

# Neurofibromatoses Type I (Von Recklinghausen's Disease) - Pipeline Review, H1 2020

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## Abstracts

Neurofibromatoses Type I (Von Recklinghausen's Disease) - Pipeline Review, H1 2020

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Neurofibromatoses Type I - Pipeline Review, H1 2020, provides an overview of the Neurofibromatoses Type I (Genetic Disorders) pipeline landscape.

Neurofibromatosis type 1 (NF1), also called von Recklinghausen's disease, is a rare genetic disorder characterized by the development of multiple noncancerous (benign) tumors of nerves and skin (neurofibromas). This is transmitted on chromosome 17 and is caused by mutation of the NF1 gene. Symptoms include liver enlargement, glioma, Lisch nodules and pheochromocytoma. Treatment includes pain medications, surgery, chemotherapy and radiation therapy.

### REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Neurofibromatoses Type I - Pipeline Review, H1 2020, provides comprehensive information on the therapeutics under development for Neurofibromatoses Type I (Genetic Disorders), complete with analysis by stage of development, drug target, mechanism of action (MoA), route of administration (RoA) and molecule type. The guide covers the descriptive pharmacological action of the therapeutics, its complete research and development history and latest news and press releases.

The Neurofibromatoses Type I (Genetic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Neurofibromatoses Type I (Von Recklinghausen's Disease) and features dormant and discontinued projects. The guide

covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Pre-Registration, Phase III, Phase II and Phase I stages are 1, 1, 3 and 1 respectively. Similarly, the Universities portfolio in Discovery stages comprises 1 molecules, respectively.

Neurofibromatoses Type I (Genetic Disorders) pipeline guide helps in identifying and tracking emerging players in the market and their portfolios, enhances decision making capabilities and helps to create effective counter strategies to gain competitive advantage. The guide is built using data and information sourced from Global Markets Direct's 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. Additionally, various dynamic tracking processes ensure that the most recent developments are captured on a real time basis.

**Note:** Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

## SCOPE

The pipeline guide provides a snapshot of the global therapeutic landscape of Neurofibromatoses Type I (Genetic Disorders).

The pipeline guide reviews pipeline therapeutics for Neurofibromatoses Type I (Genetic Disorders) by companies and universities/research institutes based on information derived from company and industry-specific sources.

The pipeline guide covers pipeline products based on several stages of development ranging from pre-registration till discovery and undisclosed stages.

The pipeline guide features descriptive drug profiles for the pipeline products which comprise, product description, descriptive licensing and collaboration details, R&D brief, MoA & other developmental activities.

The pipeline guide reviews key companies involved in Neurofibromatoses Type I (Genetic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Neurofibromatoses Type I (Genetic Disorders) therapeutics based on mechanism of action (MoA), drug target, route of administration (RoA) and molecule type.

The pipeline guide encapsulates all the dormant and discontinued pipeline projects.

The pipeline guide reviews latest news related to pipeline therapeutics for Neurofibromatoses Type I (Genetic Disorders)

## **REASONS TO BUY**

Procure strategically important competitor information, analysis, and insights to formulate effective R&D strategies.

Recognize emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage.

Find and recognize significant and varied types of therapeutics under development for Neurofibromatoses Type I (Genetic Disorders).

Classify potential new clients or partners in the target demographic.

Develop tactical initiatives by understanding the focus areas of leading companies.

Plan mergers and acquisitions meritoriously by identifying key players and it's most promising pipeline therapeutics.

Formulate corrective measures for pipeline projects by understanding Neurofibromatoses Type I (Genetic Disorders) pipeline depth and focus of Indication therapeutics.

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.

Adjust the therapeutic portfolio by recognizing discontinued projects and understand from the know-how what drove them from pipeline.

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NFlection Therapeutics Inc

Pfizer Inc

SpringWorks Therapeutics Inc

Vyriad Inc

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Featured News & Press Releases

Apr 14, 2020: KOSELUGO (selumetinib) approved for the treatment of neurofibromatosis type 1-associated plexiform neurofibromas in pediatric patients, available from Onco360

Apr 13, 2020: FDA approves AstraZeneca's Koselugo to treat rare genetic disorder

Apr 10, 2020: FDA Approves First Ever Treatment for Neurofibromatosis

Mar 18, 2020: In NIH trial, selumetinib shrinks tumors, provides clinical benefit for children with NF1

Nov 15, 2019: FDA grants priority review to tumour drug selumetinib

Oct 28, 2019: Selumetinib shows clinical benefit in adult patients with Neurofibromatosis Type 1-Associated Plexiform Neurofibromas

Oct 25, 2019: SpringWorks starts Phase IIb trial of mirdametinin in NF1-PN

Jul 30, 2019: European Commission grants Orphan Drug Designation for SpringWorks Therapeutics' MEK Inhibitor, Mirdametinin, for the treatment of Neurofibromatosis Type 1

Jun 03, 2019: SpringWorks Therapeutics Announces FDA Fast Track Designation for PD-0325901 for the Treatment of a Severe Form of Neurofibromatosis Type 1

Apr 01, 2019: FDA grants AstraZeneca-Merck's selumetinib breakthrough therapy designation

Dec 10, 2012: Study Identifies Targeted Molecular Therapy For Untreatable NF1 Tumors

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### COMPANIES MENTIONED

AstraZeneca Plc

NFlection Therapeutics Inc

Pfizer Inc

SpringWorks Therapeutics Inc

Vyriad Inc



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