Stem Cell Transplantation/Stem Cell Banking Fact Sheet

Stem cell transplant (SCT) is also known as “high-dose chemotherapy with stem cell rescue” or “bone marrow transplant.” The stem cells involved are hematopoietic stem cells, which are primitive cells capable of both self-renewal and differentiation into mature cells of the blood and immune system. Normally, these stem cells live in the bone marrow and travel in very small numbers into the blood. Because of this, bone marrow transplants used to be performed by aspirating the cells directly from the bone marrow – think bone marrow biopsy multiplied! While this technique is effective and sometimes still used, it has been largely replaced by peripheral blood stem cell harvest, made possible by the use of drugs to encourage the movement of stem cells from the bone marrow into the circulating blood. In some cases, it is desirable to save these stem cells for future use (stem cell banking), while in others, these stem cells are given to a recipient for immediate treatment of a condition.

What Conditions Are Treated by Stem Cell Transplantation:
The conditions most commonly treated by hematopoietic stem cell transplantation are multiple myeloma, leukemia, lymphoma, and aplastic anemia.

What Process is Used for Stem Cell Transplantation:
There are two major types of stem cell transplantation: autologous and allogeneic.

In ***autologous*** transplantation (also called ASCT), the patient who is to receive the stem cells is also the donor, and the source of the stem cells is the patient’s own blood. The stem cells that live in the bone marrow are induced to move into the blood by means of a biological chemical called a growth factor or cytokine. After several days of subcutaneous injection of the growth factor, the stem cells are collected from the donor by means of a technique called apheresis, a procedure easily performed that is somewhat similar to plasmapheresis. Collected stem cells can be frozen in liquid nitrogen for up to 20+ years. If the stem cells are not being used at this point, then the patient has “banked” his own stem cells for future use. If immediate transplantation follows, the goal of transplantation is to kill the cancer cells in the recipient by administering a “conditioning” or preparative regimen involving high doses of chemotherapy ± radiation therapy and subsequently replacing, or “rescuing,” the bone marrow with the stem cells previously collected from the patient. Following stem cell infusion, colonization of the infused stem cells (engraftment) in the bone marrow is rapid, usually 12-14 days. It may take approximately two to four weeks for the bacterial fighting capabilities of the immune system to redevelop and a longer time for the anti-viral and anti-fungal elements of the immune system to function adequately.

In ***allogeneic*** transplantation, the donor of the stem cells is another individual, either a relative (usually a sibling), or in some cases an unrelated individual whose tissue is a close match to that of the recipient. Allogeneic transplants are also now being performed using umbilical cord blood as the source of stem cells, although this technique has seen its share of difficulties and is infrequently used. The procedure for harvesting the stem cells from the donor is similar to that for autologous transplant, in that the stem cells are induced to migrate into the blood of the donor through the use of growth factors. In traditional allogeneic transplantation the recipient undergoes aggressive chemotherapy and possibly radiation therapy. This conditioning regimen not only eradicates the disease but also has an immunosuppressive effect that prevents rejection of the donor’s stem cells by the recipient’s immune system. Standard allogeneic transplantation is rarely considered for WM.

A new modification of allogeneic transplantation is ***non-myeloablative stem cell transplantation,*** or ***mini-allo transplantation***, whereby a reduced-intensity conditioning regimen is used that is considerably less toxic to the recipient. This reduced-intensity conditioning regimen serves not to completely eradicate the patient’s disease but rather to prepare the recipient’s immune system to receive the stem cells from the donor. The donor cells themselves constitute the primary therapy. The recipient receives a lower dose of chemotherapy, which may be coupled with radiation therapy, to cause immunosuppression of the bone marrow followed by an infusion of matched donor stem cells. After a period of several weeks, the donor stem cells replace the recipient’s immune system and ideally begin to attack the cancer cells (graft-versus-tumor effect) and replace them with healthy normal cells. The aim of this type of transplant is to provide a complete response as well as to reduce the serious side effects and toxicity of standard allogeneic transplants.

Stem Cell Transplantation Side Effects:
The conditioning regimen prior to transplantation frequently results in hair loss, loss of appetite, dry mouth, nausea, vomiting, mouth sores, diarrhea, and an increased risk of infection. Many of these side effects can be managed by medications. During the period of engraftment, the recipient's immune system is depressed, and great care must be taken to protect the recipient from infection; antibiotics, anti-viral medications, and anti-fungal medications are part of the standard care regimen. For this reason, patients are usually hospitalized during the engraftment period so that they can be closely monitored. During this time, the patient will not be able to produce red blood cells and platelets and may need to receive supportive transfusions of these substances. Fatigue is often present for months following transplant.

An important, and potentially serious, consequence of allogeneic (but not autologous) transplantation is the development of graft vs. host disease (GVHD). GVHD affects between 20-50% of patients who were transplanted with cells from a related donor. The percentage is higher in patients who were transplanted with cells from an unrelated donor. GVHD is caused by T-cells, which are a type of white blood cell. T-cells are programmed to identify what belongs in a particular person's body and what does not. When they detect something foreign in the body, they signal the immune system to destroy it. When donor cells are transplanted into a recipient, they also contain some of the donor's T-cells. The donor T-cells perceive the patient's organs and tissues as foreign material and signal the immune system to attack them. Because GVHD is relatively common in allogeneic transplantation, the medical team closely monitors the patient for signs of this complication; however, symptoms sometimes don't appear until after the patient goes home following transplant.

There are two types of GVHD: acute and chronic. Patients may develop one, both, or neither.

Acute GVHD usually occurs during the first three months after a transplant. The disease often begins as a mild or faint rash on the patient's back or abdomen; it can also appear on the hands or feet. The rash can spread and eventually resemble sunburn with peeling or blistering. Acute GVHD may also cause stomach and abdominal pain, vomiting, cramping, nausea, and watery or bloody diarrhea. It may also affect the liver and occasionally cause mouth sores. To reduce the risk of developing acute GVHD, patients are typically given powerful immunosuppressive drugs, such as a combination of cyclosporine and methotrexate, tacrolimus and methotrexate, or cyclosporine and mycophenolate mofetil. Prednisone is usually added.

If a patient develops chronic GVHD, it will usually happen 3-18 months after transplant. Patients who have had acute GVHD run the greatest risk of developing the chronic form. Chronic GVHD may last several months or even years and can affect many organs in the body but most often occurs in the mouth, in the skin, in the eyes, and/or in the lungs. The drugs used most often to control chronic GVHD are cyclosporine, prednisone, mycophenolate mofetil, and rapamycin. Additional treatments may be prescribed, depending on the particular tissue being attacked.

The use of these immunosuppressive drugs confers a higher risk for infections in transplant patients, who must be careful and vigilant about monitoring themselves for symptoms.

The Role of Stem Cell Transplantation/Stem Cell Banking in WM:
Autologous stem cell transplants have been shown to be effective in the treatment of WM for younger patients with aggressive disease and are considered appropriate salvage therapy for selected patients, are associated with a very low treatment-related mortality, and can offer long-term disease control; however autologous transplantation is not a cure for WM. Whereas allogeneic transplants have high treatment-related mortality rates (reduced in mini-allo transplants), the potential for complete and durable long-lasting responses is increased due to graft vs. tumor effect. Nonetheless, allogeneic transplants are rarely recommended for WM.

Stem cell banking, which is the collection and storage of one's own stem cells for future use, is increasingly being considered a viable option for WM patients. This option should be part of the conversation you have with your doctor when you consider any treatment, as some treatments can have an undesirable effect on the collection of stem cells.

NOTE: The information in this fact sheet is intended to be helpful and educational, but it does not constitute an endorsement by the IWMF and is not meant to be a substitute for professional medical advice.