Onpattro® (patisiran) (Intravenous)

Last Review Date: October 1, 2019	Number: MG.MM.PH.119

Medical Guideline Disclaimer

Definition

Patisiran was FDA approved for the treatment of the polyneuropathy of hATTR amyloidosis in adults. hATTR amyloidosis is a rapidly progressive, life-threatening disease caused by mutant and wild-type transthyretin (TTR) proteins forming amyloid deposits in tissues throughout the body. More than 95% of TTR circulating in the body is produced by the liver. This amyloid accumulation leads to progressive multisystem dysfunction, including polyneuropathy (e.g., sensorimotor and autonomic neuropathy) and cardiomyopathy. Patisiran is a double-stranded small interfering ribonucleic acid (siRNA) formulated as a lipid nanoparticle complex for delivery to hepatocytes. Patisiran causes the degradation of mutant and wild-type TTR mRNA through RNA interference, which results in a reduction of serum TTR protein and TTR protein deposits in tissues.

Length of Authorization

Coverage will be provided for 6 months and may be renewed.

Dosing Limits

Max Units (per dose and over time) [Medical Benefit]:

- 30 mg every 3 weeks

Guideline

I. Initial Approval Criteria

Patisiran *may be considered medically necessary if one of the below conditions are met* ***AND*** *use is consistent with the medical necessity criteria that follows:*

Polyneuropathy of hATTR amyloidosis†

- Patient must be at least 18 years old; **AND**

- Patient has a definitive diagnosis of hATTR amyloidosis/FAP as documented by identification of a pathogenic TTR variant using molecular genetic testing; **AND**
- Used for the treatment of polyneuropathy as demonstrated by at least TWO of the following criteria:
    - Subjective patient symptoms are suggestive of neuropathy
    - Abnormal nerve conduction studies are consistent with polyneuropathy
    - Abnormal neurological examination is suggestive of neuropathy; **AND**
- Patient's peripheral neuropathy is attributed to hATTR/FAP and other causes of neuropathy have been excluded; **AND**
- Baseline in strength/weakness has been documented using an objective clinical measuring tool (e.g., Medical Research Council (MRC) muscle strength, etc.); **AND**
- Patient has not been the recipient of an orthotopic liver transplant (OLT); **AND**
- Patient is receiving supplementation with vitamin A at the recommended daily allowance

† FDA-labeled indication(s);

Limitations/Exclusions

Onpattro is not considered medically necessary for indications other than those listed above due to insufficient evidence of therapeutic value.

II. RENEWAL CRITERIA

- Patient continues to meet Initial approval criteria.
- Disease response compared to baseline prior to treatment shows improvement or stabilization in one or both of the following:
    - Neuropathy signs and symptoms
    - MRC muscle strength

Dosage/Administration

Indication	Dose

hATTR Amloidosis	**Recommended dosage:**
	• Weight < 100 kg
	  ○ 0.3 mg/kg intravenously every 3 weeks
	• Weight ≥ 100 kg
	  ○ 30 mg intravenously every 3 weeks
	
	Preparing for Therapy:
	• Dosing is based on actual body weight
	• Patients should be premedicated with a corticosteroid, acetaminophen and antihistamines.
	• Infusion should be filtered and diluted and infused, via a pump, over at least 80 minutes.
	• Patients should receive vitamin A supplementation.

Applicable Procedure Codes

C9036	Injection, patisiran, 0.1mg
J0222	Effective 10/1/19, Injection, patisiran, 0.1mg

Applicable NDCs

71336-1000-01	Onpattro 10mg/5ml single-dose vial

Applicable Diagnosis Codes

ICD-10	ICD-10 Description
E85.1	Neuropathic heredofamilial amyloidosis

Revision History

8/15/2019	Added code J0222, effective 10/1/19

References

1. ONPATTRO (patisiran) [package insert]. Cambridge, MA: Alnylam Pharmaceuticals, Inc; 2018.
2. Adams D, Gonzàlez-Duarte A, O'Riordan WD, et al. Patisiran, an RNAi therapeutic, for hereditary transthyretin amyloidosis. *N Engl J Med.* 2018;379(1):11-21.
3. Adams D, Suhr OB, Dyck PJ, et al. Trial design and rationale for APOLLO, a Phase 3, placebo-controlled study of patisiran in patients with hereditary ATTR amyloidosis with polyneuropathy. BMC Neurol. 2017;17(1):181