

# Sapropterin – Factsheet

April 2022



## What is Sapropterin?

### (Brand name Kuvan)

Sapropterin is an Australian approved non-dietary treatment for PKU, which works for approximately 30%-50% of people with PKU. Sapropterin is a medicine that, in some people with PKU, reduces blood phenylalanine (Phe) levels. This has two impacts from a patient perspective:

Helps to keep Phe level within a safe range and protect the brain from both reversible and irreversible brain damage.

Increases protein tolerance meaning the opportunity for a healthier, more balanced and easier to manage treatment regime.

Sapropterin does not eliminate the need for ongoing dietary management. Your doctor and dietitian will continue to monitor diet and blood Phe levels throughout treatment.

## What are the ingredients in Sapropterin?

Active ingredient: Sapropterin dihydrochloride.

Sapropterin is currently available in tablet (soluble) form and powder sachet form.

## Who is eligible for Sapropterin?

Sapropterin is currently listed on the Pharmaceutical Benefits Scheme (PBS) for patients with PKU aged under 18 years (since 1 May 2019), and for pregnant /maternal PKU women (since 1 July 2021).

## How is responsiveness tested?

The testing protocol can be discussed with your metabolic clinic. Details on current testing protocols in Australian clinics

can be found in the ASIEM Clinical Guideline Document [“BH4 In the Management of Phenylketonuria”](#) .

## When will testing be offered?

If sapropterin is approved for use for all PKU adults the PBAC website indicates that the minimum time frame for the completion of these processes is 5 months. Further information will be provided by MDDA and clinicians once the details around testing timeframes has been determined.

## Additional Resources & Information

MDDA’s position is that **all Australians living with PKU should have access to all treatments that have been approved as safe and effective by the Therapeutic Goods Administration (TGA)**. This means that Australian clinicians should be given the choice of selecting the treatment that best meets the needs of each individual patient and will lead to optimal treatment outcomes – without any limitations or restrictions.

If you would like to be kept informed about the latest updates and next steps as they become known, please ensure your contact details are up to date with MDDA and follow our website and Facebook.

*Australian Public Assessment Report*

<https://www.tga.gov.au/sites/default/files/auspar-kuvan.pdf>

[https://www.healthdirect.gov.au/medicines/brand/amt\\_929907011000036101/kuvan](https://www.healthdirect.gov.au/medicines/brand/amt_929907011000036101/kuvan)

*Consumer Medicine Information*

<https://www.ebs.tga.gov.au/ebs/picmi/picmirepository.nsf/pdf?OpenAgent&id=CP-2016-CMI-01941-1>