

Patisiran (ONPATTRO) for Polyneuropathy of Hereditary Transthyretin-Mediated (hATTR) Amyloidosis

Criteria for Use

October 2025

VA Pharmacy Benefits Management Services and National Formulary Committee

The following recommendations are based on medical evidence, clinician input, and expert opinion. The content of the document is dynamic and will be revised as new information becomes available. The purpose of this document is to assist practitioners in clinical decision-making, to standardize and improve the quality of patient care, and to promote cost-effective drug prescribing. THE CLINICIAN SHOULD USE THIS GUIDANCE AND INTERPRET IT IN THE CLINICAL CONTEXT OF THE INDIVIDUAL PATIENT. INDIVIDUAL CASES THAT ARE EXCEPTIONS TO THE EXCLUSION AND INCLUSION CRITERIA SHOULD BE ADJUDICATED AT THE LOCAL FACILITY ACCORDING TO THE POLICY AND PROCEDURES OF ITS P&T COMMITTEE AND PHARMACY SERVICES.

The Product Information should be consulted for detailed prescribing information.

Exclusion Criteria

If the answer to ANY item below is met, then the patient should **NOT** receive patisiran

- Sensorimotor or autonomic neuropathy (including diabetic neuropathy) without also having a diagnosis of hereditary transthyretin-mediated (hATTR) amyloidosis
- Primary amyloidosis
- Leptomeningeal amyloidosis
- Patient currently receiving another therapy for hATTR amyloidosis (e.g., diflunisal, tafamidis, vutrisiran)
- Prior liver transplant
- New York Heart Association (NYHA) Class III or IV heart failure
- Known pregnancy

Inclusion Criteria

All the following must be fulfilled to receive patisiran.

- Provider is a VA or VA Community Care neurologist or locally designated hATTR amyloidosis provider
- Diagnosis of polyneuropathy of hATTR amyloidosis including clinical symptoms and genetic testing that confirms a variant in TTR
- Documented baseline Neuropathy Impairment Score (NIS) of 5 to 130, Polyneuropathy Disability Score (PND) I to IIIb or Familial Amyloid Polyneuropathy (FAP) stage 1 or 2^{1, 2}
- Age 18-85 years
- Anticipated survival > 2 years
- Contraindication, intolerance, or lack of clinical response to at least a 9-month trial of vutrisiran
- Supplementation of the recommended daily allowance of vitamin A is planned to start upon approval³
- Discussion with patient/caregiver/family member regarding realistic treatment expectations and discontinuation should be documented

Additional Inclusion Criteria

- For females who can become pregnant: Counseling provided on potential risks vs. benefits of treatment including the risk that patisiran can decrease serum vitamin A levels

Footnotes

1. Neuropathy Impairment Score (J Peripher Nerv Syst. 2005 Jun;10(2):158-73.); Polyneuropathy Disability Score (Ther Adv Neurol Disord. 2013;6(2):129-39); Familial Amyloid Polyneuropathy (Ther Adv Neurol Disord. 2013;6(2):129-39)
2. Continuation of patisiran should be assessed at 9 months and then periodically throughout treatment. Patients should only continue therapy if they continue to demonstrate positive clinical response (e.g. improved motor function, quality of life, or ambulation or decreased neurological impairment).
3. Patients should be referred to eye clinic if they develop ocular symptoms suggestive of vitamin A deficiency (e.g., night blindness).

Prepared September 2019. Updated Sept 2022, Feb 2025, Oct 2025. Contact person: N. Antonovich, PharmD, BCPS, National Clinical Pharmacy Program Manager, VA Pharmacy Benefits Management Service (12PBM)
