BlueCross BlueShield
Federal Employee Program
Federal Employee Program®
750 9th St NW
Washington, DC 20001
202.942.1000
Fax 202.942.1125

5.75.044

Section:	Prescription Drugs	**Effective Date:**	July 1, 2024
Subsection:	Neuromuscular Drugs	**Original Policy Date:**	January 12, 2024
Subject:	Wainua	**Page:**	1 of 5

Last Review Date: June 13, 2024

Wainua

Description

Wainua (eplontersen)

Background
Wainua (eplontersen) is an antisense oligonucletodie-Ga1NAc conjugate that 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 (1).

Regulatory Status
FDA-approved indication: Wainua is a transthyretin-directed antisense oligonucleotide indicated for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults (1).

The recommended dosage of Wainua if 45 mg administered by subcutaneous injection once monthly (1).

Wainua treatment leads to a decrease in serum vitamin A levels. Supplementation at the recommended daily allowance of vitamin A is advised for patients taking Wainua. Higher doses than recommended daily allowance of vitamin A should not be given to try to achieve normal serum vitamin A levels during treatment with Wainua, as serum vitamin A levels do not reflect the total vitamin A in the body. Patients should be referred to an ophthalmologist if they develop ocular symptoms suggestive of vitamin A deficiency (e.g., night blindness, dry eyes) (1).

The safety and effectiveness of Wainua in pediatric patients have not been established (1).

Related policies
Amvuttra, Onpattro, Tegsedi

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Wainua may be considered **medically necessary** if the conditions indicated below are met.

Wainua may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 18 years of age and older

Diagnosis

Patient must have the following:

Polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis

AND ALL of the following:

1. Diagnosis of hATTR confirmed by a genetic test **OR** a tissue biopsy showing amyloid deposition
2. Patient must have **ONE** of the following baseline scores:
    a. Polyneuropathy disability (PND) score ≤ IIIb (see Appendix 1)
    b. FAP Stage 1 or 2 (see Appendix 2)
3. Prescriber agrees to supplement the patient with the recommended daily allowance of Vitamin A if indicated
4. Patient has **NONE** of the following:
    a. New York Heart Association (NYHA) class III or IV heart failure
    b. Sensorimotor or autonomic neuropathy not related to hATTR amyloidosis (monoclonal gammopathy, autoimmune disease, etc.)
    c. Prior liver transplantation
5. Prescribed by or in consultation with a neurologist, or a specialist in the treatment of the patient's diagnosis
6. **NO** dual therapy with another Prior Authorization (PA) medication for polyneuropathy caused by hATTR amyloidosis (see Appendix 3)

Prior – Approval *Renewal* Requirements

Age 18 years of age and older

Diagnosis

Patient must have the following:

Polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis

AND ALL of the following:

1. Patient condition has improved or stabilized
2. Prescriber agrees to supplement the patient with the recommended daily allowance of Vitamin A if indicated
3. **NO** dual therapy with another Prior Authorization (PA) medication for polyneuropathy caused by hATTR amyloidosis (see Appendix 3)

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity 3 single-dose autoinjectors per 90 days

Duration 12 months

Prior – Approval *Renewal* Limits

Same as above

Rationale

Summary

Wainua is an antisense oligonucleotide indicated for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults. It is recommended that patients treated with Wainua be supplemented with the recommended daily allowance of vitamin A. The safety and effectiveness of Wainua in pediatric patients have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Wainua while maintaining optimal therapeutic outcomes.

References

1. Wainua [package insert]. Wilmington, DE: AstraZeneca Pharmaceuticals LP; December 2023.

Policy History

Date	Action
January 2024	Addition to PA
March 2024	Annual review
June 2024	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on June 13, 2024 and is effective on July 1, 2024.

Appendix 1 - Polyneuropathy Disability (PND) Severity Scoring System

Polyneuropathy Disability (PND) Score	
Stage 0	No impairment
Stage I	Sensory disturbances but preserved walking capability
Stage II	Impaired walking capability but ability to walk without a stick or crutches
Stage IIIA	Walking only with the help of one stick or crutches
Stage IIIB	Walking only with the help of two sticks or crutches
Stage IV	Confined to a wheelchair or bedridden

Appendix 2 - FAP Stage Severity Scoring System

FAP Stage	
Stage 0	No symptoms
Stage I	Unimpaired ambulation; mostly mild sensory, motor, and autonomic neuropathy in the lower limbs
Stage II	Assistance with ambulation required; mostly moderate impairment progression to the lower limbs, upper limbs, and trunk
Stage III	Wheelchair bound or bedridden; severe sensory, motor, and autonomic involvement of all limbs

Appendix 3 - List of PA Medications for Polyneuropathy caused by hATTR Amyloidosis

Generic Name	Brand Name
eplontersen	Wainua
inotersen	Tegsedi
patisiran	Onpattro
vutrisiran	Amvuttra