

Hereditary ATTR (hATTR) Amyloidosis Clinical Trials and Studies: EMA, PDMA, FDA Approvals, Mechanism of Action, ROA, NDA

Hereditary ATTR (hATTR) Amyloidosis Pipeline

LAS VEGAS, NV, UNITED STATES, February 13, 2025 /EINPresswire.com/ -- DelveInsight's, "Hereditary Transthyretin Amyloidosis Pipeline Insight" report provides comprehensive insights about 8+ companies and 8+ pipeline drugs in Hereditary Transthyretin Amyloidosis (hATTR) 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.

Stay ahead with the latest insights! Download DelveInsight's comprehensive Hereditary ATTR (hATTR) Amyloidosis Pipeline Report to explore emerging therapies, key Hereditary ATTR (hATTR) Amyloidosis Companies, and future STAT Inhibitors treatment landscapes @ [Hereditary ATTR \(hATTR\) Amyloidosis Pipeline Outlook Report](#)

Key Takeaways from the Hereditary ATTR (hATTR) Amyloidosis Pipeline Report

- In December 2024:- Alnylam Pharmaceuticals- The purpose of this study is to evaluate the efficacy and safety of vutrisiran (ALN-TTRSC02) in participants with hereditary transthyretin amyloidosis (hATTR amyloidosis). Participants will receive vutrisiran subcutaneous (SC) injection once every 3 months (q3M) or the reference comparator patisiran intravenous (IV) injection once every 3 weeks (q3w) during the 18 month Treatment Period. This study will use the placebo arm of the APOLLO study (NCT01960348) as an external comparator for the primary and most other efficacy endpoints during the 18 Month Treatment Period.
- DelveInsight's Hereditary ATTR (hATTR) Amyloidosis pipeline report depicts a robust space with 8+ active players working to develop 8+ pipeline therapies for Hereditary ATTR (hATTR) Amyloidosis treatment.
- The leading Hereditary ATTR (hATTR) Amyloidosis Companies such as Alnylam Pharmaceuticals, Ionis Pharmaceuticals, Eidos Therapeutics, Intellia Therapeutics, Corino Therapeutics, Novo Nordisk and others.
- Promising Hereditary ATTR (hATTR) Amyloidosis Therapies such as Patisiran, Vutrisiran, ION-682884, Eplontersen and others.

Discover how the Hereditary ATTR (hATTR) Amyloidosis treatment paradigm is evolving. Access

DelveInsight's in-depth Hereditary ATTR (hATTR) Amyloidosis Pipeline Analysis for a closer look at promising breakthroughs @ [Hereditary ATTR \(hATTR\) Amyloidosis Clinical Trials and Studies](#)

Hereditary Transthyretin Amyloidosis (hATTR) Emerging Drugs

- Vutrisiran: Alnylam Pharmaceuticals

Vutrisiran is an investigational, subcutaneously administered RNAi therapeutic in development for the treatment of ATTR amyloidosis, which encompasses both hereditary (hATTR) and wild-type (wtATTR) amyloidosis. It is designed to target and silence specific messenger RNA, blocking the production of wild-type and variant transthyretin (TTR) protein before it is made. Quarterly administration of vutrisiran may help to reduce deposition and facilitate the clearance of TTR amyloid deposits in tissues and potentially restore function to these tissues. Vutrisiran utilizes Alnylam's Enhanced Stabilization Chemistry (ESC)-GalNAc-conjugate delivery platform, designed for increased potency and high metabolic stability that allows for infrequent subcutaneous injections. HELIOS-B is a randomized, double-blind, placebo-controlled Phase 3 study of ~600 adult patients with ATTR amyloidosis with cardiomyopathy (including both hATTR and wtATTR amyloidosis). The U.S. Food and Drug Administration (FDA) has accepted the Company's New Drug Application (NDA) for vutrisiran, an investigational RNAi therapeutic for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults. The FDA has set an action date of April 14, 2022 under the Prescription Drug User Fee Act (PDUFA), and the Agency has indicated that they are not currently planning an advisory committee meeting as part of the NDA review.

- AKCEA-TTR-LRx: Ionis Pharmaceuticals

AKCEA-TTR-LRx is an antisense drug developed using Ionis' proprietary Ligand Conjugated Antisense (LICA) technology platform and is designed to inhibit production of TTR. It was discovered by Ionis and is being co-developed by Ionis and Akcea. In a Phase 1 clinical trial, patients treated with AKCEA-TTR-LRx experienced reductions in TTR of up to 94 percent at the highest dose. CARDIO-TTRransform is a global, double-blind, randomized, placebo-controlled Phase III cardiovascular outcome study. It is designed to compare AKCEA-TTR-LRx to placebo in patients with both wild type and hereditary ATTR cardiomyopathy who are on the current available standard of care.

The Hereditary ATTR (hATTR) Amyloidosis pipeline report provides insights into

- The report provides detailed insights about companies that are developing therapies for the treatment of Hereditary ATTR (hATTR) Amyloidosis with aggregate therapies developed by each company for the same.
- It accesses the Different therapeutic candidates segmented into early-stage, mid-stage, and late-stage of development for Hereditary ATTR (hATTR) Amyloidosis Treatment.
- Hereditary ATTR (hATTR) Amyloidosis Companies are involved in targeted therapeutics development with respective active and inactive (dormant or discontinued) projects.
- Hereditary ATTR (hATTR) Amyloidosis Drugs under development based on the stage of development, route of administration, target receptor, monotherapy or combination therapy, a

different mechanism of action, and molecular type.

- Detailed analysis of collaborations (company-company collaborations and company-academia collaborations), licensing agreement and financing details for future advancement of the Hereditary ATTR (hATTR) Amyloidosis market.

Get a detailed analysis of the latest innovations in the Hereditary ATTR (hATTR) Amyloidosis pipeline. Explore DelveInsight's expert-driven report today! @ [Hereditary ATTR \(hATTR\) Amyloidosis Unmet Needs](#)

Hereditary ATTR (hATTR) Amyloidosis Companies

Alnylam Pharmaceuticals, Ionis Pharmaceuticals, Eidos Therapeutics, Intellia Therapeutics, Corino Therapeutics, Novo Nordisk and others.

Hereditary Transthyretin Amyloidosis (hATTR) 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
- Intravenous
- Subcutaneous
- Topical
- Molecule Type

Hereditary ATTR (hATTR) Amyloidosis Products have been categorized under various Molecule types such as

- Monoclonal Antibody
- Peptides
- Polymer
- Small molecule
- Gene therapy
- Product Type

Download DelveInsight's latest report to gain strategic insights into upcoming Hereditary ATTR (hATTR) Amyloidosis Therapies and key Hereditary ATTR (hATTR) Amyloidosis Developments @ Hereditary ATTR (hATTR) Amyloidosis Market Drivers and Barriers, and Future Perspectives- https://www.delveinsight.com/sample-request/hereditary-transthyretin-amyloidosis-hattr-pipeline-insight?utm_source=einpresswire&utm_medium=pressrelease&utm_campaign=ypr

Scope of the Hereditary ATTR (hATTR) Amyloidosis Pipeline Report

- Coverage- Global
- Hereditary ATTR (hATTR) Amyloidosis Companies- Alnylam Pharmaceuticals, Ionis Pharmaceuticals, Eidos Therapeutics, Intellia Therapeutics, Corino Therapeutics, Novo Nordisk and others.

- Hereditary ATTR (hATTR) Amyloidosis Therapies- Patisiran, Vutrisiran, ION-682884, Eplontersen and others.
- Hereditary ATTR (hATTR) Amyloidosis Therapeutic Assessment by Product Type: Mono, Combination, Mono/Combination
- Hereditary ATTR (hATTR) Amyloidosis Therapeutic Assessment by Clinical Stages: Discovery, Pre-clinical, Phase I, Phase II, Phase III

Which companies are leading the race in Hereditary ATTR (hATTR) Amyloidosis drug development? Find out in DelveInsight's exclusive Hereditary ATTR (hATTR) Amyloidosis Pipeline Report—access it now! @ Hereditary ATTR (hATTR) Amyloidosis Emerging Drugs and Major Companies- https://www.delveinsight.com/sample-request/hereditary-transthyretin-amyloidosis-hattr-pipeline-insight?utm_source=einpresswire&utm_medium=pressrelease&utm_campaign=ypr

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List of Top Selling Market Research Reports in 2025

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