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Hereditary Transthyretin Amyloidosis (hATTR)– Pipeline Insight, 2020

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Abstracts

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DelveInsight's, "Hereditary Transthyretin Amyloidosis (hATTR)– Pipeline Insight, 2020," report provides comprehensive insights about 10+ companies and 10+ pipeline drugs in Hereditary Transthyretin Amyloidosis 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.

Geography Covered

Global coverage

Hereditary Transthyretin Amyloidosis Understanding

Hereditary Transthyretin Amyloidosis: Overview

Transthyretin (TTR)—earlier known as prealbumin—is an abundant, soluble, ?-strand rich 55 kDa homotetramer serum protein that is responsible for the transportation of both vitamin A (via retinol-binding protein) and thyroxin throughout the body. The hereditary forms of amyloidosis are autosomal dominant diseases characterized by deposition of variant proteins, in distinctive tissues. The most common hereditary form is transthyretin amyloidosis (ATTR) caused by the misfolding of protein monomers derived from the tetrameric protein transthyretin (TTR).

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Symptoms

Symptoms of hATTR include:

- Weakness, numbness or pain in the lower legs and feet
- Carpal tunnel syndrome in both wrists
- Sexual impotence
- Urinary problems, protein in the urine
- Diarrhea or constipation
- Unexplained weight loss
- Dry eyes, increased pressure in the eyes (glaucoma), seeing ‘floaters’
- Abnormal heart beat, enlarged heart
- Getting dizzy when moving from sitting to standing (orthostatic hypotension)
- Dry eyes and mouth

Diagnosis

Disease heterogeneity and its rarity make a diagnosis of hATTR amyloidosis challenging. However, making a correct diagnosis is vital to determining prognosis, treatment, and appropriate patient and family counseling. Timely diagnosis is also important because it allows patients the opportunity to receive appropriate care as early as possible in the disease course. Diagnosis can be confirmed via biopsy of the affected tissue or organ followed by staining with Congo red to confirm the presence of amyloid. Diagnosis can be established less invasively through biopsy of the salivary gland, an endoscopic biopsy of the gastric mucosa, or subcutaneous fat aspiration. Western blot analysis, immunohistochemical staining, laser microdissection, proteomics, and mass spectrometry are subsequently used to characterize amyloid type.

Treatment

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Current treatment options for patients with TTR amyloidosis are limited, with only symptomatic treatment and transplantation. For patients diagnosed with TTR-FAP who have mild or moderate disease and confirmed by genetic testing and biopsy, a liver transplant is the current standard of care. However, symptomatic treatment is used to provide immediate relief. Since variant TTR is mainly produced in the liver, transplanting a new liver should almost eliminate the production of variant protein and further halt the disease progression outside the brain and eyes. However, the liver transplant does not effectively prevent cardiomyopathy in most cases and is not recommended for patients with late-stage TTR-FAP or with leptomeningeal-type amyloidosis. Thus, for these patients, symptomatic relief is the only available treatment. The very first pharmacologic agent to receive approval for the treatment of TTR-FAP was in 2011, and many other agents are in various stages of development. cell mediators.

Hereditary Transthyretin Amyloidosis Emerging Drugs Chapters

This segment of the Hereditary Transthyretin Amyloidosis report encloses its detailed analysis of various drugs in different stages of clinical development, including phase II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological action, agreements and collaborations, and the latest news and press releases.

Hereditary Transthyretin Amyloidosis 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 mutant 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. It utilizes Alnylam’s next-generation delivery platform known as the Enhanced Stabilization Chemistry (ESC)-GalNAc-conjugate delivery platform. Also, the drug is currently in phase III stage of development.

AKCEA-TTR-LRx: Ionis Pharmaceuticals

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AKCEA-TTR-LRx is a generation 2+ ligand-conjugated antisense (LICA) drug designed to reduce the production of transthyretin, or TTR protein, to treat all types of TTR amyloidosis (ATTR). The drug is currently in phase III stage of development.

AG 10: Eidos Therapeutics

AG 10 is an orally available, small molecule transthyretin (TTR) stabilizer being developed by Eidos Therapeutics (a subsidiary of BridgeBio Pharma), for the treatment of transthyretin-related hereditary amyloidosis. AG10 was designed to mimic a naturally-occurring variant of the TTR gene (T119M) that is considered a rescue mutation because co-inheritance has been shown to prevent or ameliorate ATTR in individuals also inheriting a pathogenic, or disease-causing, mutation in the TTR gene. The drug is the only TTR stabilizer in development that has been observed to mimic the stabilizing structure of this rescue mutation and is currently in phase III stage of development.

Further product details are provided in the report

Hereditary Transthyretin Amyloidosis: Therapeutic Assessment

This segment of the report provides insights about the different Hereditary Transthyretin Amyloidosis drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Hereditary Transthyretin Amyloidosis

There are approx. 10+ key companies which are developing the therapies for Hereditary Transthyretin Amyloidosis. The companies which have their Hereditary Transthyretin Amyloidosis drug candidates in the most advanced stage, i.e. phase III include, Alnylam Pharmaceuticals, Ionis Pharmaceuticals, Eidos Therapeutics.

Phases

DelveInsight’s report covers around 10+ products under different phases of clinical development like

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Late stage products (Phase III)

Mid-stage products (Phase II)

Early-stage product (Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

Hereditary Transthyretin Amyloidosis 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

intravitreal

Subretinal

Topical.

Molecule Type

Products have been categorized under various Molecule types such as

Monoclonal Antibody

Peptides

Polymer

Small molecule

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Gene therapy

Product Type

Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.

Hereditary Transthyretin Amyloidosis: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Hereditary Transthyretin Amyloidosis therapeutic drugs key players involved in developing key drugs.

Pipeline Development Activities

The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Hereditary Transthyretin Amyloidosis drugs.

Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Hereditary Transthyretin Amyloidosis R&D. The therapies under development are focused on novel approaches to treat/improve Hereditary Transthyretin Amyloidosis.

Alnylam Pharmaceuticals announced that the FDA granted Fast Track designation to vutrisiran In April 2020, which is an investigational therapy for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults.

In April 2018 Akcea Therapeutics and Ionis Pharmaceuticals announced that the two companies had completed a previously announced transaction licensing the exclusive, worldwide rights from Ionis to Akcea for inotersen and AKCEA-TTR-LRx, formerly IONIS-TTR-LRx.

In October 2018, Eidos Therapeutics announced that the FDA had granted the

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company Orphan Drug Designation for AG10 for the treatment of ATTR.

Hereditary Transthyretin Amyloidosis Report Insights

Hereditary Transthyretin Amyloidosis Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Hereditary Transthyretin Amyloidosis Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

Unmet Needs

Key Questions

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Hereditary Transthyretin Amyloidosis drugs?

How many Hereditary Transthyretin Amyloidosis drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Hereditary Transthyretin Amyloidosis?

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What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Hereditary Transthyretin Amyloidosis therapeutics?

What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Hereditary Transthyretin Amyloidosis and their status?

What are the key designations that have been granted to the emerging drugs?

Key Players

Alnylam Pharmaceuticals

Ionis Pharmaceuticals

Eidos Therapeutics

Corino Therapeutics Inc

Prothena

Intellia Therapeutics

Regeneron Pharmaceuticals

Key Products

ALN-TTRSC02

ION-682884

AG 10

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Tolcapone

SOM0226

PRX 004

NTLA 2001

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