POLICY Document for TEGSEDI

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

EXCEPTIONS CRITERIA
TTR DISORDERS

PREFERRED PRODUCT: ONPATTRO

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 members who are new to treatment with a targeted product for the first time.

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	**Onpattro** (patisiran) injection
Targeted	**Tegsedi** (inotersen) injection

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 either of the following criteria is met:

A. Member is currently receiving treatment with the targeted product, excluding when the requested targeted product is obtained as samples or via manufacturer's patient assistance programs.
B. Member has a documented inadequate response or intolerable adverse event with the preferred product.

Section 2: Clinical Criteria

SPECIALTY GUIDELINE MANAGEMENT

TEGSEDI (inotersen)

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 Indications

Tegsedi 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. REQUIRED DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. Testing or analysis confirming a mutation of the TTR gene
B. Medical record documentation confirming the member demonstrates signs and symptoms of polyneuropathy and an improvement in these signs and symptoms since starting therapy for continuation

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 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 of 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. The member is not a liver transplant recipient.
D. The requested medication will not be used in combination with patisiran (Onpattro) or tafamidis.

V. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for the continued treatment of ATTR-FAP when all of the following criteria are met:

A. The member must have met all initial authorization criteria.
B. The member must have demonstrated a beneficial response to treatment with Tegsedi therapy 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). Documentation from the medical record must be provided.

REFERENCES

SECTION 1

1. Onpattro [package insert]. Cambridge, MA: Alnylam Pharmaceuticals, Inc.; September 2019.
2. Tegsedi [package insert]. Boston, MA: Akcea Therapeutics, Inc. October 2018.

SECTION 3

1. Tegsedi [package insert]. Boston, MA: Akcea Therapeutics, Inc. October 2018.
2. Benson MD, et. al., Inotersen Treatment for Patients with Hereditary Transthyretin Amyloidosis. N Engl J Med. 2018 Jul 5; 379(1):22-31.
3. Ando Y, Coelho T, Berk JL, Cruz MW, Ericzon BG, Ikeda S, Lewis WD, Obici L, Planté-Bordeneuve V, Rapezzi C, et al. Guideline of transthyretin-related hereditary amyloidosis for clinicians. Orphanet J Rare Dis. 2013;8:31.