

# British Columbia Cancer Agency | POLICY

<b>Title:</b> Compassionate Access Program	<b>Number:</b> III-45
<b>Effective Date:</b> 1 February 2012	<b>Approved By:</b> Provincial Systemic Program Committee

Page 1 of 8

## RATIONALE

The BCCA Compassionate Access Program (CAP) application process is intended to address the goals of the [BCCA Systemic Therapy Treatment Policy Number III-40](#). It supports flexibility in access to evidence-based treatments that are restricted in funding on the Benefit List or indicated in exceptional clinical circumstances.

## DIRECTIVE

Approval is based on individual review of each case by the Tumour Group and Systemic Therapy Program. CAP would not approve an expensive treatment for a new indication that is anticipated to involve more than 5 patients per year. Such treatment should be submitted to the iTEC or PEC for review. Approvals must be obtained prior to the patient being booked to receive the drug therapy.

The Compassionate Access Program process aims to:

- ensure optimal patient care and safety in the administration of systemic agents
- ensure that drug treatment choices are evidence-based
- ensure fiscal responsibility in utilizing the Life Support Drug Budget
- maintain the integrity of the Provincial Pharmacy drug database

## PROCEDURE

The following scenarios would require CAP applications:

- a. Drug(s) not on the **BCCA Drug Benefit List**
- b. BCCA protocol with the '**U**' designation and eligibility criteria requiring CAP approval (e.g., UGIFOLFOX).
- c. **Changes to an existing BCCA treatment protocol** including:
  - substitution of different drugs or significant revisions to dosing scheduling, unless specified in the protocol
  - use of different doses or routes
  - extensions to number of cycles/duration
  - significant revisions to eligibility criteria/indication, including significant break in the course of treatment
- d. **Class II** drug(s) used under the following circumstances:

# British Columbia Cancer Agency | POLICY

<b>Title:</b> Compassionate Access Program	<b>Number:</b> III-45
<b>Effective Date:</b> 1 February 2012	<b>Approved By:</b> Provincial Systemic Program Committee

**Page 2 of 8**

- as a single agent or in combination with other agents for which no BCCA protocol exists, or
- in a manner that does not fit the specific indications and eligibility listed in BCCA protocols and on the Class II form

e. **Class I** drug(s) being used:

- in combination with radiation for which no BCCA protocol exists
- in any circumstance where there is no information available to ensure a clinical review of the use of the drug(s) for appropriateness and safety

It is up to the prescribing physician to provide literature support for the proposed treatment when a request is made. In the absence of this, the Tumour Group Designate who authorizes the approval of such therapy can provide the reference (see [Roles and Responsibilities of CAP Pharmacist, Tumour Group Designate and PSTP](#)).

## RECURRING APPLICATION

If a non-benefit treatment is approved for more than 5 times a year through the CAP process for a specific indication, the Tumour Group chair or delegate will be requested to develop:

- a formal proposal to be submitted to:
  - the Interim Therapeutic Evaluation Committee (iTEC) for proposals with lower cost and impact on resources (more details in [H:EVERYONE\iTEC](#))
  - or
  - Priority and Evaluation Committee (PEC) if above threshold for iTEC submission
- Treatment Policy, Protocol, Provincial Pre-Printed Order AND Protocol Specific Patient Hand-Out.

The Systemic Therapy Program has the right to decline further funding if this requirement is not fulfilled within a reasonable time frame.

## CLINICAL TRIALS

Principal Investigators of clinical trials that include a study arm involving a treatment that would require CAP approval should seek pre-approval for all trial participants from the Systemic Therapy Program prior to activating the trial, so that the requirement for individual CAP submissions may be waived (see Pharmacy Directive IV-60).

# British Columbia Cancer Agency | POLICY

<b>Title:</b> Compassionate Access Program	<b>Number:</b> III-45
<b>Effective Date:</b> 1 February 2012	<b>Approved By:</b> Provincial Systemic Program Committee

Page 3 of 8

## OUTCOMES

The Systemic Therapy Program will periodically request follow-up data from the requestor or Tumour Groups on the outcomes of CAP treatment, including:

- Further literature/presentation evidence
- Benefits in BC patients given access to the drug/drugs

## CAP PROCEDURES

1. The physician wishing to request funding for a compassionate access indication drug and/or treatment will open a CAP account with the CAP office and submit a request via the online CAP system (<https://cap.phsa.ca/>). All general and specific questions pertaining to CAP must be submitted to [cap\\_bcca@bccancer.bc.ca](mailto:cap_bcca@bccancer.bc.ca). Due to the volume and complexity of CAP requests and enquiries, it will take a minimum of one working day to respond to questions on whether CAP is required for a particular patient's treatment. If a treatment is scheduled within the next three working days and/or the relevant protocols do not provide clarification, submit a CAP request.
2. Approvals must be obtained prior to the patient being scheduled to receive the drug therapy. This minimizes the confusion and stresses for patients and staff that can often occur if treatments are scheduled or delivered in the absence of a finalized outcome.
  - If a patient has been booked and CAP outcome is still pending at 3 pm the day before scheduled treatment, the appointment should be rescheduled until CAP outcome is available.
  - If a patient has been prescribed a take-home medication for which the CAP outcome is pending, the patient should be informed that s/he will be contacted when the CAP outcome is available and, if approved, when the medication is ready to be picked up.
3. CAP request is reviewed by the CAP pharmacist, Tumour Group designate and Systemic Therapy Program designate. CAP maintains anonymity of the reviewers in all written and/or verbal communication with requestors and/or designates to preserve the objectivity of the reviewers who are involved in assessing the requests, and to maintain the integrity of CAP.
4. CAP requests require a minimum of **two** working days to render a decision.

# British Columbia Cancer Agency | POLICY

<b>Title:</b> Compassionate Access Program	<b>Number:</b> III-45
<b>Effective Date:</b> 1 February 2012	<b>Approved By:</b> Provincial Systemic Program Committee

**Page 4 of 8**

- The CAP Office Staff and the Tumour Group/Systemic Therapy Program designates cannot commit to accommodate process-related delays in an expedited manner.
  - If the treatment is considered *clinically* urgent, requestor should mark the request as “Urgent” and detail the reasons in the *Rationale Section* of the request. A decision on urgent requests will usually be obtained within 24 hours.
  - For patients who need to start urgent treatment as inpatients on weekends or statutory holidays, the decision to proceed with the treatment remains within the clinical judgment of the staff and internal processes of the centre/hospital where the treatment is delivered until CAP outcome is available.
5. To maintain the integrity and completeness of the requests as submitted by the requestor/designate, CAP office will not be able to make changes to any requests. All material modifications to a request need to be resubmitted as a new request on the CAP system. Also, it is the responsibility of the requestor to email the CAP office to delete any duplicate request(s).

## **DECISION RENDERED AND APPEALS**

A CAP application can be approved, with or without specific conditions. During an appeal process, a requestor can appeal a denied outcome in the form of a new CAP application by including additional supporting clinical information and/or literature evidence. The reapplication must be marked by the requestor as an appeal of a previous application that was previously denied. The appeal will be reviewed by a different Tumour Group and/or Systemic Program Designate, and the final binding decision will be made by the Provincial Systemic Therapy Program designate.

<b>Title:</b> Compassionate Access Program	<b>Number:</b> III-45
<b>Effective Date:</b> 1 February 2012	<b>Approved By:</b> Provincial Systemic Program Committee

## Appendix. Guiding Principles to CAP Review Process

### **Background and Purpose**

The BC Cancer Agency mandate is to ensure availability and delivery of high quality cancer care to the entire BC population. The majority of systemic therapy is delivered through rigorously developed and regularly updated practice guidelines (protocol-based therapies). CAP, previously known as the Undesignated Indication Drug Request Process, is a secure web-based system by which physicians across the province can request cancer drug therapy for their patients involving treatments not formally implemented but which are sufficiently costly as to require real-time adjudication for ensuring appropriate use. CAP approval is on a case-by-case basis and is intended to support flexibility in access to evidence-based treatments that are unusual, exceptional or occasionally under review for funding decisions. Approximately 75% of CAP requests follow clearly established nearly “automatic” criteria (e.g., FOLFIRI + bevacizumab for first line treatment of metastatic colorectal cancer). The remaining requests require more formal review. Whether the latter treatments are then offered under CAP depends upon their efficacy, safety, and impact upon total resource allocation. The medical appropriateness of a proposed treatment is best adjudicated by an expert medical panel (the Tumour Group, or the TG designate), while the latter, meso-resource allocation, is adjudicated by a Systemic Therapy Program leader.

### Guiding Principles

- Drug treatment choices must be evidence-based; however, proof of efficacy alone does not guarantee CAP approval
- Fiscal responsibility must be used in making decisions, to ensure care of individual patients is balanced by concern for the health of the BC population. Specifically, decisions must support the management of the provincial oncology drug budget and maintain the integrity of the provincial systemic therapy drug database
- The approval/denial process must be fair, consistent and transparent
- The approval process may not circumvent BCCA Systemic Therapy policy to not prescribe nor administer drugs which we do not pay for
- While approval is on a case-by-case basis and technically approval for one case does not apply “automatic” approval for other, similar situations, it should be recognized that precedents are often set by CAP decisions
- It is impossible to come up with rules covering all situations; however, as previously stated, approximately 75% are quite straightforward and/or may be dealt with using

# British Columbia Cancer Agency | POLICY

<b>Title:</b> Compassionate Access Program	<b>Number:</b> III-45
<b>Effective Date:</b> 1 February 2012	<b>Approved By:</b> Provincial Systemic Program Committee

Page 6 of 8

established one-off rules for nearly automatic approval (e.g., FOLFOX for resected node positive colon cancer)

- For non-automatic approvals, the CAP process will remain a two-stage process, with different criteria being applicable at each level. The first level is represented by the Tumour Group, and the second a Provincial Systemic Therapy leader.
- If a request occurs or is expected to occur more than five times in one year for similar situations, further approvals will be suspended and full economic analysis must be carried out, utilizing the standard, formal process
- Decisions will not be made based on the adult patient's age nor social status
- CAP principles will be made publicly accessible on the BCCA website
- There must be an appeal process
- Making the changes recommended will require investment in IT, as multiple documents would need to be changed
- CAP does not address many of the problems related to delivery of drugs supplied by the pharmaceutical agency; these will need to be brought to the Systemic Therapy Committee

## *Step 1: Tumour Group Approval: Process*

The major goal of approval at the Tumour Group level is proof of efficacy, with attention also paid to safety. In general, a drug should have shown efficacy in a phase II (or III) trial to be considered. If the same drug with a different formulation has shown phase II activity in a different disease, and that drug has known phase II activity in that tumour type, the new formulation may be considered effective and approved, if it meets the other criteria listed below (e.g., nab paclitaxel has activity in breast cancer and paclitaxel in prostate cancer; nab paclitaxel may thus be requested in prostate cancer). Drugs that are related to an approved regimen, with strong phase II or greater efficacy data, may be considered if there is an additional reason to request the new drug (e.g. carboplatin for cisplatin in a lung cancer patient with impaired renal function).

Multiple case reports may rarely be considered proof of efficacy, especially in rare tumours for which phase II or greater testing may never be done.

Preclinical data will extremely rarely be considered. The rationale must be extremely strong, to include an oncogenic target hit by that drug (e.g., imatinib/KIT in GIST). Drugs for which there is only preclinical data must have been used in some other oncologic setting, to offer proof of safety.

# British Columbia Cancer Agency | POLICY

<b>Title:</b> Compassionate Access Program	<b>Number:</b> III-45
<b>Effective Date:</b> 1 February 2012	<b>Approved By:</b> Provincial Systemic Program Committee

**Page 7 of 8**

The Tumour Group's decision to approve or deny CAP requests should not be unduly influenced when there is a pending, or completed but not yet published phase III trial. The leader may, however, base his/her decision upon that factor.

Once the efficacy review has been determined to be positive, the Tumour Group must authorize an additional reason for approving a drug. These include but are not limited to:

1. Drug should be significantly less toxic than approved regimen in the proposed patient
2. Drug is significantly more efficacious than the approved drugs (e.g., targeted therapy now available for a disease in which approved chemotherapy has only a minor impact) or best supportive care
3. No approved regimen exists
4. Drugs associated with clinical trials experimental medications may be requested only if the full protocol has been submitted for review and economic assessment of the trial impact has taken place

The steering committee discussed at length the situation in which a patient pays privately for an unfunded drug, responds to the treatment, and funding for additional cycles of the drug is requested through CAP. This was ultimately deemed inappropriate (see Appendix 1).

### *Step 2: Systemic Therapy Leader Approval: Process*

On behalf of the Provincial Systemic Therapy Program, the Director of CAP or his/her designate has the responsibility to consider the efficacy of the requested drug and must balance this against financial and other resource concerns. Requests that are supported by a high level of evidence but which have significant financial impact not included in the current systemic budget may be declined. Decisions should consider benefit to the individual as well as the population.

The exception pertains to pediatric patients < 19 years of age. Most of those patients tend to already have been exposed to experimental therapies, and in general the cost implications are less. Thus, if proof of efficacy and safety is supplied for a pediatric patient, it is highly likely the CAP request will be approved by the leader.

# British Columbia Cancer Agency | POLICY

<b>Title:</b> Compassionate Access Program	<b>Number:</b> III-45
<b>Effective Date:</b> 1 February 2012	<b>Approved By:</b> Provincial Systemic Program Committee

Page 8 of 8

The Leader should keep an eye on the trends of drug use and update both the tumour groups and the systemic program of concerns.

## Appeals Process

In case of disputes regarding the final decision, an appeal may be made to the Provincial Leader, Systemic Therapy Program, whose final decision is binding (see above, under **DECISION RENDERED AND APPEALS**).

### **NOTE**

The steering committee discussed at length the situation in which a patient pays privately for an unfunded drug, responds to the treatment, and funding for additional cycles of the drug is requested through CAP. There was mixed opinion on whether this is an appropriate indication for CAP approval. Some felt that this is a stronger justification of efficacy than phase II results. On the other hand, the majority of the group felt that this violates the principles of fairness, universality, and accessibility, in that those who can pay for arbitrary drugs would do as a test. The community may markedly increase the use of one-offs, hoping for a successful drug that they can then apply to CAP for. This increased use without good safety data could actually endanger patients. Additionally, there would be a large disclosure onus in utilizing this new policy. Every drug with even potential activity would have to be mentioned to the patient, despite the fact that the BC Cancer Agency cannot give that particular drug. The group felt this issue should be explored in greater detail but also finally agreed for now this is not an indication for CAP approval.