

BC PharmaCare Drug Information

The drug below is being considered for possible coverage under the B.C. PharmaCare program. PharmaCare is a government-funded drug plan that helps British Columbians with the cost of eligible prescription drugs and specific medical supplies. For more information on PharmaCare, visit [Ministry of Health - PharmaCare](#).

PharmaCare reviews each drug for treating a specific illness or medical condition (known as an “indication”). If a decision is made to cover the drug, it will be only for that illness or condition.

In some cases, PharmaCare may cover a drug only for people who have the illness or condition and have not responded to other drugs used to treat that illness or condition.

For more information on PharmaCare’s drug coverage review process, see the last page of this information sheet.

Information about the drug	
Generic name (scientific name)	sapropterin
Brand name	Kuvan[®]
Manufacturer	BioMarin Pharmaceutical (Canada) Inc.
Indication	phenylketonuria (PKU)
Has the drug been reviewed by the Common Drug Review (CDR)? (see the note below this table.)	Yes For more information about the CDR’s review of sapropterin (Kuvan [®]), you can Search the CDR Reports .
Public input start date	Thursday June 23, 2016
Public input closing date	Thursday July 21, 2016 AT MIDNIGHT
How is the drug taken?	Kuvan [®] is taken orally (my mouth).
How often is the drug taken?	Kuvan [®] is taken once a day.

Information about the drug	
General drug and/or drug study information	<p>Patients with PKU do not have enough of the enzyme that is needed to break down a chemical called phenylalanine. The phenylalanine accumulates in the body and affects how the brain develops. Too much phenylalanine can lead to mental retardation, stunted growth and seizures.</p> <p>Sapropterin is a man-made form of the enzyme needed to break down phenylalanine. The drug is used in combination with a low phenylalanine diet. It is not a replacement for the diet.</p> <p>Studies looked at the following outcomes to assess whether the drug is helpful and safe for the treatment of PKU:</p> <ul style="list-style-type: none"> • Phenylalanine (Phe) blood level • Phe responders • Dietary Phe tolerance • Neuropsychiatric and neurocognitive effects using various instruments • Hamilton Depression and Anxiety rating scale (HAM-D and HAM-A) • Behaviour Rating Inventory of Executive Function (BRIEF) • Proportion of patients with a rating of 1 or 2 in the Clinician Global Impression – Improvement (CGI-I) • Bad reactions • Serious bad reactions • Patients leaving the trial due to bad reactions
Other considerations	None

Note:

The Common Drug Review (CDR) is a national organization that reviews drugs on behalf of Canadian public sector plans when manufacturers want to have the jurisdictions provide coverage for the drugs. For detailed information on B.C. PharmaCare’s drug review process, including the role of the CDR in that process, see [The Drug Review Process in B.C. - Overview](#).

Cost of the drug under review compared to other drugs used to treat the same indication			
generic name (Brand Name) of Drug Comparator	PharmaCare Status (if and how the drug is already covered)	Usual Dose	Annual Cost of Therapy
sapropterin (Kuvan [®])	Non-Benefit/Under Review	5 to 20 mg/kg/day	\$12,045 ^a to \$168,630 ^b

^a 11 kg child at 5 mg/kg/day

^b 68 kg person at 20 mg/kg/day

The Drug Review Process in B.C.

A manufacturer submits a request to the Ministry of Health (Ministry).

An independent group called the [Drug Benefit Council \(DBC\)](#) gives advice to the Ministry. The DBC looks at:

- whether the drug is safe and effective
- advice from a national group called the [Common Drug Review \(CDR\)](#)
- what the drug costs and whether it is a good value for the people of B.C.
- ethical considerations involved with covering or not covering the drug
- input from physicians, patients, caregivers, patient groups and drug submission sponsors

The Ministry makes PharmaCare coverage decisions by taking into account:

- the existing PharmaCare policies, programs and resources
- the evidence-informed advice of the DBC
- the drugs already covered by PharmaCare that are used to treat similar medical conditions
- the overall cost of covering the drug

For more information about the B.C. Drug Review Process, visit: [The Drug Review Process in B.C. - Overview](#).

This document is intended for information only.

It does not take the place of advice from a physician or other qualified health care provider.