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Researchers find gene that could lead to new therapies for bone marrow disease

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Van Andel Research Institute (VARI) researchers are one step closer to finding new ways to treat Myelodysplastic Syndrome (MDS), a bone marrow disease that strikes up to 15,000 people each year in the United States, and that sometimes results in acute myeloid leukemia. Researchers found that the gene RhoB is important to the disease's progression and could prove to be a therapeutic target for late-stage MDS.

"Using our genetic models, we've been able to provide a better understanding of underlying molecular defects that drive the malignant progression of MDS," said VARI Distinguished Scientific Investigator Art Alberts, Ph.D., whose laboratory recently published its findings in the journal *PLoS ONE*. "The genes that we've focused on in this study might have a role not only in leukemia, but in solid tumors as well."

Alberts' lab previously reported that the Drf1 gene is crucial to the development of MDS. In its recent study, the lab found that the RhoB gene is important as well; lack of the proteins that are the product of the gene accelerates the disease's progression. The researchers believe examining RhoB levels in samples from patients with advanced MDS could help direct them to better treatment options.

"Our goal is to identify novel therapeutic targets and develop new drugs that affect their activity, but also to find ways to improve upon existing therapeutic strategies that are often associated with deleterious side effects," said Alberts.

"This recent work of Dr. Alberts builds on earlier knowledge from his laboratory yielding important insights into how the body controls the growth of red blood cells," said VARI President and Research Director Dr. Jeffrey Trent. "This breakthrough in our knowledge has the potential to provide us with both new avenues of research, as well as new insights to develop improved treatments for MDS, and other disorders."

In MDS, cells in the bone marrow don't create enough blood cells, and many of the blood cells they do create are abnormal. The body destroys the abnormal blood cells, resulting in low blood counts that can lead to fatigue, impaired immunity, easy bruising and bleeding, and other symptoms. According to the American Cancer Society, 80 to 90 % of all patients with MDS are older than 60 years. In about one third of patients, MDS turns into acute myeloid leukemia, a fast-growing bone marrow cancer.

Lead author of the study Aaron DeWard said that the genetic models that the lab is using are key to translating their discoveries into therapies. "The models allow us to identify and test novel therapeutics and ask more clinically relevant questions," said DeWard.

Source: Van Andel Research Institute