

# Hereditary Transthyretin Amyloidosis Market Report 2032: Epidemiology, Therapies, Latest Approvals by DelveInsight

*Epidemiology assessed for the condition showed that the US, in 2020, accounted for approximately 15,312 prevalent cases of hATTR.*

LAS VEGAS, NEVADA, UNITED STATES, March 26, 2024 /EINPresswire.com/ -- DelveInsight's "Hereditary Transthyretin Amyloidosis (hATTR) Market Insights, Epidemiology, and Market Forecast-2032" report offers an in-depth understanding of the Hereditary Transthyretin Amyloidosis (hATTR), historical and forecasted epidemiology as well as the Hereditary Transthyretin Amyloidosis (hATTR) market trends in the United States, EU4 (Germany, Spain, Italy, France) the United Kingdom and Japan.

To Know in detail about the Hereditary Transthyretin Amyloidosis (hATTR) market outlook, drug uptake, treatment scenario and epidemiology trends, Click here; [Hereditary Transthyretin Amyloidosis \(hATTR\) Market Forecast](#)

Some of the key facts of the Hereditary Transthyretin Amyloidosis (hATTR) Market Report:

The Hereditary Transthyretin Amyloidosis (hATTR) market size is anticipated to grow with a significant CAGR during the study period (2019-2032).

Key Hereditary Transthyretin Amyloidosis (hATTR) Companies: Pfizer, Alnylam Pharmaceuticals, Akcea Therapeutics, Ionis Pharmaceuticals, AstraZeneca, Eidos Therapeutics, Corino Therapeutics, Prothena, Novo Nordisk, Intellia Therapeutics, Regeneron Pharmaceuticals, and others

Key Hereditary Transthyretin Amyloidosis (hATTR) Therapies: Vyndaqel, Onpattro, Tegsedi, Vutrisiran, Eplontersen, Acoramidis, CRX-1008, and others

In 2020, the total diagnosed prevalent cases of hereditary transthyretin amyloidosis (hATTR) in the 7MM (Seven Major Markets) were estimated at 13,540, with a compound annual growth rate (CAGR) of 5.05% during the study period spanning from 2018 to 2030. Epidemiological assessments revealed that the United States accounted for approximately 15,312 prevalent cases of hATTR in 2020.

Among the EU-5 countries (France, Germany, Italy, Spain, and the United Kingdom) in 2020, France had the highest number of diagnosed prevalent cases of hATTR, totaling 1,480, while Germany had the lowest number of cases at 518. Japan reported 648 diagnosed prevalent cases of hATTR in the same year.

In the US in 2020, the highest proportion of type-specific cases was observed in Familial Amyloid Polyneuropathy (FAP) compared to Familial Amyloid Cardiomyopathy (FAC) and Mixed hATTR Type. In the EU-5, NYHA Class II accounted for the highest number of cases in 2020, followed by NYHA Class III and NYHA Class I.

Regarding stage-specific diagnosed prevalent contribution, Japan reported 47 cases in stage 3 and 180 cases in stage 1 in 2020.

The Hereditary Transthyretin Amyloidosis (hATTR) market is expected to surge due to the disease's increasing prevalence and awareness during the forecast period. Furthermore, launching various multiple-stage Hereditary Transthyretin Amyloidosis (hATTR) pipeline products will significantly revolutionize the Hereditary Transthyretin Amyloidosis (hATTR) market dynamics.

## Hereditary Transthyretin Amyloidosis (hATTR) Overview

Transthyretin (formerly known as prealbumin) is a prevalent, soluble serum protein weighing 55 kDa and forming a  $\beta$ -strand rich homotetramer. Its primary function is the transportation of both vitamin A (via retinol-binding protein) and thyroxine throughout the body. Additionally, transthyretin (TTR) plays roles in binding and redistributing  $\beta$ -amyloid in the choroid plexus and retaining T4 in the cerebral spinal fluid (CSF). Under certain circumstances, TTR may dissociate into its monomeric subunits of 127 amino acids and undergo abnormal alterations, leading to the formation of amyloidogenic intermediates. These intermediates can then self-aggregate into amyloid fibrils, which accumulate as amyloid deposits throughout the body, resulting in Transthyretin Amyloidosis. This condition can be further classified into wild-type (wt) or hereditary forms, with the latter divided into familial amyloid polyneuropathy (FAP) and familial amyloid cardiomyopathy (FAC).

Hereditary amyloidosis forms are autosomal dominant disorders characterized by the deposition of variant proteins in specific tissues. The most prevalent hereditary form is transthyretin amyloidosis (ATTR), caused by misfolding of protein monomers derived from the tetrameric protein transthyretin (TTR). Mutations in the TTR gene often lead to TTR instability and subsequent fibril formation. Closely related is wild-type TTR, where the native TTR protein, especially in the elderly, can destabilize and re-aggregate, resulting in nonfamilial cases of TTR amyloidosis.

Familial transthyretin amyloidosis (FTA) is caused by mutations in the TTR gene, responsible for producing transthyretin, a protein facilitating the transport of vitamin A and thyroxine to various body parts. Mutations in TTR result in a defective transthyretin protein that forms amyloid when folded. This amyloid accumulates in different body parts, leading to nerve and tissue damage. Most individuals with FTA inherit the TTR mutation from a family member, although some may develop the condition due to new mutations (de novo) in the TTR gene, without any family history of the disease.

Get a Free sample for the Hereditary Transthyretin Amyloidosis (hATTR) Market Forecast, Size & Share Analysis Report:

## Hereditary Transthyretin Amyloidosis (hATTR) Epidemiology

The epidemiology section provides insights into the historical, current, and forecasted epidemiology trends in the seven major countries (7MM) from 2019 to 2032. It helps to recognize the causes of current and forecasted trends by exploring numerous studies and views of key opinion leaders. The epidemiology section also provides a detailed analysis of the diagnosed patient pool and future trends.

### Hereditary Transthyretin Amyloidosis (hATTR) Epidemiology Segmentation:

The Hereditary Transthyretin Amyloidosis (hATTR) market report proffers epidemiological analysis for the study period 2019–2032 in the 7MM segmented into:

Total Prevalence of Hereditary Transthyretin Amyloidosis (hATTR)

Prevalent Cases of Hereditary Transthyretin Amyloidosis (hATTR) by severity

Gender-specific Prevalence of Hereditary Transthyretin Amyloidosis (hATTR)

Diagnosed Cases of Episodic and Chronic Hereditary Transthyretin Amyloidosis (hATTR)

Download the report to understand which factors are driving Hereditary Transthyretin Amyloidosis (hATTR) epidemiology trends @ [Hereditary Transthyretin Amyloidosis \(hATTR\) Epidemiology Forecast](#)

### Hereditary Transthyretin Amyloidosis (hATTR) Drugs Uptake and Pipeline Development Activities

The drugs uptake section focuses on the rate of uptake of the potential drugs recently launched in the Hereditary Transthyretin Amyloidosis (hATTR) market or expected to get launched during the study period. The analysis covers Hereditary Transthyretin Amyloidosis (hATTR) market uptake by drugs, patient uptake by therapies, and sales of each drug.

Moreover, the therapeutics assessment section helps understand the drugs with the most rapid uptake and the reasons behind the maximal use of the drugs. Additionally, it compares the drugs based on market share.

The report also covers the Hereditary Transthyretin Amyloidosis (hATTR) Pipeline Development Activities. It provides valuable insights about different therapeutic candidates in various stages and the key companies involved in developing targeted therapeutics. It also analyzes recent developments such as collaborations, acquisitions, mergers, licensing patent details, and other information for emerging therapies.

### Hereditary Transthyretin Amyloidosis (hATTR) Therapies

Vyndaqel  
Onpattro  
Tegsedi  
Vutrisiran  
Eplontersen  
Acoramidis  
CRX-1008

## Hereditary Transthyretin Amyloidosis (hATTR) Therapies Key Companies

Pfizer  
Alnylam Pharmaceuticals  
Akcea Therapeutics  
Ionis Pharmaceuticals  
AstraZeneca  
Eidos Therapeutics  
Corino Therapeutics  
Prothena  
Novo Nordisk  
Intellia Therapeutics  
Regeneron Pharmaceuticals

Discover more about therapies set to grab major Hereditary Transthyretin Amyloidosis (hATTR) market share @ [Hereditary Transthyretin Amyloidosis \(hATTR\) Treatment Landscape](#)

## Hereditary Transthyretin Amyloidosis (hATTR) Market Outlook

Hereditary Transthyretin Amyloidosis (hATTR) market has been assessed, based on demand, prescription analysis and the annual cost of therapy of current and forecasted market value of the approved drugs, Vyndaqel, Onpattro, Tegsedi in the US, Europe, and Japan as well as forecasted patient share and the annual cost of therapy for upcoming medicines. Supportive therapies like Diflunisal, other symptomatic drugs for TTR-FAP and recommended symptomatic drugs like loop diuretics, aldosterone antagonists, angiotensin-converting enzyme inhibitors, and beta-blockers have been considered for hATTR market estimation. Liver transplantation is the most common treatment method in hATTR amyloidosis, as the liver is the main source of abnormal TTR production. This procedure is most effective in patients who are in the early stages of the disease.

In addition, only a few hATTR therapies have been approved for this indication. Each drug has a different purpose or mode of action, such as stabilizing the TTR protein, preventing the production of the TTR protein, or removing amyloid deposits. The market scenario started changing in 2018, with the launch of Tegsedi and Onpattro, and witnessed further growth in 2019, with the US launch of Vyndaqel/Vyndamax.

Tegsedi (inotersen) is a transthyretin-directed antisense oligonucleotide. In October 2018, Akcea Therapeutics and Ionis Pharmaceuticals announced the US FDA approval of the drug for the treatment of hATTR-PN. Previously, in July 2018, Tegsedi had also received approval from the EC for stage I or stage II in adult patients with hATTR-PN. Use of Inotersen has demonstrated significant benefit in Norfolk Quality of Life Questionnaire-Diabetic Neuropathy and modified Neuropathy Impairment Score +7. However, the drug has not been approved by the PMDA, Japan.

Both Tegsedi and Onpattro have received Fast Track Designation and Orphan Drug Designation for Transthyretin Amyloidosis. Onpattro has additionally received Breakthrough Therapy Designation from the FDA for the treatment of hATTR-PN. Onpattro is projected to hold a significant share of the hATTR-PN market with good safety and efficacy results even for the late stage of the hATTR-PN and may grab a large portion of the market if the cardiac population is penetrated for treatment. Tegsedi's performance is not good, and onpattro is outperforming this candidate. Hence, it will not generate significant revenue. Ionis' emerging candidate Eplontersen, however seems to be good contender in the future for hATTR.

Even though the current hATTR treatment options are limited, multiple potential therapies are emerging to help mitigate the underlying genetic mutation in patients with hATTR amyloidosis. Major potential emerging drugs are Vutrisiran, and Eplontersen

Alnylam's Vutrisiran is an investigational, subcutaneously-administered RNAi therapeutic. Alnylam has completed enrollment in its HELIOS-B Phase III study in patients with hATTR-CM. The company is expected to report 30-month endpoint top-line results from the HELIOS-B trial study in early 2024. The US FDA is currently evaluating the NDA for the drug to treat hATTR-PN. The approval of the drug for both segments of the patient will help the company grab a significant share in the market of hATTR.

Eidos Therapeutics' orally available, small molecule TTR stabilizer, AG 10, is another molecule that has demonstrated potent activity in Phase II clinical trial. The company anticipates data to be released in 4Q 2021 or early 2022. The filing for NDA submission and MAA is anticipated in mid- 2022 (Bridgebio, 2021). The company is expecting top-line data for acoramidis for Phase III clinical trials in patients with ATTR-PN (ATTRibute-PN) in 2024. In addition, BridgeBio expects to submit an application for regulatory approval of the drug in 2022 to the FDA. In addition, Alexion holds an exclusive license to develop and commercialize AG10 in Japan. It is conducting a Phase III bridging study of ALXN2060 exclusively in Japan for patients with ATTR-CM. The study is expected to be completed in April 2023.

Scope of the Hereditary Transthyretin Amyloidosis (hATTR) Market Report

Study Period: 2019–2032

Coverage: 7MM [The United States, EU5 (Germany, France, Italy, Spain, and the United Kingdom),

and Japan]

Key Hereditary Transthyretin Amyloidosis (hATTR) Companies: Pfizer, Alnylam Pharmaceuticals, Akcea Therapeutics, Ionis Pharmaceuticals, AstraZeneca, Eidos Therapeutics, Corino Therapeutics, Prothena, Novo Nordisk, Intellia Therapeutics, Regeneron Pharmaceuticals, and others

Key Hereditary Transthyretin Amyloidosis (hATTR) Therapies: Vyndaqel, Onpattro, Tegsedi, Vutrisiran, Eplontersen, Acoramidis, CRX-1008, and others

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Hereditary Transthyretin Amyloidosis (hATTR) Market Dynamics: Hereditary Transthyretin Amyloidosis (hATTR) market drivers and Hereditary Transthyretin Amyloidosis (hATTR) market barriers

Competitive Intelligence Analysis: SWOT analysis, PESTLE analysis, Porter's five forces, BCG Matrix, Market entry strategies

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### Hereditary Transthyretin Amyloidosis (hATTR) Pipeline

"Hereditary Transthyretin Amyloidosis (hATTR) Pipeline Insight, 2024" report by DelveInsight outlines comprehensive insights of present clinical development scenarios and growth prospects across the Hereditary Transthyretin Amyloidosis (hATTR) market. A detailed picture of the Hereditary Transthyretin Amyloidosis (hATTR) pipeline landscape is provided, which includes the disease overview and Hereditary Transthyretin Amyloidosis (hATTR) treatment guidelines.

### Hereditary Transthyretin Amyloidosis (hATTR) Epidemiology

DelveInsight's 'Hereditary Transthyretin Amyloidosis (hATTR) Epidemiology Forecast to 2032' report delivers an in-depth understanding of the disease, historical and forecasted Hereditary Transthyretin Amyloidosis (hATTR) epidemiology in the 7MM, i.e., the United States, EU5 (Germany, Spain, Italy, France, and the United Kingdom), and Japan.

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DelveInsight is a leading Healthcare Business Consultant, and Market Research firm focused exclusively on life sciences. It supports Pharma companies by providing comprehensive end-to-end solutions to improve their performance.

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