

COMPASSIONATE REVIEW POLICY

The Executive officer will consider requests for drugs or indications in the absence of a Committee to Evaluate Drugs (CED) review where there are rare clinical circumstances in immediately life-, limb-, or organ-threatening conditions. Requests must meet the criteria developed for the Compassionate Review Policy as described below.

No compassionate consideration will be given for a CED-reviewed drug or indication where the Executive Officer (EO) has decided not to fund.

In situations where a product has been reviewed by the CED and the ministry is in negotiations with the manufacturer but the EO has not made a final funding decision, requests will be considered on a case by case basis for those individuals who have been urgently hospitalized due to an immediate life-, limb-, or organ threatening complication. The request must be for a drug therapy that is directly related to the clinical condition that has resulted in the hospitalization and the drug in question is under review by the ministry. Interim drug funding will be limited to a maximum 6 month approval and will begin once the patient is discharged from hospital. Further coverage may not be approved once final criteria for funding have been established.

Under the Compassionate Review Policy, the Executive Officer will also consider requests for drugs without a Notice of Compliance (NOC) and Drug Identification Number (DIN) issued by Health Canada if the physician indicates in the request that approval has been obtained through the Health Canada Special Access Program (SAP). Requests must meet the criteria as described below.

Note: For cancer drugs, Cancer Care Ontario (CCO) administers the Case-by-Case Review Program (CBCRP) on behalf of the Ministry of Health and Long-Term Care. The CBCRP extends and adapts the Compassionate Review Policy to both oral therapies and injectable drugs that will be administered in cancer centres and hospitals. Program policies, FAQs, eligibility criteria and how to apply to the CBCRP online are on the CCO website at: www.cancercare.on.ca/cbcrp.

For all requests, the Executive Officer of Ontario Public Drug Programs makes all final funding decisions.

COMPASSIONATE REVIEW POLICY CRITERIA

All of the following criteria must be met for compassionate consideration:

1. DISEASE

- Incidence & Prevalence:
 - o Rare clinical circumstance (i.e., the disease itself is not rare, but the patient's comorbidities, allergies, severity of symptoms, and other factors make the clinical situation rare).
 - i.e., less than 25 similar requests per year are anticipated
- NOTE: Requests for drugs for rare diseases (DRDs) may be referred to the separate DRD review process.

AND

- Severity:
 - o Life-, limb-, or organ-threatening (threat must be immediate); OR
 - o Indirectly life-threatening (threat must be immediate)
 - e.g., a psychiatric condition causing patient to be harmful towards himself/herself or others.
 - e.g., a condition or disease with a significant public health impact.

2. EVIDENCE

- Level of evidence: minimum requirement is case-series or multiple case reports where $n \geq 25$ in total. The requesting physician should provide published evidence to support the request, which may also help expedite the review.
- AND
- Level of effect: evidence of efficacy must be overwhelmingly in favour of the drug. Ideally randomized controlled trial (RCT) data should be available; individual case reports and expert opinion provide the lowest level of evidence and may be considered insufficient data to support compassionate requests.
- AND
- Safety evidence is available demonstrating that potential benefits of treatment outweigh risks.

3. CLINICAL ALTERNATIVES

- Failure of ALL available and appropriate ODB Formulary alternatives.
- AND
- Failure of all appropriate non-pharmacological alternatives.
- AND
- No other clinically appropriate and/or less expensive alternative(s) available that may be considered through the Exceptional Access Program (EAP).

4. FUNDING ALTERNATIVES

- Cannot enroll in a clinical trial.
- AND
- No other funding options [e.g., cannot receive compassionate supply or funding from the drug manufacturer; or not covered through other programs such as Inherited Metabolic Diseases (IMD), Drugs for Rare Diseases (DRD), etc.]
 - o The Compassionate Review Policy is not intended to “bypass” the review process for drugs that would normally be considered under other OPD programs, including DRD and IMD, or under the CED/Cancer Care Ontario (CCO) review process; such requests will be referred to the appropriate program.

5. COST OF REQUESTED DRUG

- Treatment will avoid other health care costs (e.g., hospitalization, physician visits, etc.).
- In cases where the cost impact is significant, a more detailed analysis of cost and benefits may be conducted before a funding decision is made.

6. ASSESSMENT (ALL OF CRITERIA 1-5 MUST BE MET BEFORE ASSESSMENT REVIEW)

- Requests will be sent for opinions from at least three reviewers (external medical experts) and must be recommended for approval by at least 2 of 3 reviewers (where available – for some specialties, only one or two reviewers are available). At least one reviewer must be a specialist with an understanding of the disease condition for which request is being submitted. If recommended for approval by only 1 of 2 reviewers, a third review must be obtained.
- If approval is granted, a short-term trial period will be provided initially; AND there must be objective measure(s) of response provided for consideration of a request for renewal.

7. EXECUTIVE OFFICER REVIEW AND DECISION

- The Executive Officer will review each compassionate request package and make the final decision on funding.

NOTE: All requests will be treated with “rush” priority on the basis of disease severity, but additional time will be required to perform the required literature search, to obtain external reviews, and for the internal review process, including final consideration by the Executive Officer. Therefore, it should be noted that requests being considered under the Compassionate Review Policy will not be processed within the usual timeframe for rush requests. To help expedite requests, the requesting physician is encouraged to provide supporting literature evidence.

The Compassionate Review Policy and criteria are currently under review. Comments or inquiries should be directed to the Executive Officer c/o:

E-mail: EAPFeedback.MOH@ontario.ca
Phone: 416-327-8109 or 1-866-811-9893
Fax: 416-327-7526 or 1-866-811-9908

Ontario Public Drug Programs
3rd Floor, 5700 Yonge St.
North York, ON M2M 4K5