

STANDARD MEDICARE PART B MANAGEMENT

ONPATTRO (patisiran)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Onpattro is indicated for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults.

All other indications will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:

For initial requests: testing or analysis confirming a mutation of the TTR gene

For continuation requests: medical record documentation confirming the member demonstrates clinical benefit

III. CRITERIA FOR INITIAL APPROVAL

Polyneuropathy of Hereditary Transthyretin-mediated Amyloidosis

Authorization of 12 months may be granted for treatment of polyneuropathy of hereditary transthyretin-mediated amyloidosis (also called transthyretin-type familial amyloid polyneuropathy [ATTR-FAP]) when all of the following criteria are met:

- A. The diagnosis is confirmed by detection of a mutation of the TTR gene.
- B. Member exhibits clinical manifestations of ATTR-FAP (e.g., amyloid deposition in biopsy specimens, TTR protein variants in serum, progressive peripheral sensory-motor polyneuropathy).
- C. The member is not a liver transplant recipient.
- D. The requested medication will not be used in combination with inotersen (Tegsedi) or tafamidis (Vyndaqel, Vyndamax).

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization for 12 months may be granted when all of the following criteria are met:

- A. The member is currently receiving treatment with Onpattro.

Reference number(s)
4238-A

- B. Onpattro is being used for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis.
- C. There is a clinical benefit from Onpattro therapy.

V. REFERENCES

1. Onpattro [package insert]. Cambridge, MA: Alnylam Pharmaceuticals, Inc.; September 2019.
2. Adams, et al. Patisiran, an RNAi Therapeutic, for Hereditary Transthyretin Amyloidosis. *N Engl J Med.* 2018 Jul 5; 379(1):11-21.
3. Ando Y, Coelho T, Berk JL, Cruz MW, Ericzon BG, Ikeda S, Lewis WD, Obici L, Planté-Bordeneuve V, Rapezzi C, et al. Guideline of transthyretin-related hereditary amyloidosis for clinicians. *Orphanet J Rare Dis.* 2013; 8:31.
4. Sekijima Y, Yoshida K, Tokuda T, Ikeda S. Familial transthyretin amyloidosis. In: *GeneReviews*. Seattle (WA): University of Washington, Seattle. 1993-2017. <https://www.ncbi.nlm.nih.gov/books/NBK1194/>. Accessed 16 Aug 2018.