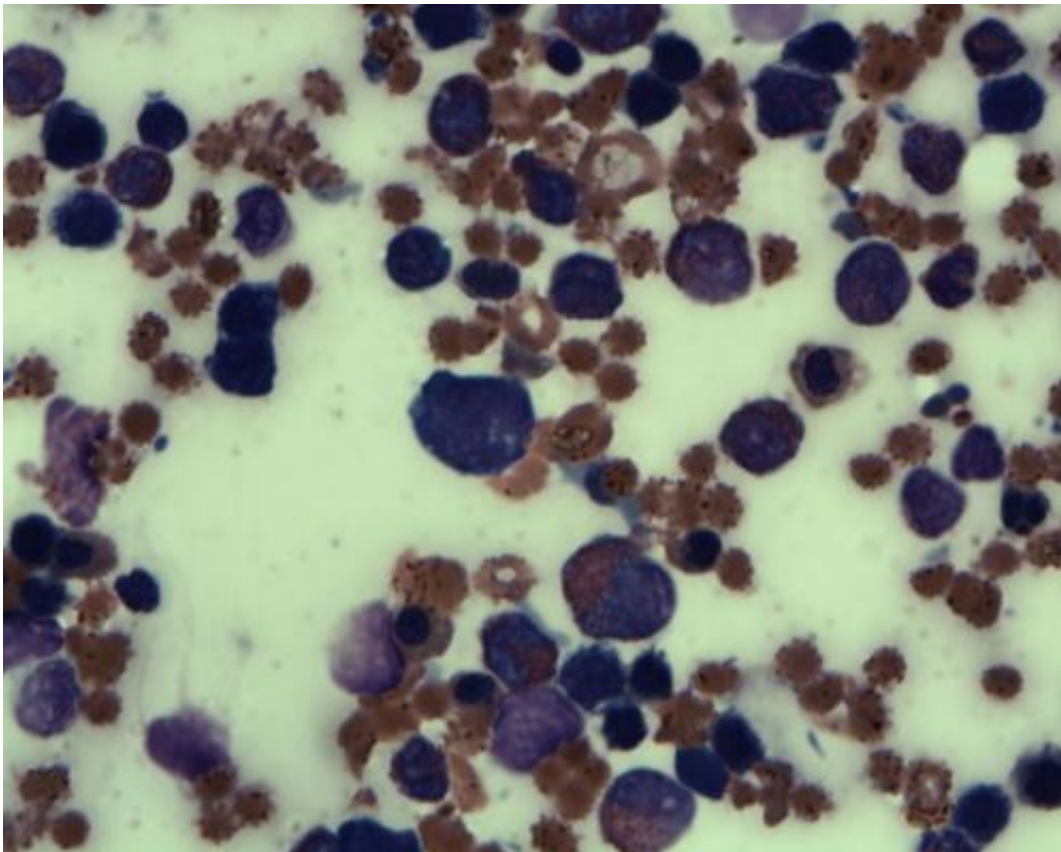


'Dramatic' early phase 1 results for AG-120 in IDH1 mutated AML

November 25 2014, by Garth Sundem



“This is one of the most exciting developments in AML in a long time,” says Daniel Pollyea, MD, MS.

Results presented November 19 by University of Colorado Cancer Center investigator Daniel Pollyea, MD, MS, at the 26th European Organisation for Research and Treatment of Cancer Symposium in

Barcelona show "extremely promising" early phase 1 clinical trial results for the investigational drug AG-120 against the subset of patients with acute myeloid leukemia (AML) harboring mutations in the gene IDH1. The finding builds on phase 1 results of a related drug, AG-221, against IDH2 mutations, presented at the most recent meeting of the American Association for Cancer Research. The IDH1 mutation is found in 15-20 percent of all cases of AML, totaling about 3,500 cases of IDH1 AML per year.

"This is one of the most exciting developments in AML in a long time," Pollyea says. He cautions that results at this stage are preliminary and based on only 17 patients, but says, "Results have been dramatic."

The gene IDH1 is normally involved in regulation of the Krebs cycle, a cellular energy pathway. When IDH1 is mutated it sets off a chain reaction that results in the failure of cells to mature, allowing for the development of AML. In preclinical studies, the [drug](#) AG-120 was shown to block the abnormal function of mutated IDH1, restoring natural development and leading to the orderly death of leukemia cells.

Based on strong phase 1 clinical trial results, the U.S. Food and Drug Administration recently granted Fast Track designation to the sister drug, AG-221, targeting the IDH2 mutation. There is reason to be equally optimistic about AG-120. "This is a high-risk population and we hope that with continued successes we'll be able to offer this therapy to more and more patients participating in this clinical trial," Pollyea said.

Pollyea explains that the clinical trial offered at the University of Colorado Hospital, the primary clinical care partner of the University of Colorado Cancer Center, is mainly for AML patients that have relapsed after previous treatments. "The test for the IDH mutation is done next door by the molecular lab at Children's Hospital Colorado and we now offer the assay as a standard test for [patients](#) diagnosed with AML so

that in the case of relapse we can quickly offer the clinical trial," Pollyea says.

The AG-120 phase 1 clinical trial for IDH1 mutant AML is also offered at Dana-Farber Cancer Institute and Memorial Sloan Kettering Cancer Center, as well as a select number of other academic hospitals.

Provided by University of Colorado Denver

Citation: 'Dramatic' early phase 1 results for AG-120 in IDH1 mutated AML (2014, November 25) retrieved 19 April 2024 from <https://medicalxpress.com/news/2014-11-early-phase-results-ag-idh1.html>

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