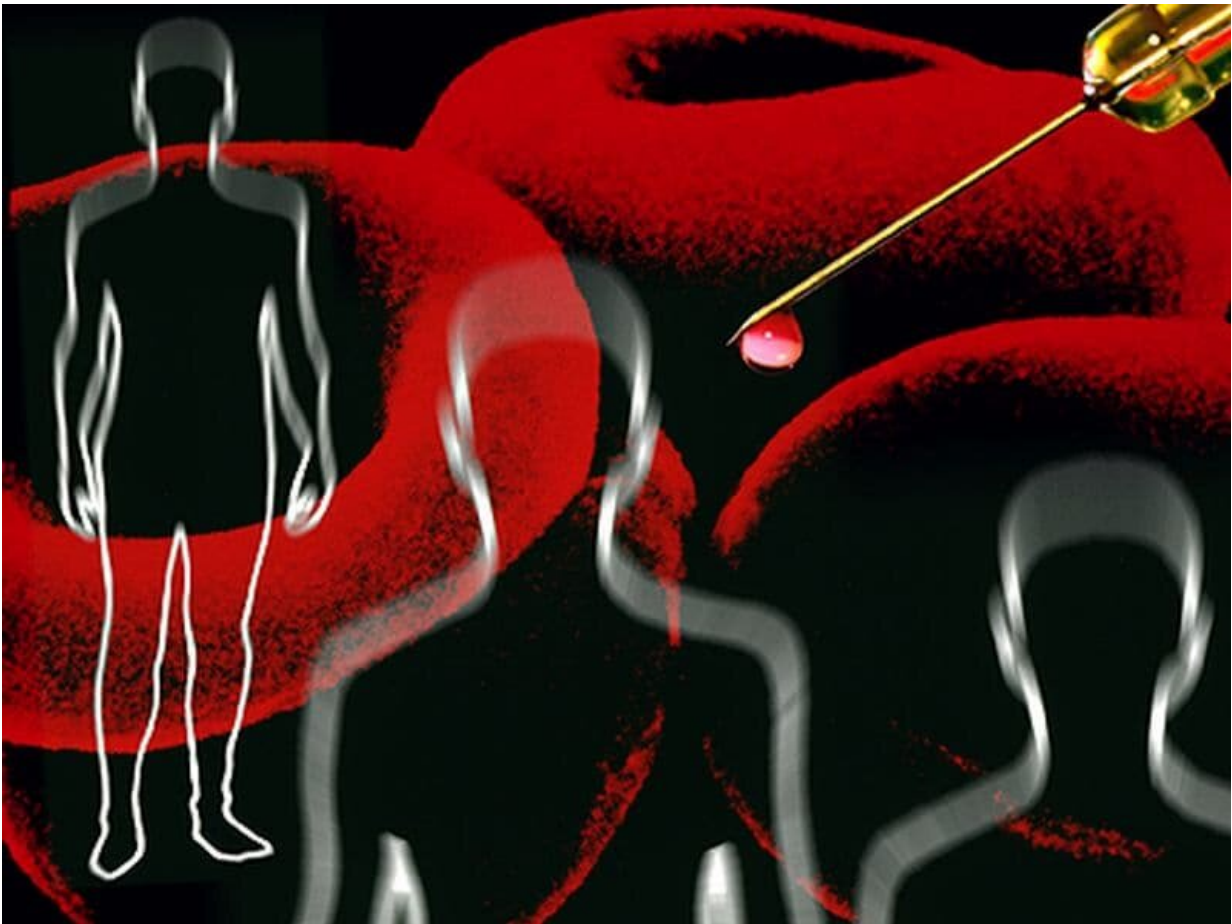


# Gene therapy may help fight tough-to-treat blood cancer

May 2 2019, by Amy Norton, Healthday Reporter

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(HealthDay)—A gene therapy that tweaks the immune system might

offer hope to people with blood cancer that has resisted standard treatments, a new preliminary trial suggests.

The [cancer](#), called multiple [myeloma](#), arises in certain [white blood cells](#). It is currently incurable, but there are treatments that can help people live with the disease for years.

However, most people eventually progress, and some fail to respond to the available therapies at all.

The new study involved 33 patients just like that: They'd typically had seven to eight rounds of various treatments and were out of options. So researchers tried a recently developed approach that harnesses the [immune system](#)'s cancer-killing potential: CAR T-cell therapy.

It involves removing immune system T-cells from a patient, then genetically altering them to be armed with chimeric antigen receptors, or CARs.

That allows the T-cells to recognize and attack cancer cells once they are infused back into the blood, said senior researcher Dr. James Kochenderfer.

CAR T-cell therapy is already approved for certain cases of leukemia and lymphoma—two other types of [blood cancer](#).

But the approach is not one-size-fits-all. CARs have to target a protein specific to the cancer, explained Kochenderfer, a scientist with the U.S. National Cancer Institute.

In this study, his team used CARs that recognize a protein on multiple myeloma cells, called BCMA.

The investigators found that the therapy appeared safe. It caused short-term side effects in all patients, but they were manageable.

There were also early signs that the therapy helped. Most patients—85%—saw their tumors shrink or go away, and they typically went one year before the cancer started to progress again.

"This therapy is totally different than other myeloma therapies," Kochenderfer said. "So myeloma resistant to other therapies might respond to anti-BCMA CAR T-cells."

His team reported the findings in the May 2 issue of the *New England Journal of Medicine*. The trial was funded by Bluebird Bio and Celgene, the companies developing the therapy.

Multiple myeloma is relatively uncommon, based on American Cancer Society statistics: The average person has less than a 1% chance of developing it. But for those who do, the disease is often deadly. Just under half of Americans diagnosed with multiple myeloma die within five years, according to the cancer institute.

Treatment options range from chemotherapy to "targeted" drugs that zero in on certain abnormalities in the cancer, to stem cell transplants.

Some patients respond well to them, said Dr. Melissa Alsina, who heads the multiple myeloma transplant program at Moffitt Cancer Center, in Tampa, Fla. Others, such as those in this trial, do not.

The fact that 85% responded to the CAR T-cell therapy is "impressive," said Alsina, who was not involved in the trial.

She said it's "somewhat disappointing" that the disease typically progressed after one year. But overall, Alsina said, the results are

"exciting," and it seems likely that the therapy will eventually be submitted for approval by the U.S. Food and Drug Administration.

An important question for the future is whether CAR T-cell therapy would be more effective if it's begun earlier in the course of multiple myeloma, Alsina added.

The therapy carries risks. One is cytokine release syndrome—where the body produces a massive amount of inflammatory substances (cytokines) that cause high fever, low blood pressure and other symptoms. Three-quarters of patients in this trial developed cytokine release syndrome—but it was usually mild and able to be treated within a few days.

Neurological issues—like dizziness, confusion and memory problems—are another concern. Forty-two percent of patients had those types of side effects, but they were mild in all cases but one, the researchers said.

Kochenderfer noted that more research is "clearly needed," and the companies developing this particular CAR T-cell therapy are moving it into further trials. His lab at the cancer institute is working on a slightly different CAR T-cell [therapy](#), also targeting BCMA.

For now, Alsina said, multiple myeloma patients can only get the treatment by enrolling in a clinical trial. One way to find out about trials is by contacting the cancer institute.

**More information:** The U.S. National Cancer Institute has more on [CAR T-cell therapy](#).

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