sapropterin (sa-prop-te-rin)

**Classification**
Therapeutic: antihyperphenylalaninemics
Pharmacologic: synthetic BH4

**Pregnancy Category:** C

**Indications**
To reduce phenylalanine (Phe) levels in patients with hyperphenylalaninemia (HPA) caused by tetrahydrobiopterin (BH4) deficiency due to a genetically impaired enzyme (CAPI TALS indicate l ife-threatening, underlines indicate most frequent. Strikethrough indicates discontinued).

**Action**
Acts as a synthetic form of the cofactor (BH4) for the enzyme phenylalanine hydroxylase (PAH). PAH converts phenylalanine to tyrosine. In PKU patients, activity of PAH is deficient. BH4 helps to activate PAH and thus reduce Phe levels. **Therapeutic Effects:** Preservation of brain function by lowering Phe levels.

**Pharmacokinetics**

**Absorption:** Well absorbed following oral administration; food increases absorption.

**Distribution:** Unknown.

**Metabolism and Excretion:** Unknown.

**Half-life:** 6.7 hr.

**TIME/ACTION PROFILE (effect on Phe levels)**

<table>
<thead>
<tr>
<th>ROUTE</th>
<th>ONSET</th>
<th>PEAK</th>
<th>DURATION</th>
</tr>
</thead>
<tbody>
<tr>
<td>PO</td>
<td>within 24 hr</td>
<td>up to one mo</td>
<td>unknown</td>
</tr>
</tbody>
</table>

**Contraindications/Precautions**

**Contraindicated in:** Hypersensitivity to any component of the drug.

**Use Cautiously in:** Renal or hepatic dysfunction; Concurrent use of levodopa; OB: Use during pregnancy only if clearly needed; Pedi: Safety and effectiveness in children ≤ 3 yrs not established.

**Adverse Reactions/Side Effects**

**CNS:** headache.

**EENT:** pharyngolaryngeal pain.

**GI:** abdominal pain, diarrhea, nausea, vomiting. 

**Hemat:** neutropenia.

**Interactions**

**Drug-Drug:** Concurrent use of medications known to inhibit folate metabolism including methotrexate can decrease BH4 levels; use cautiously. Concurrent use of medications known to affect nitric oxide-mediated vasorelaxation including sildenafil, vardenafil, or tadalafil could increase hypotension. Concurrent use of levodopa may risk cholinergic over-stimulation and instability; use cautiously.

**Route/Dosage**

**PO (Adults):** 10 mg/kg once daily, titrated on the basis of Phe levels (range 5–20 mg/kg/day).

**NURSING IMPLICATIONS**

**Assessment**

- Assess dietary intake and any dietary changes. Provide nutrition counseling. All patients with PKU should maintain a Phe-restricted diet. During dose titration, dietary Phe intake must remain stable to determine effectiveness of sapropterin.

- Monitor for signs of allergic reaction (rash).

- Lab Test Considerations:
- Monitor blood Phe levels after 1 wk of treatment and periodically for up to 3 mos. If blood Phe does not decrease from baseline at 10 mg/kg/day after 1 wk, may increase to 20 mg/kg/day. Patients whose blood Phe does not decrease within 1 mo of treatment with 20 mg/kg/day dosages are considered non-responders and therapy should be discontinued.
- Prolonged elevations of Phe in patients with PKU can result in neurologic damage including mental retardation, microcephaly, delayed speech, seizures, and behavioral abnormalities. Prolonged levels that are too low can cause catabolism and protein breakdown.

**Potential Nursing Diagnoses**

- Imbalanced nutrition: more than body requirements (Indications)
- Deficient knowledge, related to diet and medication regimen (Patient/Family Teaching)

**Implementation**

- PO: Administer with foods to increase absorption. Dissolve tablet in 4–8 oz of water or apple juice and administer within 15 min of dissolution. Tablets may take

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several minutes to dissolve; stirring or crushing tablets may make dissolution faster. Small pieces floating on top of water or apple juice are normal and safe for patients to swallow. If small pieces remain in glass after drinking medicine, add more water or apple juice to make sure complete dose is administered. Protect tablets from moisture, do not remove dessicant packet. Color of tablets may change over time to light yellow; this is normal and tablets are safe. Do not use tab-
lets that have expired.

Patient/Family Teaching

- Instruct patient to take saropterin as directed at the same time each day. Take missed doses as soon as remembered that day; do not take 2 doses in the same day, omit dose if remembered next day. Instruct patient to read the Patient Information guide prior to taking saropterin and with each Rx refill, in case of new information.
- Advise patient to avoid making changes to dietary Phe without consulting health care professional; any dietary changes may affect Phe level.
- Instruct patient to notify health care professional if fever or illness occurs; dose may need to be adjusted.
- Advise patient to consult health care professional prior to taking other Rx, OTC, or herbal products.
- Advise female patients to notify health care professional if pregnancy is planned or suspected or if breast feeding.

Evaluation/Desired Outcomes

- Reduction of Phe levels.

Why was this drug prescribed for your patient?