Effective Date: 08/01/2024
Reviewed: 5/24 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 hereditary transthyretin-mediated amyloidosis in adults.

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

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

A. Initial requests:
   1. Chart notes or medical record documentation confirming diagnosis of hereditary transthyretin-mediated amyloidosis (hATTR) as documented by amyloid deposition on tissue biopsy and identification of a pathogenic *TTR* variant using molecular genetic testing
   2. Chart notes or medical record documentation supporting signs and symptoms of polyneuropathy caused by hATTR, including baseline clinical scores (e.g., modified Neuropathy Impairment Scale+7 (mNIS+7) composite score, Norfolk Quality of Life-Diabetic Neuropathy (QoL-DN) total score, etc.)
B. Continuation requests: Chart notes or medical record documentation supporting benefit from therapy

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by, or in consultation with a neurologist, or physician specializing in the treatment of amyloidosis related to hATTR/FAP.

IV. CRITERIA FOR INITIAL APPROVAL

Polyneuropathy due to Hereditary Transthyretin-Mediated Amyloidosis (hATTR-PN)/Familial Amyloidotic Polyneuropathy (FAP)
An authorization of 6 months may be granted for the treatment of hATTR-PN when all the following criteria are met:

A. Patient is at least 18 years of age
B. Patient has a definitive diagnosis of hATTR amyloidosis/FAP as documented by amyloid deposition on tissue biopsy and identification of a pathogenic *TTR* variant using molecular genetic testing

C. Patient has polyneuropathy as demonstrated by at least TWO of the following criteria:
    a. Subjective patient symptoms are suggestive of neuropathy
    b. Abnormal nerve conduction studies are consistent with polyneuropathy
    c. Abnormal neurological examination is suggestive of neuropathy
D. Patient's peripheral neuropathy is attributed to hATTR/FAP and other causes of neuropathy have been excluded
E. 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.)
F. Patient is receiving supplementation with vitamin A at the recommended daily allowance
G. The requested medication will not be used concurrently with other transthyretin (TTR) reducing or stabilizing agents (e.g., Onpattro (patisiran), Amvuttra (vutrisiran), Vyndaqel/Vyndamax (tafamadis), etc.)
H. 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
    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)

V. 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 hereditary transthyretin-mediated amyloidosis when all of the following criteria are met:

A. Patient 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. Patient continues to receive supplementation with vitamin A at the recommended daily allowance
D. The requested medication will not be used concurrently with with other transthyretin (TTR) reducing or stabilizing agents (e.g., Onpattro (patisiran), Amvuttra (vutrisiran), Vyndaqel/Vyndamax (tafamadis), etc.)

VI. 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

VII. 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.