

**Policy: MBP 188.0**

**Section: Medical Benefit Pharmaceutical Policy**

**Subject: Onpattro (patisiran)**

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### **I. Policy:**

Onpattro (patisiran)

### **II. Purpose/Objective:**

To provide a policy of coverage regarding Onpattro (patisiran)

### **III. Responsibility:**

- A. Medical Directors
- B. Medical Management
- C. Pharmacy Department

### **IV. Required Definitions**

1. Attachment – a supporting document that is developed and maintained by the policy writer or department requiring/authoring the policy.
2. Exhibit – a supporting document developed and maintained in a department other than
3. the department requiring/authoring the policy.
4. Devised – the date the policy was implemented.
5. Revised – the date of every revision to the policy, including typographical and grammatical changes.
6. Reviewed – the date documenting the annual review if the policy has no revisions necessary.

### **V. Additional Definitions**

Medical Necessity or Medically Necessary means Covered Services rendered by a Health Care Provider that the Plan determines are:

- a. appropriate for the symptoms and diagnosis or treatment of the Member's condition, illness, disease or injury;
- b. provided for the diagnosis and the direct care and treatment of the Member's condition, illness disease or injury;
- c. in accordance with current standards good medical treatment practiced by the general medical community;
- d. not primarily for the convenience of the Member, or the Member's Health Care Provider; and
- e. the most appropriate source or level of service that can safely be provided to the Member. When applied to hospitalization, this further means that the Member requires acute care as an inpatient due to the nature of the services rendered or the Member's condition, and the Member cannot receive safe or adequate care as an outpatient

### **Medicaid Business Segment**

Medically Necessary — A service, item, procedure, or level of care compensable under the Medical Assistance program that is necessary for the proper treatment or management of an illness, injury, or disability is one that:

- i. Will, or is reasonably expected to, prevent the onset of an illness, condition, injury or disability.
- ii. Will, or is reasonably expected to, reduce or ameliorate the physical, mental or developmental effects of an illness, condition, injury or disability.
- iii. Will assist the Member to achieve or maintain maximum functional capacity in performing daily activities, taking into account both the functional capacity of the Member and those functional capacities that are appropriate for Members of the same age.

**DESCRIPTION:**

Onpattro (patisiran) is a double-stranded small interfering ribonucleic acid (siRNA) that causes degradation of mutant and wild-type transthyretin (TTR) mRNA through RNA interference, which results in a reduction of serum TTR protein and TTR protein deposits in tissues. Serum TTR is a carrier of retinol binding protein, which is involved in the transport of vitamin A in the blood.

**CRITERIA FOR USE: Requires Prior Authorization by Medical Director or Designee**

Onpattro (patisiran) will be considered medically necessary for all lines of business when ALL of the following criteria are met:

- Prescription written by or in consultation with a neurologist, specialist with experience in the treatment of hereditary transthyretin-mediated amyloidosis (hATTR), or geneticist **AND**
- Medical record documentation of age greater than or equal to 18 years **AND**
- Medical record documentation of a diagnosis of hereditary transthyretin-mediated amyloidosis as confirmed by genetic testing to confirm a pathogenic mutation in TTR **AND** one of the following:
  - Biopsy of tissue/organ to confirm amyloid presence **OR**
  - A clinical manifestation typical of hATTR (Neuropathy and/or CHF) without a better alternative explanation

**AND**

- Medical record documentation of Onpattro being used to treat polyneuropathy **AND**
- Medical record documentation of familial amyloid polyneuropathy (FAP) stage 1-2 and/or polyneuropathy disability score (PND) indicating the patient is not wheelchair bound or bedridden **AND**
- Medical record documentation that Onpattro will not be used in combination with other RNA interference treatment

**Note:**FAP stage:

- 1- unimpaired ambulation
- 2- assistance with ambulation
- 3- wheelchair-bound or bedridden

Polyneuropathy disability score:

- I- preserved walking, sensory disturbances
- II- impaired walking without need for stick/crutches
- IIIa- walking with 1 stick/crutch
- IIIb- walking with 2 sticks/crutches
- IV- wheelchair-bound or bedridden

Polyneuropathy disability score (used in Neuro-TTR trial for Tegsedi):

- I- preserved walking, sensory disturbances
- II- impaired walking without need for stick/crutches
- III- walking with 1 stick/crutch
- IV- walking with 2 sticks/crutches
- V- wheelchair-bound or bedridden

**AUTHORIZATION DURATION:** Initial approval will be for 12 months or less if the reviewing provider feels it is medically appropriate. Subsequent approvals will be for an additional 12 months or less if the reviewing provider feels it is medically appropriate. The medication will no longer be covered if the member progresses to FAP stage 3 and/or polyneuropathy disability score indicating the patient is wheelchair-bound or bedridden.

**QUANTITY LIMIT:** 15 mL per 21 days

Note: For Medicaid (GHP Family), any requests for services that do not meet criteria set in the PARP will be evaluated on a case-by-case basis.

**LINE OF BUSINESS:**

**Eligibility and contract specific benefit limitations and/or exclusions will apply. Coverage statements found in the line of business specific benefit document will supersede this policy.**

**REFERENCES:**

1. Onpattro [prescribing information]. Cambridge, MA: Alnylam Pharmaceuticals Inc; January 2023.

This policy will be revised as necessary and reviewed no less than annually.

**Devised:** 1/15/19

**Revised:** 3/19/19 (clarified criteria, Tegsedi), 1/19/23 (LOB carve out, Medicaid PARP statement, Medicaid business segment), 12/29/23 (references added),

**Reviewed:** 2/1/20, 1/28/21, 1/21/22, 1/15/24

**MA UM Committee approval:** 12/31/23