POLICY Document for WAINUA (eplontersen)

The overall objective of this policy is to support the appropriate and cost-effective use of the medication, specific to use of preferred medication options, and overall, clinically appropriate use. This document provides specific information to both sections of the overall policy.

Section 1: Preferred Product
- Policy information specific to preferred medications

Section 2: Clinical Criteria
- Policy information specific to the clinical appropriateness for the medication

Section 1: Preferred Product

CAREFIRST: EXCEPTIONS CRITERIA
hATTR DISORDERS

PREFERRED PRODUCTS: ONPATTRO, AMVUTTRA

POLICY
This policy informs prescribers of preferred products and provides an exception process for targeted products through prior authorization.

I. PLAN DESIGN SUMMARY

This program applies to the products for the treatment of polyneuropathy of hereditary transthyretin-mediated amyloidosis specified in this policy. Coverage for the targeted product is provided based on clinical circumstances that would exclude the use of the preferred product and may be based on previous use of a product. The coverage review process will ascertain situations where a clinical exception can be made. This program applies to all members who are requesting treatment with the targeted products

Each referral is reviewed based on all utilization management (UM) programs implemented for the client.

Table. Polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis Products

	Product(s)
Preferred*	• **Amvuttra** (vutrisiran) injection • **Onpattro** (patisiran) injection
Targeted	• **Tegsedi** (inotersen) injection • **Wainua** (eplontersen) injection

*: Medications considered formulary or preferred on your plan may still require a clinical prior authorization review.

II. EXCEPTION CRITERIA

This program applies to members requesting treatment for an indication that is FDA-approved for the preferred product.

Coverage for the targeted product is provided when member has a documented inadequate response, contraindication, or intolerable adverse event to all preferred products.

Section 2: Clinical Criteria

SPECIALTY GUIDELINE MANAGEMENT

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. Testing or analysis confirming a mutation in the TTR gene.
    2. Medical record documentation confirming the member demonstrates signs and symptoms of polyneuropathy.
B. Continuation of therapy requests: Chart notes or medical record documentation supporting clinical benefit of therapy compared to baseline.

III. PRESCRIBER SPECIALTIES

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

IV. CRITERIA FOR INITIAL APPROVAL

Polyneuropathy of Hereditary Transthyretin-mediated Amyloidosis
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:
A. The diagnosis is confirmed by detection of a mutation in the TTR gene.
B. Member exhibits clinical manifestations of ATTR-FAP (e.g., amyloid deposition in biopsy specimens, TTR protein variants in serum, progressive peripheral sensory-motor polyneuropathy).
C. Member is not a liver transplant recipient.
D. The requested medication will not be used in combination with any other medication approved for the

treatment of hereditary transthyretin-mediated amyloidosis (e.g., Amvuttra, Onpattro, Tegsedi, Vyndamax, Vyndaqel).

V. CONTINUATION OF THERAPY

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

A. Member must have met all initial authorization criteria.
B. 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:

SECTION 1

1. Amvuttra [package insert]. Cambridge, MA: Alnylam Pharmaceuticals, Inc.; February 2023.
2. Onpattro [package insert]. Cambridge, MA: Alnylam Pharmaceuticals, Inc.; January 2023.
3. Tegsedi [package insert]. Waltham, MA: Sobi Inc; January 2024.
4. Wainua [package insert]. Wilmington, DE: AstraZeneca Pharmaceuticals LP: September 2024.

SECTION 2

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. Sekijima Y. Hereditary Transthyretin Amyloidosis. 2001 Nov 5 [Updated 2021 June 17]. 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 1, 2024.