

Onpattro™ (patisiran)



When requesting Onpattro™ (patisiran), the individual requiring treatment must be diagnosed with an FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-approved Indication

Onpattro is indicated for the treatment of polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults.

Coverage Guidelines

The individual must meet **all** of the following criteria for approval:

- Diagnosis of hereditary transthyretin-mediated amyloidosis is confirmed by genetic testing;
- Has symptomatic polyneuropathy (e.g., reduced motor strength/coordination, impaired sensation [e.g., pain, temperature, vibration, touch]);
(Note: Examples of assessments for symptomatic disease include history and clinical exam, electromyography, or nerve conduction velocity testing.)
- Is at least 18 years of age;
- Onpattro is prescribed by or in consultation with a neurologist, geneticist, or a physician who specializes in the treatment of amyloidosis.

Approval duration: 12 months

Dosing Recommendations

- For patients weighing < 100 kg, administer 0.3 mg/kg as an IV infusion once every 3 weeks.
- For patients weighing ≥ 100 kg, administer 30 mg as an IV infusion once every 3 weeks.

References

1. Onpattro® [prescribing information]. Cambridge, MA: Alnylam; January 2023.
2. Alcantara M, Mezi MM, Baker SK, et al. Canadian guidelines for hereditary transthyretin amyloidosis polyneuropathy management. *Can J Neuro Sci.* 2022;49:7-18.
3. Kittleson MM, Maurer MS, Ambardekar AV, et al; on behalf of the American Heart Association Heart Failure and Transplantation Committee of the Council on Clinical Cardiology. AHA scientific statement: cardiac amyloidosis: evolving diagnosis and management. *Circulation.* 2020;142:e7-e22.
4. McDonagh TA, Metra M, Adamo M, et al. 2021 ESC guidelines for the diagnosis and treatment of acute and chronic heart failure. *Eur Heart J.* 2021;42:3599-3726.

5. Lin H, Merkel M, Hale C, Marantz JL. Experience of patisiran with transthyretin stabilizers in patients with hereditary transthyretin-mediated amyloidosis. *Neurodegener Dis Manag.* 2020;10(5):289-300.
6. Schmidt HH, Wixner J, Plante-Bordeneuve V; on behalf of the Patisiran Post-LT Study Group. Patisiran treatment in patients with hereditary transthyretin-mediated amyloidosis with polyneuropathy after liver transplantation. *Am J Transplant.* 2022;22:1646-1657.