Once a new drug or procedure has been clinically tested and approved by the FDA, doctors and patients often rush to use it. "But just because a treatment is new doesn’t mean it’s better," said Cary P. Gross, MD, Associate Professor of Medicine and Director of the COPPER (Cancer Outcomes, Public Policy and Effectiveness Research) Center at Yale Cancer Center. "Even if a treatment is effective for patients enrolled in a clinical trial, sometimes it’s not better for patients outside the research setting. We often don’t know the true risks of these new therapies until they are used in actual clinical practice."

COPPER’s researchers apply scientific skepticism to new medical treatments and technologies being used on patients. "As the practice of medicine enters the digital age, there is increasing interest in analyzing the resulting ‘Big Data’ – large databases that contain important clinical information from thousands or even millions of patients, but have been stripped of patient identifying information. We analyze ‘Big Data’ to determine what happens to cancer patients in actual clinical practice who are receiving these new approaches," Dr. Gross said.

For example, one of the more than 30 papers published last year by COPPER researchers showed that a drug frequently given to breast cancer patients – trastuzumab – increased the risk of heart failure in older women by up to 20 percent when combined with anthracycline, a common chemotherapy. As with many new drugs, the use of trastuzumab climbed steeply, from 2.6 percent of women with breast cancer in 2000 to 22.6 percent in 2007.

So why was the drug being used among increasing numbers of older women even though the risks were unclear? There was little data available to inform decisions. Researchers typically prefer not to complicate cancer trials by including patients who have other medical conditions. Such patients are often older, with problems such as diabetes, heart disease, or kidney damage that can make them more susceptible to complications from new cancer treatments. Yet the treatments get approved on the basis of trials that exclude them. "The patients in research studies often don’t reflect the patients we see in actual clinical practice," Dr. Gross explained.

Dr. Gross was the lead author of another COPPER paper published last year that looked at brachytherapy, an increasingly popular treatment for women with breast cancer. The standard therapy for women with breast cancer is surgery followed by radiation. In brachytherapy, by contrast, radiation is delivered to localized sites in high doses via a catheter. It’s highly targeted, and takes less time. Consequently more and more women have been opting for it.

Dr. Gross and his colleagues looked at data on about 30,000 breast cancer patients nationwide, one year after they received radiation treatment. In some areas of the country up to 70 percent of women got brachytherapy compared to external beam radiation.

"Our study doesn’t close the door on brachytherapy," Dr. Gross said, "but it does raise the importance of looking at outcomes so we can tell if a new therapy really is better and should be widely used."

"The ability of the scientific community to generate new knowledge is dramatically outpacing our ability to test whether these new treatments are effective," Dr. Gross explained. "Once a new treatment appears to be effective in a trial setting, we shouldn’t stop studying it. We need to bridge the gap between research and clinical practice, using ‘Big Data’ to help our patients make educated choices about their care. When patients are making critically important decisions about their health, we need to be able to say ‘data shows that for patients like you – a 75 year old women with stage III breast cancer and a history of disease and diabetes – therapy X tends to work better than therapy Y.’ We don’t have that capability yet, but we are on the cusp of a new era that will get us there in the not-too-distant future."