

Canadian cord-blood procedure passes key milestone in cancer trial

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Anne Marinier and Guy Sauvageau confer at their research laboratory at the University of Montreal on Tuesday, June 26, 2018. Dr. Marinier and Dr. Sauvageau discovered the molecule that has led to new technology used in treating cancer patients.
DARIO AYALA/THE GLOBE AND MAIL

Researchers behind a made-in-Canada technology for multiplying the number of stem cells that can be derived from donated cord blood say they are ready to move on to the next phase in their effort to prove the technology can save lives.

Last week, the 25th and final patient to be admitted into a clinical trial of the technology was given a transfusion of stem cells derived from cord blood. The stem cells are used to regenerate the body's capacity to make healthy blood cells in patients with severe forms of blood cancer including acute leukemia.

Stem cells harvested from the umbilical cord blood of newborns are often favoured for transplants into adults because they can be more easily matched to recipients with less chance of complications. The problem is that there are often too few stem cells in a single cord-blood donation to help an adult patient.

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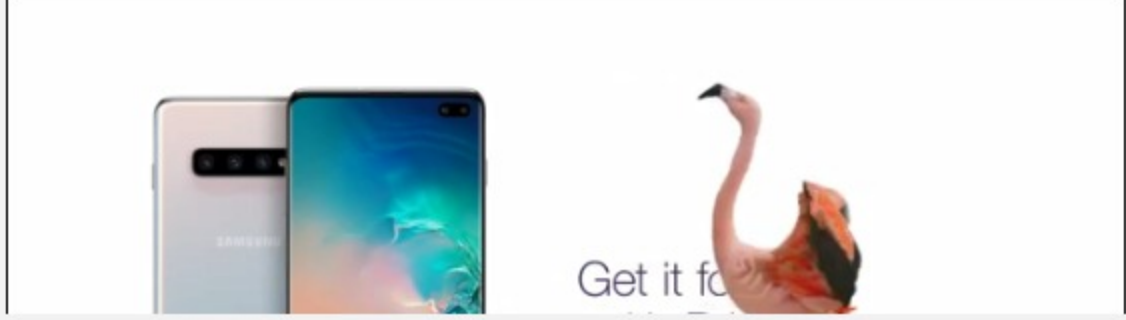
"You get what you get. You can't call up the baby and say 'I want more cells,'" said Sandra Cohen, a haematologist at the Maisonneuve-Rosemont Hospital in Montreal who is leading the trial.

But researchers have found a way to increase the stem cells from a single unit of cord blood by 35 times in a seven-day period. It's this "expanded" form of cord blood that is now being tested in the Phase I/II clinical trial.

While those who participated in the trial will need to be monitored for a total of three years, researchers say they are already encouraged by the results that suggest that the cells are working and that in many cases recovery is accelerated compared with conventional cord transplants.

"It looks really promising but we need more patients and longer follow-ups to confirm it," Dr. Cohen said.

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The overall survival rate after 12 months for patients who received the expanded cord transplant is currently 75 per cent, compared with 60 per cent for conventional cord transplants. Dr. Cohen and her collaborators now hope to launch a second clinical trial for high-risk leukemia patients who would otherwise have a very low chance of survival.

The basis of the technology is a small molecule, dubbed UMI71, discovered by Guy Sauvageau and Anne Marinier at the University of Montreal. The molecule binds to cells and keeps new blood cells from specializing. Instead, they remain stem cells, and continue to proliferate until they are harvested from a laboratory medium. After they are injected into a patient, the stem cells take up residence and can generate all the cell types that are required to restore a healthy blood stream.



Dr. Marinier at her research laboratory at the University of Montreal on Tuesday, June 26, 2018.
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According to Dr. Marinier, an additional advantage of the method is that the compound used to expand the cord blood is not used directly in patients, which translates into fewer steps required to show its efficacy and safety.

If the results continue to be favourable, the method stands to greatly increase the number of cord blood donors that a patient in need could be potentially matched to.

"With our procedure I think we can get to 50 per cent of the cord units available worldwide," Dr. Marinier said.

Together with Peter Zandstra, a researcher in stem-cell bioengineering at the University of British Columbia, Dr. Marinier and Dr. Sauvageau have launched a company, ExCellThera, in hopes of bringing the procedure to market and competing with other approaches to expanding cord blood being developed outside of Canada.

Heidi Elmoazzen, who oversees the National Public Cord Blood Bank in Ottawa, said that on any given day, hundreds of Canadians are looking for a blood stem cell donor match. The problem is compounded in this country by an ethnically diverse population, which lowers the chances of finding a well-matched adult donor.

Dr. Elmoazzen welcomed the prospect of new technologies for increasing the number of possible matches with a cord blood donor.

"You're providing more opportunities for patients to get a transplant," she said. "If it can be made affordable and accessible, this could be a game changer for cord blood."

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