Generic Name:
Patisiran
Trade Name:
Onpattro
Company:
Alnylam Pharmaceuticals
Notes:

FDA approved patisiran infusion for the treatment of peripheral nerve disease (polyneuropathy) caused by hereditary transthyretin-mediated amyloidosis (hATTR) in adult patients.

This is the first FDA-approved treatment for patients with polyneuropathy caused by hATTR, a rare, debilitating, and often fatal genetic disease characterized by the buildup of abnormal amyloid protein in peripheral nerves, the heart, and other organs. It is also the first FDA approval of a new class of drugs called small interfering ribonucleic acids (siRNAs).

siRNAs work by silencing a portion of RNA involved in causing the disease. More specifically, patisiran encases the siRNA into a lipid nanoparticle to deliver the drug directly into the liver, in an infusion treatment, to alter or halt the production of disease-causing proteins.

The agent is designed to interfere with RNA production of an abnormal form of the protein transthyretin (TTR). By preventing the production of TTR, the drug can help reduce the accumulation of amyloid deposits in peripheral nerves, improving symptoms and helping patients better manage the condition.

Efficacy was shown in a clinical trial involving 225 patients, 148 of whom were randomly assigned to receive a patisiran infusion once every three weeks for 18 months, and 77 of whom were randomly assigned to receive a placebo infusion at the same frequency. The patients who received patisiran had better outcomes on measures of polyneuropathy, including muscle strength, sensation (pain, temperature, numbness), reflexes, and autonomic symptoms (blood pressure, heart rate, digestion) compared with those receiving the placebo infusions. Patisiran-treated patients also scored better on assessments of walking, nutritional status, and the ability to perform activities of daily living.

The most common adverse reactions reported by patients in clinical trials included flushing, back pain, nausea, abdominal pain, dyspnea, and headache. All patients who participated in the clinical trials received premedication with a corticosteroid, acetaminophen, and antihistamines (H1 and H2 blockers) to reduce the occurrence of infusion-related reactions.

Patients may also experience vision problems, including dry eyes, blurred vision, and eye floaters (vitreous floaters). Use of the agent can cause a decrease in serum vitamin A levels, so patients should take a daily Vitamin A supplement at the recommended daily allowance.

Medication Monitor Categories:
New Drug Approvals