

Consumer Medicine Information (CMI) summary

The [full CMI](#) on the next page has more details. If you are worried about using this medicine, speak to your doctor or pharmacist.

1. Why am I using Kuvan?

Kuvan contains the active ingredient sapropterin dihydrochloride which is a synthetic copy of the body's own substance called tetrahydrobiopterin. Kuvan is used to treat hyperphenylalaninemia (HPA) by reducing blood phenylalanine levels in patients with phenylketonuria (PKU) or tetrahydrobiopterin (BH4) deficiency. For more information, see Section [1. Why am I using Kuvan?](#) in the full CMI.

2. What should I know before I use Kuvan?

Do not use if you have ever had an allergic reaction to Kuvan or any of the ingredients listed at the end of the CMI.

Talk to your doctor if you have any other medical conditions, take any other medicines, or are pregnant or plan to become pregnant or are breastfeeding. For more information, see Section [2. What should I know before I use Kuvan?](#) in the full CMI.

3. What if I am taking other medicines?

Some medicines may interfere with Kuvan and affect how it works. A list of these medicines is in Section [3. What if I am taking other medicines?](#) in the full CMI.

4. How do I use Kuvan?

- Take Kuvan with meals.
- Place the number of tablet(s) or sachet(s) your doctor has told you to take in a glass or cup of water or apple juice and stir until dissolved. Your doctor will tell you how much water or juice to use to dissolve the tablet(s) or powder, and the amount of the medicine mixture to take. You must take Kuvan exactly as explained by your doctor.
- You may mix the crushed tablets in a small amount of soft food such as yoghurt or mashed banana.

More instructions can be found in Section [4. How do I use Kuvan?](#) in the full CMI.

5. What should I know while using Kuvan?

Things you should do	<ul style="list-style-type: none"> • Remind any doctor, dentist or pharmacist you visit that you are using Kuvan • Keep all your doctor's appointments so your progress can be checked. • Call your doctor straight away if you become ill for any reason.
Things you should not do	<ul style="list-style-type: none"> • Do not change your diet without contacting your doctor. • Do not stop using this medicine unless your doctor tells you to.
Driving or using machines	<ul style="list-style-type: none"> • Be careful before you drive or use any machines or tools until you know how Kuvan affects you.
Looking after your medicine	<ul style="list-style-type: none"> • Keep the tablets in the bottle and sachets in a cool dry place below 25°C. • Keep the bottle tightly closed to protect the tablets from moisture. • Do not use the tablets if more than 2 months has passed since first opening the bottle.

For more information, see Section [5. What should I know while using \[insert medicine\]?](#) in the full CMI.

6. Are there any side effects?

All medicines can have side effects. If they do occur, they are usually minor and temporary. Do not be alarmed by this list. You may not experience any of them.

The common side effects include headache, runny nose, sore throat, blocked nose, throat infection, mouth and throat pain, cough, pain in the oesophagus, oesophagitis (inflammation of the lining of the food pipe), gastritis (inflammation of the lining of the stomach), gastrointestinal problems (e.g. vomiting, upset stomach, nausea, diarrhoea, stomach ache) and blood Phe levels that are too low. The serious side effects are symptoms of an allergic reaction (shortness of breath, wheezing, difficulty breathing or a tight feeling in your chest, swelling of the face, lips, tongue, or other parts of the body, rash, itching, hives, or flushed red skin), and symptoms of severe gastritis (inflammation of the stomach lining) or oesophagitis (inflammation of the lining of the food pipe), e.g. severe upper stomach area discomfort or pain, nausea and vomiting, blood in your vomit or stool, black or tarry stools, swallowing that is difficult or painful.

For more information, including what to do if you have any side effects, see Section [6. Are there any side effects?](#) in the full CMI.

Active ingredient: *sapropterin dihydrochloride*

Consumer Medicine Information (CMI)

This leaflet provides important information about using Kuvan. **You should also speak to your doctor or pharmacist if you would like further information or if you have any concerns or questions about using Kuvan.**

Where to find information in this leaflet:

- [1. Why am I using Kuvan?](#)
- [2. What should I know before I use Kuvan?](#)
- [3. What if I am taking other medicines?](#)
- [4. How do I use Kuvan?](#)
- [5. What should I know while using Kuvan?](#)
- [6. Are there any side effects?](#)
- [7. Product details](#)

1. Why am I using Kuvan?

Kuvan contains the active ingredient **sapropterin dihydrochloride** which is a synthetic copy of the body's own substance called **tetrahydrobiopterin (BH4)**. Kuvan is identical to the body's own natural BH4 except that it is made in a laboratory. BH4 is used by the body to process phenylalanine (Phe) in order to build another amino acid called tyrosine, which is a building block for several important brain chemicals.

Kuvan is used to treat **hyperphenylalaninemia (high blood phenylalanine levels) in patients with phenylketonuria (PKU) or tetrahydrobiopterin (BH4) deficiency**. People with PKU or BH4 deficiency have abnormally high levels of Phe in the blood. Phe is a necessary amino acid but it is harmful in excessive amounts and people with PKU or BH4 deficiency cannot process Phe normally. A Phe restricted diet will reduce Phe levels in the body and some people also need treatment with Kuvan.

Kuvan is used together with dietary restrictions to reduce Phe in the blood. Not everyone with PKU or BH4 deficiency responds to Kuvan but if you do respond, you may be able to tolerate more Phe in your diet than you could without treatment.

2. What should I know before I use Kuvan?

Warnings

Do not use Kuvan if:

- you are allergic to sapropterin dihydrochloride, or any of the ingredients listed at the end of this leaflet.
- Always check the ingredients to make sure you can use this medicine.

Check with your doctor if you:

- have any other medical conditions, in particular you:
 - have or have had kidney or liver disease
 - have or have had convulsions ('fits')
 - have gastritis (inflammation of the stomach lining)
 - have oesophagitis (inflammation of the food pipe [gullet])
 - do not feel well before you start to take Kuvan. The levels of Phe in your blood may be higher if you are currently ill
- are over 50 years old
- your child is less than 1 month old. More frequent monitoring is recommended in children
- take any medicines for any other condition

During treatment, you may be at risk of developing certain side effects. It is important you understand these risks and how to monitor for them. See additional information under Section [6. Are there any side effects?](#)

Pregnancy and breastfeeding

Check with your doctor if you are pregnant or intend to become pregnant. It is very important to control blood Phe during pregnancy as high Phe can harm you or your baby. Dietary management is the first option. Your doctor will consider the use of Kuvan only if strict dietary management does not adequately reduce your blood Phe levels.

Talk to your doctor if you are breastfeeding or intend to breastfeed. It is not known whether Kuvan passes into breast milk. You should not take Kuvan if you are breastfeeding.

3. What if I am taking other medicines?

Tell your doctor or pharmacist if you are taking any other medicines, including any medicines, vitamins or supplements that you buy without a prescription from your pharmacy, supermarket or health food shop.

Tell your doctor or pharmacist if you are taking any of the following medicines:

- methotrexate (a medicine used to treat arthritis, psoriasis and some cancers)
- trimethoprim (an antibiotic)
- any heart medicine or angina treatment, e.g. glyceryl trinitrate, isosorbide dinitrate or sodium nitroprusside
- any medicine for the treatment of impotence, such as sildenafil, vardenafil, or tadalafil
- minoxidil (a medicine used to treat hair loss and high blood pressure)
- levodopa (a medicine used to treat Parkinson's disease)

These medicines may be affected by Kuvan or may affect how well it works. You may need to use different amounts of your medicines, or take different medicines. Your doctor will advise you.

Your doctor or pharmacist has more information on medicines to be careful with or avoid while taking Kuvan.

Check with your doctor or pharmacist if you are not sure about what medicines, vitamins or supplements you are taking and if these affect Kuvan.

4. How do I use Kuvan?

Follow carefully all directions given to you by your doctor, dietitian or pharmacist.

Kuvan is taken as tablet(s) or powder for oral solution in sachet(s) dissolved in water or apple juice.

It is not possible to know whether or not Kuvan will work for you until you start taking it. Your doctor will monitor your blood Phe levels when you start taking Kuvan to see if it is working.

How much to take

Your doctor will tell you how many tablets or sachets to take. **You must take Kuvan exactly as explained by your doctor.**

PKU

The usual starting dose of Kuvan in adults and children with PKU is 10 mg for each kg of body weight per day. Your doctor may adjust your dose depending on your condition, response to treatment, and as your child grows. The recommended daily dose is between 5 mg and 20 mg for each kg of body weight per day.

BH4 deficiency

The usual starting dose of Kuvan in adults and children with BH4 deficiency is 2 mg to 5 mg for each kg of body weight per day. Your doctor may adjust your dose depending on your condition and response to treatment. The recommended daily dose is between 2 mg and 20 mg for each kg of body weight per day.

When to take Kuvan

PKU

Take the tablet(s) or sachet(s) dissolved in water or apple juice as a single daily dose with a meal and at the same time each day, preferably in the morning.

BH4 deficiency

Take the tablet(s) or sachet(s) dissolved in water or apple juice with a meal. Divide the total daily dose into 2 or 3 doses, distributed over the day, to achieve the best treatment effect.

How to take Kuvan

Tablets

- Place the number of tablet(s) your doctor has told you to take in a glass or cup of water or apple juice and stir until dissolved. Your doctor will tell you how

much water or juice to use to dissolve the tablet(s). It may take a few minutes for the tablets to dissolve.

- To make the tablets dissolve faster you can crush them.
- Don't worry if there are small particles in the solution. This will not affect how well it works.
- Only prepare the solution when you are ready to take it. Kuvan solution should be drunk within 15 to 20 minutes after preparing it. Discard any unused solution.
- You may mix the crushed tablets in a small amount of soft food such as yoghurt or mashed banana. Make sure all the soft food is consumed so the full dose of the medicine is taken.

Sachets

- Open the sachet(s) of Kuvan powder for oral solution by folding and tearing or cutting at the dotted line in the upper right corner of the sachet.
- Empty the contents of the sachet(s) into a glass or cup of water or apple juice. Your doctor will tell you how much water or juice to use to dissolve the powder. After dissolving Kuvan powder in water or apple juice, the solution must be clear and colourless to yellow.
- Open the sachet(s) only when you are ready to use them. Drink the solution within 30 minutes. Discard any unused solution.

Adults and Children above 20 kg body weight

Place the number of tablet(s) or sachet(s) your doctor has told you to take in a glass or cup (120 - 240 mL) of water or apple juice and stir until dissolved.

Be sure that you know what dose of Kuvan powder your doctor prescribed. For higher doses, your doctor may also prescribe Kuvan 500 mg powder for oral solution. Be sure you know whether to use Kuvan 100 mg powder for oral solution, or both medicines to prepare your dose.

Children up to 20 kg body weight

If you are using Kuvan sachet to prepare the dose, only use the 100 mg sachet to prepare Kuvan for children weighing up to 20 kg body weight.

The dose of Kuvan is based on body weight. This will change as your child grows. Your doctor will tell you:

- the number of Kuvan tablets or 100 mg sachet(s) needed for one dose
- the amount of water or apple juice needed to mix one dose of Kuvan, and
- the amount of the mixture (tablet(s) or 100 mg sachet(s) and water or apple juice) to give your child for their prescribed dose.

You will need to use an accurate measuring device (e.g. medicine cup or oral syringe) with suitable graduations for this purpose. Ask your pharmacist for an accurate measuring device. For each dose, a fresh solution should be prepared.

Ask your doctor or pharmacist if you have any questions about how to prepare Kuvan or how much to take.

Do not swallow the desiccant capsule contained in the bottle of tablets.

If you forget to use Kuvan

Kuvan should be used regularly at the same time(s) each day. If you miss your dose at the usual time, take it as soon as you remember on the same day.

If it is almost time for your next dose, skip the dose you missed and take your next dose when you are meant to.

Do not take a double dose to make up for the dose you missed.

At your next appointment, tell your doctor if you forgot to take Kuvan.

If you use too much Kuvan

If you think that you have used too much Kuvan, you may need urgent medical attention.

Symptoms of an overdose with Kuvan may include headache and dizziness.

You should immediately:

- phone the Poisons Information Centre (In Australia telephone **131 126**. In New Zealand telephone **0800 POISON** or **0800 764 766**), or
- contact your doctor, or
- go to the Emergency Department at your nearest hospital.

You should do this even if there are no signs of discomfort or poisoning.

5. What should I know while using Kuvan?

Things you should do

- Keep all your doctor's appointments so your progress can be checked.
While you are taking Kuvan, your doctor will test your blood regularly to check your levels of Phe and tyrosine and may decide to adjust the dose of Kuvan or your diet if needed.

Call your doctor straight away if you:

- become ill for any reason even if the illness is not related to the reason you are taking Kuvan.
Your doctor may want to check your Phe levels as other illnesses may increase blood Phe.

Remind any doctor, dentist or pharmacist you visit that you are using Kuvan.

Things you should not do

- Do not change your diet without contacting your doctor.
Any change you make to your diet may affect your blood Phe level. You must continue your diet treatment as recommended by your doctor.

- Do not stop taking Kuvan unless your doctor tells you to.
The Phe levels in your blood may increase. Your doctor will need to carefully monitor your blood Phe levels if you stop taking Kuvan.
- Do not give Kuvan to anyone else, even if they have the same condition as you.
- Do not take Kuvan to treat any other complaints unless your doctor tells you to.

Driving or using machines

Be careful before you drive or use any machines or tools until you know how Kuvan affects you.

The effect of Kuvan on your ability to drive a car or operate machinery has not been studied.

Looking after your medicine

- Keep the tablets in the bottle and sachets in a cool dry place where the temperature stays below 25°C.
- Keep the bottle tightly closed to protect the tablets from moisture. Keep the desiccant tube in the bottle.
- Follow the instructions in the carton on how to take care of your medicine properly.

Store it in a cool dry place away from moisture, heat or sunlight; for example, do not store it:

- in the bathroom or near a sink, or
- in the car or on window sills.

Keep it where young children cannot reach it.

When to discard your medicine

Do not use the tablets if more than 2 months has passed since first opening the bottle.

Getting rid of any unwanted medicine

If you no longer need to use this medicine or it is out of date, take it to any pharmacy for safe disposal.

Do not use this medicine after the expiry date.

6. Are there any side effects?

All medicines can have side effects. If you do experience any side effects, most of them are minor and temporary. However, some side effects may need medical attention.

See the information below and, if you need to, ask your doctor or pharmacist if you have any further questions about side effects.

Less serious side effects

Less serious side effects	What to do
Ear, nose or throat related <ul style="list-style-type: none">• runny nose• sore throat• blocked or stuffy nose• throat infection (pharyngitis)• cough	Speak to your doctor if you have any of these less serious side

<ul style="list-style-type: none"> • mouth and throat pain <p>Nervous system related</p> <ul style="list-style-type: none"> • headache <p>Gastrointestinal related</p> <ul style="list-style-type: none"> • vomiting • upset stomach • nausea • diarrhoea (loose stools) • stomach ache • oesophagitis (inflammation of the lining of the food pipe) • pain in the oesophagus • gastritis (inflammation of the lining of the stomach) <p>Blood-test related</p> <ul style="list-style-type: none"> • levels of Phe in blood tests that are too low 	<p>effects and they worry you.</p>
--	---

Serious side effects

Serious side effects	What to do
<p>Allergic reaction related:</p> <ul style="list-style-type: none"> • shortness of breath, wheezing, difficulty breathing or a tight feeling in your chest • swelling of the face, lips, tongue, or other parts of the body • rash, itching, hives or flushed, red skin <p>Gastrointestinal related:</p> <ul style="list-style-type: none"> • symptoms of severe gastritis or oesophagitis, e.g. severe upper stomach area (abdominal) discomfort or pain, nausea and vomiting, blood in your vomit or stool, black or tarry stools, swallowing that is difficult or painful. 	<p>Call your doctor straight away, or go straight to the Emergency Department at your nearest hospital if you notice any of these serious side effects.</p>

Tell your doctor or pharmacist if you notice anything else that may be making you feel unwell.

Other side effects not listed here may occur in some people.

Some side effects may be found only by having a blood test, e.g. levels of Phe in blood tests that are too low.

Reporting side effects

After you have received medical advice for any side effects you experience, you can report side effects to the Therapeutic Goods Administration online at www.tga.gov.au/reporting-problems. By reporting side effects, you can help provide more information on the safety of this medicine.

Always make sure you speak to your doctor or pharmacist before you decide to stop taking any of your medicines.

7. Product details

This medicine is only available with a doctor's prescription.

What Kuvan contains

Active ingredient (main ingredient)	sapropterin dihydrochloride <ul style="list-style-type: none"> • 100 mg in each soluble tablet • 100 mg or 500 mg in each sachet of powder
Other ingredients (inactive ingredients)	<p>Tablets:</p> <ul style="list-style-type: none"> • mannitol • calcium hydrogen phosphate • crospovidone • ascorbic acid • sodium stearyl fumarate • riboflavin <p>Sachets:</p> <ul style="list-style-type: none"> • mannitol • potassium citrate • sucralose • ascorbic acid

Do not take this medicine if you are allergic to any of these ingredients.

What Kuvan looks like

Kuvan soluble tablets are off-white to light yellow with "177" printed on one face. The tablets are supplied in a bottle of 30 tablets with a child-resistant closure. Each bottle contains a small plastic tube of desiccant (silica gel). (AUST R 165738).

Kuvan powder for oral solution is off-white to light yellow. Each carton contains 30 sachets. (100 mg: AUST R 297728, 500 mg: AUST R 297734).

Who distributes Kuvan

BioMarin Pharmaceutical Australia Pty Ltd
119 Willoughby Road
Crows Nest NSW 2065

Kuvan tablets are supplied in New Zealand by:
Pharmacy Retailing (NZ) Limited t/a Healthcare Logistics
58 Richard Pearse Drive
Airport Oaks 2022
Auckland

For enquiries about Kuvan, contact medinfoasia@bmrn.com
To report adverse events, contact drugsafety@bmrn.com
Alternatively, call BioMarin:

Australia: 1800 387 876
New Zealand: 0800 882 012

This leaflet was prepared in August 2021.

® Registered trademark of BioMarin Pharmaceutical Inc.,
USA