**What is Sapropterin?**

Sapropterin is the only Australian approved non-dietary treatment for PKU, which works for approximately **30%-50%** of people with PKU.

Sapropterin is a medicine that, in responsive PKU patients, reduces blood 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 your child’s treatment.

**What are the ingredients in Sapropterin?**

Active ingredient: Sapropterin dihydrochloride.

Sapropterin is currently available in tablet (soluble) form.

**Who is eligible for Sapropterin?**

Children and adolescents less than 18 years of age who have been shown to respond to Sapropterin will be eligible for the PBS subsidy. This will be tested by your clinic using protocols that have been established through agreement between clinical experts and the PBAC. Those children and adolescents who start taking Sapropterin before the age of 18 will be eligible to continue receiving PBS subsidised Sapropterin into adulthood.

**How is responsiveness tested?**

Further information will be provided by MDDA and metabolic clinicians in due course as to how Sapropterin responsiveness testing will be conducted. Further details on current testing best practices can be found in the below mentioned BH4 guideline document – however specific initial testing procedures are yet to be communicated.

**When will testing be offered?**

There are several more steps in the process before Sapropterin is listed on the PBS. 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.

**More Information**

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

**The ASIEM Clinical Guideline Document “BH4 In the Management of Phenylketonuria” can be found** [**here**](https://www.mdda.org.au/wp-content/uploads/2017/10/Australasian-BH4-Guidelines-Document-ASIEM-Endorsed-06.09.2017.pdf)**.**

*MDDA strongly support the PBAC recommendation and while it is well-understood within the PKU community that Sapropterin does not benefit all PKU patients, MDDA will continue to advocate to ensure* ***ALL*** *patients who would benefit from Kuvan are able to access it.*