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## **THE BIOMARKERS CONSORTIUM LAUNCHES I-SPY 2 BREAST CANCER CLINICAL TRIAL**

### **Highly Anticipated Multi-Agent Trial Opens at Major U.S. Medical Sites**

*Groundbreaking Public-Private Collaboration Combines Personalized Medicine  
and Novel Trial Design to Develop Potentially Life Saving New Breast Cancer Drugs*

BETHESDA, MD – March 17, 2010 – [The Biomarkers Consortium](#), a unique public-private partnership that includes the U.S. Food and Drug Administration (FDA), the National Institutes of Health (NIH), and major pharmaceutical companies, led by the Foundation for the National Institutes of Health (FNIH), today announced the launch of a highly anticipated clinical trial to help screen promising new drugs being developed for women with high risk, fast-growing breast cancers—women for whom an improvement over standard treatment could dramatically change the odds of survival.

[The I-SPY 2 trial](#) will employ a groundbreaking clinical trial model that uses genetic or biological markers (“biomarkers”) from individual patients’ tumors to screen promising new treatments, identifying which treatments are most effective in specific types of patients. In addition, an innovative adaptive trial design will enable researchers to use early data from one set of patients to guide decisions about which treatments might be more useful for patients later in the trial, and eliminate ineffective treatments more quickly.

“I-SPY 2 promises to leverage convergence of progress on a number of research fronts to speed the evaluation of promising new breast cancer drugs using molecular cancer biomarkers to identify those agents that are effective in specific subpopulations of breast cancer patients,” said Anna D. Barker Ph.D., Deputy Director, National Cancer Institute, and Co-Chair of The Biomarkers Consortium Cancer Steering Committee. “This will allow us to finally design advanced, smaller and less expensive Phase III trials that test the right drugs in the right patients.”

The large-scale trial involves a unique collaboration by scientists from the National Cancer Institute (NCI), FDA, and nearly 20 major cancer research centers across the country. Study results will be made broadly available to the entire cancer research and development community.

“The I-SPY 2 trial explores a whole new way to rapidly screen new cancer treatments and match the therapy to specific markers,” said Janet Woodcock, M.D., Director, Center for Drug Evaluation and Research at the U.S. Food and Drug Administration. “Developing individualized medicines needs a solution bigger than any one group can generate. The Biomarkers Consortium is a public-private collaboration of scores of organizations working together to achieve this critical mission. It is a model for the future and FDA is proud to be a founding member.”

I-SPY 2 has the potential to significantly reduce the cost of drug development and speed the process of screening drugs with the goal of bringing safe and effective new drugs to market more efficiently. Currently, it takes over \$1 billion, 12 to 15 years, and thousands of patient volunteers to get a single drug to market. I-SPY 2 was developed to allow the activity of drugs to be assessed much earlier in the research process, potentially enabling drugs to be developed and approved using fewer patients, less time and far fewer resources. The goal is to shave several years and hundreds of millions of dollars off the current process.

The I-SPY 2 trial will focus on treatment in the neoadjuvant therapy setting, in which chemotherapy is given to patients to reduce tumor size before surgery. All patients will receive the current standard of care and most participants will receive one investigational drug. A distinctive feature of the trial is that it will screen multiple drugs from multiple companies—up to 12 different cancer drugs over the course of the trial. In order to do this, FNIH received a master Investigational New Drug (IND) approval from the FDA—which allows the I-SPY 2 TRIAL team to graduate, drop and add drugs seamlessly throughout the course of the trial without having to stop the trial to write a whole new protocol. This will dramatically reduce the time it takes to move from one drug to another in the trial.

Five new investigational agents currently in development by three major pharmaceutical companies have already been selected for testing as part of the first phase of the trial, and will be donated by the companies with each agent representing a different drug class or type of chemical mechanism for attacking cancer. The first agents expected to be tested include:

- **ABT-888 (veliparib)**, a PARP inhibitor being developed by Abbott Laboratories, Abbott Park, IL
- **AMG 655 (conatumumab)**, an APO/TRAIL inhibitor and **AMG 386**, an angiogenesis inhibitor, both under development at Amgen, Thousand Oaks, CA
- **CP-751,871 (figitumumab)**, an IGFR inhibitor and **HKI-272 (neratinib)**, a Pan ErbB inhibitor both under development at Pfizer, Inc., New York, NY

I-SPY 2 will be coordinated by two principal investigators, Laura Esserman, M.D., M.B.A., Professor and Director, Carol Franc Buck Breast Care Center at the University of California, San Francisco (UCSF), and Donald Berry, Ph.D., Professor and Chair, Department of Biostatistics, Division Head, Division of Quantitative Sciences at The University of Texas M.D. Anderson Cancer Center. Clinical operations of the trial will be managed by Angie DeMichele, M.D., M.S.C.E., Associate Professor of Medicine and Epidemiology of the Abramson Cancer Center at the University of Pennsylvania Medical Center. Nola Hylton, Ph.D., Professor of Radiology and Director of the Breast MRI Research Program at UCSF developed new tools to use MRI as a quantitative measure of response to therapy developed in a previous research study, I-SPY 1; these tools will be an integral part of the I-SPY 2 trial and will help validate whether MRI tumor volume change, rather than surgery, can be used as a way of determining patients' response to treatment.

"I-SPY 2 will provide a path to personalized medicine," said Dr. Esserman, a breast cancer surgeon and researcher at UCSF. "The collaborative power behind this trial is truly transformational for breast cancer patients and for cancer research as a whole. We have set up a system where everyone can learn faster and, together, we can dramatically reduce the amount of time and the cost to bring those drugs to market that can make a difference in whether women live or die."

"A considerable advantage for trial participants in I-SPY 2 is that drugs and drug combinations can be given to more patients in the trial as soon as they are proven to be clearly beneficial," added Dr. Berry, who supervised development of the innovative Bayesian adaptive design for I-SPY 2. "By the same token, drugs that are ineffective in the trial can be dropped just as quickly, which increases the safety of the study."

I-SPY 2 is expected to cost approximately \$26 million over five years. Funding will come from a variety of sources, and [Safeway, Inc.](#), one of the largest food and drug retailers in North America, has stepped up as a significant seed funder. The corporation will contribute a sizeable portion of proceeds from the [Safeway Foundation's](#) annual chain-wide October Breast Cancer Awareness fundraising initiative to I-SPY 2. A major foundational investment has also been secured from [Johnson & Johnson](#), and the project is being developed in part with funds from [Genentech](#) and [Lilly](#). FNIH is actively working to raise the remaining funds from pharmaceutical and other companies, non-profit cancer organizations and philanthropic foundations and individuals.

I-SPY 2 has benefited from the unprecedented involvement of dozens of breast cancer advocates in helping to design the trial. The advocates—many of them former patients—have helped create brochures, a website, and DVD to inform patients about the trial. They have worked to ensure that the design of the trial is as convenient for patients as possible.

All results from the trial will be published by the investigators via articles in peer-reviewed scientific journals. The large amount of valuable data expected to be generated by the project will be stored in a database at UCSF and

M.D. Anderson using tools developed as part of the NCI's Cancer Bioinformatics Grid (caBIG) initiative. In order to maximize public health benefit, the non-profit Foundation for the NIH will serve as a trusted third party to manage data and intellectual property arising from the trial.

FNIH will manage the trial as part of The Biomarkers Consortium, a public-private biomedical research partnership that endeavors to develop and qualify biomarkers to speed the development of medicines and therapies for detection, prevention, diagnosis, and treatment of disease and improve patient care. Members of the Consortium include over fifty partners including the NIH, FDA, the Pharmaceutical Research and Manufacturers of America (PhRMA), the Centers for Medicare & Medicaid Services, the Biotechnology Industry Organization (BIO), major pharmaceutical companies, and numerous non-profit medical research organizations.

Up to 20 of the nation's leading cancer centers, including many of NCI's Comprehensive Cancer Centers, will recruit and treat patients as part of the trial. Currently selected centers include:

- [UCSF Helen Diller Family Comprehensive Cancer Center, University of California, San Francisco, CA](#)
- [Abramson Cancer Center, University of Pennsylvania, Philadelphia, PA](#)
- [University of Minnesota Medical Center, Minneapolis, MN](#)
- [Moores UC San Diego Cancer Center, University of California, San Diego, La Jolla, CA](#)
- [The University of Texas M.D. Anderson Cancer Center, Houston, TX](#)
- [University of Colorado Cancer Center, Aurora, CO](#)
- [Mayo Clinic, Scottsdale, AZ](#)
- [Mayo Clinic, Rochester, MN](#)
- [OHSU Knight Cancer Institute, Oregon Health and Science University, Portland, OR](#)
- [Inova Health System, Falls Church, VA](#)
- [The University of Chicago Comprehensive Cancer Center, Chicago, IL](#)
- [The Harold C. Simmons Comprehensive Cancer Center, University of Texas Southwestern Medical Center, Dallas, TX](#)
- [The USC Norris Comprehensive Cancer Center, University of Southern California, Los Angeles, CA](#)
- [Winship Cancer Institute of Emory University, Atlanta, GA](#)
- [The University of Kansas Cancer Center, Kansas City, KS](#)
- [Cardinal Bernardin Cancer Center, Loyola University Chicago Health System, Maywood, IL](#)

Technologies from Agendia (Huntington Beach, CA) and Sentinelle Medical Inc. (Toronto, Canada) will be used to measure biomarkers in the trial.

**For more information:**

[www.ispy2.org](http://www.ispy2.org)

Clinical Pharmacology & Therapeutics 86, 97–100 (1 July 2009);

*I-SPY 2: An Adaptive Breast Cancer Trial Design in the Setting of Neoadjuvant Chemotherapy*; AD Barker , CC Sigman , GJ Kelloff , NM Hylton , DA Berry & LJ Esserman; <http://www.nature.com/clpt/journal/v86/n1/full/clpt200968a.html>

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**About the Foundation for the NIH**

The Foundation was established by the United States Congress to support the mission of the NIH – improving health through scientific discovery. The Foundation identifies and develops opportunities for innovative public-private partnerships involving industry, academia, and the philanthropic community. A non-profit, 501(c)(3) corporation, the Foundation raises private-sector funds for a broad portfolio of unique programs that complement and enhance NIH priorities and activities. The Foundation's Web site address is <http://www.fnih.org>.