Reference number(s)
6314-A

Specialty Guideline Management
Wainua

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Wainua	eplontersen

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[1]

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.

Documentation

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

- Initial requests:
  - Testing or analysis confirming a pathogenic variant in the TTR gene.
  - Medical record documentation demonstrating clinical manifestations of transthyretin-type familial amyloid polyneuropathy [ATTR-FAP] (e.g., amyloid deposition in biopsy

specimens, TTR protein variants in serum, progressive peripheral sensory-motor polyneuropathy).
    - Medical record documentation confirming the member demonstrates signs and symptoms of polyneuropathy.
- Continuation requests: Chart notes or medical record documentation supporting clinical benefit of therapy compared to baseline.

Prescriber Specialties

This medication must be prescribed by or in consultation with a neurologist, geneticist, or physician specializing in the treatment of amyloidosis.

Coverage Criteria

Polyneuropathy of Hereditary Transthyretin-mediated Amyloidosis[1-3]

Authorization of 12 months may be granted for the 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:

- Member is 18 years of age or older.
- The diagnosis is confirmed by detection of a pathogenic variant in the TTR gene.
- Member exhibits clinical manifestations of ATTR-FAP (e.g., amyloid deposition in biopsy specimens, TTR protein variants in serum, progressive peripheral sensory-motor polyneuropathy).
- Member is not a liver transplant recipient.
- The requested medication will not be used in combination with vutrisiran (Amvuttra), patisiran (Onpattro), inotersen (Tegsedi), tafamidis meglumine (Vyndaqel), tafamidis (Vyndamax), or acoramidis (Attruby).

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria section when all of the following criteria are met:

- Member must have met all requirements in the coverage criteria section.
- Member must have demonstrated a beneficial response to treatment with the requested medication compared to baseline (e.g., improvement of neuropathy severity and rate of disease

progression as demonstrated by the modified Neuropathy Impairment Scale+7 (mNIS+7) composite score, the Norfolk Quality of Life-Diabetic Neuropathy (QoL-DN) total score, polyneuropathy disability (PND) score, FAP disease stage, manual grip strength).

References

1. Wainua [package insert]. Wilmington, DE: AstraZeneca Pharmaceuticals LP; Spetember 2024.
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. Sekijima Y. Hereditary Transthyretin Amyloidosis. 2001 Nov 5 [Updated 2024 May 30]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2024. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1194/. Accessed March 18, 2025.