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Promising drug a 'new paradigm' for treating leukemia

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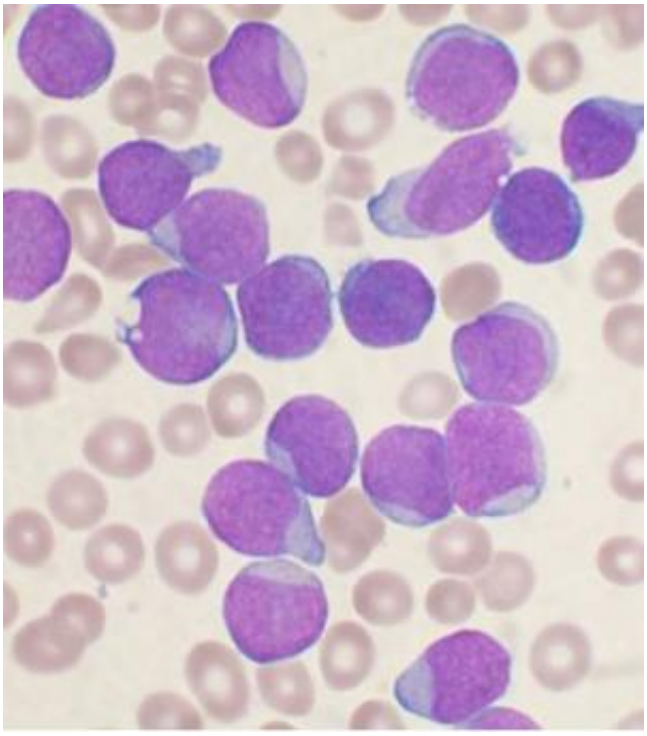

A Wright's stained bone marrow aspirate smear from a patient with precursor B-cell acute lymphoblastic leukemia. Credit: VashiDonsk/Wikipedia

Researchers at the University of Virginia School of Medicine have developed a compound that delays leukemia in mice and effectively kills leukemia cells in human tissue samples, raising hopes that the drug could lead to improved treatments in people. The researchers call it an exciting "new paradigm" for treating leukemia.

The compound works by disabling an altered cellular protein that drives one type of acute myeloid leukemia, the most common form of adult leukemia. By blocking that protein, the drug allows a cancerous cell to detect that it has problems and die, rather than continue to grow and spread. In essence, the compound blocks the cellular machinery that the cancer has highjacked.

"This drug that we've developed is ... targeting a class of proteins that hasn't been targeted for drug development very much in the past. It's really a new paradigm, a new approach to try to treat these diseases," said researcher John H. Bushweller, PhD, of the UVA Department of Molecular Physiology and Biological Physics. "This class of proteins is very important for determining how much of many other proteins are made, so it's a unique way of changing the way the cell behaves."

The drug is notable because of its specificity, killing cancerous cells but not healthy cells. "It's what we call a targeted agent. It hits one specific protein," Bushweller said. "It's not a killer of many other types of cells. As far as we can tell, it only really kills the leukemia cells that have this particular altered protein in them."

In accomplishing that, Bushweller and his team have overcome a major challenge in the effort to develop a new cancer treatment. "When you target a mutated protein in a cancer, you would ideally like to inhibit that mutated form of the protein but not affect the normal form of the protein that's still there," he explained. "In the case of this drug, we've achieved that. We have an inhibitor that turns off the mutated form of the protein but does not affect the so-called wildtype, or normal, form of the protein." As a result, this drug does not show the toxicity and side-effects associated with the traditional chemotherapy drugs used to treat acute myeloid leukemia.

Having shown the effectiveness of the compound in mouse models and

human patient samples, Bushweller must now develop it further so it can be tested in people. To do so, Bushweller could license the drug to a pharmaceutical company, start his own company, or seek further support from the National Institutes of Health.

The drug research has been published in the journal *Science*.

Provided by University of Virginia

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