Budesonide add-on therapy improves markers of disease activity but fails to improve histology

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The addition of budesonide is associated with clinically meaningful improvements in biochemical markers of disease activity but no improvement in liver histology in high-risk patients with primary biliary cholangitis (PBC) experiencing a sub-optimal response to ursodeoxycholic acid (UDCA), according to the results of a study presented today. The placebo-controlled study, which randomized 62 patients with PBC, was terminated early because of slow recruitment and as a result, insufficient power to detect a significant histological difference between treatment groups.

PBC is an autoimmune liver disease that is characterized by the progressive destruction of the small bile ducts, resulting in intrahepatic cholestasis, parenchymal injury, and, ultimately, end-stage liver disease. The condition typically occurs in middle-aged women, with features frequently including fatigue, pruritis, jaundice, xanthomas, osteoporosis, and dyslipidaemia. Ursodeoxycholic acid is the first-line therapy for PBC; however, up to 40% of patients have an insufficient response to this therapy. Second-line licensed therapy is with obeticholic acid. Previous studies evaluating the combination of budesonide and UDCA in patients with PBC reported promising results, although relevant budesonide toxicity was reported in patients with late-stage disease.

The study presented today at The International Liver Congress 2018 in Paris, France, represents an important, long-awaited, placebo-controlled

trial evaluating patients with PBC at high risk of progression. Patients were required to have histologically confirmed PBC and inflammatory activity according to the Ishak score, failure to achieve serum alkaline phosphatase (ALP)