| Effective Date: 08/01/2024 |
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| Reviewed: 5/2024, 5/2025, 4/2026<br>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)

## 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 |
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| 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

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