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Stem cells apparently cure boy's fatal disease

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The treatment uses umbilical and marrow cells to help develop normal skin. Doctors say it may move his genetic disorder, recessive epidermolysis bullosa, 'off the incurable list' for other patients.

By Thomas H. Maugh II, Los Angeles Times Staff Writer
June 7, 2008

Using stem cells from umbilical cord blood and bone marrow, researchers have apparently cured a fatal genetic disease in a 2-year-old Minneapolis boy, which could open the door for other stem cell treatments.

For the first time in his life, [Nate Liao](#) is wearing normal clothes, eating food that has not been pureed, and playing with his siblings.

"Nate's quality of life is forever changed," said Dr. John Wagner of the University of Minnesota Medical School, who performed the treatment. "Maybe we can take one more disorder off the incurable list."

The team later treated Nate's 5-year-old brother, Jacob, and is preparing to treat 9-month-old Sarah Rose Mooreland of Folsom, Calif. Hopes are high for them as well.

Nate suffers from recessive epidermolysis bullosa, which affects 1 in 100,000 children. They lack a critical protein called collagen type VII that anchors the skin and lining of the gastrointestinal system to the body.

Their skin is extraordinarily fragile. Tearing and blistering occur with minimal friction, leading to painful wounds and scarring. Solid food produces erosion of the esophagus. Death usually results from malnutrition, infections or aggressive skin cancer.

The only treatment previously has been to wrap the skin in bandages.

The idea of using circulating stem cells to treat the condition was developed by Dr. Angela M. Christiano of Columbia University Medical Center. This is the first time that cells from bone marrow and cord blood have been used to treat a condition that does not involve blood.

Seven months after treatment, Nate's body is making collagen type VII, Wagner said at a news conference Tuesday. His face has plumped up and he has fewer blisters. "I have watched Nate improve every day," said his mother, Theresa.

The results will be published in a future issue of the New England Journal of Medicine.

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