

# Familial Amyloid Neuropathies - Pipeline Review, H1 2020

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## Abstracts

Familial Amyloid Neuropathies - Pipeline Review, H1 2020

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Familial Amyloid Neuropathies - Pipeline Review, H1 2020, provides an overview of the Familial Amyloid Neuropathies (Metabolic Disorders) pipeline landscape.

Familial amyloid neuropathy is a slowly progressive condition characterized by the buildup of abnormal deposits of a protein called amyloid (amyloidosis) in the body's organs and tissues. Symptoms include numbness, tingling, pins and needles in the feet and hands, weakness and pain in the arms and legs, loss of sensation, urinary retention, reduced sweating, ankle swelling, fatigue, nausea, weight loss, dizziness and fainting. Supportive treatment includes diuretics, calcium channel blockers, beta blockers, angiotensin receptor blockers and angiotensin converting enzyme (ACE) inhibitors.

### REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Familial Amyloid Neuropathies - Pipeline Review, H1 2020, provides comprehensive information on the therapeutics under development for Familial Amyloid Neuropathies (Metabolic 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 Familial Amyloid Neuropathies (Metabolic Disorders) pipeline guide also reviews of

key players involved in therapeutic development for Familial Amyloid Neuropathies 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, Phase I, Preclinical and Discovery stages are 1, 2, 2, 2, 2 and 2 respectively. Similarly, the Universities portfolio in Phase II and Preclinical stages comprises 1 and 1 molecules, respectively. Familial Amyloid Neuropathies (Metabolic 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 Familial Amyloid Neuropathies (Metabolic Disorders).

The pipeline guide reviews pipeline therapeutics for Familial Amyloid Neuropathies (Metabolic 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 Familial Amyloid Neuropathies (Metabolic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Familial Amyloid Neuropathies (Metabolic 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 Familial Amyloid Neuropathies (Metabolic 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 Familial Amyloid Neuropathies (Metabolic 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 Familial Amyloid Neuropathies (Metabolic 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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Featured News & Press Releases

Dec 06, 2018: Eidos Therapeutics Appoints William Lis to Board of Directors

Nov 13, 2017: European Medicines Agency (EMA) Grants Alnylam Accelerated Assessment of Patisiran for Patients with Hereditary ATTR (hATTR) Amyloidosis

Nov 02, 2017: Sanofi and Alnylam Present Positive Complete Results from APOLLO Phase 3 study of Investigational Patisiran in Hereditary ATTR (hATTR) Amyloidosis Patients with Polyneuropathy

Sep 20, 2017: Arbutus' LNP Licensee Alnylam Announces Positive Phase 3 Results for LNP-Enabled Patisiran Program

Sep 20, 2017: Sanofi and Alnylam Report Positive Topline Results from APOLLO Phase 3 Study of Patisiran in Hereditary ATTR (hATTR) Amyloidosis Patients with Polyneuropathy

Apr 26, 2017: Alnylam Reports Final 24-Month Results from Phase 2 Open-Label Extension Study of Patisiran, an Investigational RNAi Therapeutic in Development for the Treatment of Hereditary ATTR (hATTR) Amyloidosis

Oct 10, 2016: Data Monitoring Committee Recommends Continuation of APOLLO Phase 3 Clinical Trial of Patisiran for Hereditary ATTR Amyloidosis with Polyneuropathy (hATTR-PN)

Jul 01, 2016: Alnylam Reports New Results on patisiran at the XV International Symposium on Amyloidosis

Jun 27, 2016: Alnylam to Report New Patisiran Results at the XV International Symposium on Amyloidosis

Apr 20, 2016: Alnylam Reports Complete 18-Month Data from Ongoing Phase 2 Open-Label Extension Study of Patisiran, an Investigational RNAi Therapeutic Targeting Transthyretin for the Treatment of Hereditary ATTR Amyloidosis with Polyneuropathy (hATTR-PN)

Feb 01, 2016: Alnylam Completes Enrollment in APOLLO Phase 3 Study with Patisiran, an Investigational RNAi Therapeutic for Patients with Transthyretin (TTR)-Mediated Amyloidosis (ATTR Amyloidosis)

Nov 03, 2015: Alnylam Reports Positive Results from Ongoing Phase 2 Open-Label Extension Studies for Patisiran in Development for the Treatment of Transthyretin (TTR)-Mediated Amyloidosis (ATTR Amyloidosis)

Sep 28, 2015: Alnylam and Collaborators Report First-Ever Evidence that Patisiran Reduces Pathogenic, Misfolded Transthyretin Monomers and Oligomers in TTR-Mediated Amyloidosis Patients with Familial Amyloidotic Polyneuropathy

Jul 20, 2015: Alnylam Initiates Phase 3 Open Label Extension Study with Patisiran, an Investigational RNAi Therapeutic Targeting Transthyretin for the Treatment of TTR-Mediated Amyloidosis

Apr 21, 2015: Alnylam Reports 12-Month Clinical Data from Phase 2 Open Label Extension Study of Patisiran, an Investigational RNAi Therapeutic for Patients with Familial Amyloidotic Polyneuropathy

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Familial Amyloid Neuropathies - Pipeline by Regeneron Pharmaceuticals Inc, H1 2020

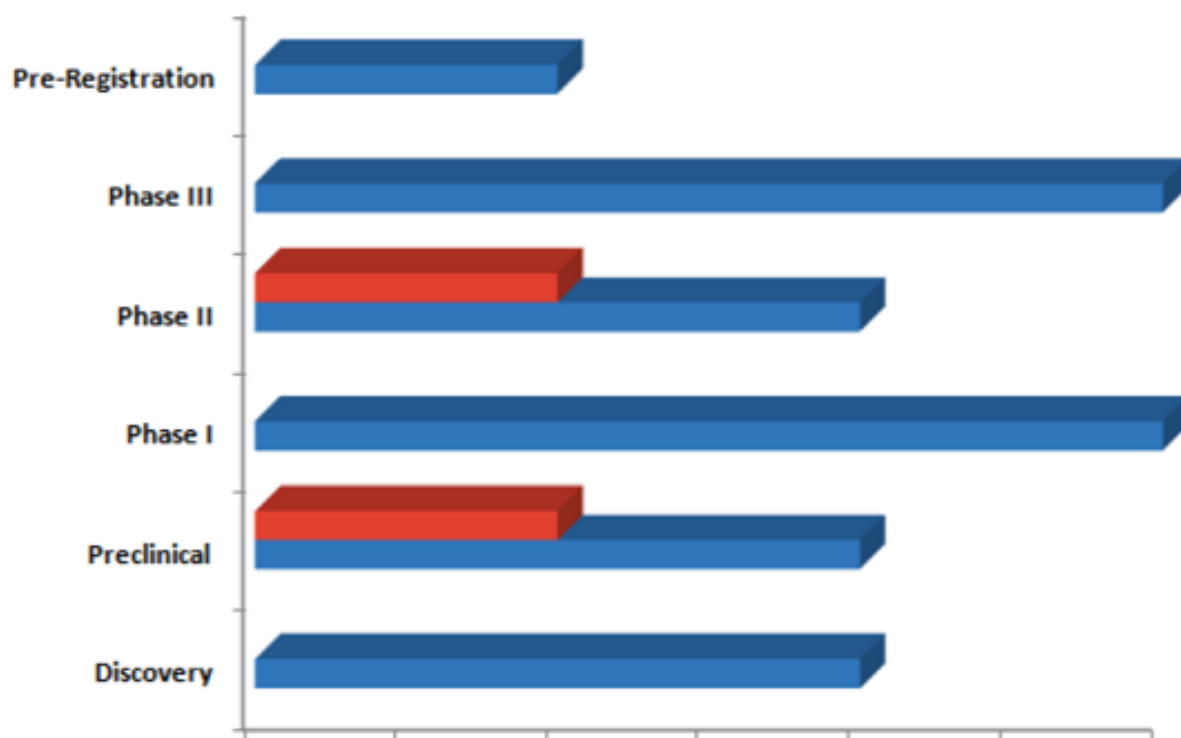
Familial Amyloid Neuropathies - Dormant Projects, H1 2020

Familial Amyloid Neuropathies - Discontinued Products, H1 2020

## About

Familial Amyloid Neuropathies is a slowly progressive condition characterized by the buildup of abnormal deposits of a protein called amyloid (amyloidosis) in the body's organs and tissues. Symptoms include numbness, tingling, pins and needles in the feet and hands, weakness and pain in the arms and legs, loss of sensation, urinary retention, reduced sweating, ankle swelling, fatigue, nausea, weight loss, dizziness and fainting. Supportive treatment includes diuretics, calcium channel blockers, beta blockers, angiotensin receptor blockers and angiotensin converting enzyme (ACE) inhibitors.

Familial Amyloid Neuropathies pipeline therapeutics constitutes close to 13 molecules.



Global Markets Direct's latest report, 'Familial Amyloid Neuropathies - Pipeline Review, H1 2020', provides comprehensive information on the therapeutics under development for Familial Amyloid Neuropathies, complete with analysis by stage of development, drug target, mechanism of action (MoA), route of administration (RoA) and molecule type.

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