

Hematopoietic cell transplantation for treatment of patients with primary immunodeficiencies and other nonmalignant inherited disorders using low-dose TBI and fludarabine with or without Campath. (2007-FH)

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Synopsis:

This study is to find out if we can transplant blood stem cells in a safer way. Stem cells are “seed cells” necessary to make blood cells. Transplanting blood stem cells (which include blood stem cells taken from the bone marrow, vein, or umbilical cord) is the only way to cure some diseases. In the past most patients were given very high doses of chemotherapy and radiation before the transplant to prevent rejection of the donor stem cells. The high-dose therapy is risky and can cause serious organ damage or death. The high dose therapy is more likely to cause serious problems in some patients. These tend to be patients who are older or who have infections or poor organ function.

There is a newer type of transplant, called a nonmyeloablative transplant. This type of transplant is being studied to see if it is safer. A nonmyeloablative transplant uses lower doses of radiation and chemotherapy before the transplant. These doses are not strong enough to completely destroy your immune system. Other immune suppressing drugs are given after the transplant to help you accept the donor stem cells. These immune suppressing drugs also are given to help prevent graft-versus-host disease. Graft-versus-host disease is a problem that can happen after any type of transplant (high-dose or nonmyeloablative). It is caused by certain donor cells attacking parts of your body.

We have studied nonmyeloablative transplant for treatment of diseases of the blood and immune systems. We found that there was less chance for serious organ damage or death after the transplant. We found that some patients accepted all of the donor cells, whereas other patients accepted part of the donor cells. This mixture of the patient’s own cells with the donor cells is called *mixed-chimerism*. In some patients with mixed chimerism their original disease symptoms went away, but other patients continued to have symptoms. We also found that graft versus host disease was a problem for many patients.

The purpose of this study is to increase the number of donor cells that are accepted by the patient and to decrease the chance for graft versus host disease.