

Onpattro® (patisiran)

When requesting Onpattro® (patisiran), the individual requiring treatment must be diagnosed with the following 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

Polyneuropathy of Hereditary Transthyretin-Mediated Amyloidosis

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

- Has a transthyretin pathogenic variant as confirmed by genetic testing;
- Has symptomatic polyneuropathy (e.g., reduced motor strength/coordination, impaired sensation [e.g., pain, temperature, vibration, touch]);
- Is 18 years of age or older;
- Does not have a history of liver transplantation;
- 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

Onpattro is administered via intravenous infusion. Dosing is based on actual body weight.

- For patients weighing less than 100kg, the recommended dosage is 0.3 mg/kg once every 3 weeks.
- For patients weighing 100 kg or more, the recommended dosage is 30 mg once every 3 weeks.

References

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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.
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7. Maurer MS, Kale P, Fontana M, et al; for the APOLLO-B Trial Investigators. Patisiran treatment in patients with transthyretin cardiac amyloidosis. *N Engl J Med*. 2023;389(17): 1553-1565.
8. Alnylam announces receipt of complete response letter from U.S. FDA for supplemental new drug application for patisiran for the treatment of the cardiomyopathy of ATTR amyloidosis [press release]. Cambridge, MA: Alnylam; October 6, 2023. Available at: <https://investors.alnylam.com/press-release?id=27741>. Accessed on: December 3, 2024.
9. Kittleson M, Ruberg FL, Ambardekar AV, et al. A report of the American College of Cardiology Solution Set Oversight Committee. 2023 ACC expert consensus decision pathway on comprehensive multidisciplinary care for the patient with cardiac amyloidosis. *JACC*. 2023;81(11):1076-1126.