New clinical trial design promises to accelerate cancer drug approvals

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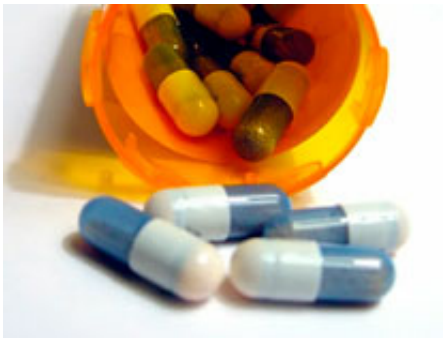

Patients with early-stage breast cancer usually have to wait years to receive new cancer drugs but new guidance from the Food and Drug Administration (FDA) promises to reduce substantially the time and cost of getting new treatments to patients. The approach is based on a trial design being tested in the I-SPY 2 TRIAL, an innovative Phase II breast cancer trial.

University of Colorado Cancer Center is a designated study site for I-SPY 2 (Investigation of Serial Studies to Predict Your Therapeutic Response with Imaging and Molecular Analysis 2). The trial evaluates which medical treatments are most effective for different types of breast tumors.

“New cancer drugs are usually tested first in patients with advanced stage disease and then approved for use in early stage cancer but only after additional clinical trials. It can take hundreds of thousands of dollars and several years to study one drug,” said Anthony Elias, MD, breast cancer program director and associate director for clinical research at CU Cancer Center. “I-SPY 2 shows us that we can find the treatments that work and get them to patients that need them efficiently and safely."

The new FDA recommendations, discussed in the current issue of The New England Journal of Medicine, would speed up approval of drugs tested prior to surgical removal of tumors in certain types of high-risk patients with localized, early stage disease. The guidance centers on neoadjuvant therapy for breast cancer -- the administration of therapeutic agents prior to surgery. The FDA said it may now grant approval to medications that have shown clinical benefit, based on data from patients receiving this type of neoadjuvant treatment whose invasive cancers have disappeared by the time of surgery, termed “pathologic complete response.”

“Better options for patients with high-risk breast cancer are urgently needed," said Janet Woodcock, MD, Director of the Center for Drug Evaluation and Research at FDA. “The FDA guidance explains how a promising drug identified in trials such as I-SPY 2 could be evaluated for FDA approval, so patients could have rapid access if the drug proved better than current treatments."

The I-SPY 2 Trial, led by Laura Esserman, MD, MBA, at the University of California at San Francisco (UCSF) and Donald Berry, MD, at MD Anderson Cancer Center in Houston, uses specific genetic signatures – biomarkers – in the tumors of patients to select those most likely to benefit from testing using the new approaches. The biomarkers are also incorporated into a unique “adaptive” trial design that allows researchers to measure the relative benefit of treating patients with different tumor

profiles with a specific drug and guide treatment assignments for subsequent trial participants.

I-SPY 2 can test new treatments with significantly fewer participants and in half the traditional time, which will dramatically lower costs under the new guidance.

The use of the I-SPY 2 design as a basis for accelerated drug approvals was first discussed in an article in the December issue of the Journal of the American Medical Association co-authored by Esserman and Woodcock.

“We are truly excited to see that the FDA is supportive of trials like I-SPY 2," said Esserman, the co-principal investigator of I-SPY 2. “This really moves us much closer to getting the right drugs to the right patients now, and at a time when they can be cured."

The trial, which was launched two years ago, is screening multiple cancer drugs at 19 major cancer research centers across the country, including CU Cancer Center. Scientists from the National Cancer Institute, FDA, pharmaceutical and biotechnology companies, as well as breast cancer patient advocates also contributed to the design of the trial, which is managed by FNIH and Quantum Leap Healthcare Collaborative with support from Quintiles, a global biopharmaceuticals services provider. Funding for I-SPY 2 is provided by non-profit foundations including The Safeway Foundation, several pharmaceutical companies, and other private sector and philanthropic donors.

FDA is accepting public comment on the new recommendations through July.

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