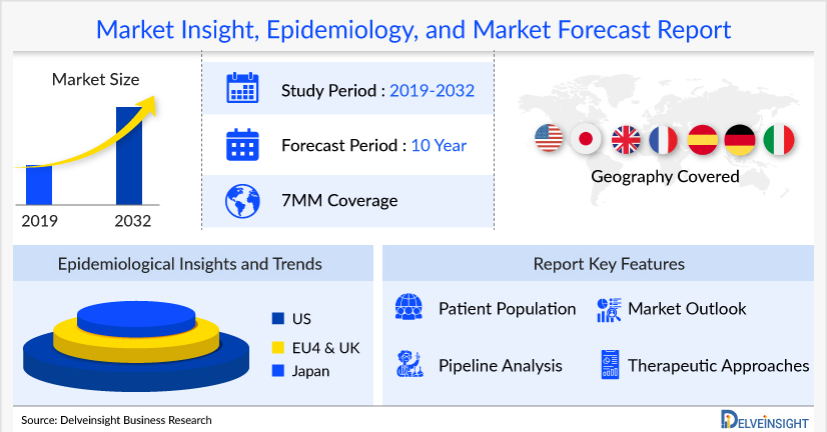
Hereditary Transthyretin Amyloidosis Market Report 2032: Epidemiology Data, Therapies, Latest Approvals by DelveInsight

Hereditary Transthyretin Amyloidosis companies are Takeda, Amgen, Novartis, AVEO Pharmaceuticals, AbbVie, Bristol-Myers Squibb, Abbott, and others.



Hereditary Amyloidosis Market Outlook Report

LAS VEGAS, NEVADA, UNITED STATES, June 24, 2024 /EINPresswire.com/ -- DelveInsight's "Hereditary Transthyretin Amyloidosis Market Insights, Epidemiology, and Market Forecast-2032" report offers an in-depth understanding of the Hereditary Transthyretin Amyloidosis, historical and forecasted epidemiology as well as the Hereditary Transthyretin Amyloidosis 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 market outlook, drug uptake, treatment scenario and epidemiology trends, Click here; Hereditary Transthyretin Amyloidosis Market Forecast

Some of the key facts of the Hereditary Transthyretin Amyloidosis Market Report:
The Hereditary Transthyretin Amyloidosis market size is anticipated to grow with a significant CAGR during the study period (2019-2032).
Key Hereditary Transthyretin Amyloidosis 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 Therapies: Vyndaqel, Onpattro, Tegsedi, Vutrisiran, Eplontersen, Acoramidis, CRX-1008, and others
In 2020, the total diagnosed prevalent cases of Hereditary Transthyretin Amyloidosis in the Seven Major Markets (7MM) were estimated at 13,540, with a compound annual growth rate (CAGR) of 5.05% projected from 2018 to 2030. Epidemiological assessments revealed that the United States had 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 reported the highest number of diagnosed prevalent cases of hATTR at 1,480, while Germany had the lowest at 518. Japan reported 648 diagnosed prevalent cases of hATTR in the same year.
In the US in 2020, Familial Amyloid Polyneuropathy (FAP) accounted for the highest proportion of type-specific cases compared to Familial Amyloid Cardiomyopathy (FAC) and Mixed hATTR Type.
In the EU-5, NYHA Class II had the highest number of cases in 2020, followed by NYHA Class III and NYHA Class I. Regarding stage-specific diagnosed prevalent cases, Japan reported 47 cases in stage 3 and 180 cases in stage 1 in 2020.
The Hereditary Transthyretin Amyloidosis market is expected to surge due to increasing disease prevalence and awareness during the forecast period. Additionally, the introduction of multiple-stage hATTR pipeline products is anticipated to significantly transform market dynamics.

Hereditary Transthyretin Amyloidosis Overview

Transthyretin (formerly known as prealbumin) is a prevalent, soluble serum protein with a molecular weight of 55 kDa, forming a β-strand rich homotetramer. Its primary function is to transport vitamin A (via retinol-binding protein) and thyroxin throughout the body. Additionally, transthyretin (TTR) binds and redistributes β-amyloid in the choroid plexus and retains T4 in the cerebrospinal fluid (CSF). Under certain conditions, TTR can dissociate into its monomeric subunits, each consisting of 127 amino acids, and undergo abnormal alterations, leading to the formation of amyloidogenic intermediates. These intermediates can self-aggregate into amyloid fibrils, which accumulate as amyloid deposits throughout the body, resulting in transthyretin amyloidosis. This condition can be classified into wild-type (wt) or hereditary forms, with the latter further 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 the misfolding of protein monomers derived from the tetrameric transthyretin (TTR). Mutations in the TTR gene often lead to TTR instability and subsequent fibril formation. Similarly, in wild-type TTR, the native 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, which encodes transthyretin, a protein that facilitates the transport of vitamin A and thyroxin. Mutations in TTR result in defective transthyretin that forms amyloid fibrils when misfolded. These amyloid deposits accumulate in various parts of the body, 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 (de novo) mutations in the TTR gene, without any family history of the disease.

Hereditary Transthyretin Amyloidosis 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 Epidemiology Segmentation:

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

Total Prevalence of Hereditary Transthyretin Amyloidosis
Prevalent Cases of Hereditary Transthyretin Amyloidosis by severity
Gender-specific Prevalence of Hereditary Transthyretin Amyloidosis
Diagnosed Cases of Episodic and Chronic Hereditary Transthyretin Amyloidosis

Hereditary Transthyretin Amyloidosis 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 market or expected to get launched during the study period. The analysis covers Hereditary Transthyretin Amyloidosis 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 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 Therapies

Vyndaqel
Onpattro
Tegsedi
Vutrisiran
Eplontersen
Acoramidis
CRX-1008

Hereditary Transthyretin Amyloidosis 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 market share @ Hereditary Transthyretin Amyloidosis Treatment Landscape

Hereditary Transthyretin Amyloidosis Market Outlook

Hereditary Transthyretin Amyloidosis 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 therpaies 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 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 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 Therapies: Vyndaqel, Onpattro, Tegsedi, Vutrisiran, Eplontersen, Acoramidis, CRX-1008, and others
Hereditary Transthyretin Amyloidosis Therapeutic Assessment: Hereditary Transthyretin

Amyloidosis current marketed and Hereditary Transthyretin Amyloidosis emerging therapies
Hereditary Transthyretin Amyloidosis Market Dynamics: Hereditary Transthyretin Amyloidosis market drivers and Hereditary Transthyretin Amyloidosis market barriers
Competitive Intelligence Analysis: SWOT analysis, PESTLE analysis, Porter's five forces, BCG Matrix, Market entry strategies
Hereditary Transthyretin Amyloidosis Unmet Needs, KOL's views, Analyst's views, Hereditary Transthyretin Amyloidosis Market Access and Reimbursement

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Hereditary Transthyretin Amyloidosis Pipeline

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

Hereditary Transthyretin Amyloidosis Epidemiology

DelveInsight's 'Hereditary Transthyretin Amyloidosis Epidemiology Forecast to 2032' report delivers an in-depth understanding of the disease, historical and forecasted Hereditary Transthyretin Amyloidosis 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.

It also offers Healthcare Consulting Services, which benefits in market analysis to accelerate the business growth and overcome challenges with a practical approach.

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