

# Enhanced cord blood stem cell transplants safe in long-term studies

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An innovative experimental treatment for boosting the effectiveness of blood stem-cell transplants with umbilical cord blood has a favorable safety profile in long-term studies. The initial testing made use of zebrafish models. "This is the first time a compound discovered in zebrafish has received a nod from the FDA for a clinical trial," said researcher Wolfram Goessling. File photo by Justin Ide/

(Medical Xpress) -- An innovative experimental treatment for boosting the effectiveness of stem-cell transplants with umbilical cord blood has a favorable safety profile in long-term animal studies, report scientists from Dana-Farber Cancer Institute, Beth Israel Deaconess Medical Center (BIDMC), and Children's Hospital Boston (CHB).

Analysis of long-term safety testing in nonhuman primates, published online by the journal *Cell Stem Cell*, revealed that, after one year following transplant, umbilical cord blood units treated with a signaling

molecule called 16,16-dimethyl PGE2 reconstituted all the normal types of blood cells, and none of the animals receiving treated cord blood units developed cancer. Wolfram Goessling, MD, PhD, of Dana-Farber and Brigham and Women's Hospital, is the first author of the paper, and Trista North, PhD, of BIDMC is the senior author.

The results of long-term safety studies in mice were previously submitted to the [Food and Drug Administration](#) to gain permission for a Phase 1 clinical trial under an Investigational New Drug (IND) application. Principal investigator, Corey Cutler, MD, a Dana-Farber transplant specialist, initiated the trial in 2009 at Dana-Farber and the Massachusetts General Hospital. The IND is sponsored by Fate Therapeutics, Inc. of San Diego.

Goessling and North were post-doctoral fellows in the laboratory of co-author Leonard Zon, MD, a stem cell researcher at CHB and a scientific founder of Fate Therapeutics, when they hit upon 16,16-dimethyl PGE2 while looking for compounds that could regulate the production of [hematopoietic stem cells](#). The initial testing made use of zebra fish models. Goessling commented that "this is the first time a compound discovered in zebra fish has received a nod from the FDA for a clinical trial."

One of the limitations of cord blood as a transplant source is the cells engraft, or "take," in the recipient's bone marrow more slowly than matched donor cells form bone marrow. In addition, there is a higher failure rate for cord blood transplants. Thus there is a need for ways to improve the speed and quality of cord blood transplantation.

Provided by Dana-Farber Cancer Institute

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