

# 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)	<b>iptacopan</b>
Brand name	<b>TBC</b>
Manufacturer	Novartis Pharmaceuticals Canada Inc.
Indication	Iptacopan is indicated for the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH) who have an inadequate response to, or are intolerant of, a complement 5 (C5) inhibitor.
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 iptacopan (TBC), <a href="#">Search the CDA-AMC Reports</a> .
Public input start date	<b>Monday, December 23, 2024</b>
Public input closing date	<b>Wednesday, February 5, 2025, at 11:59 PM</b>
How is the drug taken?	Iptacopan is taken orally (through the mouth).
How often is the drug taken?	Iptacopan is taken twice per day.

General drug and/or drug study information	<p>Iptacopan is used to treat paroxysmal nocturnal hemoglobinuria (PNH) in patients who have an inadequate response to, or are intolerant of, a C5 inhibitor. PNH is a rare blood disease caused by a genetic mutation in bone marrow stem cells. Bone marrow is responsible for making red blood cells (RBCs), which carry oxygen to tissues in the body, platelets, which help blood to clot, and white blood cells, which fight infections. PNH therefore causes impaired bone marrow function, the destruction of RBCs, and blood clots.</p> <p>When RBCs break apart, the hemoglobin (Hb) inside is released. Hb is the red part of RBCs that carries oxygen around the body. The loss of RBCs and release of Hb causes many of the PNH symptoms, including red urine, anemia, abdominal pain, fatigue, difficulty swallowing, and erectile dysfunction. More serious complications of this disease can include dangerous blood clots, chronic kidney disease, and pulmonary hypertension, which is a type of high blood pressure that affects the arteries in the lungs and the right side of the heart.</p> <p>Iptacopan prevents your body's immune system from destroying your RBCs.</p> <p>Studies looked at the following:</p> <ul style="list-style-type: none"> <li>• Increase and change from baseline Hb levels, including those in the absence of RBC transfusion.</li> <li>• The number of patients who stopped needing blood transfusions (transfusion avoidance)</li> <li>• Change from baseline in lactate dehydrogenase (LDH), as high levels of LDH indicate the breakdown of RBCs.</li> <li>• Occurrences of breakthrough hemolysis (BTH) and major adverse vascular events (MAVEs)</li> <li>• Change from baseline in health-related quality of life (HRQoL) as measured by the European Organisation for Research and Treatment of Cancer Questionnaire (EORTC QLQ-C30).</li> <li>• Change from baseline in symptoms of PNH as measured by the Functional Assessment of Chronic Illness Therapy-Fatigue (FACIT-Fatigue) questionnaire.</li> <li>• Bad reactions</li> <li>• Serious bad reactions</li> <li>• Patients leaving the trial due to bad reactions or death.</li> <li>• Bad reactions of special interest, including infections from encapsulated bacteria, serious or severe infections, PNH hemolysis and thrombosis, testicular effects, thyroid changes, and decreased platelets</li> </ul>
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](#).

<b>Cost of the drug compared to other drugs used to treat the same indication<sup>a</sup></b>				
<b>Generic Name (Brand Name) of Drug Comparator</b>	<b>PharmaCare Status (if and how the drug is already covered)</b>	<b>Dosage Form</b>	<b>Usual Dose</b>	<b>Cost of Therapy</b>
iptacopan (TBC)	Under Review	Capsule	Twice Daily	TBC (to be confirmed)
<b>Current Comparators</b>				
pegcetacoplan (Empaveli)	<a href="#">Exceptional, case-by-case coverage provided through the BC PharmaCare EDRD process</a> for PNH	Single-dose vial for subcutaneous infusion	First four weeks: twice weekly in addition to patient's current dose of eculizumab Subsequent weeks: twice weekly as monotherapy	First Year: \$558,707 <sup>b</sup> Subsequent Years: \$518,655 <sup>b</sup>
danicopan (Voydeya)	Under Review	Tablet	Three Times Daily	\$75,525 to \$100,699 <sup>b</sup>
danicipan + eculizumab				First Year: \$621,350 to \$646,524 <sup>b</sup> Subsequent Years: \$597,986 to \$623,161 <sup>b</sup>

<sup>a</sup> All prices as per PharmaCare formulary, unless otherwise specified. Weight-based dosing assumes a weight of 65 kg; dosing based on body surface area assumes an area of 1.8 m<sup>2</sup>.

Cost of the drug compared to other drugs used to treat the same indication <sup>a</sup>				
Generic Name (Brand Name) of Drug Comparator	PharmaCare Status (if and how the drug is already covered)	Dosage Form	Usual Dose	Cost of Therapy
eculizumab (Soliris)	<a href="#">Exceptional, case-by-case coverage provided through the BC PharmaCare EDRD process</a> for PNH and atypical hemolytic uremic syndrome	Single use vial for intravenous infusion	Loading dose, once per week for four weeks, maintenance dose one week later, then maintenance dose every two weeks thereafter	First Year: \$545,825 Subsequent years: \$522,461

<sup>a</sup> All prices as per PharmaCare formulary, unless otherwise specified. Weight-based dosing assumes a weight of 65 kg; dosing based on body surface area assumes an area of 1.8 m<sup>2</sup>.

<sup>b</sup> Price as per CDA Pharmacoeconomic Review Report for iptacopan (TBC) PNH.

### 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 [Canada's Drug Agency-L'Agence des médicaments du Canada \(CDA-AMC\)](#)
- 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 PharmaCare coverage decisions by taking into account:

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

For more information about the drug review process in B.C., visit: the [How PharmaCare decides which drugs to cover](#).

**This document provides information only.**

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