# Testing Cord Blood Transplants as a Cure for Leukemia—and HIV

A new clinical trial at several U.S. cancer centers will enroll people living with HIV and advanced leukemia. Here's how to get more info.

March 16, 2022 By Sabin Russell at Fred Hutch News Service

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When the news came out last month that a New York woman was likely cured of HIV/AIDS by the same cord blood transplant she had received to save her from leukemia, Dr. Filippo Milano's telephone at Seattle's Fred Hutchinson Cancer Research Center began to ring.

The unnamed New York patient had been given a transplant in August 2017 using donated umbilical cord blood with genes naturally resistant to HIV, the virus that causes AIDS. The transplant cured her cancer, as anticipated, and more than four years later her doctors at Weill Cornell Medicine are confident it cured her HIV as well.

Although HIV is a treatable condition for those who have access to antiviral drugs, as soon as a person stops that treatment, the virus rebounds. Four-and-a-half years post-transplant, and with no detectable virus 14 months after stopping her drugs, the New York patient appears to be only the third person ever cured of HIV, which has infected 80 million worldwide since 1981.

For Milano, the story was electrifying and well-timed. He was not surprised that doctors and potential patients began calling him.

As director of the Hutch's Cord Blood Transplant Program, he also had been working for years to launch a clinical trial of a similar approach that might cure patients of both leukemia and HIV.

The trial had only recently cleared its final authorizations. It is to begin enrolling this month.

"The first thing we will tell the patient is that the transplant is to cure your cancer, not your HIV. But while we are curing your cancer with the transplant, it might be possible to cure your HIV," said Milano. The trial will be conducted at Fred Hutch and at least three other medical centers across the country.

With funding from the National Heart, Lung, and Blood Institute, this Phase 2 trial aims to enroll 10 patients with leukemia who are also HIV-positive. As was done in New York, they will receive, as a treatment for their cancer, transplanted stem cells from cord blood. Because that cord blood in

this trial will come from donors with rare genes that confer resistance to HIV, the hope is that the procedure will give them immunity against HIV as well.

Participants in the trial also get a second infusion of cord blood-derived cells meant to give patients a burst of protection against infection during several vulnerable weeks before the transplanted stem cells engraft and rebuild a fully functioning immune system.

The New York patient also received a bridge of infection-protection prior to engraftment, but in her case, it was an infusion of immune cells donated by a close relative — a so-called haploidentical transplant.

“In New York they used two different stem cell sources, but the idea behind it is just like what we have in our clinical trial,” Milano said.

Conventional bone marrow or blood stem cell transplantation is a complex and risky procedure for people facing death from blood cancers. It swaps out a patient’s diseased blood-forming cells with healthy replacements from a donor. First, the patient’s own immune system is destroyed with radiation and chemotherapy drugs, then a unit of blood-forming cells from the donor is infused. If these transplanted cells take root, or engraft in the patient, the person has a chance to live cancer-free.

Transplants require that donor and recipient carry closely matching tissue types, which are inherited traits — proteins that dot your cells like flags or family crests proclaiming allegiance to you. Otherwise, the donated immune cells will encounter the patient’s cells and healthy tissues, assume they are foreign, and attack them. Such transplants are likely to fail, unable to engraft a replacement immune system.

While siblings are often good matches, 70% of the time patients need to access registries of potential donors in hopes of finding a more compatible tissue type. Compatibility is linked to similarities in the genetic heritage of both donor and recipient, which traces back to the ancient, geographic origins of their ancestors. So, tissue matches are more likely to be found among people of similar races and ethnicities. Because most donors signed up in bone marrow registries are white, this poses a serious barrier to transplantation for racial and ethnic minorities in the U.S.

Be the Match, which operates the world’s most ethnically diverse bone marrow registry, found that a white person has a 79% chance of finding a compatible donor, while the chances for a Black person are only 29%.

Concerned about patients who cannot find a suitable donor, cancer physicians including Milano developed umbilical cord blood transplantation as an alternative. Cord blood produces immature immune cells that do not require the kind of tissue-type matching required of conventional transplants. It can be a lifesaver for patients who have difficulty finding a close match in donor registries, particularly for people of color.

Milano and the Hutch program team perform more than 30 transplants per year — about 700 since

the program launched in 2006. Nearly two-thirds of their patients came from minority communities. While transplants are difficult, desperate options for patients at risk of dying, more than 70% of those patients survived the procedure.

“That means over 300 people belonging to minorities have been cured because of cord blood,” he said. “I think that is outstanding.”

He is hopeful that, if transplants can cure both leukemia and HIV in patients who have both diseases, the use of cord blood will assure that option is available to patients of diverse ethnic backgrounds who have both HIV and leukemia. That was the case with the New York patient, who was described by her physician as a person of “mixed race.”

To find compatible donors for the new trial, Milano is collaborating with the Cleveland Cord Blood Center to screen donated umbilical cords for the rare HIV resistance traits. The center stores blood from those donations, collected from all over the country. Milano estimates about one million cord blood units are available worldwide in public banks, and about 1% of those could contain the needed mutations.

## From transplant to gene editing, the long road to elusive HIV cures

Milano had been researching the possibility of infusing cord blood carrying HIV resistance traits as early as 2016, when there was only one patient in the world who had been cured of HIV/AIDS: Timothy Ray Brown, a Seattle native who was living in Berlin when he developed acute myeloid leukemia.

In 2006, Brown knew that to survive his leukemia he would need a transplant. However, his German physician, Dr. Gero Hütter, thought he might also be able to control Brown’s HIV if the transplant came from a donor who was naturally resistant to the virus. What if he transplanted stem cells lacking a cell-surface receptor known as CCR5, which HIV uses like a trap door to slip into cells?

For this trait to block HIV, the donor would have had to have inherited from both parents a rare mutation known as a CCR5 delta-32 deletion. Only about 1% of northern Europeans (Brown’s ancestry) carry two copies of the flawed gene, but Hütter found a donor.

Brown had his transplant in February 2007, and when his leukemia recurred in 2008, he needed a second one from the same donor. The second transplant was so traumatic he barely survived. Having never restarted his antiviral medications, yet remaining HIV-negative, in 2010 his apparent cure was announced. At the time he remained anonymous, known only as the “Berlin Patient.”

While his HIV never returned, Brown died on Sept. 29, 2020, due to a relapse of leukemia. He was 54.

For most of his years after the transplant, Brown was the only person in the world known to have

been cured of HIV. But he lived long enough to know he was not alone. On March 4, 2019, a second man, Adam Castillejo — then known only as the "London Patient" — was declared cured after a similar transplant.

Fred Hutch transplant physician Dr. Hans-Peter Kiem, who is co-principal investigator for defeatHIV, a Hutch-based research coalition dedicated to finding an HIV cure, has worked closely with Milano in developing the new cord blood trial. He was encouraged by the success of the New York patient.

"That study is very important, because it supports the basic concept that HIV can be controlled or cured with protected immune cells," Kiem said.

He noted that these transplants, however exciting, can only be provided to patients who need them to survive a lethal cancer. That is because transplants themselves remain "highly risky, and very hard for patients to endure."

But the research that has now protected three patients from HIV is informing a greater effort to engineer T cells (key cancer- and infection-fighting blood components of the immune system) with HIV-blocking traits. Kiem's dream is to develop a single injection that could modify a patient's own cells to make them resistant to HIV, which could potentially be used in patients in low- or middle-income countries.

Milano was preparing to launch the cord blood trial as far back as 2019, but the project encountered multiple delays — most recently a two-year setback due to COVID-19's chilling effect on many clinical studies.

The launch had been delayed earlier by difficulties with a frozen cord blood product that was to serve as the protective "bridge" to engraftment. His newly authorized clinical trial will be using a reformulated version of that product, which is made of cells processed from multiple cord blood donors. The product contains cells that fight off infections for several weeks and then die, without trying to rebuild an array of T cells and B cells in the patient's new immune system. That reconstitution is the job of the transplanted cord blood cells, carrying their secret cargo of HIV-resistance traits.

Patients participating in the study will be randomly assigned to different doses of radiation and chemotherapy — the rigorous "conditioning" performed on the patient to wipe out the leukemia and perhaps destroy cells harboring HIV — prior to the infusion of the cord blood.

Patient participants will continue taking antiviral drugs after their transplants, and their health will be closely monitored by their hospitals. The transplant recipients will regularly give blood samples so doctors can track their leukemia and check for reservoirs of HIV.

While the conditioning regimes may be sufficient to wipe out lingering reservoirs of HIV, that alone has not worked in other efforts to replicate Brown's experience. Doctors suspect that, just as the engrafted cells control leukemia by detecting and killing blood cells that are malignant, the

transplanted immune cells resistant to HIV may control further outbreaks by finding and attacking cells harboring latent virus.

Drs. Timothy Henrich, at the University of California at San Francisco, and Rafick-Pierre Sékaly, of Emory University, in Atlanta, will perform sophisticated analyses of participant blood specimens to determine when, if ever, individual patients can stop taking their HIV medication and to detect any early return of the virus, should that occur.

Note: Scientists at Fred Hutch played a role in developing these discoveries, and Fred Hutch and certain of its scientists may benefit financially from this work in the future.

To learn more about the recent HIV cure case in the woman who had leukemia, see the POZ article "New York Woman May Be Cured of HIV After Stem Cell Transplant."

Patients who have HIV and are in need of a transplant for leukemia and would like more information about the trial may email principal investigator Dr. Filippo Milano at fmilano@fredhutch.org.

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