FDA Approves Genzyme’s Mozobil

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

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Genzyme Corporation (Nasdaq: GENZ) announced today that the U.S. Food and Drug Administration has granted marketing approval for Mozobil™ (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 stems 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 for patients in the Mozobil plus G-CSF and placebo plus G-CSF arms were comparable.

In addition to its expected benefits for patients with NHL and MM, Mozobil may offer economic benefits for transplant centers. The product has the potential to decrease the number of apheresis days and provide transplant centers with predictable and efficient use of the apheresis center. Mozobil may also reduce the number of patients who require a second mobilization procedure due to a failure to mobilize sufficient numbers of cells with current therapy of G-CSF alone. 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 will commercialize Mozobil in the United States through a blood and marrow transplant sales force that is part of the company’s Transplant and Oncology business unit. This team of dedicated specialists will receive support from the business unit’s oncology sales force to reach a targeted group of hematologists/oncologists and transplant specialists nationwide. This coordinated approach will support physicians and patients across the complementary customer base for these businesses. Genzyme also markets Clolar® (clofarabine), a product indicated for the treatment of pediatric patients with relapsed or refractory acute lymphoblastic leukemia after at least two prior regimens. The company recently filed a supplemental new drug application to expand the indication for Clolar to treat adult patients with acute myeloid leukemia. Genzyme’s drugs Campath® (alemtuzumab) and Thymoglobulin® (Anti-thymocyte Globulin [Rabbit]) are also used in the hematology/oncology setting.

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. Approximately 55,000 hematopoietic stem cell transplants are performed each year.
globally for multiple myeloma, Hodgkin's and non-Hodgkin's lymphoma, and other conditions. Genzyme expects that over time and with further clinical development, Mozobil will be used in the majority of these procedures. Peak sales of the product in the transplant setting are projected to reach $400 million annually.

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.

“Mozobil is an exciting and innovative new treatment that expands Genzyme’s contribution to the field of Hematology and Oncology,” said Joseph Lobacki, senior vice president and general manager, Genzyme Transplant and Oncology. “We look forward to strengthening our partnership with the blood marrow transplant community to make this product broadly available to patients who are facing transplantation procedures for non-Hodgkin’s lymphoma or multiple myeloma.”

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 (≥ 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.

About Genzyme

One of the world's leading biotechnology companies, Genzyme is dedicated to making a major positive impact on the lives of people with serious diseases. Since 1981, the company has grown from a small start-up to a diversified enterprise with more than 10,000 employees in locations spanning the globe and 2007 revenues of $3.8 billion. In 2007, Genzyme was chosen to receive the National Medal of Technology, the highest honor awarded by the President of the United States for technological innovation.

With many established products and services helping patients in nearly 90 countries, Genzyme is a leader in the effort to develop and apply the most advanced technologies in the life sciences. The company's products and services are focused on rare inherited disorders, kidney disease, orthopaedics, cancer, transplant, and diagnostic testing. Genzyme's commitment to innovation continues today with a substantial development program focused on these fields, as well as immune disease, infectious disease, and other areas of unmet medical need.

This press release contains forward-looking statements, including statements regarding: Genzyme's expectations regarding the use of the product in various treatment regimens, its expectations for product adoption, sales and the effectiveness of its coordinated commercialization efforts, its regulatory strategy with respect to marketing authorization applications, its plans for further clinical development, the possibility of additional therapeutic applications of Mozobil, the ability to work together with the bone marrow transplant community and the expected economic benefits to transplant centers through a reduction in the number of apheresis days and the predictability of mobilization of stem cells resulting in a more efficient use of the apheresis center. These forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially. These risks and uncertainties include, among others: the adoption rate or acceptance of the product within the bone marrow transplant community, the effectiveness of Genzyme's regulatory, clinical and commercialization efforts, the ability to develop additional indications or applications for the product, and the risks and uncertainties described in reports filed by Genzyme with the U.S. Securities and Exchange Commission, including without limitation the factors discussed under the caption "Risk Factors" in Genzyme's Quarterly Report on Form 10-Q for the quarter ended September 30, 2008. We caution investors not to place undue reliance on the forward-looking statements contained in this press release. These statements speak only as of the date of this press release, and we undertake no obligation to update or revise the statements.

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