

Lay Abstract

CML is a lethal leukemia which can be cured by treating the patient with chemotherapy and infusing normal stem cells from a donor (transplant). For patients who do not have a donor, we propose to use cells that can be recovered from the patient's own blood that have been enriched for normal, non-leukemic stem cells by giving the patient chemotherapy before the cells are collected. Since CML may return even after the patient is treated with lethal doses of chemotherapy, we will examine the possibility of introducing a novel gene in the patient's normal marrow which will make it resistant to chemotherapy. This will then make it possible to continue chemotherapy after the transplant. Since a small number of leukemic cells will likely be present in the stem cells used for transplant, the gene will also contain a component that can turn off the leukemia in leukemic stem cells. Patients will therefore receive blood stem cells with this new gene. After transplant, they will be treated with chemotherapy to eliminate leukemia that survived the transplant procedure.