MedicalXpress

UM171 molecule saves another life

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In a world first, a young man suffering from severe aplastic anemia who could not be helped by standard treatments has been given a life-saving blood transplant with the made-in-Canada UM171 molecule.

The procedure was done by a medical team at the Institute of Hemato-oncology and Cellular Therapy (iHOTC) of Maisonneuve-Rosemont Hospital and the Institute for Research in Immunology and Cancer, both affiliated with Université de Montréal.

The young man's case history, including the lifesaving transplant, was recently published in the scientific journal *European Journal of Haematology*, highlighting the unique and revolutionary properties of the UM171 molecule.

An autoimmune disease, severe aplastic anemia destroys stem cells in bone marrow and leads to a halt in the production of red blood cells, white blood cells and platelets. For allografting (grafting between individuals) for this disease, the donor's stem cells must be as compatible as possible with those of the recipient to avoid the risk of immunological complications.

No donor option

If no compatible family or unrelated donor can be found, stem cells from a semi-identical family donor, also known as a haplo-identical donor, may be considered, under certain conditions, as an alternative source of cells. However, a family member must be healthy and

available for such a procedure; the young man in this case did not have that option.

Cord blood transplantation, which is less demanding in terms of compatibility, is a good option for many patients requiring a stem cell transplant. On the other hand, cord blood generally does not contain enough stem cells for an adult patient weighing more than 70 kg; it produces a slow rise in white blood cells with an increased risk of often fatal infections.

In addition, the rate of graft rejection—the destruction of infused cord cells by the recipient's immune system—is very high in patients with severe aplastic anemia who have received multiple blood transfusions.

"It was after having exhausted all our treatment options that UM171, which had already proven itself in a clinical trial in blood cancer patients, came into play," said UdeM medical professor Jean Roy, a hematologist and clinical researcher at the MRH.35-fold increase

"As well as increasing the number of stem cells in a unit of umbilical cord blood by an average of 35 times, it greatly reduces the risk of a frequent long-term immunological complication (graft-versus-host disease) requiring years of use of toxic immunosuppressive drugs."

The researchers' success confirms the excellent performance of UM171, which has already been demonstrated in two other studies conducted by iHOTC research teams with very encouraging results. A third study is now underway.

"This young man's story and the other studies with UM171 clearly demonstrate how innovative clinical research, set up by local investigators, can create a culture of excellence and improve care to save more lives," said IHOTC director Denis Claude Roy.

"The future will certainly bring us more such accomplishments, and that's very encouraging."

More information: Jean-Sébastien Claveau et al, Single UM171-expanded cord blood transplant can cure severe idiopathic aplastic anemia in absence of suitable donors, *European Journal of Haematology* (2020). DOI: 10.1111/ejh.13504

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