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Fast Facts

NCI 9671: Exceptional Responders Pilot study: Molecular profiling of tumors from cancer patients who are Exceptional Responders

Exceptional responders: patients who meet the following criteria:

It is recognized that whether or not a response is exceptional depends on an individual patient's tumor, stage, performance status, previous treatment history and perhaps other factors, as well as on the particular regimen the patient received. For this protocol, we give the following guidance as to Exceptional Response. These criteria will be considered minimum criteria.

1. Complete response to a regimen in which complete response is expected in < 10 % of similarly treated patients
2. PR > 6 months in a regimen in which PRs > 6 months are expected in < 10% of patients with similar disease treated with same or similar regimen
3. CR or PR of unusual duration, such that the internal review committee considers it to be an exceptional response. Examples below
   a. PR of duration > 3 x the median expected PR duration (in cases where PR is expected in > 10% of patients with the same disease treated with the same regimen)
   b. CR of duration > 3x the median expected CR duration (in cases where CR may be seen in > 10% of patients with same disease treated with same regimen)
   c. The observed duration of CR (or PR) is longer than expected for 90% of patients with same disease treated with same regimen.

Selection of Patients and Tumors

Eligibility Criteria

1. Documented exceptional response (as defined above). Reports of radiologic scans or other evidence documenting response will be submitted for review. Cases where response is not assessable (e.g. adjuvant treatment) will not be eligible because the outcome cannot be attributed to a specific treatment.

2. Treatment history must be available, for prior treatment and for the drug to which the exceptional response occurred.

3. Patient must meet consent criteria detailed in section VII.2.0. This requires: (i) current ER consent by a living participant not lost to follow-up, (ii) prior consent for future research by a participant not known to be deceased, but lost to follow-up, or (iii) if patient is deceased and did not decline to participate in research at the time of tissue removal for any tissue that would be used in this study, then no consent is required. A flow diagram is provided in Appendix 9.

4. Tumor sample available that meets study requirements (see below).

5. Required tumor samples MUST exist and be able to be submitted. Investigators wishing to submit samples must not have made agreements that would prohibit the free use of data from such samples. The NCI will provide investigators with a letter for the collaborator amending their existing agreement to allow for the case to be submitted.

a) Tumor tissue from prior to administration of the drug to which the exceptional response occurred is required. Ideally this sample will have been collected just prior to treatment, but other prior tissue will be considered. Tissue may be fresh frozen or formalin-fixed paraffin embedded.
b) Tumor tissue amount must be at least a core biopsy, and meet minimum specimen requirements as described below in METHODS (section IV).

6. Encouraged: Normal tissue sample: (optional): Blood or other specimen source for germline sequencing.

7. The tumor samples and clinical data submitted to the Exceptional Responders Database in dbGaP will need to have appropriate agreements in place to allow for the submission. The Exceptional Responders Database can accept clinical data and samples from cases enrolled on a CTEP sponsored clinical trial and cases that were not enrolled on any clinical trial. If the response occurred on a trial that was not CTEP-sponsored, there are existing agreements between the submitting site and the pharmaceutical company. If existing agreements do not allow for the submission of sample and clinical data, the NCI will provide the investigators with a letter that allows the tissue to be used for the Exceptional Responders study if signed by the appropriate collaborator. The letter modifies the existing agreement to include the CTEP IP Option language (See Section VIII) that would allow the case to be submitted to the Exceptional Responders Database. If the existing agreement cannot be modified and the letter cannot be signed, the proposed case will not be accepted.

NOTE: As stated above, the patient does not need to have been enrolled on a clinical trial to be eligible for the exceptional responders study.

Ineligibility Criteria

Cases or tissue meeting these requirements will not be accepted into this study:

1. Patient's response did not meet criteria for an exceptional response
    a) Patient's treatment regimen is expected to lead to CR or durable PR in > 10% of patients.
    b) Patient’s duration of response is not >3x expected median length of response
    c) Response not evaluable or not able to be attributed to systemic treatment (e.g. adjuvant treatment)

2. Patient refused consent for use of tissue for research activities included in the Exceptional Responders study. (see section VII)

3. Tumor sample from prior to the exceptional response is not available, or does not meet quality metrics