

Bone marrow transplant in womb hailed as success

SUMMARY: A bone marrow transplant within the womb has apparently cured a deadly immune system disorder in a child now 18 months old.

By **DANIEL G. HANEY**
of The Associated Press

BOSTON — In a medical first, a 4-month-old fetus doomed to have a disastrously weak immune system was cured by a bone marrow transplant given while he was still in the womb.

The baby was born healthy after

an otherwise normal pregnancy. At 18 months of age, he shows no signs of his life-threatening inherited disease, known as severe combined immunodeficiency syndrome, or SCIDS.

Doctors said the approach is especially noteworthy because it eliminated the disease before it even began. They hope the same technique can be used to head off some other genetic illnesses, including sickle cell anemia.

The first successful use of the technique in the womb was per-

formed by Alan W. Flake, a pediatric surgeon at Children's Hospital of Philadelphia. It was described in today's issue of the New England Journal of Medicine.

SCIDS is extremely rare, striking about one in every 100,000 babies, most of them boys. The best-known victim was David, Houston's famous "bubble boy."

Victims cannot fight off microbes, and even a minor infection can be fatal. The babies fail to make T cells, a variety of white blood cell that is essential for warding off in-

fection.

In recent years, doctors have attempted to cure these babies by giving them bone marrow transplants shortly after birth. When all goes well, the transplanted marrow produces the blood cells that they lack.

However, about one-third of the transplants fail. David, who spent his life inside a germ-free plastic tent, died in 1984 at age 12 after such an attempt.

Giving the transplant before

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Alan W. Flake says prenatal treatment has several advantages.

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birth is intended to get around many of the problems and hazards of the treatment.

The boy who received the transplant in the womb is the second son of a woman who carried a mutant gene that causes the disorder. Her first son died of it at age 7 months.

Doctors performed genetic testing on the second pregnancy and found this fetus, too, had the bad gene. The family decided to let doctors try to fix the defect before birth. They removed some of the father's bone marrow and injected it into the fetus' abdomen in three treatments one week apart.

Since birth, the boy has caught two colds and recovered from them normally. His bloodstream carries the usual number of T cells.

"By all appearances, he is cured of his disease," Flake said.

Flake said the prenatal treatment has several advantages over waiting until birth to do the transplant:

■ The fetal immune defenses are immature, so the fetus does not reject the transplanted tissue.

■ After birth, doctors have to give chemotherapy to destroy some of the baby's marrow to make room for the transplant. In the fetal stage, this is not necessary.

■ When treated after birth, babies have to be kept in germ-free surroundings for three or four months until the transplanted tissue begins to work. But this too is unnecessary before birth, since the fetus is already in a sterile environment — the womb.

William Shearer, who was David's primary physician at Texas Children's Hospital, called the fetal transplants an important advance. "It's a high-risk procedure, but in competent hands it opens up a new avenue for therapy," he said.