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	omaveloxolone
Brand name	Skyclarys™
Dosage form(s)	50 mg capsule, oral administration
Manufacturer	Biogen Canada Inc.
Submission type	**New Submission**
Indication reviewed	For the treatment of Friedreich's ataxia in patients 16 years of age and older.
Canada's Drug Agency (CDA-AMC) Reimbursement Review 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 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 66 Your Voice patient input questionnaire responses from patients, caregivers, or patient groups, and responses to clinical questions from 4 specialists. The Ministry also reviewed a recommendation provided from the Drug Benefit Council.
Drug Coverage Decision	**Non-benefit**
Date	July 8, 2026
Reason(s)	• Omaveloxolone demonstrated a small magnitude of benefit compared to placebo. However, the evidence was highly uncertain, specifically in regards to long-term efficacy and the impact of treatment on health-related quality of life.

	• At the submitted price, omaveloxolone was not considered cost-effective for the treatment of Friedreich's ataxia in patients 16 years of age and older. The Ministry participated in the pan-Canadian Pharmaceutical Alliance negotiations with the manufacturer which were not able to address the concerns identified by the CDEC with respect to the cost-effectiveness and value for money. Negotiation was closed without an agreement.

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 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.

This document is intended for information only.
It does not take the place of advice from a physician or other qualified health care provider.

Appendix

CONFIDENTIAL

Drug Benefit Council (DBC) Recommendation and Reasons for Recommendation

FINAL

omaveloxolone (Skyclarys™)
Biogen Canada Inc.

Description:

Drug review of **omaveloxolone (Skyclarys™)** for the following Health Canada-approved indication:

For the treatment of Friedreich's ataxia (FA) in patients 16 years of age and older.

In its review, the DBC considered the Final Recommendation completed by Canada's Drug Agency (CDA-AMC), posted on July 10, 2025, which included clinical and pharmacoeconomic evidence reviews and recommendations from the Canadian Drug Expert Committee (CDEC). The DBC received Your Voice Patient Input Questionnaire responses from one Patient Group, three patients and 62 caregivers. The DBC also considered patient input provided to the CDEC, including input from four patient groups, input by clinical experts consulted by CDA-AMC, and a Budget Impact Assessment (BIA).

Dosage Form:

The recommended dose for omaveloxolone is 150 mg taken orally once a day. Omaveloxolone is available as 50 mg capsules.

Recommendation:

The Drug Benefit Council (DBC) recommends not to list omaveloxolone for the indication under review at the submitted price.

Reasons for the Recommendation:

1. **Summary**

- Results from one phase II, double-blind, randomized, placebo-controlled trial (MOXIe Part 2, N=103) demonstrated that, compared to placebo, treatment with omaveloxolone may have slowed the worsening of neurological function as measured by the modified Friedreich's Ataxia Rating Scale (mFARS) relative to placebo over 48 weeks in patients with FA aged 16 years and older.
- There is no estimated minimal clinically important difference for the mFARS score, therefore, the actual clinical significance of observed changes in mFARS scores throughout the trial is uncertain. However, experts suggested a difference in 2 points as clinically meaningful.

- No statistically significant improvements in upper extremity function, mobility, or fall frequency were reported. Improvements in activities of daily living were observed.
- The impact of omaveloxolone on health-related quality of life (HRQoL) was evaluated as an exploratory end point using the Short Form Health Survey-36 (SF-36). Changes in SF-36 scores from baseline to week 48 were compared with no difference found between the omaveloxolone and placebo groups.
- A price reduction of 97% would be required for omaveloxolone plus standard of care (SoC) to achieve an incremental cost-effectiveness ratio (ICER) of $50,000 per quality-adjusted life year (QALY) gained compared to SoC alone.
- There is a high level of unmet need for patients with FA as no other disease-modifying therapies exist for this disease.

2. Clinical Efficacy

- MOXIe Part 2 consisted of a 48-week placebo-controlled period. Additional long-term information was provided by a three-year open-label extension study.
- MOXIe Part 2 reported study results based on a Full Analysis Set (FAS) that included all patients without severe pes cavus (n = 83). This analysis population was used to conduct the efficacy analyses, even though it did not include all patients who were randomized in the trial. The All Randomized Population (n = 103) included patient with and without severe pes cavus, but this population was analyzed descriptively only.
- The primary end point of MOXIe Part 2 was the change in mFARS at week 48 in patients who were randomized to omaveloxolone compared to patients who were randomized to placebo.
- Secondary end points of MOXIe Part 2 included changes in patient global impression of change (PGIC), clinical global impression of change (CGIC), change in performance in upper extremity function (9-Hole Peg Test), mobility (25-foot timed walk test), frequency of falls, change in peak work during maximal exercise testing and in FA-Activities of Daily Living at week 48.
- Videotaping of normal walking, change in SF-36 and distribution of change in mFARS at week 48 were evaluated as exploratory end points.
- In the FAS population, patients receiving omaveloxolone had a mean change from baseline of -1.45 points (SD 4.209), while those receiving placebo had a mean change of +0.90 points (SD 4.305) at week 48, a mean difference of -2.40 (95% CI: -4.31 to -0.50, p=0.0141).
- Additional information can be found in the CDA-AMC Recommendation.

3. Safety

- 100% of the omaveloxolone group and 100% of the placebo group experienced a treatment-emergent adverse event (TEAE). After initiating omaveloxolone, compared to the placebo group, the omaveloxolone group experienced higher

proportions of nausea, abdominal pain, diarrhea, vomiting, fatigue, muscle spasms, back pain, headaches, oropharyngeal pain, coughs and decreased appetite.
- Serious adverse events (SAEs) were reported in five patients in the omaveloxolone group and three patients in the placebo group during the placebo-controlled period.
- Adverse events (AEs) of interest included increases in alanine aminotransferase (ALT) and aspartate aminotransferase (AST). 19 patients (37.3%) and 11 patients (21.6%) in the omaveloxolone group reported ALT and AST increases, respectively, after initiating omaveloxolone. Only 1 patient in the placebo arm (1.9%) experienced an increase in ALT and another experienced an AST increase.
- There were no deaths reported in either group.

4. Economic Considerations
- At the submitted price, omaveloxolone costs $399,180 per patient annually.
- CDA could not conduct a reanalysis of the BIA to address uncertainty in the market share or drug acquisition costs of omaveloxolone due to the uncertainty of clinical evidence.
- Using the sponsor submitted price for omaveloxolone and publicly listed prices for all other drugs, the ICER for omaveloxolone plus SoC was $1,534,503 per QALY gained compared to SoC alone.
- Omaveloxolone is not cost-effective at the Willingness-to-Pay threshold of $50,000 per QALY for patients aged 16 years and older.
- A price reduction of 97% would be required for omaveloxolone plus SoC to be considered cost-effective at a Willingness-To-Pay threshold of $50,000 per QALY gained versus SoC alone.
- The long-term relative efficacy of omaveloxolone versus SoC is also uncertain as there is no direct comparative data beyond 48 weeks.

5. Of Note
- Input from patient groups and clinical experts underscore the severe burden of FA, the profound impact on HRQoL, and the absence of effective disease-modifying therapies. Current SoC for FA is symptoms and comorbidity management as there are no other pharmacological therapies to treat FA.
- Further price reductions may be required due to the uncertainty in both the clinical evidence and the economic analyses.
- The DBC also noted the lack of improvement in HRQoL outcomes relative to placebo.