PRECISION for medicine

CASE STUDY

A “basket” trial and an accelerated pathway to proof of concept

Study synopsis

Precision for Medicine has been working with a sponsor since 2013 on a trial with an adaptive approach to exploring multiple tumor types concurrently, as an accelerated pathway to proof-of-concept.

Targeted patient: The study targets subjects with ERBB/EGRF–specific mutations. The protocol identifies a list of possible mutations. All patients are required to have genomic assays completed with the mutation identified prior to consideration for enrollment.

Study Design: The initial protocol had 8 different tumor-specific mutation cohorts in a Simon’s 2-stage design. For the first stage, 7 patients are enrolled; if 1 response is observed the cohort expands to stage 2. If no responses are identified, the cohort may be closed, with new tumor types identified. Tumor types are ruled “in” for further exploration (up to the point of accelerated approval or phase 3 investment) or “out” within this trial.

The trial involves patients with specific mutations and/or amplifications that account for 2% to 8% of all solid tumors

Challenges

The primary study challenges are:

- The patient population is limited (2% to 8% of all solid tumors)
- Genomic profiling with molecular pathology is not “standard of care” for all cancers targeted; therefore, lack of insurance coverage makes profiling prohibitive for the general population
- Strict adherence to a complex screening process was essential to ensure that only “driver” mutations approved by the sponsor were identified and enrolled into the trial
- Site study staff experience with the highly technical genomic data can vary

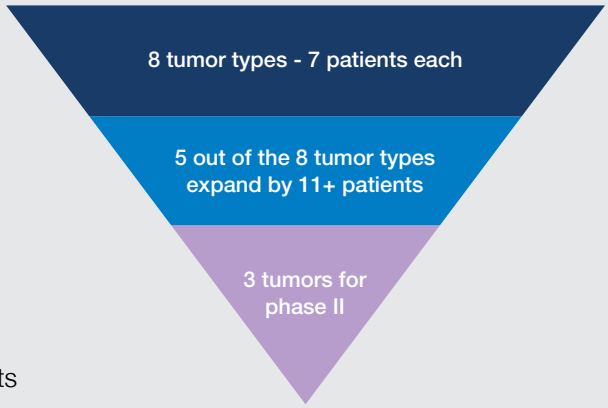
Solutions

In order to have the optimal sites on this study to reach patient enrollment goals, it was critical to recruit sites that have a commitment to personalized medicine and are practiced at conducting genomic assays for the broader patient population through other research studies. To broaden the potential for patient contribution, sites were targeted for participation if they had established sequencing capabilities or partnerships with sequencing laboratories and conducted sequencing within the identified patient populations as part of their routine patient evaluations.

The following tactics were implemented in order to facilitate patient enrollment and continued study engagement at sites:

- A multi-step process was developed for sites to obtain pre-approval for known genomic mutations prior to patient enrollment
- A dedicated and experienced oncology monitor was assigned to each site as a single point of contact to simplify communication for all site management, including on-site and remote monitoring activities
- Weekly news flashes were issued to all sites to ensure ongoing knowledge of enrollment and status of patients
- Relationships with the investigators were fostered to keep sites engaged and motivated to contribute patients across tumor types
- Investigator focus group meetings were held with lead investigators around national congresses to discuss data trends and strategically decide on next steps in the adaptive design

Sample of Multiple Cohort Exploration Based on Genomic Mutation



Results

To date, a total of 20 tumor-specific cohorts have been pursued and 7 treatment approaches have been eliminated. The trial continues to add cohorts and tumor-specific explorations, both as single and combination treatment approaches. The trial started as 100 patients across 6 sites and is now being conducted at ~50 sites globally with 250+ patients enrolled.

This study has focused enrollment at key study locations with the capabilities of and committment to precision medicine; the personalized approach of the Precision team is enabling success at the site level by targeting patient populations and optimizing sponsor investment.

For more information about our clinical trial solutions, please contact us at info@precisionformedicine.com, or visit precisionformedicine.com

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