

Effective Date: 08/01/2024
Reviewed: 5/2024, 5/2025
Scope: Medicaid

Wainua (eplontersen)

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 Indication

Wainua is indicated for the treatment of the polyneuropathy of variant/hereditary transthyretin-mediated amyloidosis in adults.

All other indications are considered experimental/investigational and not medically necessary.

II. CRITERIA FOR INITIAL APPROVAL

Polyneuropathy due to Variant/Hereditary Transthyretin-Mediated Amyloidosis (hATTR-PN)

An authorization of 6 months may be granted for the treatment of hATTR-PN when all the following criteria are met:

- A. Wainua is prescribed by, or in consultation with a neurologist, or physician specializing in the treatment of amyloidosis related to hATTR
- B. Member is at least 18 years of age
- C. Documentation that member has a definitive diagnosis of hATTR amyloidosis as documented by amyloid deposition on tissue biopsy and identification of a pathogenic *TTR* variant using molecular genetic testing
- D. Member has polyneuropathy as demonstrated by at least TWO of the following criteria:
 - a. Subjective member symptoms are suggestive of neuropathy
 - b. Abnormal nerve conduction studies are consistent with polyneuropathy
 - c. Abnormal neurological examination is suggestive of neuropathy
- E. Member's peripheral neuropathy is attributed to hATTR and other causes of neuropathy have been excluded
- F. Documentation of baseline in strength/weakness has been documented using an objective clinical measuring tool (e.g. Medical Research Council (MRC) muscle strength, modified Neuropathy Impairment Scale+7 (mNIS+7) composite score, etc.)
- G. Member is receiving supplementation with vitamin A at the recommended daily allowance
- H. The requested medication will not be used concurrently with other transthyretin (TTR) reducing or stabilizing agents (e.g., patisiran (Onpattro), vutisiran (Amvuttra), acoramidis (Attruby), or tafamidis (Vyndaqel/Vyndamax))
- I. If the request is for Wainua to be used in combination with a TTR-stabilizer (e.g., acoramidis (Attruby), tafamidis (Vyndaqel/Vyndamax)) to treat overlapping ATTR-CM and ATTR-PN, the member has a documented inadequate response, intolerance, or contraindication to monotherapy vutisiran (Amvuttra)
- J. Coverage will not be provided in the following circumstances:
 - a. Prior or planned liver transplant
 - b. Severe renal impairment or end-stage renal disease
 - c. New York Heart Association (NYHA) heart failure classification >2 (i.e., NYHA class III or IV)
 - d. Other known causes of neuropathy (i.e., uncontrolled diabetes, sensorimotor or autonomic neuropathy not related to hATTR amyloidosis)
 - e. Primary or leptomeningeal amyloidosis
 - f. Cardiomyopathy hATTR (hATTR-CM)

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III. CONTINUATION OF THERAPY

If member has not been approved for this drug by Neighborhood in the past, clinician must submit documentation that initial criteria is met. An authorization of 6 months may be granted for all adults who are using the requested medication for treatment of polyneuropathy due to variant/hereditary transthyretin-mediated amyloidosis when all of the following criteria are met:

- A. Documentation that member has achieved or maintained a positive clinical response compared to pre-treatment baseline as evidenced by stabilization or improvement in one or more of the following:
 - a. Signs and symptoms of neuropathy (e.g., improved ambulation, improvement in neurologic symptom burden, improvement in activities of daily living)
 - b. Documented improvement of clinical response compared to baseline (e.g., modified Neuropathy Impairment Scale+7 (mNIS+7) composite score, Norfolk Quality of Life-Diabetic Neuropathy (QoL-DN) total score, etc.)
- B. Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include ocular symptoms related to hypovitaminosis A, etc.
- C. Member continues to receive supplementation with vitamin A at the recommended daily allowance
- D. The requested medication will not be used concurrently with other transthyretin (TTR) reducing or stabilizing agents (e.g., patisiran (Onpattro), vutisiran (Amvuttra), acoramidis (Attruby), or tafamidis (Vyndaqel/Vyndamax))

IV. QUANTITY LIMIT

Drug	Quantity Limit	FDA-Recommended Dosing
Wainua (eplontersen)	1 auto-injector pen (45mg/0.8mL) every 28 days (daily dose of 0.03 mL)	45mg administered by subcutaneous injection once monthly

V. REFERENCES

- Wainua [package insert]. Wilmington, DE: AstraZeneca Pharmaceuticals LP; December 2023.
- Ando Y, Coelho T, Berk JL, et. al. Guideline of transthyretin-related hereditary amyloidosis for clinicians. Orphanet J Rare Dis. 2013;8(31).
- Coelho T, Marques W Jr, Dasgupta NR, et al. Eplontersen for Hereditary Transthyretin Amyloidosis with Polyneuropathy. JAMA. 2023;330(15):1448-1458.