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
Medical Necessity shall mean a service or benefit that is compensable under the Medical Assistance
Program and if it meets any one of the following standards:

(i) the service or benefit will, or is reasonably expected to, prevent the onset of an illness, condition or disability.

(ii) the service or benefit will, or is reasonably expected to, reduce or ameliorate the physical, mental or
development effects of an illness, condition, injury or disability.

(iii) the service or benefit 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 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

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.

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

Devised: 1/15/19
Revised: 3/19/19 (clarified criteria, Tegsedi)
Reviewed: 2/1/20