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# Patisiran

National Cancer Institute

## Source

National Cancer Institute. *Patisiran*. NCI Thesaurus. Code C116792.

A lipid nanoparticle (LNP)-based formulation consisting of small-interfering RNAs (siRNAs) directed against transthyretin (TTR) encapsulated in lipids, which has potential use in the treatment of TTR-mediated amyloidosis (ATTR). Upon intravenous administration of patisiran, the lipid formulation promotes the uptake by cells. The siRNAs bind to TTR mRNAs, which may result in the inhibition of both the translation and expression of the TTR protein. ATTR is caused by mutations in the TTR gene and results in the formation of abnormal amyloid proteins that accumulate in and cause damage to various body organs and tissues.