



Drug Coverage Decision for BC PharmaCare

About PharmaCare

BC PharmaCare is a publicly funded drug plan that helps B.C. residents pay for most prescription drugs and pharmacy services, and some medical devices and supplies.

Details of Drug Reviewed

Drug	cipaglicosidase alfa and miglustat
Brand name	Pombiliti® and Opfolda®
Dosage form(s)	cipaglicosidase alfa is 105 mg/vial powder for intravenous infusion miglustat is a 65 mg capsule for oral administration
Manufacturer	Amicus Therapeutics Canada Inc.
Submission type	New Submission
Indication reviewed	Cipaglicosidase alfa is indicated for use in combination with the enzyme stabilizer miglustat (65 mg miglustat capsule) for the treatment of adult patients with late-onset Pompe disease (acid α -glucosidase [GAA] deficiency) weighing ≥ 40 kg.
Canada's Drug Agency (CDA-AMC) Reimbursement Reviews Process	The Canadian Drug Expert Committee (CDEC) recommended: to Reimburse with clinical criteria and/or conditions . Visit the CDA-AMC website for more details .
Ministry of Health (the Ministry) Review	The Ministry reviewed clinical and pharmacoeconomic combined reports prepared by the CDA-AMC's reimbursement review process, including clinical and pharmacoeconomic evidence review material and the recommendations of the CDEC. The Ministry also considered patient input provided to CDEC and a budget impact assessment. The Ministry received four Your Voice patient input questionnaire responses from patients and patient groups.
Drug Coverage Decision	Exceptional Coverage Through the Expensive Drugs for Rare Diseases (EDRD) Process
Date	August 19, 2026
Reason(s)	Drug coverage decision is consistent with the CDEC recommendation.

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| | <ul style="list-style-type: none">• A phase III, randomized, double-blind multicentre trial demonstrated that treatment with cipaglucosidase alfa and miglustat was as good as alglucosidase alfa (Myozyme®) for a walking distance outcome, and no difference to minimal improvement for a breathing outcome. There was not enough evidence to suggest that Pombiliti and Opfolda provided an advantage over Myozyme in addressing patients' unmet needs. However, the CDEC noted that cipaglucosidase alfa and miglustat offers a potential alternative for patients with late-onset Pompe disease who have developed anti-drug antibodies to other enzyme replacement therapies.• The Ministry participated in the pan-Canadian Pharmaceutical Alliance negotiations with the manufacturer which were able to address the concerns identified by the CDEC with respect to the cost-effectiveness and value for money. |
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The drug review process in B.C.

A manufacturer submits a request to the Ministry of Health (the 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 funding it provides good value
- ethical considerations of 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
- the outcomes of pan-Canadian Pharmaceutical Alliance (pCPA) negotiations with manufacturers

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

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