

# Transthyretin (ATTR or Prealbumin or TBPA or TTR) - Pipeline Review, H1 2018

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## Abstracts

Transthyretin (ATTR or Prealbumin or TBPA or TTR) - Pipeline Review, H1 2018

### SUMMARY

Transthyretin (ATTR or Prealbumin or TBPA or TTR) - Transthyretin is a transport protein. It transports thyroid hormones in the plasma and cerebrospinal fluid, and also transports retinol (vitamin A) in the plasma.

The diseases caused by mutations include amyloidotic polyneuropathy, euthyroid hyperthyroxinaemia, amyloidotic vitreous opacities, cardiomyopathy, oculoleptomeningeal amyloidosis, meningocerebrovascular amyloidosis and carpal tunnel syndrome.

Transthyretin (ATTR or Prealbumin or TBPA or TTR) pipeline Target constitutes close to 14 molecules. Out of which approximately 13 molecules are developed by companies and remaining by the universities/institutes. The molecules developed by companies in Pre-Registration, Filing rejected/Withdrawn, Phase II, Phase I and Preclinical stages are 2, 1, 1, 3 and 6 respectively.

Similarly, the universities portfolio in Phase II stages comprises 1 molecules, respectively. Report covers products from therapy areas Metabolic Disorders, Central Nervous System and Cardiovascular which include indications Familial Amyloid Neuropathies, Amyloidosis, Amyloid Cardiomyopathy, Familial Amyloid Cardiomyopathy, Alzheimer's Disease, Cardiomyopathy and Neuropathy.

The latest report Transthyretin - Pipeline Review, H1 2018, outlays comprehensive

information on the Transthyretin (ATTR or Prealbumin or TBPA or TTR) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type. It also reviews key players involved in Transthyretin (ATTR or Prealbumin or TBPA or TTR) targeted therapeutics development with respective active and dormant or discontinued projects.

The report is built using data and information sourced from 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.

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

## SCOPE

The report provides a snapshot of the global therapeutic landscape for Transthyretin (ATTR or Prealbumin or TBPA or TTR)

The report reviews Transthyretin (ATTR or Prealbumin or TBPA or TTR) targeted therapeutics under development by companies and universities/research institutes based on information derived from company and industry-specific sources

The report covers pipeline products based on various stages of development ranging from pre-registration till discovery and undisclosed stages

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

The report reviews key players involved in Transthyretin (ATTR or Prealbumin or TBPA or TTR) targeted therapeutics and enlists all their major and minor projects

The report assesses Transthyretin (ATTR or Prealbumin or TBPA or TTR) targeted therapeutics based on mechanism of action (MoA), route of administration (RoA) and molecule type

The report summarizes all the dormant and discontinued pipeline projects

The report reviews latest news and deals related to Transthyretin (ATTR or Prealbumin or TBPA or TTR) targeted therapeutics

## **REASONS TO BUY**

Gain strategically significant competitor information, analysis, and insights to formulate effective R&D strategies

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

Identify and understand the targeted therapy areas and indications for Transthyretin (ATTR or Prealbumin or TBPA or TTR)

Identify the use of drugs for target identification and drug repurposing

Identify potential new clients or partners in the target demographic

Develop strategic initiatives by understanding the focus areas of leading companies

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

Devise corrective measures for pipeline projects by understanding Transthyretin (ATTR or Prealbumin or TBPA or TTR) development landscape

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

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Arcturus Therapeutics Ltd

Ionis Pharmaceuticals Inc

Neurimmune Holding AG

Pfizer Inc

Prothena Corp Plc

Regeneron Pharmaceuticals Inc

Transthyretin (ATTR or Prealbumin or TBPA or TTR) - Drug Profiles

AG-10 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

AKCEA-TTR-LRx - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

ALN-TTRsc02 - Drug Profile

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Mechanism Of Action

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doxycycline hyclate - Drug Profile

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tafamidis meglumine - Drug Profile

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Product Description

Mechanism Of Action

R&D Progress

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Featured News & Press Releases

Apr 03, 2018: Silence Therapeutics: Litigation Update (Portugal and US)

Mar 29, 2018: Pfizer Announces Positive Topline Results From Phase 3 ATTR-ACT Study Of Tafamidis In Patients With Transthyretin Cardiomyopathy

Mar 28, 2018: Alnylam Presents New Clinical Results from the APOLLO Phase 3 Study of Patisiran at the 16th International Symposium on Amyloidosis

Mar 28, 2018: Alnylam Presents New data from the Phase 1 study of ALN-TTRsc02 at the 16th International Symposium on Amyloidosis

Mar 28, 2018: Proclara Biosciences Presents Preclinical Data Supporting the Development of GAIM-Based Therapies for Peripheral Amyloidoses at the 16th International Symposium on Amyloidosis

Mar 26, 2018: Ionis and Akcea Present New Data from ATTR Amyloidosis Program at 16th International Symposium on Amyloidosis

Mar 20, 2018: Eidos Therapeutics to Host Symposium on TTR Stabilization at the 16th International Symposium on Amyloidosis

Mar 14, 2018: Alnylam to Present Clinical Trial Data on Amyloidosis Drug Candidate ALN-TTRsc02 at the 16th International Symposium on Amyloidosis

Mar 14, 2018: Alnylam to Report New Clinical Results from the APOLLO Phase 3 Study of Patisiran at the 16th International Symposium on Amyloidosis

Feb 27, 2018: Intellia Therapeutics Announces Publication in Cell Reports of Preclinical Data Demonstrating Effective CRISPR/Cas9 Genome Editing Using Lipid Nanoparticle (LNP) Delivery Technology

Feb 01, 2018: Alnylam Announces FDA Acceptance of New Drug Application (NDA) and Priority Review Status for Patisiran, an Investigational RNAi Therapeutic for the Treatment of Hereditary ATTR (hATTR) Amyloidosis

Jan 25, 2018: Alnylam Announces EMA Acceptance of Marketing Authorisation Application (MAA) for Patisiran for the Treatment of Hereditary ATTR (hATTR) Amyloidosis

Jan 08, 2018: Ionis Inotersen NDA Accepted for Priority Review by the FDA

Dec 18, 2017: Sanofi and Alnylam Submit Marketing Authorization Application to the EMA for Patisiran for the Treatment of Hereditary ATTR Amyloidosis

Dec 12, 2017: Alnylam Completes Submission of New Drug Application to U.S. Food and Drug Administration (FDA) for Patisiran for the Treatment of Hereditary ATTR

(hATTR) Amyloidosis

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Arcturus Therapeutics Ltd  
Ionis Pharmaceuticals Inc  
Neurimmune Holding AG  
Pfizer Inc  
Prothena Corp Plc  
Regeneron Pharmaceuticals Inc

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