PHARMACY POLICY STATEMENT
Georgia Medicaid

DRUG NAME	Wainua (eplontersen)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Wainua, approved by the FDA in 2023, is a transthyretin-directed antisense oligonucleotide indicated for treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults. It causes degradation of mutant and wild-type TTR mRNA through binding to the TTR mRNA, which results in a reduction of serum TTR protein and TTR protein deposits in tissues. Efficacy was demonstrated in the NEURO-TTRansform clinical trial. Wainua is a monthly self-administered subcutaneous injection.
hATTR is a rare and progressive inherited disorder where misfolded TTR accumulates as amyloid fibrils in the body. In polyneuropathy of hATTR (hATTR-PN), these fibrils deposit in the peripheral nerves which leads to pain, muscle weakness, and autonomic dysfunction.

Wainua (eplontersen) will be considered for coverage when the following criteria are met:

Hereditary Transthyretin Amyloidosis (hATTR Amyloidosis): Polyneuropathy

For **initial** authorization:
1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a neurologist; AND
3. Member has a diagnosis of hATTR amyloidosis with documentation of a transthyretin (TTR) mutation confirmed by genetic testing; AND
4. Member has signs/symptoms of polyneuropathy; AND
5. Member must have documentation of familiar amyloid polyneuropathy (FAP) Cutinho stage 1 (ambulatory without assistance) or stage 2 (ambulatory with assistance); AND
6. Wainiua will NOT be used in combination with another TTR silencer or a TTR stabilizer.
7. **Dosage allowed/Quantity limit:** 45 mg injected subQ once monthly. (1 syringe per 28 days)

If all the above requirements are met, the medication will be approved for 9 months.

For **reauthorization**:
1. Chart notes must include documentation of positive clinical response to therapy such as improvement or stabilization of neuropathy impairment.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Wainua (eplontersen) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
01/26/2024	New policy for Wainua created.
04/03/2025	Updated references. Removed liver transplant exclusion (Karam 2024, Alcantara 2022)

References:

1. Wainua [prescribing information]. Wilmington, DE: AstraZeneca Pharmaceuticals LP; 2024.
2. Coelho T, Marques Jr W, Dasgupta NR, et al. Eplontersen for Hereditary Transthyretin Amyloidosis With Polyneuropathy. *JAMA*. 2023;330(15):1448-1458. doi:10.1001/jama.2023.18688
3. Coelho T, et al. Characteristics of Patients with Hereditary Transthyretin Amyloidosis-Polyneuropathy (ATTRv-PN) in NEURO-TTRansform, an Open-label Phase 3 Study of Eplontersen. *Neurol Ther*. 2023;12:267–287
4. Ando Y, Coelho T, Berk JL, et al. Guideline of transthyretin-related hereditary amyloidosis for clinicians. Orphanet J Rare Dis. 2013;8:31
5. National Institutes of Health (NIH). Transthyretin amyloidosis. Available at: https://ghr.nlm.nih.gov/condition/transthyretin-amyloidosis.
6. Amyloid transthyretin (ATTR) Amyloidosis: Signs, symptoms, and diagnostic workup. 2018 Akcea Therapeutics, Inc. Available at: https://www.hattrguide.com/wp-content/uploads/2018/04/Diagnostic-Card.pd
7. Stages of FAP. FAP News Today. Accessed February 1, 2024. https://fapnewstoday.com/stages-of-familial-amyloid-polyneuropathy/
8. Adams D, Ando Y, Beirão JM, et al. Expert consensus recommendations to improve diagnosis of ATTR amyloidosis with polyneuropathy. *J Neurol*. 2021;268(6):2109-2122. doi:10.1007/s00415-019-09688-0
9. Karam C, Mauermann ML, Gonzalez-Duarte A, et al. Diagnosis and treatment of hereditary transthyretin amyloidosis with polyneuropathy in the United States: Recommendations from a panel of experts. *Muscle Nerve*. 2024;69(3):273-287. doi:10.1002/mus.28026
10. Alcantara M, Mezei MM, Baker SK, et al. Canadian Guidelines for Hereditary Transthyretin Amyloidosis Polyneuropathy Management. *Can J Neurol Sci*. 2022;49(1):7-18. doi:10.1017/cjn.2021.34

Effective date: 03/01/2026
Revised date: 04/03/2025