

Orphacol

Cholic acid. From 1 month of age.



General description:

Orphacol (cholic acid) is an orphan drug indicated for the treatment of inborn errors in primary bile acid synthesis, caused by deficiency in the enzyme 3β -Hydroxy- Δ^5 -C27-steroid dehydrogenase/isomerase (3β -HSD) or in the enzyme Δ^4 -3-Oxo-steroid- 5β -reductase (Δ^4 -3-oxoR). Orphacol is available as hard capsules, ready to take. Suitable for infants, children and adolescents aged 1 month to 18 years and adults.



Presentation and Protein Equivalent (PE):

Orphacol is available in two formats – 50 mg and 250 mg cholic acid capsules. Each box contains 30 capsules.



Important information:

Treatment must be initiated and monitored by an experienced gastroenterologist/hepatologist or a paediatric gastroenterologist/hepatologist in the case of paediatric patients. During the initiation of therapy with Orphacol and dose adjustment, serum and/or urine bile acid levels should be monitored intensively (at least every three months during the first year of treatment, every six months during the second year). The lowest dose of cholic acid that effectively reduces the bile acid metabolites to as close to zero as possible should be chosen. After the initiation period, serum and/or urine bile acids (using suitable analytical techniques) and liver parameters should be determined annually, at a minimum, and the dose adjusted accordingly.



Dosage and Administration:

The dose must be adjusted for each patient in a specialised unit according to blood and/or urine chromatographic bile acid profiles. The daily dose ranges from 5 to 15 mg/kg in infants, children, adolescents and adults. In all age groups, the minimum dose is 50 mg. The dose is adjusted in 50 mg steps. In adults, the daily dose should not exceed 500 mg. For enteral use only.








Preparation:

Orphacol capsules must be taken with food at approximately the same time each day, in the morning and/or evening. Capsules must be swallowed whole with water, without chewing. For infants and children who cannot swallow capsules, the capsules may be opened, and the content added to infant formula or juice.



Storage:

Keep it in a cool, dry place.

 Product	 Treatment of	 Presentation and PE	 Age	 Preparation
Orphacol (cholic acid)	Primary Bile Acid Synthesis Disorders (DSABP) - deficiency in 3β - HSD or Δ^4 -3-oxoR	Orphan drug in hard capsules form. Each box contains 30 capsules. 50 or 250 mg	From 1 month of age	Tablets ready to take with water or other suitable liquid

