

## The BC Cancer Agency Compassionate Access Program (CAP)

### 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 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.

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.

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.

## **APPENDIX 1**

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.