Long-term Efficacy and Safety of Odevixibat, an Ileal Bile Acid Transporter Inhibitor in Children With Progressive Familial Intrahepatic Cholestasis: Interim Results From PEDIFC2, an Open-Label Phase 3 Trial


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INTRODUCTION

• Progressive familial intrahepatic cholestasis (PFIC) is a group of rare, inherited cholestatic liver disorders that result in obstructive jaundice with cholestasis and cirrhosis. Multiple cholestasis phenotypes have been described including PFIC1, PFIC2, and PFIC3 with two additional PFIC subtypes (PFIC4 and PFIC5, respectively).
• Odevixibat (RO7-2634), a small molecule bile acid sequestrant, has been approved by the FDA for the treatment of PFIC1 and PFIC2.
• PFIC2 is the most common form of PFIC, estimated to occur in 1 in 100,000 live births.
• Commonly, available therapy options are limited to nutritional support and liver transplantation to treat patients with advanced cirrhosis.

• This study is an open-label, phase 3 trial that examines the safety and efficacy of odevixibat in patients aged 1-18 years with PFIC2.

RESULTS

• 120 children and adolescents aged 1-18 years with PFIC2 were randomized to receive odevixibat 1 or 2 mg twice daily, or to placebo twice daily for 24 weeks.

• 93% of patients in both active treatment groups remained on study medication, compared to 81% in the placebo group.

• There were no serious adverse events related to study medication.

METHODS

• Study Population: Children and adolescents aged 1-18 years with PFIC2 were eligible for inclusion.

• Study Design: This was a multicenter, double-blind, placebo-controlled, randomized, phase 3 trial.

• Study Outcomes: Primary outcomes were the changes in serum bile acids and pruritus scores from baseline to week 24.

• Statistical Analysis: Analysis was performed on the intent-to-treat population.

REFERENCE