

# Amyloidosis Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

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## **Abstracts**

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### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Amyloidosis - Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update, provides an overview of the Amyloidosis (Metabolic Disorders) pipeline landscape.

Abnormal accumulation of amyloid protein in the tissues results in a group of diseases known as amyloidosis. It may affect the shape and functioning of the organ eventually leading to organ failure. Predisposing factors are misfolding of abnormal protein called the light chain and its subsequent deposition in the body. It may also occur as a result of dialysis. Symptoms include fatigue, chest pain, swelling and breathing problems. Amyloidosis can be managed by transplant and, medication such as diuretics and chemotherapy drugs.

### **REPORT HIGHLIGHTS**

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Amyloidosis - Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update, provides comprehensive information on the therapeutics under development for Amyloidosis (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 Amyloidosis (Metabolic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Amyloidosis and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies /Universities /Institutes, the molecules developed by Companies in Pre-Registration, Filing rejected/Withdrawn, Phase III, Phase II, Phase I, Phase 0, Preclinical and Discovery stages are 1, 1, 7, 11, 9, 1, 14 and 12 respectively. Similarly, the Universities portfolio in Phase III, Phase II, Phase 0, Preclinical and Discovery stages comprises 1, 1, 1, 1 and 1 molecules, respectively.

Amyloidosis (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 Amyloidosis (Metabolic Disorders).

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

The pipeline guide evaluates Amyloidosis (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 Amyloidosis (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 Amyloidosis (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 Amyloidosis (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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- Jan 24, 2022: Eplontersen granted Orphan Drug Designation in the US for transthyretin amyloidosis
- Jan 21, 2022: Alnylam presents positive 18-Month results from HELIOS-A phase 3 study of investigational Vutrisiran in patients with hATTR amyloidosis with polyneuropathy
- Jan 14, 2022: Alnylam to present full 18-month results from the HELIOS-A phase 3 study of vutrisiran at the Soci?t? Francophone du Nerf P?riph?rique Annual Meeting Dec 27, 2021: BridgeBio Pharma reports month 12 topline results from phase 3 ATTRibute-CM study
- Dec 27, 2021: BridgeBio Pharma reports month 12 topline results from phase 3 ATTRibute-CM study
- Dec 13, 2021: Attralus presents positive new data at 2021 American Society of Hematology Annual Meeting
- Nov 22, 2021: Intellia Therapeutics announces expansion of ongoing phase 1 study of NTLA-2001 to include adults with transthyretin amyloidosis with cardiomyopathy (ATTR-



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Oct 21, 2021: Intellia Therapeutics receives U.S. FDA Orphan Drug Designation for

NTLA-2001, an investigational CRISPR therapy for the treatment of transthyretin (ATTR) amyloidosis

Oct 11, 2021: Tegsedi receives innovative drug category pricing in Brazil Sep 13, 2021: Alnylam submits marketing authorization application to the European Medicines Agency for investigational vutrisiran for the treatment of hereditary ATTR amyloidosis with polyneuropathy

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