Autologous stem sell transplant

Patient Informations Booklet

This brochure is intended for families whose child will undergo an autologous hematopoietic stem cell transplant (autologous transplant). Its purpose is to explain the reasons for this type of treatment and its main side effects. This brochure does not replace the meeting you will have with the transplant team's doctor, but is intended as a supplementary document.

What is an Autologous Stem Cell Transplant?

Autologous transplantation is a treatment that involves infusing the patient with their own stored stem cells intravenously in order to revive their bone marrow.

Purpose of Stem Cell Autotransplantation

Autologous hematopoietic stem cell transplantation allows patients to receive very high doses of chemotherapy to treat their cancer. The aim of this very intense chemotherapy is to destroy any cancer cells that may still be present. However, it will have the same effect on the patient's hematopoietic stem cells. Without an autologous stem cell transplant, the patient would be left without bone marrow and therefore without blood cells permanently, with a very high risk of infection and bleeding. An autologous transplant, given after intensive chemotherapy, allows the patient to recover normal blood cell production within 3 to 4 weeks.

Autologous hematopoietic stem cell transplantation is a standard treatment for certain cancers such as metastatic neuroblastoma and medulloblastoma. It is also a possible treatment for refractory or relapsed cancers, when treatment needs to be intensified, such as in cases of relapsed lymphoma.

The Different Stages

Stem cell collection

At a time deemed appropriate by the medical team, hematopoietic stem cells will be collected for autologous transplantation. A consultation with the transplant team physician will allow you to understand the procedure. In addition, you will be given an explanatory brochure entitled "Hematopoietic Cell Collection." Once collected, the stem cells will be kept safely in our freezers until the time of autologous transplantation.

Pre-transplant assessment

In the weeks leading up to the autotransplant, a series of tests will be performed to ensure that the body is ready to receive the upcoming treatments. Imaging tests and other specialized tests will be necessary to check the condition of certain organs such as the kidneys, heart, ears, and lungs. Blood tests will also be performed to ensure that there are no infections. Specialists will also help you prepare for the transplant. You will meet with, among others, dentists, nutritionists specializing in bone marrow transplants, pharmacists, and infectious disease specialists.

Installation of a double-lumen central catheter

Prior to admission to the bone marrow transplant unit, a double central venous catheter (or two single catheters) will be inserted under sedation in the angiography room. PiccLine, Broviacr, or PAC catheters are most commonly used. These long-term catheters are necessary for various blood tests and for the administration of fluids, supportive medications, and chemotherapy throughout the hospital stay.

Hospitalisation

Duration

Autologous transplantation requires a 4-6 week hospital stay in a room specially designed for this type of procedure. It includes chemotherapy days (between 2-7 days), stem cell infusion (one day) followed by monitoring for possible complications and bone marrow reconstitution. Patients are in insolation in their rooms from admission until their neutrophil count is above "0.5" for two consecutive days following the autologous transplant.

Chemotherapy before autologous transplantation

Treatment prior to autologous transplantation consists of two to four different chemotherapy agents administered over two to five days. The type of chemotherapy used and the number of days of treatment depends on the type of cancer being treated. These high-dose chemotherapy drugs are designed to destroy as many cancer cells as possible. It takes 1 to 3 days for the body to eliminate the chemotherapy drugs. The stem cells are then thawed in the room and administered as a blood transfusion through the central catheter. Chemotherapy is responsible for the symptoms and complications experienced during autologous transplant treatment. The side effects of chemotherapy begin during the days of administration, but are most intense during the 2 to 3 weeks following the end of chemotherapy.

Isolation

All isolation measures surrounding the transplant process are designed to limit the risk of infection to the patient while waiting for their bone marrow and immune system to resume normal function.

Any person entering the patient's room (parents, visitors, hospital staff, etc.) must wash their hands and put on a gown and mask before entering the room to avoid contaminating the child.

Visitors are limited to six people, who must be registered on a pre-established list, for the entire duration of the hospital stay. After discharge, public places (e.g., schools, shopping centers, restaurants) should be avoided until your attending physician or nurse gives you permission (approximately three months after the autologous transplant).

Food safety

The nutritionist specializing in bone marrow transplants is responsible for teaching you how to cook, prepare, and store food in the context of the transplant in order to prevent the spread of bacteria and thus avoid infections. These measures must be maintained for up to 3 months after the transplant or until your doctor or nurse authorizes you to stop.

Autotransplantation — Cell Administration

Unlike solid organ transplants, stem cell transplants do not involve surgery. The transplant takes place in the patient's room, under the supervision of a specially trained nurse. The cells arrive at the room still frozen. A technician from the cell therapy laboratory thaws them at the patient's bedside in a water bath prepared for this purpose.

Once thawed, the stem cells are quickly infused via the patient's central venous catheter by a certified nurse. The entire process takes less than 30 minutes. In some cases, the stem cells may need to be frozen in several bags. The 30-minute process is then repeated for each bag until all the necessary cells have been administered.

The possible side effects during stem cell administration are mainly related to the cell preservation product called DMSO (dimethyl sulfoxide). This product is added to each bag of stem cells before the freezing process to ensure the cells can withstand the freezing and thawing process. It is therefore administered to the patient at the same time as the stem cells. It can occasionally cause side effects such as allergic reactions, increased or decreased blood pressure, rapid or slow heartbeat, skin redness, difficulty breathing or nausea. These effects are temporary and easily treatable with

medication. In order to quickly detect these possible adverse effects, vital signs are closely monitored during and after the autologous transplant using a cardiac monitor for a period of 12 hours.

Since DMSO is eliminated through the skin and respiration, patients emit a distinctive odour in the hours following stem cell infusion. Often described as smelling like canned corn, this odour may cause discomfort in children, leading to nausea or vomiting. Medication is administered to prevent possible side effects. However, you can offer your child chewing gum or hard candy to reduce this discomfort.

Resumption of Bone Marrow Activity

Once in the bloodstream, the stem cells know what to do and return to the bones, where they rebuild the patient's bone marrow. It usually takes 2 to 4 weeks before the bone marrow produces enough blood cells again. Blood samples are taken daily via the catheter to monitor the increase in red blood cells (measured by haemoglobin levels), platelets and various types of white blood cells, particularly neutrophils.

The period during which the bone marrow is deficient is called the aplastic period. This term refers to the absence of cells in the blood. During the aplastic period, the patient may require repeated transfusions of red blood cells and platelets. It is not possible to give white blood cell transfusions, but the patient receives subcutaneous injections of a white blood cell production stimulator (G-CSF). Usually, neutrophils are the first blood cells to be produced in sufficient quantities, 10 to 12 days after the autologous transplant.

Complications

As mentioned above, complications encountered during the autologous transplant process are delayed toxicities from high doses of chemotherapy.

The most common are:

- **Nausea and vomiting**

  To prevent nausea and vomiting, anti-nausea medication is administered regularly to the patient. Other non-drug-based methods may be used by the treatment team, in combination with medication, to provide greater relief to the patient.

- **Mucositis**

  Mucositis is defined as the presence of ulcerations or lesions in one or more parts of the digestive tract. These lesions are usually painful. They can appear in the mouth, oesophagus, stomach, throughout the intestine and up to the rectum or anus.

  Depending on the location, symptoms may vary: loss of taste, loss of appetite, pain, inability to swallow food and saliva, inability to speak, nausea, vomiting, abdominal bloating, abdominal cramps or diarrhea. Pain can be relieved with painkillers (e.g. acetaminophen, morphine). The pain clinic team is often consulted as well to help relieve pain as much as possible.

  If the patient is unable to eat sufficiently for several days, the nursing team may need to insert a tube through the nose into the stomach (nasogastric tube or NGT). It is then possible to administer liquid nutrition via tube feeding (enteral nutrition). This form of alternative nutrition is preferred as it allows for faster refeeding. If enteral feeding is not tolerated (repeated vomiting, severe diarrhoea), intravenous feeding may be used (parenteral feeding or IVF). This second form of feeding may be associated with a slightly increased risk of infectious complications or complications for the liver. It is therefore only used as a second option if the patient is unable to eat sufficiently.

  Mucositis begins 3 to 4 days after the end of chemotherapy and reaches its peak 8 to 10 days after the end of chemotherapy. It then remains stable for about a week before disappearing completely during the third week. There is no effective treatment to prevent it; pain must be relieved and nutrition ensured. Mucositis will disappear when white blood cell counts return to normal. Its severity varies greatly from one patient to another and depends on the chemotherapy protocol used.

- **Infection**

  Given the absence of an immune system during the period of aplasia, the risk of infection by bacteria, viruses or fungi is greatly increased. Close monitoring of temperature (every 4 hours), as well as other signs associated with infection, is essential for early detection of the onset of infection. Medications are given during this period to prevent certain infections. Despite all these precautions, it is common for patients to develop fever during the period of aplasia. In fact, patients often become infected with their own bacteria that are naturally present on their skin and in their digestive tract. The decrease in immune defences and the presence of mucositis create entry points for bacteria from the digestive tract into the bloodstream, promoting infections. Once neutrophil counts start to increase and mucositis has healed, the risk of developing bacterial infections decreases significantly. However, the risk of viral infections persists for at least 3 months after autologous transplantation.

Venous Occlusive Disease (VOD)

Venous occlusive disease (VOD) is characterised by obstruction of the blood vessels in the liver. It is a delayed side effect of certain high-dose chemotherapies (particularly Busulfan or Melphalan). It occurs within 21 days of the end of chemotherapy and manifests as:

- ▪ Weight gain due to water retention in the body, developing over several days and leading to generalised oedema.
- ▪ A gradual increase in liver size. This swelling of the liver is called hepatomegaly and is often painful.
- ▪ A disturbance in liver enzymes in the blood. These tests are called ALT, AST and bilirubin.
- ▪ Yellowing of the eyes and skin, known as jaundice, and brown dis-colouration of the urine
- ▪ Significant need for platelet transfusions once or twice a day
- ▪ In more severe cases, accumulation of fluid in the abdomen (ascites) or around the lungs (pleural effusion), which can lead to breathing difficulties.

The patient's weight is closely monitored twice a day in order to quickly detect any increase. An ultrasound scan of the liver is sometimes necessary to make the diagnosis.

MVO occurs in varying degrees. It is very common in patients treated for neuroblastoma and rare in other types of cancer that receive different types of chemotherapy. Its intensity varies greatly. It generally resolves without any lasting effects. In mild cases, it can resolve spontaneously within a few days. In more severe cases, treatment involves various drugs to increase water elimination in the urine (called diuretics) and a drug to help improve blood circulation in the liver vessels. This drug, called Defibrotide®, is administered through a catheter four times a day for 10 to 20 days. Sometimes, the doctor needs to insert a drain into the abdomen or lungs to remove the accumulated fluid and help the patient breathe more easily. The most severe forms may require transfer to intensive care for a few days to get through the acute period of breathing difficulties with the help of breathing apparatus. Exceptionally severe forms can be life-threatening, but this is very rare.

Thrombotic Microangiopathy (TMA)

Thrombotic microangiopathy (TMA) is another delayed toxicity of high-dose chemotherapy. It is caused by damage to blood vessels inside the kidneys but can spread to other organs (lungs, brain). It manifests itself as:

- Weight gain due to generalised water retention in the body, developing over several days and leading to generalised oedema
- Very high blood pressure requiring several treatments for hypertension.

- Abnormal presence of protein in the urine
- Low platelet and red blood cell counts in the blood that do not recover well after transfusions, leading to an increased risk of bleeding
- Renal failure

MAT is not very common and its intensity varies greatly from one patient to another. For the most severe forms, specific treatment involving intravenous injections over several weeks is available.

Reproductive system

The cells responsible for reproduction are very sensitive to chemotherapy. High-dose chemotherapy therefore carries a significant risk of causing difficulties for patients in having children naturally (infertility) or a complete inability to have children (sterility) in the future.

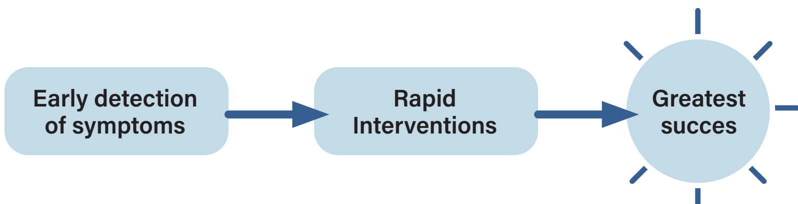
Before proceeding with an autologous transplant, depending on the type of cancer and treatment received, as well as the patient's age and gender, fertility preservation options may be available.

Do not hesitate to discuss this with your doctor before the transplant. Even if the patient is sterile or infertile, their ability to have a sexual life will be preserved.

Conclusion

Autologous transplantation is a treatment with a high risk of complications given the intensity and toxicity of the chemotherapy drugs used. It is a regulary used treatment and the protocols are well established. The risk of death from complications is low (1 to 5%) in children and is mainly secondary to resistant infections or organ toxicity (liver, kidney, lungs, blood vessels). Nevertheless, this risk is much lower than the risk of death from cancer in the absence of high-dose chemotherapy, which is why the treating team recommends this treatment.

Every effort will be made to prevent these complications. Increased monitoring by healthcare staff, combined with cooperation from the patient and their parents, is essential in order to identify these complications and treat them quickly. The patient and their parents are the best sources of information about the patient's state of health. They are considered essential partners for the treatment team in order to optimise treatment as much as possible.



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Notes

CHU Sainte-Justine
3175, chemin de la Côte-Sainte-Catherine
Montréal (Québec) H3T 1C5
Téléphone : 514 345-4931

chusj.org

Created by
Gabrielle Méthot, B.Sc.inf

In collaboration with
Johanne Richer B.Sc.inf, Cell Therapy Programme Coordinator
Coordinatrice du programme de thérapie cellulaire CHUSJ
Maude Lessard, M.Sc.inf, Nursing consultant
Dr Pierre Teira, Medical Director of the Hematology-Oncology Program CHUSJ
Dr Henrique Bittencourt, Medical Director of the Cell Therapy Program CHUSJ
Mme Ligia Prince Torres, partner mom

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