BlueCross BlueShield Federal Employee Program

SAPROPTERIN

Kuvan, Javygtor (**sapropterin**)

Preferred product: generic sapropterin

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Prolonged high blood phenylalanine (Phe) levels are neurotoxic and lead to impairment of intelligence and other brain functions (such as attentiveness). Reduction of blood Phe levels through dietary control is an important determinant of long-term neurologic outcome in phenylketonuria (PKU) patients, and reduction of blood Phe levels in patients with PKU has been shown to decrease the long-term risk of neurologic injury. It is difficult for many patients to maintain reduced blood Phe, and many patients with PKU experience some degree of neurological impairment despite efforts to maintain dietary Phe control (1-2).

Response to treatment cannot be pre-determined by laboratory testing (e.g., genetic testing), and can only be determined by a therapeutic trial of sapropterin. Although long-term assessment of neurologic function in patients with PKU receiving sapropterin for the treatment of elevated blood Phe has not been done, sapropterin may help maintain reduced blood Phe levels as an adjunct to a Phe-controlled diet (1-2).

Regulatory Status

FDA-approved indication: Sapropterin is indicated to reduce blood phenylalanine (Phe) levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive phenylketonuria (PKU). Sapropterin is to be used in conjunction with a Phe-restricted diet (1-2).

The most common side effects of sapropterin are headache, vomiting, diarrhea, runny nose, cough, and sore throat. Most of these side effects were mild and did not result in patients stopping sapropterin treatment (1-2).

During clinical trials, gastritis was reported as a serious adverse reaction. Monitor patients for signs and symptoms of gastritis (1-2).

Pediatric patients with PKU, 1 month to 16 years of age, have been treated with sapropterin in clinical studies (1-2).

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Summary

Reduction of blood Phe levels in patients with PKU has been shown to decrease the long-term risk of neurologic injury. In clinical trials of sapropterin in patients with PKU, reductions in blood Phe levels were observed in some patients. Pediatric patients with PKU, 1 month to 16 years of age, have been treated with sapropterin in clinical studies (1-2).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of sapropterin while maintaining optimal therapeutic outcomes.

References

1. Kuvan [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; August 2024.
2. Javygtor [package insert]. Princeton, NJ: Dr. Reddy's Laboratories Inc.; October 2024.