

Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease - Pipeline Insight, 2021

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Abstracts

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DelveInsight's, "Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease - Pipeline Insight, 2021," report provides comprehensive insights about 10+ companies and 10+ pipeline drugs in Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease 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 (A1ATD) Liver Disease Understanding

Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease: Overview

Alpha-1 antitrypsin is a large, 52?kDa serum glycoprotein and a serine protease inhibitor. Alpha-1 antitrypsin deficiency disease is an inherited metabolic disorder in which this protein is absent or defective. The protein Alpha-1 antitrypsin (AAT) is primarily synthesized and secreted by liver hepatocytes. Primary function depends on its secretion from the liver and its physiological action in the lungs to prevent excessive tissue destruction by neutrophil elastase. Alpha-Antitrypsin (AAT) Deficiency occurs when the body is unable to produce sufficient amounts of AAT protein, exposing the

organs to harmful effects of proteolytic enzymes, such as neutrophil elastase. 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 panniculitis.

'Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease pipeline landscape is provided which includes the disease overview and Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease treatment guidelines. The assessment part of the report embraces, in depth Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease commercial assessment and clinical assessment of the pipeline products under development. In the report, detailed description of the drug is given which includes mechanism of action of the drug, clinical studies, NDA approvals (if any), and product development activities comprising the technology, Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease collaborations, licensing, mergers and acquisition, funding, designations and other product related details.

Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease R&D. The therapies under development are focused on novel approaches to treat/improve Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease.

Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease Emerging Drugs Chapters

This segment of the Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease 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 (A1ATD) Liver Disease Emerging Drugs

ARO-AAT: Arrowhead Pharmaceuticals

ARO-AAT is a subcutaneous RNAi-based therapeutics, being developed to treat the liver disease associated with alpha-1 antitrypsin deficiency (AATD). 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 and repair. ARO-AAT is currently in Phase II/III clinical developmental stage for the treatment of Alpha-1 Antitrypsin Deficiency Associated Liver Disease (AATD).

PHP 303: PH Pharmaceutical

PHP-303 is a novel neutrophil elastase (NE) inhibitor. NE is a major inflammatory factor involved in the progression of NASH and AATD. Inhibition of NE improves inflammatory response and, especially in NASH, insulin resistance. PH Pharma has expanded PHP-303 indication to include the genetic disease alpha-1 antitrypsin deficiency (AATD) and completed a Phase 2 clinical study in EU.

ZF874: Centessa Pharmaceuticals

ZF874 is a small molecule chemical chaperone that specifically rescues the folding of the Z variant of alpha-1-antitrypsin (A1AT). It is being developed for the treatment of alpha-1-antitrypsin deficiency (AATD) caused by the Z-mutation. It is a novel compound that acts as a molecular 'patch' for the faulty protein, allowing it to fold correctly, thereby simultaneously relieving the liver burden of polymer accumulation and providing fully-functional Z-A1AT in the circulation to protect the lungs. The drug is currently in phase I stage of development for the treatment of Alpha1 Anti-Trypsin Deficiency.

Further product details are provided in the report

Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease: Therapeutic Assessment

This segment of the report provides insights about the different Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease

There are approx. 10+ key companies which are developing the therapies for Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease. The companies which have their Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease drug candidates in the most advanced stage, i.e. phase II/III include, Arrowhead Pharmaceuticals.

Phases

DelveInsight's report covers around 10+ 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

Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease 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

Subcutaneous

Molecule Type

Products have been categorized under various Molecule types such as

Gene therapy

RNAi

Small interfering RNA

Small molecule

Product Type

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

Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease: 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 (A1ATD) Liver Disease 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 (A1ATD) Liver Disease drugs.

Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease Report Insights

Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease 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 Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease drugs?

How many Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease 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 (A1ATD) Liver Disease?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease 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 (A1ATD) Liver Disease and their status?

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

Key Players

Arrowhead Pharmaceuticals

PH Pharmaceutical

Centessa Pharmaceuticals

Novartis

Vertex Pharmaceuticals

Dicerna Pharmaceuticals

Alnylam Pharmaceuticals

Apic Bio

LogicBio Therapeutics

Key Products

ARO-AAT

PHP 303

ZF874

Carbamazepine

VX-864

Belcesiran

ALN-AAT02

APB-101

LB 201

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Assessment by Stage and Route of Administration

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Comparative Analysis

ARO-AAT: Arrowhead Pharmaceuticals

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.....

Early Stage Products (Phase I)

Comparative Analysis

ZF874: Centessa Pharmaceuticals

Product Description

Research and Development

Product Development Activities
Drug profiles in the detailed report.....

Inactive Products

Comparative Analysis

Alpha-1 Antitrypsin Deficiency (A1ATD) Liver Disease Key Companies

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