

## Patisiran (Onpattro™)

### **What is patisiran?**

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

### **How does patisiran work?**

Patisiran 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 patisiran, 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 patisiran?**

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'. Currently, these infusions must be administered in a hospital or clinic setting.

### **What side effects might you expect?**

The most commonly observed side effects seen in patients who participated in the APOLLO Phase III trial - a randomized, double-blind, placebo-controlled, global study to evaluate the efficacy and safety of patisiran 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 patisiran?**

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

- 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 patisiran?**

Patisiran was approved by the FDA on August 10, 2018 for the treatment of hATTR amyloidosis polyneuropathy in the United States. However, patisiran may still not be available while the drug company gets it ready for commercialization. Once ready for market, you or a loved one who has hATTR polyneuropathy will be able to ask your physician to prescribe it.

Insurance coverage of patisiran will vary depending on the particular plan. If your insurance does not provide enough coverage, there are additional programs that may be able to help with the cost. These include co-pay assistance programs by the pharmaceutical company, Alynlam.

### **More information**

For more information you can visit us at [www.arci.org](http://www.arci.org) or Alynam's website at [www.alnylam.com](http://www.alnylam.com). If you have further questions you can contact us by phone at 617-467-5170 or by email at [arc@arci.org](mailto:arc@arci.org).