

## SPECIALTY GUIDELINE MANAGEMENT

### CHOLBAM (cholic acid)

#### POLICY

##### I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

##### FDA-Approved Indications

Cholbam is indicated for:

1. Treatment of bile acid synthesis disorders due to single enzyme defects (SEDs)
2. Adjunctive treatment of peroxisomal disorders (PDs) including Zellweger spectrum disorders in patients who exhibit manifestations of liver disease, steatorrhea or complications from decreased fat soluble vitamin absorption

*Limitation of use: The safety and effectiveness of Cholbam on extrahepatic manifestations of bile acid synthesis disorders due to SEDs or PDs including Zellweger spectrum disorders have not been established.*

All other indications are considered experimental/investigational and are not a covered benefit.

##### II. CRITERIA FOR INITIAL APPROVAL

###### A. Bile acid synthesis disorders due to single enzyme defects (SEDs)

Authorization of 6 months may be granted for treatment of bile acid synthesis disorders due to single enzyme defects when the diagnosis is confirmed by mass spectrometry or other biochemical testing or genetic testing.

###### B. Peroxisomal disorders (PDs) including Zellweger spectrum disorders

Authorization of 6 months may be granted for adjunctive treatment of peroxisomal disorders when the diagnosis is confirmed by mass spectrometry or other biochemical testing or genetic testing.

##### III. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must meet additional authorization criteria below:

###### A. Bile acid synthesis disorders due to SEDs

Authorization of 12 months may be granted to members who have achieved and maintained improvement in liver function.

###### B. Peroxisomal disorders (PDs) including Zellweger spectrum disorders

Authorization of 12 months may be granted to members continuing adjunctive treatment with Cholbam who have achieved and maintained improvement in liver function.

#### IV. REFERENCES

1. Cholbam [package insert]. San Diego, CA: Retrophin, Inc.; March 2015.
2. Gonzales E, Gerhardt MF, Fabre M et al. Oral cholic acid for hereditary defects of primary bile acid synthesis: a safe and effective long-term therapy. *Gastroenterology*. 2009;137:1310-1320.
3. Heubi J, Setchell KDR, Bove KE. Inborn errors of bile acid metabolism. *Seminars Liver Dis*. 2007;27:282-294.
4. Poll-The BT, Gartner J. Clinical diagnosis, biochemical findings and MRI spectrum of peroxisomal disorders. *Biochim Biophys Acta*. 2012;1822:1421-1429.