In Brief: Cholic Acid (Cholbam) for Bile Acid Synthesis Disorders

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IN BRIEF

Cholic Acid (Cholbam) for Bile Acid Synthesis Disorders

The FDA has approved oral cholic acid (Cholbam – Retrophin) for treatment of children and adults with bile acid synthesis disorders caused by single enzyme defects and for adjunctive treatment of peroxisomal disorders such as Zellweger spectrum disorders in patients who have liver disease, steatorrhea, or complications from fat-soluble vitamin malabsorption. Patients with these rare inborn errors of bile acid metabolism cannot synthesize primary bile acids such as cholic acid, resulting in reduced bile flow, decreased absorption of fat and fat-soluble vitamins, and development of liver disease, which can be fatal. Cholbam is the first drug to be approved in the US for these indications.

FDA approval was based on a long-term, single-arm clinical trial, an extension of that trial, and case reports in a total of 77 patients with bile acid synthesis disorders and 34 patients with peroxisomal disorders. Administration of cholic acid appeared to decrease hepatic injury and increase height and weight; 67% of patients with bile acid synthesis disorders and 42% of those with peroxisomal disorders treated in the clinical trials survived for more than 3 years.1 Some of these survivors have been treated successfully for more than 20 years. Diarrhea has been the most common adverse effect.

Cholbam is available in 50- and 250-mg capsules. The usual dosage is 10-15 mg/kg taken once daily or in two divided doses. The cost of 30 days’ treatment for a 50-kg patient at a daily dose of 10 mg/kg is about $50,000.2

2. Approximate WAC. WAC = wholesaler acquisition cost or manufacturer’s published price to wholesalers; WAC represents a published catalogue or list price and may not represent an actual transactional price. Source: AnalySource® Monthly. April 5, 2016. Reprinted with permission by First Databank, Inc. All rights reserved. ©2016 www.fdbhealth.com/policies/drug-pricing-policy.