

BC PharmaCare Drug Information

The drug below is being considered for coverage under the BC PharmaCare program. PharmaCare is a government-funded drug plan that helps B.C. residents with the cost of eligible prescription drugs and medical supplies. For more information about PharmaCare, visit the [PharmaCare website](#).

PharmaCare reviews each drug for treating a specific illness or medical condition (also called an “indication”). If PharmaCare decides to cover a drug, that coverage applies only to the indication(s) specified. In some cases, PharmaCare covers a drug only for people who have not responded to other drugs that treat the same indication.

More information about the PharmaCare drug review process is provided on the last page of this document.

Drug information	
Generic name (scientific name)	sepiapterin
Brand name	Sephience™
Manufacturer	PTC Therapeutics
Indication	For the treatment of hyperphenylalaninemia (HPA) in adult and pediatric patients one month of age and older with sepiapterin-responsive phenylketonuria (PKU). Sephience™ is indicated in conjunction with a phenylalanine (Phe)-restricted diet (PRD).
Has the drug been reviewed by CDA-AMC, or will CDA-AMC be reviewing it? (See note below.)	Yes For more information about the CDA-AMC Reimbursement Review (CRR) of sepiapterin (Sephience™), Search the CDA-AMC Reports .
Public input start date	Wednesday, September 30 , 2026
Public input closing date	Tuesday, October 27 , 2026, at 11:59 pm
How is the drug taken?	sepiapterin is taken orally (through the mouth).
How often is the drug taken?	sepiapterin is taken once a day.

Drug information	
General drug and/or drug study information	HPA is a group of conditions characterized by high levels of phenylalanine (Phe) in the blood. Phe is an amino acid found in many protein-containing foods. If left untreated, Phe can build up in the blood and brain, potentially causing serious problems with brain development, learning, and behaviour. PKU is a rare, inherited metabolic disorder and the most severe form of HPA. PKU is usually identified through newborn screening. Treatment aims to keep Phe within a safe range, and the standard of care is a low-Phe diet. Sepiapterin (Sephience) helps the body break down Phe more effectively by improving the function of the enzyme responsible for processing Phe. It does this by increasing levels of BH4, a naturally occurring substance that helps the enzyme work properly. Together, these effects can reduce the buildup of Phe in the blood. Studies looked at the following: • Phe levels, as measured by: ○ Average change from baseline Phe ○ Percent change from baseline Phe ○ Proportion of patients with baseline Phe ≥ 600 $\mu\text{mol/L}$ who achieved < 600 $\mu\text{mol/L}$ ○ Proportion of patients with baseline Phe ≥ 360 $\mu\text{mol/L}$ who achieved < 360 $\mu\text{mol/L}$ ○ Proportion of patients with baseline Phe ≥ 120 $\mu\text{mol/L}$ who achieved < 120 $\mu\text{mol/L}$ • Quality of life, as measured by: ○ Change from baseline in Phenylketonuria Quality of Life questionnaire (PKU-QOL) response • Bad reactions • Serious bad reactions • Patients leaving the trial due to bad reactions • Bad reactions of special interest: diarrhea, gastroenteritis, vomiting, upper abdominal pain, and nausea
Other considerations	None

Note:

CDA-AMC ([Canada's Drug Agency-L'Agence des Médicaments du Canada](#)) is a national organization that reviews drugs on behalf of Canadian public sector plans when drug manufacturers want those plans to provide coverage for the drug. For detailed information about the PharmaCare drug review process, including the role of the CDA-AMC Reimbursement Review (CRR) in that process, visit [How PharmaCare Decides Which Drugs to Cover](#).

Table of Comparators Used to Treat the Same Indication		
Generic Name (Brand Name) of Drug Comparator	Dosage Form	PharmaCare Status (if and how the drug is already covered)
sepiapterin (Sephience™)	Powder for oral suspension	Under Review
<i>Synthetic BH4 precursor</i>		
Sapropterin dihydrochloride (generic)	Powder for oral suspension	Special Authority

The Drug Review Process in B.C.

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

An independent national organization called [Canada's Drug Agency \(CDA-AMC\)](#) provides evidence-based recommendations to public drug plans across Canada through its reimbursement review process.

As part of the CDA-AMC's Clinical Reimbursement Review process, the Canadian Drug Expert Committee (CDEC) makes reimbursement recommendations for non-oncology pharmaceuticals to the participating federal, provincial, and territorial publicly funded drug plans. In developing its recommendations, the CDEC considers:

- whether the drug is safe and effective
- what the drug costs and whether it is a good value to the citizens of B.C.
- ethical considerations related to covering or not covering the drug
- input from physicians, patients, caregivers, patient groups and drug submission sponsors

The Ministry makes a BC PharmaCare coverage decision by considering:

- existing BC PharmaCare policies, programs and resources
- the evidence-informed advice of the CDA-AMC
- the recommendations and reimbursement conditions of the CDEC
- if a Ministry Initiated review, the advice of an independent expert group called the Drug Benefit Council (DBC)
- BC-specific patient input collected through the Your Voice website
- drugs already covered by BC PharmaCare that treat similar medical conditions
- the overall cost of covering the drug

Visit [BC PharmaCare](#) and [Drug reviews](#) for more information.

This document provides information only.

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