

Transthyretin (ATTR or Prealbumin or TBPA or TTR) - Pipeline Review, H2 2018

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Abstracts

Transthyretin (ATTR or Prealbumin or TBPA or TTR) - Pipeline Review, H2 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 The molecules developed by companies in Pre-Registration, Filing rejected/Withdrawn, Phase II, Phase I and Preclinical stages are 2, 1, 2, 4 and 4 respectively.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, H2 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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Assessment by Route of Administration

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Transthyretin (ATTR or Prealbumin or TBPA or TTR) - Companies Involved in
Therapeutics Development

Akcea Therapeutics Inc

Alnylam 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

Product Description

Mechanism Of Action

R&D Progress

doxycycline hyclate - Drug Profile

Product Description

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EDE-1307 - Drug Profile

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R&D Progress

inotersen sodium - Drug Profile

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NI-301 - Drug Profile

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NPT-189 - Drug Profile

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NTLA-1001 - Drug Profile

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patisiran - Drug Profile

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Mechanism Of Action

R&D Progress

Peptides to Inhibit TTR for Familial Amyloid Neuropathies - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

PRX-004 - Drug Profile

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R&D Progress

tafamidis meglumine - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

tolcapone - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

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Featured News & Press Releases

Oct 04, 2018: Akcea and Ionis announce approval of TEGSEDI (inotersen injection) in Canada

Oct 03, 2018: FDA grants Orphan Drug Designation to Eidos Therapeutics product candidate, AG10, for treatment of transthyretin amyloidosis

Oct 03, 2018: Kaskela Law LLC Announces Investor Class Action Lawsuit Against Alnylam Pharmaceuticals, Inc. and Encourages Investors with Losses in Excess of \$100,000 to Contact the Firm

Oct 02, 2018: Alnylam launches ONPATTRO (patisiran), the first-ever RNAi therapeutic, in Germany

Sep 28, 2018: Alnylam announces submission of New Drug Application in Japan for ONPATTRO (patisiran sodium) for treatment of Hereditary ATTR Amyloidosis

Sep 28, 2018: Alnylam Announces Submission of New Drug Application in Japan for ONPATTRO™ (patisiran sodium) for Treatment of Hereditary ATTR Amyloidosis

Sep 24, 2018: Alnylam Pharmaceuticals receives Priority Review in Canada for Patisiran, an investigational RNAi Therapeutic for the treatment of Hereditary Transthyretin-mediated Amyloidosis (hATTR amyloidosis) with Polyneuropathy

Sep 18, 2018: New sub-group analyses from the Tafamidis phase 3 Transthyretin Amyloid Cardiomyopathy (ATTR-ACT) study presented at 2018 HFSA Annual Scientific Meeting

Sep 17, 2018: Eidos Therapeutics presents data from its Phase 1 Clinical Trial of AG10 at the 22nd Annual Scientific Meeting of the Heart Failure Society of America

Sep 14, 2018: Alnylam announces publication in circulation of exploratory cardiac endpoint data from APOLLO phase 3 study of Patisiran

Aug 30, 2018: Alnylam receives approval of ONPATTRO (patisiran) in Europe

Aug 28, 2018: Pfizer reports data from tafamidis' rare heart disease trial

Aug 23, 2018: Intellia Therapeutics presents progress in lead in vivo program at cold spring harbor laboratory's fourth meeting on genome engineering: the CRISPR-Cas revolution

Aug 23, 2018: Eidos Therapeutics announces publication of AG10 molecular design in journal of medicinal chemistry

Aug 20, 2018: Veristat congratulates Alnylam on the FDA approval of ONPATTRO (patisiran)

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COMPANIES MENTIONED

Akcea Therapeutics Inc

Alnylam Pharmaceuticals Inc

Neurimmune Holding AG

Pfizer Inc

Prothena Corp Plc

Regeneron Pharmaceuticals Inc

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