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Alpha-1 antitrypsin deficiency (AATD) – Pipeline Insight, 2020

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Abstracts

This report can be delivered to the clients within 72 Hours

DelveInsight's, "Alpha-1 antitrypsin deficiency (AATD) – Pipeline Insight, 2020," report provides comprehensive insights about 25+ companies and 25+ pipeline drugs in Alpha-1 antitrypsin deficiency 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

Alpha-1 antitrypsin deficiency Understanding

Alpha-1 antitrypsin deficiency: Overview

Alpha-1 antitrypsin deficiency is an inherited disorder that may cause lung disease and liver disease. The signs and symptoms of the condition and the age at which they appear vary among individuals. Alpha1-antitrypsin is a protein made by the liver whose function is to protect the lungs. If these proteins are malformed or deficient, the impact is a predisposition for obstructive pulmonary disease and liver disease. This deficiency may predispose an individual to several illnesses and most commonly manifests as chronic obstructive pulmonary disease (including bronchiectasis) and liver disease (especially cirrhosis and hepatoma), or more rarely, as a skin condition called

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panniculitis. A deficiency of A1AT allows substances that break down proteins (so-called proteolytic enzymes) to attack various tissues of the body. The attack results in destructive changes in the lungs (emphysema) and may also affect the liver and skin.

Symptoms

People with alpha-1 antitrypsin deficiency usually develop the first signs and symptoms of lung disease between ages 20 and 50. The earliest symptoms are shortness of breath following mild activity, reduced ability to exercise, and wheezing. Other signs and symptoms can include unintentional weight loss, recurring respiratory infections, fatigue, and rapid heartbeat upon standing. Affected individuals often develop emphysema, which is a lung disease caused by damage to the small air sacs in the lungs (alveoli). Characteristic features of emphysema include difficulty breathing, a hacking cough, and a barrel-shaped chest. Smoking or exposure to tobacco smoke accelerates the appearance of emphysema symptoms and damage to the lungs.

Diagnosis

The diagnosis of A1AD is based on a low concentration of A1AT blood plasma in combination with a high-risk phenotype (demonstrated by isoelectric focusing) or genotype (by specific allele analysis [usually for the Z and S alleles and sometimes for the F and I alleles on commercial tests]). In some instances, further testing to sequence the A1AT gene is needed to establish a firm diagnosis (i.e., mapping all the chemical elements [called nucleotides] that make up the A1AT gene).

Treatment

Treatment of pulmonary disease is with purified human alpha-1 antitrypsin (60 mg/kg IV over 45 to 60 minutes given once a week or 250 mg/kg over 4 to 6 hours given once a month [pooled only]), which can maintain the serum alpha-1 antitrypsin level above a target protective level of 80 mg/dL (35% of normal). Treatment is expensive and is therefore reserved for nonsmoking patients who have two abnormal alleles, mild to moderately abnormal pulmonary function, and confirmation of diagnosis by low serum alpha-1 antitrypsin levels.

Smoking cessation, use of bronchodilators, and early treatment of respiratory infections are particularly important for patients with alpha-1 antitrypsin deficiency and emphysema.

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For severely impaired people
Gene therapy is under study.

Treatment of liver disease is supportive. Enzyme replacement does not help because the disease is caused by abnormal processing rather than by enzyme deficiency. Liver transplantation may be used for patients with liver failure.

Alpha-1 antitrypsin deficiency Emerging Drugs Chapters

This segment of the Alpha-1 antitrypsin deficiency 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.

Alpha-1 antitrypsin deficiency Emerging Drugs

DCR-A1AT: Dicerna

DCR-A1AT is a subcutaneously administered ribonucleic acid interference (RNAi) therapeutic that is being investigated for the treatment of liver disease in patients with alpha-1 antitrypsin (A1AT) deficiency. DCR-A1AT, which incorporates Dicerna’s proprietary GalXC technology, is designed to target SERPINA1, reducing production of abnormal A1AT in the liver. A clinical trial program investigating the safety and efficacy of DCR-A1AT is currently underway. The U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to Dicerna’s DCR-A1AT for the treatment of alpha-1 antitrypsin (A1AT) deficiency.

ARO-AAT: Arrowhead Pharmaceuticals

ARO-AAT is being developed to treat the liver disease associated with alpha-1 antitrypsin deficiency (AATD), a rare genetic disorder that severely damages the liver and lungs of affected individuals. ARO-AAT is designed to knock down the hepatic production of the mutant alpha-1 antitrypsin (Z-AAT) protein, the cause of progressive liver disease in AATD patients. Reducing production of the inflammatory Z-AAT protein is expected to halt the progression of liver disease and potentially allow it to regenerate

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and repair.

Further product details are provided in the report

Alpha-1 antitrypsin deficiency: Therapeutic Assessment

This segment of the report provides insights about the different Alpha-1 antitrypsin deficiency drugs segregated based on following parameters that define the scope of the report, such as:

- Major Players in Alpha-1 antitrypsin deficiency

There are approx. 25+ key companies which are developing the therapies for Alpha-1 antitrypsin deficiency. The companies which have their Alpha-1 antitrypsin deficiency drug candidates in the mid to advanced stage, i.e. phase III and Phase II include, Kamada, Grifols Therapeutics, CSL Behring, Inhibrx, Alnylam Pharmaceuticals etc.

Phases

DelveInsight's report covers around 25+ products under different phases of clinical development like

- Mid-stage products (Phase II and Phase I/II)
- Early-stage products (Phase I/II and Phase I) along with the details of
- Pre-clinical and Discovery stage candidates
- Discontinued & Inactive candidates
- Route of Administration

Alpha-1 antitrypsin deficiency pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

- Subcutaneous

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Intravenous

Intramuscular

Oral

Molecule Type

Products have been categorized under various Molecule types such as

Blood proteins

Gene therapies

Small interfering RNA

Small molecules

Product Type

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

Alpha-1 antitrypsin deficiency: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Alpha-1 antitrypsin deficiency 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 Alpha-1 antitrypsin deficiency drugs.

Report Highlights

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The companies and academics are working to assess challenges and seek opportunities that could influence Alpha-1 antitrypsin deficiency R&D. The therapies under development are focused on novel approaches to treat/improve Alpha-1 antitrypsin deficiency.

March 2020: Dicerna receives Orphan Drug Designation from U.S. Food and Drug Administration for DCR-A1AT for treatment of Alpha-1 Antitrypsin deficiency.

April 2020: - Alnylam Pharmaceuticals, Inc. and Dicerna Pharmaceuticals, Inc. announced the formation of a development and commercialization collaboration on investigational RNAi therapeutics for the treatment of alpha-1 antitrypsin (A1AT) deficiency-associated liver disease (alpha-1 liver disease).

Alpha-1 antitrypsin deficiency Report Insights

Alpha-1 antitrypsin deficiency Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Alpha-1 antitrypsin deficiency Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

Unmet Needs

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Key Questions

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Alpha-1 antitrypsin deficiency drugs?

How many Alpha-1 antitrypsin deficiency drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Alpha-1 antitrypsin deficiency?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Alpha-1 antitrypsin deficiency 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 Alpha-1 antitrypsin deficiency and their status?

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

Key Players

Octapharma

Dicerna Pharmaceuticals

Alnylam Pharmaceuticals

Arrowhead Pharmaceuticals

Apollo Therapeutics

Vertex Pharmaceuticals

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AstraZeneca

Kamada, Ltd.

Key Products

OctaAlpha1

DCR-A1AT

ALN-AAT02

ARO-AAT

Research programme: genetic disorder therapeutics

VX-814

Alvelestat

Inhaled AAT

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