

Onpattro™ (Patisiran)

What is Onpattro?

Onpattro is an RNA interference (RNAi) drug developed for treating patients with nerve damage (polyneuropathy) due to hereditary TTR (hATTR) amyloidosis. Onpattro is designed to reduce the production of the protein transthyretin (TTR) that causes the disease.

How does Onpattro work?

Onpattro is a type of 'RNA interference' drug, which aims to stop the production of amyloid-producing proteins by 'silencing' the TTR gene through an RNA mechanism. RNA functions as a genetic template and messenger that transports genes from our DNA and translates them into specific proteins. RNA interference drugs are designed to identify and destroy a specific type of RNA. In the case of Onpattro, it works by destroying the RNA associated with the TTR gene. Once the RNA has been removed, it can no longer create amyloid-producing TTR.

If you or someone you care for takes Onpattro

You will receive the drug through intravenous infusion (fluids into a vein) once every three weeks. Each infusion takes just over an hour to administer and is preceded by a combination of pre-drugs to help reduce the risk of a negative 'infusion reaction'. Onpattro should be administered by a healthcare professional. Administration is usually done in a hospital or clinic setting, but home infusion may be an option for some patients. The decision for a patient to receive hospital/clinic or home infusions should be made after an evaluation and recommendation by the treating physician. Home infusion may not be covered by all insurance plans.

What side effects might you expect?

The most commonly observed side effects in patients on the APOLLO Phase III trial - a randomized, double-blind, placebo-controlled, global study to evaluate the efficacy and safety of Onpattro in patients with hATTR amyloidosis with polyneuropathy - were diarrhea, swelling in the lower legs and ankles (edema), and symptoms related to receiving intravenous infusions, called 'infusion-related

reactions'. These reactions are often characterised by fever, chills, cough, nausea, changes in blood pressure, flushing, rash and fatigue, which occurred in 19% of patients.

What improvements might you see with Onpattro?

In the APPOLLO Phase III clinical trial, 225 hATTR amyloidosis patients with polyneuropathy were given either Onpattro or placebo (inactive drug) over 18 months. Results from the trial have shown that, compared to those on placebo, patients on Onpattro:

- saw statistically significant improvements in their polyneuropathy
- had statistically significant improvements in other measures looked at in the study, including overall quality of life and ability to carry out daily activities including walking

How can you or someone you care for get access to Onpattro?

Onpattro was approved by the FDA on August 10, 2018 for the treatment of hATTR amyloidosis polyneuropathy in the United States.

Insurance coverage of Onpattro will vary depending on the particular plan. If insurance does not provide enough coverage, there are additional programs that can be looked in to. These include co-pay assistance programs by the pharmaceutical company, Alnylam. More information about co-pay assistance and other programs can be found on Alnylam's patient support website at: www.alnylamassist.com.

More information

For more information you can visit us at www.arci.org, Alnylam's website at www.alnylam.com or the Onpattro website at www.onpattro.com. If you have further questions you can contact us by phone at 617-467-5170 or by email at arc@arci.org.

