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Foundation for the NIH Announces First Results of I-SPY 2 Breast Cancer Clinical Trial

I-SPY 2 Graduates Two Promising Drugs that Show Improved Response for Women with HER2-Positive and Triple-Negative Breast Cancer

Bethesda, MD (December 26, 2013) – The I-SPY 2 TRIAL, a randomized phase II clinical trial for breast cancer launched through a unique partnership with the [Foundation for the National Institutes of Health](#) (FNIH) Biomarkers Consortium and sponsored by [QuantumLeap Healthcare Collaborative](#) has graduated the first two drugs from its innovative, multidrug standing platform trial.

I-SPY 2 data presented recently at the [San Antonio Breast Cancer Symposium](#) shows that when added to standard, pre-surgery chemotherapy, the molecularly targeted experimental drug veliparib ([AbbVie Inc.](#)) in combination with carboplatin improved the rate of tumor response in patients with triple-negative breast cancer – a cancer in which the most successful treatment targets (estrogen, progesterone and HER2 receptors) are not present. Additional data from the trial was announced on December 4th, highlighting another experimental drug, neratinib ([Puma Biotechnology](#)), which has graduated from the trial as well, based on having a high probability of success in Phase III with a signature of HER2-positive/HR-negative disease (positive for HER2 receptor, but negative for estrogen and progesterone receptors).

The [I-SPY 2](#) trial uses a unique adaptive design to match experimental therapies with patients. Genetic or biological markers (“biomarkers”) from individual patients’ tumors are used to screen promising new treatments, identifying which treatments are most effective in specific types of patients. The adaptive trial design enables 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, while minimizing the exposure of patients to treatments that do not benefit them.

Eligible patients enrolled in the trial are randomly assigned to standard neoadjuvant chemotherapy, including paclitaxel, followed by anthracycline-based chemotherapy, or they receive paclitaxel in combination with a novel agent followed by anthracycline-based chemotherapy before surgery. Each woman has a four-to-one chance of being randomized to receive an experimental agent.

“The I-SPY 2 trial is among our most ambitious partnership projects,” said Dr. Maria Freire, FNIH President and Executive Director. “This first success reinforces the collaborative approach spearheaded by the Biomarkers Consortium as a model for research among scientists and the pharmaceutical industry to help speed the development of medicines and therapies and improve patient care.”

The trial’s adaptive statistical design was developed by the overall principal investigators for the I-SPY trial, [Laura J. Esserman, M.D., M.B.A.](#), professor of surgery and radiology and director of the Carol Frank Buck Breast Care Center at UCSF Helen Diller Family Comprehensive Cancer Center in San Francisco, and [Donald A. Berry, Ph.D.](#), professor in the Department of Biostatistics at The University of Texas MD Anderson Cancer Center, and founder of Berry Consultants.

“What’s so exciting about these two I-SPY 2 graduations—especially looking at challenges of triple-negative and HER2 medicine—is that we are making significant inroads into not only understanding the cancers, but providing women with promising new options to standard treatment, much more quickly, that could dramatically change their odds of survival,” said Dr. Esserman.

The I-SPY 2 trial has the ability to stop assigning a therapy to subtypes of patients for whom it is offering no benefit, while at the same time, increasing its use to other subtypes where it is performing very well,” said Dr. Berry. “This has actually happened in the trial. The design is great for patients in the trial, while at the same time accelerating drug development and making it more accurate. A principal goal is to enable small, focused phase III trials in early breast cancer, where therapies are more effective.”

The large-scale trial involves a unique collaboration of scientists from the National Cancer Institute (NCI), FDA, industry, 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.

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. I-SPY 2 was designed to address the problem that historically it has taken over \$1 billion, 12 to 15 years, and thousands of patient volunteers to get a single drug to market. The I-SPY 2 design allows the activity of drugs to be assessed much earlier in the research process, potentially enabling drugs to be developed and approved using fewer patients, potentially shaving several years and hundreds of millions of dollars off the current process..

A distinctive feature of the trial is that it screens multiple drugs from multiple companies—up to 12 different cancer drugs over the course of the trial. This allows the I-SPY 2 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, a process that dramatically reduces the time it takes to move from one drug to another in the trial.

“The I-SPY 2 standing trial is a direct path to personalized medicine,” said Dr. Esserman. “The approach that we’ve developed through QuantumLeap Healthcare Collaborative means that everyone can learn faster together, allowing us to dramatically reduce the amount of time and cost involved with bringing drugs to market. This isn’t just a matter of more efficient R&D, it is critical for women with fast-acting cancers whose lives are on the line until we find tolerable treatments targeted to their specific genetics and disease.”

I-SPY 2, which is now sponsored by QuantumLeap, was launched in 2010 through the Foundation for the National Institutes of Health (FNIH) Biomarkers Consortium. Funding comes from a variety of sources, including pharmaceutical and other companies, non-profit cancer organizations and philanthropic foundations and individuals. [Safeway, Inc.](#), one of the largest food and drug retailers in North America, was a significant and early seed funder. The trial 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 a 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.

Results from the trial will be published by the investigators via articles in peer-reviewed scientific journals. The large amount of valuable biomarker and clinical data to be generated by the project will be stored in a database at UCSF to be used by investigators and by other researchers.

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About the FNIH Biomarkers Consortium

The Biomarkers Consortium is a public-private biomedical research partnership managed by the [Foundation for the National Institutes of Health](#) that endeavors to discover, develop, and seek regulatory approval for biological markers (biomarkers) to speed the development of medicines and therapies for detection, prevention, diagnosis and treatment of disease and improve patient care. For additional information about the Biomarkers Consortium, please visit www.biomarkersconsortium.org.

About QuantumLeap Healthcare Collaborative

QuantumLeap Healthcare Collaborative was established in 2005 as a collaboration between medical researchers at University of California at San Francisco, and Silicon Valley entrepreneurs. QuantumLeap’s mission is to accelerate transfer of high-impact research in clinical processes and systems technology into widespread adoption so that patients and physicians can benefit from the research as soon as practicable. For more information, visit: <http://www.quantumleaphealth.org/>.