

# Alpha- Antitrypsin Deficiency - Pipeline Review, H1 2018

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## Abstracts

Alpha%li%Antitrypsin Deficiency - Pipeline Review, H1 2018

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Alpha%li%Antitrypsin Deficiency - Pipeline Review, H1 2018, provides an overview of the Alpha%li%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%li%Antitrypsin Deficiency - Pipeline Review, H1 2018, provides comprehensive information on the therapeutics under development for Alpha%li%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%li%Antitrypsin Deficiency (Genetic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Alpha%li%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Development by Companies/Universities/Institutes, the molecules developed by Companies in Filing rejected/Withdrawn, Phase III, Phase II, Phase I, IND/CTA Filed, Preclinical, Discovery and Unknown stages are 1, 1, 3, 3, 1, 11, 4 and 1 respectively. Similarly, the Universities portfolio in Discovery stages comprises 1 molecules, respectively.

Alpha%li%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%li%Antitrypsin Deficiency (Genetic Disorders).

The pipeline guide reviews pipeline therapeutics for Alpha%li%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%li%Antitrypsin Deficiency (Genetic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Alpha%li%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%li%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%li%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%li%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.

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Applied Genetic Technologies Corp

Arrowhead Pharmaceuticals Inc

AstraZeneca Plc

Carolus Therapeutics Inc

Cevec Pharmaceuticals GmbH

Editas Medicine Inc

Grifols SA

iBio Inc

Inhibrx LP

Intellia Therapeutics Inc

International Stem Cell Corp

Kamada Ltd

Octapharma AG

OncBioMune Pharmaceuticals Inc

Promethera Biosciences SA

ProMetic Life Sciences Inc

rEVO Biologics Inc

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Z Factor Ltd

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alpha-1 proteinase inhibitor (human) - Drug Profile

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Featured News & Press Releases

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

Aug 30, 2016: Kamada Meets Primary Endpoint of U.S. Phase 2 Study of Inhaled Alpha-1 Antitrypsin for the Treatment of Alpha-1 Antitrypsin Deficiency

May 05, 2016: Investigational Gene-Based Therapy for Alpha-1 Antitrypsin Deficiency Demonstrates Durable Response at Five Years

Jan 20, 2016: AGTC Announces Data Evaluating Novel AAV-Based Gene Therapy as a Potential Treatment for Alpha-1 Antitrypsin Deficiency

Dec 08, 2015: Kamada Completes Enrollment of U.S. Phase 2 Study of Inhaled Alpha-1 Antitrypsin for the Treatment of AAT Deficiency

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Alnylam Pharmaceuticals Inc

Applied Genetic Technologies Corp

Arrowhead Pharmaceuticals Inc

AstraZeneca Plc

Carolus Therapeutics Inc

Cevec Pharmaceuticals GmbH

Editas Medicine Inc

Grifols SA

iBio Inc

Inhibrx LP

Intellia Therapeutics Inc

International Stem Cell Corp

Kamada Ltd

Octapharma AG

OncBioMune Pharmaceuticals Inc

Promethera Biosciences SA

ProMetic Life Sciences Inc

rEVO Biologics Inc

Therapure Biopharma Inc

Z Factor Ltd

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