

## Sickle cell gene therapy trial halted after two patients develop cancer, though the link is not certain

A patient who was treated [with gene therapy for sickle cell disease] five and a half years ago has developed myelodysplastic syndrome, a form of cancer that is often a precursor to leukemia, [Bluebird Bio reported](#), while another has developed acute myeloid leukemia.

Concerned by the diagnoses, the National Institutes of Health has [halted a similar trial at Boston's Children's Hospital](#) pending further investigation.

It is not clear whether the cancers are linked to the experimental gene therapy. But the sudden setback is a disappointment to many sickle cell experts and patients, mostly African-Americans, who [had hoped that a cure was on the horizon](#).

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It is not yet clear what caused the cancers. One possibility is that the disabled virus used to deliver the gene therapy treatment damaged crucial DNA in blood-forming cells in the patients' bone marrows. That would be the worst-case scenario, said Dr. John F. Tisdale.

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But there is also the likelihood that both cancers were caused by a powerful drug, busulfan, which is used to clear bone marrow in order to make space for new cells modified by gene therapy. Busulfan is known to confer a blood cancer risk, Dr. Tisdale noted. If it turns out to be the culprit in Bluebird Bio's trials, "We are back to what we know," he said.

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