

# Research offers hope for basal cell carcinoma

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(PhysOrg.com) -- People with a genetic condition called basal cell nevus syndrome develop hundreds or even thousands of skin cancers, or basal cell carcinomas, each year. Now Jean Tang, MD, PhD, assistant professor of dermatology, is reporting that a drug produced by Genentech called GDC-0449 significantly slowed or stopped the development of tumors in each of 24 patients who received the drug, and caused existing tumors to shrink in size.

The results were so compelling that the randomized, double-blinded, [placebo](#) controlled trial was stopped early to allow all the patients access to the drug.

Tang is the first author of the phase-2 study of the drug that was presented in abstract form at the opening plenary session of the annual meeting of the American Association for Cancer Research in Orlando, Fla., earlier this month.

“This could be a life-changing result for our patients with a heavy burden of skin tumors,” she said. “These patients have suffered from repeated surgeries every month and their scars have left them with a poor quality of life. This drug prevented almost all of our study participants from undergoing surgery.”

According to the abstract, 12 patients receiving a placebo developed about 1.74 new cancers per month during the study, while 24 patients receiving GDC-0449 developed about 0.07 new cancers each month.

Additionally, the total area of cancer on the patient's body decreased by about 24 square centimeters with GDC-0449 vs. about 3 square centimeter decrease for those receiving placebo.

None of the patients who received GDC-0449 required surgical removal of any [basal cell carcinoma](#) during the course of the study — a significant finding for these patients who can develop many lesions that require surgical removal multiple times a month.

GDC-0449 belongs to a class of drugs that inhibit a biological cascade called the hedgehog signaling pathway. Hedgehog family members play a role in cell division and have been implicated in many types of cancers. A mutation in a hedgehog pathway protein called Patched is responsible for basal cell nevus syndrome, which can be passed from parent to child.

“Many of our patients enrolled in this trial not because of their own disease but for the benefit of their children who have inherited the same mutation,” Tang said. “They and we are hopeful that this drug may someday be used to reverse their genetic disease.”

The senior author of the study, Ervin Epstein, Jr., MD, from Children's Hospital of Oakland Research Institute, presented the results in a press conference at the AACR annual meeting on April 2 and in the opening session on April 3. Epstein is a consultant for Genentech and Novartis. Tang declared no conflicts of interest.

The trial was initiated and designed by the researchers and funded by Genentech.

Provided by Stanford University Medical Center

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