

The Medical Letter[®]

on Drugs and Therapeutics

Volume 58

April 25, 2016

ISSUE No.
1493

IN THIS ISSUE

In Brief: Cholic Acid (*Cholbam*) for Bile Acid Synthesis Disorders p 56

Important Copyright Message

FORWARDING OR COPYING IS A VIOLATION OF U.S. AND INTERNATIONAL COPYRIGHT LAWS

The Medical Letter, Inc. publications are protected by U.S. and international copyright laws. Forwarding, copying or any distribution of this material is prohibited.

Sharing a password with a non-subscriber or otherwise making the contents of this site available to third parties is strictly prohibited.

By accessing and reading the attached content I agree to comply with U.S. and international copyright laws and these terms and conditions of The Medical Letter, Inc.

For further information click: [Subscriptions](#), [Site Licenses](#), [Reprints](#)
or call customer service at: 800-211-2769

The Medical Letter®

on Drugs and Therapeutics

Volume 58 (Issue 1493)

April 25, 2016

Take CME Exams

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 ■

1. FDA. Medical review: cholic acid (Cholbam). Available at: www.accessdata.fda.gov/drugsatfda_docs/nda/2015/205750Orig1s000MedR.pdf. Accessed April 14, 2016.
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.

Follow us on Twitter  Like us on Facebook 

PRESIDENT: Mark Abramowicz, M.D.; **VICE PRESIDENT AND EXECUTIVE EDITOR:** Gianna Zuccotti, M.D., M.P.H., F.A.C.P., Harvard Medical School; **EDITOR IN CHIEF:** Jean-Marie Pflomm, Pharm.D.; **ASSOCIATE EDITORS:** Susan M. Daron, Pharm.D., Amy Faucard, MLS, Corinne Z. Morrison, Pharm.D., Michael P. Viscusi, Pharm.D.; **CONSULTING EDITORS:** Brinda M. Shah, Pharm.D., F. Peter Swanson, M.D.

CONTRIBUTING EDITORS: Carl W. Bazil, M.D., Ph.D., Columbia University College of Physicians and Surgeons; Vanessa K. Dalton, M.D., M.P.H., University of Michigan Medical School; Eric J. Epstein, M.D., Albert Einstein College of Medicine; Jane P. Gagliardi, M.D., M.H.S., F.A.C.P., Duke University School of Medicine; David N. Juurlink, BPhM, M.D., Ph.D., Sunnybrook Health Sciences Centre; Richard B. Kim, M.D., University of Western Ontario; Franco M. Muggia, M.D., New York University Medical Center; Sandip K. Mukherjee, M.D., F.A.C.C., Yale School of Medicine; Dan M. Roden, M.D., Vanderbilt University School of Medicine; Esperance A.K. Schaefer, M.D., M.P.H., Harvard Medical School; F. Estelle R. Simons, M.D., University of Manitoba; Neal H. Steigbigel, M.D., New York University School of Medicine; Arthur M. F. Yee, M.D., Ph.D., F.A.C.R., Weill Medical College of Cornell University

MANAGING EDITOR: Susie Wong; **ASSISTANT MANAGING EDITOR:** Liz Donohue; **EDITORIAL ASSISTANT:** Cheryl Brown

EXECUTIVE DIRECTOR OF SALES: Gene Carbona; **FULFILLMENT AND SYSTEMS MANAGER:** Cristine Romatowski; **EXECUTIVE DIRECTOR OF MARKETING AND COMMUNICATIONS:** Joanne F. Valentino; **VICE PRESIDENT AND PUBLISHER:** Yosef Wissner-Levy

Founded in 1959 by
Arthur Kallet and Harold Aaron, M.D.

Copyright and Disclaimer: The Medical Letter, Inc. is an independent nonprofit organization that provides healthcare professionals with unbiased drug prescribing recommendations. The editorial process used for its publications relies on a review of published and unpublished literature, with an emphasis on controlled clinical trials, and on the opinions of its consultants. The Medical Letter, Inc. is supported solely by subscription fees and accepts no advertising, grants, or donations. No part of the material may be reproduced or transmitted by any process in whole or in part without prior permission in writing. The editors do not warrant that all the material in this publication is accurate and complete in every respect. The editors shall not be held responsible for any damage resulting from any error, inaccuracy, or omission.

Subscription Services

Address:
The Medical Letter, Inc.
145 Huguenot St. Ste. 312
New Rochelle, NY 10801-7537
www.medicalletter.org

Customer Service:
Call: 800-211-2769 or 914-235-0500
Fax: 914-632-1733
E-mail: custserv@medicalletter.org

Permissions:
To reproduce any portion of this issue,
please e-mail your request to:
permissions@medicalletter.org

Subscriptions (US):
1 year - \$129; 2 years - \$232;
3 years - \$345, \$65 per year
for students, interns, residents, and
fellows in the US and Canada.
Reprints - \$12 each.

Site License Inquiries:
E-mail: info@medicalletter.org
Call: 800-211-2769 ext. 315
Special rates available for bulk
subscriptions.

Get Connected:  

Copyright 2016. ISSN 1523-2859

The
Medical
Letter