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A New Clinical Trial Design Promises to Accelerate Cancer Drug Approvals

BETHESDA, MD (June 1, 2012) – The Food and Drug Administration (FDA) has drafted a regulatory guidance describing a new way of conducting breast cancer drug trials that 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 being conducted under the auspices of the Biomarkers Consortium, a public-private partnership led by the Foundation for the National Institutes of Health (FNIH) that includes representatives from NIH, FDA, and multiple pharmaceutical companies and academic research centers.

Patients with early-stage breast cancer have typically had to wait for years to receive new cancer drugs, which are usually tested first in patients with later stage metastatic disease and approved for use in more curable early stage cancer only after additional randomized clinical trials. The draft guidance, which is described in the current issue of the New England Journal of Medicine, establishes a potential new pathway for accelerated approval of drugs tested prior to surgical removal of tumors in certain types of high-risk patients with localized, early-stage disease. FDA signaled it may now grant approval of new drugs that have shown clinical benefit, based on data from patients receiving this type of "neoadjuvant" treatment whose invasive cancer has disappeared by the time of surgery ("pathologic complete response"). I-SPY 2 results, together with an appropriately powered follow-on phase III study in the biomarker populations identified in I-SPY 2, could be sufficient for accelerated regulatory approval of an investigational agent under the new guidance.

"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, which is being led by Laura Esserman, MD, MBA at the University of California at San Francisco (UCSF) and Dr. Donald Berry, MD at MD Anderson Cancer Center in Houston, uses specific genetic signatures – biomarkers – in the tumors of patients to select those who are 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 Drs. Esserman and Woodcock.

“We are truly excited to see that the FDA is supportive of trials like I-SPY 2,’’ said Dr. 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. 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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