

## 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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