Maralixibat treatment response is associated with improved health-related quality of life in patients with bile salt export pump deficiency


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Objectives

The aim was to evaluate the relationship between baseline characteristics and health-related quality of life (HRQoL) response to maralixibat in patients with bile salt export pump deficiency (BSEP deficiency). The Multidimensional Fatigue Scale and the Pediatric Fatigue Questionnaire were used to assess fatigue.

Methods

Study design and participants

• Inclusion criteria: Patients with BSEP deficiency undergoing treatment with maralixibat were included. BSEP deficiency is a rare, inherited disorder characterized by bile acid accumulation and a broad range of manifestations that usually present in early childhood, including jaundice, pruritus, failure to thrive, and progressive liver disease. The disease is due to defects in the bile salt export pump (BSEP), which is a key transporter that clears bile acids from the liver into the bile ducts.

• Exclusion criteria: Patients with significant comorbidities or those who were non-responders to maralixibat were excluded.

• Data collection: Retrospective analysis of prospectively collected data from patients previously treated with maralixibat for up to 72 weeks. All data were collected from previous studies funded by Mirum Pharmaceuticals, Inc.

• Multivariate linear regression models were used to assess the relationship between the response to maralixibat and baseline characteristics. The relationship between the response to maralixibat and baseline characteristics was evaluated.

• The minimal clinically important difference (MCID) for the HRQoL assessments were selected independently by caregivers at baseline and week 48, and analyzed retrospectively.

• The study was approved by the Institutional Review Board (IRB) of the Centers for Disease Control and Prevention (CDC), and all patients provided written informed consent.

• All data are from a 30-center, multinational, open-label, multicenter, dose-ranging, Phase 2 study of patients with BSEP deficiency previously enrolled in the INDIGO and INDIGO-II trials.

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