

Clinical trial delivers good results in leukemia patients

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Huntsman Cancer Institute (HCI) researchers Michael Deininger, M.D., Ph.D., and Thomas O'Hare, Ph.D., were part of a team that found a potent oral drug, ponatinib, effective in patients who have developed resistance to standard treatments for chronic myeloid leukemia (CML) and Philadelphia chromosome positive acute lymphoblastic lymphoma (Ph+ ALL). The *New England Journal of Medicine* released results of the trial today.

In the phase I clinical trial conducted at five cancer centers nationwide, ponatinib was highly active in patients with CML and Ph+ ALL who had developed resistance to currently approved [tyrosine kinase inhibitors](#) (TKIs), the standard treatment for CML and Ph+ ALL. Ponatinib is a rationally developed drug, designed in the labs of study sponsor ARIAD Pharmaceuticals, Inc. to address limitations of currently available treatments. Deininger, professor and chief of Hematology and [Hematologic Malignancies](#) at Huntsman Cancer Institute at the University of Utah, and O'Hare, a research associate professor, helped to develop ponatinib, and predicted its efficacy against all known mutant forms of BCR-ABL1, the abnormal protein tyrosine kinase that causes CML.

"Ponatinib is arguably the most potent and broadest BCR-ABL1 available thus far, covering even the T315I mutant, which is completely resistant against all approved TKIs", says Deininger, a senior author on the study who leads an ongoing ponatinib trial at Huntsman Cancer Institute. "The results of this study as well as preliminary data from a

larger phase 2 trial show that ponatinib has remarkable activity in patients with resistant CML and Ph+ ALL, suggesting that this new TKI will expand our therapeutic armamentarium very significantly. It is a poster child for personalized cancer therapy."

Provided by University of Utah Health Sciences

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