPS-0373

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Assessment by Stage and Molecule Type

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KPS-0373: Kissei Pharmaceutical

Product Description

Research and Development

Product Development Activities

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Product Description

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