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ATTR Amyloidosis – Pipeline Insight, 2020

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Abstracts

This report can be delivered to the clients within 48-72 Hours

DelveInsight's, "ATTR Amyloidosis – Pipeline Insight, 2020," report provides comprehensive insights about 8+ companies and 8+ pipeline drugs in ATTR 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

ATTR Amyloidosis Understanding

ATTR Amyloidosis: Overview

ATTR Amyloidosis is a disorder of protein misfolding. Normally, liver made Transthyretin (TTR) proteins is made up of four identical parts and helps in carrying of thyroid hormone and vitamin A in the blood. However, in ATTR amyloidosis, the protein becomes unstable, breaks apart, and deposits in the heart and/or the nerves. ATTR amyloidosis can be either hereditary or acquired (non-hereditary). Hereditary ATTR amyloidosis is caused due to inheritance of mutated DNA while in Wild-type ATTR amyloidosis, normal TTR protein becomes unstable, misfolds and forms amyloid fibrils.

Symptoms

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The symptoms of ATTR Amyloidosis depends on the organ involved and include:

Carpal tunnel syndrome.

Numbness, burning and/or tingling (peripheral neuropathy).

Biceps tendon rupture.

Lumbar spinal stenosis.

Swelling of the feet or legs.

Eye floaters (vitreous opacities).

Shortness of breath.

Palpitations.

Chest pain.

Diarrhea or constipation.

Diagnosis

A diagnosis of ATTR Amyloidosis is based upon patients' symptoms, findings on physical examination and sometimes family history. The diagnosis can be confirmed (or eliminated) by certain tests like Tissue biopsy, Genetic testing, and Imaging studies.

Treatment

The goals for ATTR amyloidosis treatment is to stop disease progression by limiting TTR deposits, and minimize the effects that ATTR has on your body. The specific therapeutic treatment used depend upon several factors, including the severity of the disorder. Therapies that are used to treat ATTR Amyloidosis are focused on principles like Small interfering RNA and Antisense oligonucleotides.

ATTR Amyloidosis Emerging Drugs Chapters

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This segment of the ATTR 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.

ATTR Amyloidosis Emerging Drugs

Acoramidis - Eidos Therapeutics

Acoramidis (formerly AG10) is an investigational, orally-administered small molecule designed to potently stabilize tetrameric transthyretin, or TTR. Acoramidis 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 ATTR in individuals also inheriting a pathogenic, or disease-causing, mutation in the TTR gene. Acoramidis is currently being studied in a Phase 3 clinical trial. Eidos Therapeutics get acoramidis license from Stanford University in 2016.

AKCEA-TTR-LRx: Ionis Pharmaceuticals

AKCEA-TTR-LRx is an antisense drug that uses Ionis' advanced LIgand Conjugated Antisense, or LICA, technology. AKCEA-TTR-LRx inhibits the production of the transthyretin (TTR) protein at its source. It is in development to treat a broad population of patients with both hereditary and wild-type forms of transthyretin amyloidosis, or ATTR amyloidosis. It was originally discovered by Ionis and is being co-developed by Ionis and Akcea. On 12 Oct, 2020 Akcea Therapeutics has been acquired by Ionis Pharmaceuticals.

Further product details are provided in the report

ATTR Amyloidosis: Therapeutic Assessment

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

Major Players in ATTR Amyloidosis

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There are approx. 8+ key companies which are developing the therapies for ATTR Amyloidosis. The companies which have their ATTR Amyloidosis drug candidates in the most advanced stage, i.e. phase III include, Eidos Therapeutics.

Phases

DelveInsight's report covers around 8+ products under different phases of clinical development like

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

ATTR 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.

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Molecule Type

Products have been categorized under various Molecule types such as

Monoclonal Antibody

Peptides

Polymer

Small molecule

Gene therapy

Product Type

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

ATTR Amyloidosis: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses ATTR 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 ATTR Amyloidosis drugs.

Report Highlights

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

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ATTR Amyloidosis Report Insights

- ATTR Amyloidosis Pipeline Analysis
- Therapeutic Assessment
- Unmet Needs
- Impact of Drugs

ATTR 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 ATTR Amyloidosis drugs?
- How many ATTR Amyloidosis drugs are developed by each company?
- How many emerging drugs are in mid-stage, and late-stage of development for the treatment of ATTR Amyloidosis?
- What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the ATTR Amyloidosis

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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 ATTR Amyloidosis and their status?

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

Key Players

Eidos Therapeutics

Ionis Pharmaceuticals

Alnylam Pharmaceuticals

Intellia Therapeutics

Prothena

Corino Therapeutics

SOM Biotech

Neurimmune Therapeutics

Key Products

Acoramidis

AKCEA-TTR-LRx

Vutrisiran

NTLA-2001

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PRX004

Tolcapone

Revusiran (ALN-TTRSC)

NI-006

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