

Mozobil approved by US FDA for use to mobilize stem cells for transplant

This could be of benefit to those of us who have been treated with nucleoside analog drugs and may have diminished our potential to produce stem cells for possible transplant...it is anticipated that it will also be approved in countries other than the US sometime next year (read below)

FDA Approves Genzyme's Mozobil

Product provides enhanced mobilization of stem cells for autologous transplantation in Non-Hodgkin's Lymphoma and Multiple Myeloma patients

CAMBRIDGE, Mass., Dec 15, 2008 (BUSINESS WIRE - Genzyme press release)

Genzyme announced today that the U.S. Food and Drug Administration has granted marketing approval for Mozobil(TM) (plerixafor injection), a drug intended to be used in combination with granulocyte-colony stimulating factor (G-CSF) to mobilize hematopoietic stem cells to the bloodstream for collection and subsequent autologous transplantation in patients with non-Hodgkin's lymphoma (NHL) and multiple myeloma (MM). The product has also been granted orphan drug designation.

"Mozobil is an important advancement in the treatment of patients with certain types of cancer who require a stem cell transplant," said John F. DiPersio, M.D., Ph.D., professor, Washington University, St. Louis. "This product should become an integral part of the treatment regimen for transplantation because of the benefits it offers to patients, physicians and transplant centers."

Mozobil is designed to mobilize hematopoietic stem cells from the bone marrow into the bloodstream where they can be collected, making it more likely for patients with certain types of cancers to proceed to transplant. Currently, before a transplant can take place, patients may receive a prescribed dose of chemotherapy and/or other drugs called growth factors to help mobilize their hematopoietic stem cells into the bloodstream. Once the cells are released into the bloodstream, they are collected in preparation for a transplant.

In order for the transplant to take place, a minimum number of approximately 2 million stem cells per kilogram of body weight must be collected. For many patients, this process can take three or four hours over multiple days to complete. Even then, some patients are not able to mobilize enough cells, and a transplant is not possible.

"For many cancer patients, moving on to a transplant is their only hope for remission or a cure," added Dr. DiPersio.

In the pivotal studies of Mozobil, 59 percent of patients with NHL who received Mozobil and G-CSF collected the target number of at least 5 million stem cells/kg of body weight in four or fewer apheresis sessions compared with 20 percent of placebo patients. The median number of days to reach the target cell count was three days for the Mozobil group and not evaluable in the placebo group. Seventy-two percent of patients with MM who received Mozobil and G-CSF collected the target number of at least 6 million stem cells/kg of body weight in two or fewer apheresis sessions

compared to 34 percent of placebo patients. The median number of days to reach the target cell count was one day for the Mozobil group and four days for the placebo group. The target numbers of stem cells in the pivotal studies were chosen based on literature that suggests that reaching these targets can help to facilitate engraftment. Updated 12-month follow-up findings showed that graft durability rates f!

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ents in the Mozobil plus G-CSF and placebo plus G-CSF arms were comparable.

More than 1,000 patients have already received Mozobil through a compassionate use program in the United States. An additional 250 patients have received the product through similar compassionate use programs in Europe since they began 6 months ago. These patients had failed to mobilize enough cells for transplantation using the current standards of care.

Genzyme has submitted an application in Europe for approval of Mozobil and expects approval of the product in the second half of 2009. Genzyme recently filed applications in Australia and Brazil, and additional global applications in up to 60 countries are planned. Mozobil has received orphan drug designation in Mexico which allows the product to be commercialized in the country upon U.S. approval.

Genzyme believes that Mozobil may have broad application outside the current indication. Early preclinical and clinical investigations are already underway to explore additional therapeutic indications for Mozobil, including mobilization of hematopoietic stem cells in allogeneic stem cell transplants and tumor sensitization in oncology/hematology treatments such as adult myeloid leukemia.

About Mozobil

Mozobil, a novel small molecule CXCR4 chemokine receptor antagonist, has been shown in multiple earlier studies to rapidly and effectively increase the number of stem cells in circulation in the blood in patients with non-Hodgkin's lymphoma and multiple myeloma. Once circulating in the blood, stem cells can be collected for use in an autologous stem cell transplant. Genzyme has been developing Mozobil since its acquisition of AnorMED, Inc. in 2006.

Important Safety Information

Mozobil is indicated for use in combination with granulocyte-colony stimulating factor (G-CSF) to mobilize hematopoietic stem cells to the bloodstream for collection and subsequent autologous transplantation in patients with non-Hodgkin's lymphoma and multiple myeloma. Prescribing physicians and patients should be aware of the potential for tumor cell mobilization in leukemia patients, increased circulating leukocytes and decreased platelet counts, splenic enlargement, and fetal harm when administered to pregnant women. The most common adverse reactions (a%JPY 10%) reported in patients who received plerixafor in conjunction with G-CSF that were more frequent than in patients who received placebo were diarrhea, nausea, fatigue, injection site reactions, headache, arthralgia, dizziness and vomiting. For full prescribing information, please visit www.genzyme.com.