



Preferred Drug List

NEW DRUG REVIEW

Proprietary Name: Kuvan™

Common Name: Sapropterin dihydrochloride

PDL Category: MISCELLANEOUS

<u>Comparable Products</u>	<u>Preferred Drug List/ Recommended Drug List Status</u>
N/A	

Summary

Indications and Usage: Reduce blood phenylalanine levels in patients with hyperphenylalaninemia due to tetrahydrobiopterin responsive phenylketonuria.

Mechanism of Action: Activates residual phenylalanine hydroxylase enzyme which improves the normal oxidative metabolism of phenylalanine resulting in a decrease of phenylalanine levels.

Dosage Forms: Tablets: 100mg

Recommended Dosage: 10mg/kg/day taken once daily; may be adjusted up to 20mg/kg/day.

Common Adverse Drug Reactions: Headache, diarrhea, abdominal pain, URI, pharyngolaryngeal pain, vomiting, nausea

Contraindications: None listed

Manufacturer: BioMarin Pharmaceutical Inc.

Analysis: Phenylketonuria is a rare genetic disorder that affects approximately 50,000 patients world wide. A deficiency of the enzyme, phenylalanine hydroxylase, causes the disease. Prior to the development of Kuvan™, patients with phenylketonuria had to rely primarily on diet restrictions to keep phenylalanine levels low. Other options were for patients to import tetrahydrobiopterin preparations from European laboratories at an extremely high cost with unknown quality controls. Kuvan™ was granted orphan drug status from the FDA as an investigational small molecule for the treatment of phenylketonuria. BioMarin estimates that 30-50% of the 50,000 affected patients could benefit from use of Kuvan™. There are four published clinical trials evaluating the effectiveness of Kuvan™. One of which was a multicenter, double-blind, placebo controlled study of 88 patients who had previously responded to Kuvan™. Following a wash-out period, patients were randomized to receive placebo or the active drug. After six weeks of therapy, the group that received Kuvan™ recorded an adjusted mean change in blood phenylketonuria from baseline of -239 µmol/L. It is estimated that there are 80 patients in Iowa who are affected with this disorder. Since only a small group of patients who have phenylketonuria respond to therapy with Kuvan™, and its high cost, it is recommended that Kuvan™ be added to the Preferred Drug List as a non-preferred drug.

IME Recommendation:

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| <input type="checkbox"/> Preferred Drug | <input type="checkbox"/> Recommended Drug |
| <input checked="" type="checkbox"/> Non-Preferred Drug | <input type="checkbox"/> Non-Recommended Drug |
| <input type="checkbox"/> Preferred Drug with Conditions | |