

What is hematopoietic stem cell transplantation? Highly personalized medicine ... and more?

Hematopoietic stem cell transplantation (HSCT) is a transplant of hematopoietic stem cells. We talk about allograft because the transplant comes from another person, called the donor, as opposed to an autograft where the patient's own bone marrow is used. In a graft, donor stem cells can come from many different origins: bone marrow, peripheral stem cells, or placental blood (also known as cord blood).

What is bone marrow? Concretely, bone marrow is a liquid found in the bones of the body. The bone marrow contains all the mother cells, that is, the hematopoietic stem cells. The latter continuously manufacture blood cells (white and red blood cells, platelets). White blood cells are a person's immune system. Red blood cells contain hemoglobin, which transports oxygen from the air throughout the body. Platelets are used for coagulation, that is to say to prevent bleeding (for example by creating a crust on a wound). The bone marrow must therefore be replaced when the cells that compose it are sick, in particular to strengthen its immune system and make sure the proper functioning and good manufacture of blood cells, otherwise the disease may prevail. Hematopoietic stem cells in good health will therefore be taken from a donor compatible with the recipient (often a brother or sister but not necessarily) to then be grafted (injected) in the patient. He will be able to make healthy blood cells again.

Thus, in the case of leukemia in a child, it will be necessary to reduce as much as possible the diseased cells, that is to say cancer cells, which are called blasts. In general, we first try to destroy these blasts by chemotherapy, then we use the transplant (HSCT), an immunotherapy. In short, we first practice a conditioning regime (based on chemotherapy) that destroys the cancerous cells in the bone marrow to prepare for the transplant. Then healthy donor stem cells are injected into the patient's body. Conditioning is a very important stage, because we have to make sure that the child is well prepared for the transplant, so that his body does not make a rejection (side effect). The infected marrow will therefore be destroyed, most of the time by chemotherapy or sometimes also by radiotherapy, or even immunotherapy, in order to replace it with healthy marrow. This transfusion is done intravenously. The manufacture of new healthy cells can then take a long time, during which the child is constantly under observation in a protected environment at the Hospital.

The risks when practicing a transplant are very individual and can totally vary from person to person. The cure rate after HSCT has increased dramatically in recent years, but there is still room for improvement, not only in terms of healing, but also in terms of side effects, relapses, etc.

This is why medical research must be able to continue moving forward. The CANSEARCH Foundation (www.cansearch.ch) has made it its main mission since its creation in 2011. It financially supports a research platform in oncology and pediatric hematology (CANSEARCH Research Laboratory), set up in 2011 by Prof. Marc Ansari, Head of the Pediatric Oncology-Hematology Unit of the Geneva University Hospitals (HUG), in collaboration with them and with the Faculty of Medicine of the University of Geneva. CANSEARCH has many ongoing research projects in the field of individualized therapies, including its pharmacogenomics project, which focuses on the conditioning regimen to be given before HSCT and more specifically on the correct dose of Busulfan (the main chemotherapeutic



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agent) to be given to the patient in preparation for the transplant, if he should receive one, so that he can avoid maximum toxicity or side effects. For more details:

<https://cansearch.ch/en/recherche/research-projects/>

The Pediatric Oncology and Hematology Unit of the HUG is accredited by the European organization JACIE in order to carry out this particular treatment for more than 10 years on more than 100 children suffering from either cancer or non-malignant disease such as sickle cell anemia, thalassemia, immune deficiency. It has also been recognized by the Swiss Confederation as a highly specialized unit in the field of pediatric oncology and hematology.