PACKAGE LEAFLET: INFORMATION FOR THE USER

Orphacol 50 mg hard capsules
Orphacol 250 mg hard capsules
Cholic acid

▼This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you may get. See the end of section 4 for how to report side effects.

Read all of this leaflet carefully before you start taking this medicine because it contains important information for you.
- Keep this leaflet. You may need to read it again.
- If you have any further questions, ask your doctor or pharmacist.
- This medicine has been prescribed for you. Do not pass it on to others. It may harm them, even if their signs of illness are the same as yours.
- If you get any side effects, talk to your doctor or pharmacist. This includes any possible side effects not listed in this leaflet. See section 4.

What is in this leaflet:
1. What Orphacol is and what it is used for
2. What you need to know before you take Orphacol
3. How to take Orphacol
4. Possible side effects
5. How to store Orphacol
6. Contents of the pack and other information

1. What Orphacol is and what it is used for

Orphacol contains cholic acid, a bile acid which is normally produced by the liver. Certain medical conditions are caused by defects in bile acid production and Orphacol is used to treat infants from one month to 2 years of age, children, adolescents and adults with these medical conditions. The cholic acid contained in Orphacol replaces the bile acids that are missing due to the defect in bile acid production.

2. What you need to know before you take Orphacol

Do not take Orphacol
- if you are allergic to cholic acid or any of the other ingredients of this medicine (listed in section 6).
- if you are taking phenobarbital or primidone, a medicine to treat epilepsy.

Warnings and precautions
During your treatment, your doctor will carry out various blood and urine tests at different time to see how your body is handling this medicine and to help work out the dose that you need. More frequent tests will be needed if you are growing fast, if you are ill (if you have e.g. liver problems), or if you are pregnant.

Other medicines and Orphacol
Tell your doctor or pharmacist if you are taking, have recently taken any or might take other medicines.

Some medicines used to lower cholesterol levels, so-called bile acid sequestrants (cholestyramine, colestipol, colesevelam), and medicines to treat heartburn that contain aluminium may lessen the effect of

Orphacol. If you take these medicines, take Orphacol at least 5 hours before or at least 5 hours after taking these other medicines.
Ciclosporin (a medicine used to suppress the immune system) may also change the effect of Orphacol. Please tell your doctor if you are taking ciclosporin.

Ursodeoxycholic acid may lessen the effect Orphacol if both medicines are taken at the same time. If you are prescribed ursodeoxycholic acid along with Orphacol in single doses, take one product in the morning and the other product in the evening. If you are prescribed divided doses of ursodeoxycholic acid and/or Orphacol, as these products should be administered separately by several hours, please ask for the advice of your doctor or pharmacist about proper sequence of administration.

Pregnancy and breast-feeding
Please consult your doctor if you plan to become pregnant. Take a pregnancy test as soon as you suspect you may be pregnant. It is very important to continue taking Orphacol during pregnancy.

If you become pregnant during treatment with Orphacol, your doctor will decide which treatment and dose is best in your situation. As a precaution, you and your unborn child should be closely monitored during pregnancy.

Orphacol can be used during breast-feeding. Tell your doctor if you plan to breast-feed or are breast-feeding before you take Orphacol.

Ask your doctor or pharmacist for advice before taking any medicine.

Driving and using machines
Orphacol is not expected to have an effect on the ability to drive and use machines.

Orphacol contains lactose
Orphacol contains a certain sugar (lactose monohydrate). If you have been told by your doctor that you have an intolerance to some sugars, contact your doctor before taking Orphacol.

3. How to take Orphacol

Always take Orphacol exactly as your doctor has told you. Check with your doctor if you are not sure.

The usual starting dose is 5 to 15 mg per kilogram body weight every day in infants, children, adolescents and adults.

Before treatment your doctor will make an assessment from your laboratory tests to determine the correct dose for you. The dose will then be further adjusted by your doctor depending on your body's response.

Orphacol capsules are taken by mouth with a meal at approximately the same time each day, in the morning and/or evening. Taking Orphacol at regular times with a meal will help you remember to take this medicine, and may help your body to better take it up. Capsules must be swallowed whole with water. Do not chew.

If your doctor has prescribed a dose which requires you to take more than one capsule per day, you and your doctor can decide how this will be taken during the day. You may for example take one capsule in the morning and one in the evening. This way, you will have to take fewer capsules at once. However, this might not be possible if you have been prescribed at the same time another medicine containing ursodeoxycholic acid. In this case, you should seek advice from your doctor or pharmacist about proper sequence of administration of ursodeoxycholic acid and Orphacol over the day, as these products should be administered separately by several

hours (see section 2).

Use in children
For babies and children who cannot swallow capsules, the capsule can be opened and its contents added to baby formula or apple/orange or apple/apricot juice adapted for small children.

If you take more Orphacol than you should
If you have taken more Orphacol than you should, contact your doctor as soon as possible. He will assess your laboratory test results and advise you when you should resume your treatment with your normal dose.

If you forget to take Orphacol
Take your next dose when you would normally take it. Do not take a double dose to make up for a forgotten dose.

If you stop taking Orphacol
There is a risk of permanently damaging your liver if you stop taking Orphacol. You should never stop taking Orphacol unless your doctor advises you to do so.

If you have any further questions on the use of this medicine, ask your doctor.

4. Possible side effects

Like all medicines, this medicine can cause side effects, although not everybody gets them.

Several patients have experienced itching and/or diarrhoea, however, it is not known how likely this is to occur (frequency cannot be estimated from the available data). If itching and/or diarrhoea last for more than three days, tell your doctor.

For several patients an increase of liver enzymes (serum transaminases) have been reported during the treatment with Orphacol (frequency cannot be estimated from the available data). Your doctor will decide what to do if this happens to you.

Gallstones have been reported after long-term therapy with Orphacol.

Reporting of side effects
If you get any side effects, talk to your doctor. This includes any possible side effects not listed in this leaflet. You can also report side effects directly via the Yellow Card Scheme. Website: www.mhra.gov.uk/yellowcard or search for MHRA Yellow Card in the Google Play or Apple App Store. By reporting side effects you can help provide more information on the safety of this medicine.

5. How to store Orphacol

Keep out of the reach and sight of children.

Do not use Orphacol after the expiry date which is stated on the carton and blister after EXP. The expiry date refers to the last day of that month.

Store below 30°C.

Medicines should not be disposed of via wasterwater or household waste. Ask your pharmacist how to dispose of medicines no longer required. These measures will help to protect the environment.

6. Contents of the pack and other information

What Orphacol contains

- The active substance is cholic acid.
  Orphacol 50 mg: Each hard capsule contains 50 mg of cholic acid.
  Orphacol 250 mg: Each hard capsule contains 250 mg of cholic acid.
- The other ingredients are:
  Content of capsules: Lactose monohydrate (see section 2 under 'Orphacol contains lactose' for more information), colloidal anhydrous silica, magnesium stearate
  Capsule shell:
  Orphacol 50 mg: gelatin, titanium dioxide (E171), carmine blue (E132);
  Orphacol 250 mg: gelatin, titanium dioxide (E171), carmine blue (E132), yellow iron oxide (E172).

What Orphacol looks like and contents of the pack
Orphacol is available as hard capsules (capsules) of oblong shape. Capsules of 50 mg cholic acid are blue and white and capsules of 250 mg cholic acid are green and white. They are contained in blisters of 10 capsules each.
Pack sizes are available in 30, 60 and 120 capsules.
Not all pack sizes may be marketed.

Marketing Authorisation Holder and Manufacturer

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This leaflet was last revised in September 2023

This medicine has been authorised under "Exceptional Circumstances".
This means that because of the rarity of this disease and for ethical reasons it has been impossible to get complete information on this medicine.
The Medicines and Healthcare products Regulatory Agency will review any new information on the medicine every year and this leaflet will be updated as necessary.