

PHARMACY POLICY STATEMENT

Indiana Medicaid

DRUG NAME	Onpattro (patisiran)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Onpattro contains a transthyretin-directed small interfering RNA (siRNA) and is indicated for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults. It is an RNA interference (RNAi) drug that causes degradation of mutant and wild-type TTR mRNA, which results in a reduction of serum TTR protein and TTR protein deposits in tissues by targeting the liver where TTR protein is synthesized. In the APOLLO clinical trial, changes from baseline to Month 18 on both the mNIS+7 (primary endpoint) and the Norfolk QoL-DN significantly favored Onpattro, as well as changes in modified body mass index (mBMI) and gait speed (10-meter walk test). Changes were evident at 9 months.

hATTR is a rare and progressive inherited disorder where misfolded TTR accumulates as amyloid fibrils in the body. In polyneuropathy of hATTR (hATTR-PN), these fibrils deposit in the peripheral nerves which leads to pain, muscle weakness, and autonomic dysfunction.

