

# Transthyretin (ATTR or Prealbumin or TBPA or TTR) Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

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## Abstracts

Transthyretin (ATTR or Prealbumin or TBPA or TTR) Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

### 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 17 molecules. Out of which approximately 16 molecules are developed by companies and remaining by the universities/institutes. The molecules developed by companies in Pre-Registration, Phase III, Phase II, Phase I, Preclinical and Discovery stages are 1, 3, 3, 4, 3 and 2 respectively. Similarly, the universities portfolio in Phase III stages comprises 1 molecule, respectively. Report covers products from therapy areas Metabolic Disorders, Ophthalmology, Central Nervous System, Gastrointestinal, Immunology and Infectious Disease which include indications Familial Amyloid Neuropathies, Familial Amyloid Cardiomyopathy, Amyloid Cardiomyopathy, Amyloidosis, Coronavirus Disease 2019 (COVID-19) Associated Acute Respiratory Distress Syndrome, Crohn's Disease (Regional Enteritis), Glaucoma, Juvenile Macular Degeneration (Stargardt Disease), Keratoconjunctivitis Sicca (Dry Eye), Multiple Sclerosis, Rheumatoid Arthritis, Ulcerative Colitis and Unspecified Ophthalmological

Disorders.

The latest report *Transthyretin - Drugs In Development, 2022*, 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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Featured News & Press Releases

Aug 18, 2022: Alnylam to present data from the APOLLO-B phase 3 study of patisiran in patients with ATTR amyloidosis with cardiomyopathy at the 18th International Symposium on amyloidosis

Aug 03, 2022: Alnylam reports positive topline results from APOLLO-B phase 3 study of patisiran in patients with ATTR amyloidosis with cardiomyopathy

Jul 22, 2022: Alnylam receives positive CHMP opinion for vutrisiran for the treatment of hereditary transthyretin-mediated (hATTR) amyloidosis in adult patients with stage 1 or stage 2 polyneuropathy

Jul 20, 2022: Orsini Specialty Pharmacy selected as limited distribution partner for AMVUTTRA (vutrisiran)

Jun 24, 2022: Intellia and Regeneron present updated interim data from phase 1 Study of CRISPR-based NTLA-2001 for the treatment of transthyretin (ATTR) amyloidosis

demonstrating that deep serum TTR reductions remained durable after a single dose  
Jun 22, 2022: AstraZeneca-Ionis' eplontersen meets endpoints in Phase III ATTRv-PN trial

Jun 13, 2022: Alnylam announces FDA approval of AMVUTTRA (vutrisiran), an RNAi therapeutic for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults

Jun 08, 2022: Intellia Therapeutics to present updated interim clinical data from ongoing phase 1 study of NTLA-2001 for the treatment of transthyretin (ATTR) amyloidosis at the International Liver Congress 2022

May 23, 2022: Alnylam presents new 18-month results from exploratory cardiac endpoints in HELIOS-A phase 3 study of investigational vutrisiran

Apr 04, 2022: Alnylam announces 3-Month extension of review period for New Drug Application for Vutrisiran

Apr 03, 2022: BridgeBio Pharma presents updated results from phase 2 open-label extension study of Acoramidis in transthyretin amyloid cardiomyopathy (ATTR-CM)

Mar 28, 2022: BridgeBio Pharma to present updated results from phase 2 open-label extension study of Acoramidis in Transthyretin Amyloid Cardiomyopathy (ATTR-CM) at the American College of Cardiology (ACC) Annual Scientific Session & Expo

Mar 01, 2022: Intellia, Regeneron report positive Phase I transthyretin amyloidosis data

Feb 17, 2022: Intellia Therapeutics announces two upcoming investor events in February 2022

Jan 24, 2022: Eplontersen granted Orphan Drug Designation in the US for transthyretin amyloidosis

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