The Washington Post

To Your Health

A new way to study cancer and its treatments

By Lenny Bernstein June 1

CHICAGO--The National Cancer Institute announced Monday the launch of a nationwide research study that will sort patients into treatment groups based on genetic mutations in their tumors, rather than by cancer type.

The precision medicine study seeks to take advantage of progress in the last decade at identifying molecular abnormalities in cancers to determine whether drugs are more effective when targeted at those changes , rather than at longstanding labels of cancer types.

So, for example, a patient with a kidney tumor might be assigned to a group that is being treated with a drug traditionally used for another form of cancer--if DNA tests showed a likelihood that the drug might work on his tumor's makeup. Researchers believe treatment could be more effective if directed this way.

The project is part of NCI's "precision medicine" efforts and a larger shift in the field toward designing cancer trials that are faster and more efficient and that better match drugs with patients most likely to benefit from them. It could receive additional money from the precision medicine initiative the Obama administration is hoping Congress will fund.

[*Advance in treatment of late-stage melanoma confirmed in large-scale study*]

"We are truly in a paradigm change," said James H. Doroshow, director of the division of cancer treatment and diagnosis at the National Cancer Institute. Now research is asking "when is histology [the microscopic structure of cancers] important, and when isn't it?," he said.

He said at a news briefing Monday that the effort is "the largest and most rigorous precision oncology trial that's ever been attempted." He estimated the current cost, which could change as the project continues over a number of years, at $30 million to $40 million.

The American Society of Clinical Oncology announced the launch of a separate project here Monday that will provide patients with drugs targeted at similar molecular abnormalities and collect the data from oncologists providing their care, to better understand the effectiveness of the treatments. The organization is wrapping up its annual meeting, which drew 35,000 people here.

D. Neil Hayes, an associate professor in the University of North Carolina's School of Medicine who is not part of the National Cancer Institute's project, said it represents the future of cancer research. It no longer makes sense to categorize and treat cancer based on the site in the body where it originates when we know it is a disease of DNA mutations that modern technology allows us to understand, he said.

The National Cancer Institute's project will begin screening patients for eligibility July 1. About 3,000 will be tested at 2,400 sites around the country to find about 1,000 who meet the eligibility criteria. Initially, they will be sorted into approximately 20 treatment "arms" of 30 to 35 patients. Each group will receive a different drug provided by pharmaceutical companies that are part of the effort. Drugs may be added to or dropped from the research as the project continues in coming years.

Some of the drugs already have approval by the Food and Drug Administration. Others are under investigation but have undergone some research into their safety. Officials acknowledge that, as with any trial, some patients are likely to suffer side effects, possibly severe ones.Though they have cancers, the patients must be ambulatory and in relatively good health otherwise.

[*Combo therapies effective in forms of leukemia, lymphoma and breast cancer*]

The project will pay for their biopsies and medication, but the patients and their insurance companies will be responsible for other costs. They will remain in the care of their own doctors. If their disease progresses beyond a certain point, they will be dropped from that study, but may be eligible for tests of other drugs, said Alice Chen, acting head of the early clinical trial development program at the National Cancer Institute.

"If at any point it looks like the cancer is growing and the drug is not controlling the cancer, we take them off the drug," she said.

Hayes cautioned that it can be difficult to find enough subjects for some of the more rare mutations that the project may want to treat and study. Smaller attempts to recruit subjects for the same kind of studies have run into what he called "real world problems," including preserving cells from biopsies.

But even if the numerical goals aren't fully met, Hayes said, researchers will learn valuable lessons about how to conduct this new kind of research and what information can be gleaned from it.

One quarter of the slots in the Molecular Analysis for Therapy Choice (NCI-MATCH) study will be reserved for people with rare cancers, so that the project examines abnormalities found beyond the most common tumors--non-small cell lung cancer, prostate cancer, breast cancer and colon cancer.

Ultimately the success of a drug will be measured by whether it shrinks a tumor 30 percent or more over a certain

time period in 16 percent to 25 percent of the patients receiving that medication. If less than 5 percent see their tumors shrink, the attempt will be considered unsuccessful.

All the data collected will be made public for use in future cancer research, Doroshow said.

Lenny Bernstein writes the To Your Health blog. He started as an editor on the Post's National Desk in 2000 and has worked in Metro and Sports.
