

Novel cancer drug proves safe for leukemia patients

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Results of a Phase I clinical trial showed that a new drug targeting mitochondrial function in human cancer cells was safe and showed some efficacy. The findings, reported by doctors at Wake Forest Baptist Medical Center, are published in the current online edition of the journal *Clinical Cancer Research*.

"This drug is selectively taken up by <u>cancer cells</u> and then shuts down the production of energy in the mitochondria," said Timothy Pardee, M.D., Ph.D., director of leukemia translational research at Wake Forest Baptist and principal investigator of the trial. "This is the first drug to inhibit mitochondria in this way and if it proves effective in further <u>clinical</u> <u>trials</u>, it will open up a whole new approach to fighting cancer."

Mitochrondria, which are the powerhouses of the cell, are used differently by healthy cells and cancer cells. Healthy cells use mitochondria as a source of energy to carry out essential functions, whereas leukemia cells use it to reproduce faster and to repair damage from chemotherapy, Pardee said.

In the Phase I trial, 26 patients with relapsed or refractory hematological malignancies were enrolled and given the new drug, CPI-613, as a two-hour infusion on days one and four for three weeks every 28 days. Of the 21 patients who completed the trial, 29 percent saw a benefit from the drug, including one who achieved a complete remission that has lasted more than three years and another who had a partial response that has lasted more than two years.



These data suggest that agents targeting mitochondrial metabolism can be safely administered and have activity in blood-related cancers like leukemia, the researchers reported.

Based on these findings, a Phase II clinical trial is underway at Wake Forest Baptist.

Provided by Wake Forest University Baptist Medical Center

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