Generic Name:
Givosiran
Trade Name:
Givlaari
Company:
Alnylam Pharmaceuticals
Notes:

FDA approved givosiran for adults with acute hepatic porphyria (AHP).

Efficacy was evaluated in ENVISION, a randomized, double?blind, placebo?controlled, multinational trial enrolling 94 patients with AHP. Patients were randomized (1:1) to receive once-monthly S.C. injections of givosiran 2.5 mg/kg or placebo during a 6?month double?blind period.

The primary efficacy outcome measure was the rate of porphyria attacks requiring hospitalizations, urgent health care visit, or I.V. hemin administration at home. The mean rates of attacks over a 6-month time period were 1.9 (95% CI1.3?2.8) for patients receiving givosiran and 6.5 (95% CI 4.5?9.3) for those on placebo. On average, patients with AHP on givosiran experienced 70% (95% CI 60%, 80%) fewer porphyria attacks compared with placebo.

The most common adverse reactions included nausea and injection site reactions. The label contains warnings for anaphylactic reactions, hepatic and renal toxicities, and injection site reactions. Hepatic toxicity was mostly transaminase elevation. Renal toxicity was mostly serum creatinine elevation and decreases in estimated glomerular filtration rate.

The recommended givosiran dose is 2.5 mg/kg once monthly by S.C. injection.

Medication Monitor Categories:
New Drug Approvals