



Guidelines for Expanded Access Protocols

UBC –BCCA REB

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Background

In the stepwise process of new treatment approval in Canada new therapeutic agents may reach a point when safety and efficacy have been demonstrated but the new treatment has still not received approval from Health Canada. Such a new treatment meets the following criteria at that time.

1. The new treatment has been shown to have sufficient effectiveness for a given indication that oncologic specialists with experience treating that condition would consider it a reasonable choice to treat a patient with that condition. Usually this situation arises when no alternative effective safe remedy is available for that condition.
2. The new treatment has been studied sufficiently that its safety profile can be characterized well enough that a subject can understand the risks of taking the new treatment.

At such time as a new treatment meets these criteria one mechanism to obtain a supply of the treatment for a specific patient is to apply through the Special Access Program (SAP) of Health Canada for a supply of the drug or permission to use the new treatment for that individual patient. This is sometimes referred to as “Compassionate Use”. However, Health Canada often wishes to be provided with more information about this use of the new treatment than can be obtained through the reporting associated with use through the SAP. In such cases Health Canada may insist that a company wishing to make the new treatment available in Canada must do so through an Expanded Access Protocol, sometimes referred to as a “treatment use” protocol. Under these circumstances oncologic specialists wishing to obtain a supply of the new treatment for a patient who may be helped by it must activate the Expanded Access Protocol at their center in order to be allowed to receive and dispense the new treatment.

Expanded Access Protocols differ in several ways from standard Phase I to Phase IV clinical trials. Their core purpose is to provide a mechanism for access to a potentially effective new treatment for patients who may be helped by it, have no effective alternative and who wish to receive the new treatment even after being informed of its still incomplete evaluation and its known risks. In addition, because Expanded Access Protocols are being conducted to make a new treatment available rather than to conduct additional evaluations of the treatment’s effectiveness, they allow individual investigators to gather follow-up assessments according to the investigator’s individual practice. Although not as rigorous as the data gathered in standard phase I to IV trials, descriptive information gathered during administration of a new treatment under an Expanded Access Protocol can contribute additional useful information about a new treatment’s effectiveness and safety and provides an opportunity for investigators to gain additional experience administering the new agent.

Guidelines

Although the core intent of an Expanded Access Protocol is to provide access to a potentially useful new treatment such protocols must still conform to current ethical guidelines. The UBC – BCCA REB expects Expanded Access Protocols to conform to the following guidelines.

1. There is no standard treatment for the specific oncologic problem currently available.
2. There is no standard phase II – IV clinical trial currently available to that patient at BCCA that would make the new treatment available.
3. The new treatment has been sufficiently tested to establish that there is a reasonable chance that it will prove helpful for the condition to be treated.
4. The new treatment has been sufficiently tested so that the subject can be reasonably informed of the potential side effects, toxicity and risks of taking it.
5. Conditions of eligibility and ineligibility for participation are stated in the appropriate section of the consent form.
6. Fully informed consent is obtained from the subject. In addition to discussion with the study doctor, informed consent requires a written consent document that adequately describes the intent of the protocol (to provide access to the new treatment and additional experience for the investigators); the voluntary nature of participation in the protocol; the mechanisms that will be in place to protect confidentiality; the nature of the experimental treatment including the time it will take to receive it; the monitoring tests that will be performed; and the potential side effects, toxicity and risks of taking the new treatment. Applicable standard language from UBC – BCCA REB consent templates should be used.
7. Access to a continued supply of the study medication must be assured, if it proves effective and acceptably tolerated.
8. The appropriate Tumor Group or Program must approve the protocol.
9. Monitoring must, at a minimum, include SAEs, time to progression and overall survival.