

# Alpha- Antitrypsin Deficiency - Pipeline Review, H2 2018

<https://marketpublishers.com/r/A0FFE477946EN.html>

Date: August 2018

Pages: 83

Price: US\$ 2,000.00 (Single User License)

ID: A0FFE477946EN

## Abstracts

Alpha-Antitrypsin Deficiency - Pipeline Review, H2 2018

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Alpha-Antitrypsin Deficiency - Pipeline Review, H2 2018, provides an overview of the Alpha-Antitrypsin Deficiency (Genetic Disorders) pipeline landscape.

Alpha-antitrypsin deficiency is an inherited disease that occurs due to lack of alpha-1 antitrypsin (AAT), a protein that protects the lungs. Symptoms include shortness of breath and wheezing, lung infections, rapid heartbeat, weight loss and vision problems. Predisposing factors include smoking, exposure to dust, chemical fumes and infections.

### REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Alpha-Antitrypsin Deficiency - Pipeline Review, H2 2018, provides comprehensive information on the therapeutics under development for Alpha-Antitrypsin Deficiency (Genetic Disorders), complete with analysis by stage of development, drug target, mechanism of action (MoA), route of administration (RoA) and molecule type. The guide covers the descriptive pharmacological action of the therapeutics, its complete research and development history and latest news and press releases.

The Alpha-Antitrypsin Deficiency (Genetic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Alpha-Antitrypsin Deficiency and features dormant and discontinued projects. The guide covers therapeutics under

Development by Companies/Universities/Institutes, the molecules developed by Companies in Filing rejected/Withdrawn, Phase III, Phase II, Phase I, Preclinical, Discovery and Unknown stages are 1, 1, 3, 5, 12, 2 and 1 respectively. Similarly, the Universities portfolio in Discovery stages comprises 1 molecules, respectively.

Alpha-Antitrypsin Deficiency (Genetic Disorders) pipeline guide helps in identifying and tracking emerging players in the market and their portfolios, enhances decision making capabilities and helps to create effective counter strategies to gain competitive advantage. The guide is built using data and information sourced from Global Markets Direct's proprietary databases, company/university websites, clinical trial registries, conferences, SEC filings, investor presentations and featured press releases from company/university sites and industry-specific third party sources. Additionally, various dynamic tracking processes ensure that the most recent developments are captured on a real time basis.

**Note:** Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

## SCOPE

The pipeline guide provides a snapshot of the global therapeutic landscape of Alpha-Antitrypsin Deficiency (Genetic Disorders).

The pipeline guide reviews pipeline therapeutics for Alpha-Antitrypsin Deficiency (Genetic Disorders) by companies and universities/research institutes based on information derived from company and industry-specific sources.

The pipeline guide covers pipeline products based on several stages of development ranging from pre-registration till discovery and undisclosed stages.

The pipeline guide features descriptive drug profiles for the pipeline products which comprise, product description, descriptive licensing and collaboration details, R&D brief, MoA & other developmental activities.

The pipeline guide reviews key companies involved in Alpha-Antitrypsin Deficiency (Genetic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Alpha-Antitrypsin Deficiency (Genetic Disorders) therapeutics based on mechanism of action (MoA), drug target, route of administration (RoA) and molecule type.

The pipeline guide encapsulates all the dormant and discontinued pipeline projects.

The pipeline guide reviews latest news related to pipeline therapeutics for Alpha-Antitrypsin Deficiency (Genetic Disorders)

## **REASONS TO BUY**

Procure strategically important competitor information, analysis, and insights to formulate effective R&D strategies.

Recognize emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage.

Find and recognize significant and varied types of therapeutics under development for Alpha-Antitrypsin Deficiency (Genetic Disorders).

Classify potential new clients or partners in the target demographic.

Develop tactical initiatives by understanding the focus areas of leading companies.

Plan mergers and acquisitions meritoriously by identifying key players and it's most promising pipeline therapeutics.

Formulate corrective measures for pipeline projects by understanding Alpha-Antitrypsin Deficiency (Genetic Disorders) pipeline depth and focus of Indication therapeutics.

Develop and design in-licensing and out-licensing strategies by identifying prospective partners with the most attractive projects to enhance and expand business potential and scope.

Adjust the therapeutic portfolio by recognizing discontinued projects and

understand from the know-how what drove them from pipeline.

## Contents

Introduction

Global Markets Direct Report Coverage

Alpha- Antitrypsin Deficiency - Overview

Alpha- Antitrypsin Deficiency - Therapeutics Development

Pipeline Overview

Pipeline by Companies

Pipeline by Universities/Institutes

Products under Development by Companies

Products under Development by Universities/Institutes

Alpha- Antitrypsin Deficiency - Therapeutics Assessment

Assessment by Target

Assessment by Mechanism of Action

Assessment by Route of Administration

Assessment by Molecule Type

Alpha- Antitrypsin Deficiency - Companies Involved in Therapeutics Development

Adverum Biotechnologies Inc

Alnylam Pharmaceuticals Inc

Applied Genetic Technologies Corp

Arrowhead Pharmaceuticals Inc

AstraZeneca Plc

Carolus Therapeutics Inc

Editas Medicine Inc

Grifols SA

Inhibrx LP

Intellia Therapeutics Inc

International Stem Cell Corp

Kamada Ltd

Octapharma AG

OncBioMune Pharmaceuticals Inc

PlantForm Corp

Promethera Biosciences SA

ProMetic Life Sciences Inc

rEVO Biologics Inc

Santhera Pharmaceuticals Holding AG

Therapure Biopharma Inc

Z Factor Ltd

Alpha- Antitrypsin Deficiency - Drug Profiles

ADVM-043 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

AGTC-0106 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

ALNAAT-02 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

alpha-1 proteinase inhibitor (human) - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

alpha-1 proteinase inhibitor (human) second generation - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

alvelestat - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

APB-101 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

ARO-AAT - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

CT-2009 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Drug for Chronic Obstructive Pulmonary Disease and Alpha-1 Antitrypsin Deficiency -  
Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Fusion Protein for Alpha-1 Antitrypsin Deficiency - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy for Alpha-1 Antitrypsin Deficiency - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate Alpha 1 Antitrypsin for Alpha- Antitrypsin Deficiency - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

H2Stem - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Oligonucleotides to Activate SERPINA1 for Alpha-Antitrypsin Deficiency - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Oligonucleotides to Inhibit Alpha-1 Antitrypsin for Alpha-Antitrypsin Deficiency - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

POL-6014 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Recombinant Human Alpha-1 Antitrypsin - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Recombinant Protein for Alpha 1 Antitrypsin Deficiency - Drug Profile

Product Description

Mechanism Of Action

## R&D Progress

Small Molecules for Alpha-1 Antitrypsin Deficiency - Drug Profile

Product Description

Mechanism Of Action

## R&D Progress

Small Molecules to Activate SERPINA1 for Alpha-1 Antitrypsin Deficiency - Drug Profile

Product Description

Mechanism Of Action

## R&D Progress

Small Molecules to Inhibit Alpha-1 Antitrypsin Polymerization for Alpha-1 Antitrypsin Deficiency - Drug Profile

Product Description

Mechanism Of Action

## R&D Progress

Stem Cell Therapy for Alpha-Antitrypsin Deficiency, Crigler-Najjar Syndrome and Liver Cirrhosis - Drug Profile

Product Description

Mechanism Of Action

## R&D Progress

Alpha- Antitrypsin Deficiency - Dormant Projects

Alpha- Antitrypsin Deficiency - Discontinued Products

Alpha- Antitrypsin Deficiency - Product Development Milestones

## Featured News & Press Releases

Jul 10, 2018: Kamada Receives Positive Scientific Advice from European Medicines Agency on a New Phase 3 Study Design for Inhaled AAT

Jun 29, 2018: Arrowhead reports initial positive results from AROAAT1001 trial

Jun 27, 2018: Arrowhead Receives Positive EMA Opinion on Orphan Designation for ARO-AAT for Treatment of Congenital Alpha-1 Antitrypsin Deficiency

Mar 12, 2018: Arrowhead Begins Dosing in Phase 1 Study of ARO-AAT for Treatment of Alpha-1 Liver Disease

Feb 15, 2018: Arrowhead Pharmaceuticals Receives Orphan Drug Designation for ARO-AAT

Dec 20, 2017: Arrowhead Pharmaceuticals Files for Regulatory Clearance to Begin Phase 1 Study of ARO-AAT

Oct 23, 2017: Arrowhead Presents Promising Preclinical Data on Development of ARO-AAT for Treatment of Alpha-1 Liver Disease at Liver Meeting 2017

Oct 10, 2017: Arrowhead Pharmaceuticals to Present Preclinical Data on ARO-AAT at The Liver Meeting

Sep 25, 2017: Alnylam Continues Leadership in RNAi Technologies and Delivery with



New Pre-Clinical Data Presented on 'Enhanced Stabilization Chemistry Plus' GalNAc-siRNA Conjugate Platform at 13th Annual Meeting of the Oligonucleotide Therapeutics Society

Sep 22, 2017: FDA approves Grifols Prolastin-C Liquid [alpha-1 proteinase inhibitor, liquid] for the treatment of alpha-1 antitrypsin deficiency

Aug 22, 2017: Apic Bio Launches to Advance First-in-Class Gene Therapy for Treatment of Alpha-1 Antitrypsin Deficiency

Jul 20, 2017: Kamada Submits Proposed Phase 3 Protocol to FDA for Inhaled Alpha-1 Antitrypsin for Treatment of Alpha-1 Antitrypsin Deficiency Disease

Jun 22, 2017: Kamada Announces Withdrawal of European Marketing Authorization Application for Inhaled Alpha-1 Antitrypsin for Treatment of Alpha-1 Antitrypsin Deficiency Disease

Jun 08, 2017: Data Published in Molecular Therapy Demonstrate Five-Year Response Following a Single Dose of an Investigational Gene-Based Therapy for Alpha-1 Antitrypsin (AAT) Deficiency

May 24, 2017: Kamada Presents Updated Data from Phase 2 Clinical Trial of Inhaled Alpha-1-Antitrypsin for Treatment of Alpha-1 Antitrypsin Deficiency at 2017 American Thoracic Society International Conference

Appendix

Methodology

Coverage

Secondary Research

Primary Research

Expert Panel Validation

Contact Us

Disclaimer

## List Of Tables

### LIST OF TABLES

Number of Products under Development for Alpha- Antitrypsin Deficiency, H2 2018  
Number of Products under Development by Companies, H2 2018  
Number of Products under Development by Companies, H2 2018 (Contd.1), H2 2018  
Number of Products under Development by Universities/Institutes, H2 2018  
Products under Development by Companies, H2 2018  
Products under Development by Companies, H2 2018 (Contd.1), H2 2018  
Products under Development by Universities/Institutes, H2 2018  
Number of Products by Stage and Target, H2 2018  
Number of Products by Stage and Mechanism of Action, H2 2018  
Number of Products by Stage and Route of Administration, H2 2018  
Number of Products by Stage and Molecule Type, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Adverum Biotechnologies Inc, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Alnylam Pharmaceuticals Inc, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Applied Genetic Technologies Corp, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Arrowhead Pharmaceuticals Inc, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by AstraZeneca Plc, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Carolus Therapeutics Inc, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Editas Medicine Inc, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Grifols SA, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Inhibrx LP, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Intellia Therapeutics Inc, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by International Stem Cell Corp, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Kamada Ltd, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Octapharma AG, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by OncBioMune Pharmaceuticals Inc, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by PlantForm Corp, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Promethera Biosciences SA, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by ProMetic Life Sciences Inc, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by rEVO Biologics Inc, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Santhera Pharmaceuticals Holding AG, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Therapure Biopharma Inc, H2 2018  
Alpha- Antitrypsin Deficiency - Pipeline by Z Factor Ltd, H2 2018  
Alpha- Antitrypsin Deficiency - Dormant Projects, H2 2018  
Alpha- Antitrypsin Deficiency - Discontinued Products, H2 2018



## List Of Figures

### LIST OF FIGURES

Number of Products under Development for Alpha- Antitrypsin Deficiency, H2 2018

Number of Products under Development by Companies, H2 2018

Number of Products by Targets, H2 2018

Number of Products by Stage and Targets, H2 2018

Number of Products by Mechanism of Actions, H2 2018

Number of Products by Stage and Mechanism of Actions, H2 2018

Number of Products by Routes of Administration, H2 2018

Number of Products by Stage and Routes of Administration, H2 2018

Number of Products by Molecule Types, H2 2018

Number of Products by Stage and Molecule Types, H2 2018

### COMPANIES MENTIONED

Adverum Biotechnologies Inc

Alnylam Pharmaceuticals Inc

Applied Genetic Technologies Corp

Arrowhead Pharmaceuticals Inc

AstraZeneca Plc

Carolus Therapeutics Inc

Editas Medicine Inc

Grifols SA

Inhibrx LP

Intellia Therapeutics Inc

International Stem Cell Corp

Kamada Ltd

Octapharma AG

OncBioMune Pharmaceuticals Inc

PlantForm Corp

Promethera Biosciences SA

ProMetic Life Sciences Inc

rEVO Biologics Inc

Santhera Pharmaceuticals Holding AG

Therapure Biopharma Inc

Z Factor Ltd

## I would like to order

Product name: Alpha- Antitrypsin Deficiency - Pipeline Review, H2 2018

Product link: <https://marketpublishers.com/r/A0FFE477946EN.html>

Price: US\$ 2,000.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer Service:

[info@marketpublishers.com](mailto:info@marketpublishers.com)

## Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <https://marketpublishers.com/r/A0FFE477946EN.html>

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name:  
Last name:  
Email:  
Company:  
Address:  
City:  
Zip code:  
Country:  
Tel:  
Fax:  
Your message:

**\*\*All fields are required**

Customer signature \_\_\_\_\_

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at <https://marketpublishers.com/docs/terms.html>

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970