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FDA Gives Approval to Genzyme's Mozobil

Genzyme Corporation announced on December 15, 2008 that the FDA has granted marketing approval for Mozobil, 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 for patients with non-Hodgkin's lymphoma and multiple myeloma.

This product was also granted orphan drug designation by the FDA.

John DiPersio, M.D., Ph.D. professor, at Washington University, St. Louis said, "Mozobil is an important advancement in the treatment of patients with certain types of cancer who require a stem cell transplant. 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."

Currently a prescribed dose of chemotherapy and other growth factor drugs are used to help mobilize hematopoietic stem cells into the bloodstream. 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 to proceed to transplant.

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A minimum number of 2 million stem cells per kilogram of body weight must be harvested in order for a transplant to take place. Harvesting this many stem cells can take multiple apheresis sessions lasting three to four hours a session. Studies show that 59 percent of non-Hodgkins

lymphoma patients who received Mozobil in combination with G-CSF collected the targeted 5 million stem cells/kg of body weight in four or fewer sessions. Seventy-two percent of patients with multiple myeloma collected the target number of cells in two or fewer apheresis sessions. This result

is expected to offer economic benefits for transplant centers and patients as fewer sessions may be required to harvest the required amount of stem cells.

More than 1,000 patients have received Mozobil through a compassionate use program in the United States. These patients had previously failed to mobilize enough cells for transplantation using the current standard of care.

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Specialty Drug Spend Projected to Reach \$99B

Greater use of biologic medications is the driving factor in higher prescription cost projections over the next two years. The Food and Drug Administration approved eight new biopharmaceuticals from 2005 to 2007, however, it is expected that new products in the ever expanding specialty pipeline will contribute heavily to the growth. New indications for use for existing approved specialty drugs, is also pushing more utilization onto the market.

A trend toward so-called personalized medicine, tailored use of cancer drugs to people with certain genetic profiles, will also help fuel the rising cost. FDA advisers state that companies looking to receive approval of these medications will need to run larger studies to gather enough data for review. Drugs based upon individual genetic makeup will require enough patients and tissue samples to analyze genetic issues.

Specialty drugs accounted for 10 to 20 percent of overall drug spend in 2006. Predictions are that by 2010 that number will rise to 25% of overall drug spend or \$99 billion. By the year 2020 that number could very well reach 37 percent.

According to Datamonitor, a business intelligence company, drugs like Herceptin, Remicade, Avastin, Humira and Rituxan, all of which treat chronic conditions or cancer, will have the largest growth.

Will Genetic Tests Soon Be Required Prior to Treatment?

Officials from the Food and Drug Administration met in December to determine whether physicians should look for the KRAS gene mutation before prescribing two drugs for colon cancer, Erbitux and Vectibix. People who have this gene mutation will not experience any effect from the drugs, which can cost up to \$10,000 per month. Both drugs are biologics that belong to a class known as monoclonal antibodies and have best helped people who have a normal version of the KRAS gene. The KRAS gene helps regulate cell division.

ImClone and Amgen, the manufacturers of Erbitux and Vectibix, respectively, are seeking FDA permission to tell doctors that patients with mutant KRAS genes should not be treated with their drugs. According to Bristol-Myers, which sells Erbitux, about 40 percent of patients have mutant KRAS tumors.

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integrated pharmacy services

Specialty Pharmacy Pipeline Drugs to Watch

SPECIALTY PIPELINE UPDATE:

New Indications in the Pipeline

| Drug Name | Current Indication | Investigational Indication | Route of Administration | Comments |
|-----------------------------------|---|---|-------------------------|--|
| Infergen (interferon alfacon-1) | Treatment of hepatitis C | Treatment of chronic hepatitis C in combination with ribavirin after failure to respond to previous course of pegylated interferon plus ribavirin | SC injection | Clinical trials are ongoing |
| Rituxan (rituximab) | Treatment of non-Hodgkin's lymphoma and treatment of moderately to severely active RA in patients who have had an inadequate response to one or more TNF inhibitors | For the treatment of moderately to severely active RA in patients who have had an inadequate response to prior treatment with a disease modifying anti-rheumatic drug | IV infusion | Supplemental Biologic License Application filed October 2008 |
| Cimzia (certolizumab pegol) | Treatment of Crohn's disease | For the treatment of moderate to severe or active RA and moderate to severe psoriasis | SC injection | Manufacturer is evaluating further development or alternatives in psoriasis |
| Treanda (bendamustine) | Chronic lymphocytic leukemia | Treatment of non-Hodgkins lymphoma in patients who failed Rituxan | IV infusion | FDA approved new indication October 31, 2008 |
| Avastin (bevacizumab) | Treatment of breast cancer, colorectal cancer and non-small cell lung cancer | First-line treatment of renal cell carcinoma (in combination with interferon alfa-2a) and for treatment of relapsed glioblastoma multiforme | IV infusion | Supplemental Biologic License Application filed October 2008 |
| Tasigna (nilotinib) | Treatment of chronic and accelerated phase Philadelphia chromosome positive chronic myelogenous leukemia | Treatment of gastrointestinal stromal tumor in patients who have failed both Gleevec and Sutent | Oral | Supplemental New Drug Application filing anticipated in 2009 |
| Sutent (sunitinib) | For treatment of gastrointestinal stromal tumor and advanced renal cell carcinoma | Treatment of colorectal cancer, metastatic breast cancer and non-small cell lung cancer | Oral | Phase III trials ongoing |
| PegIntron (peginterferon alfa-2b) | For treatment of chronic hepatitis C | Adjuvant treatment of stage III melanoma | SC injection | FDA postponed planned review of Supplemental Biologic License Application. No new review date has been set |

New Indications in the Pipeline, continued:

| | | | | |
|------------------------------|--|--|--------------|---|
| Forteo (teriparatide) | Treatment of men and postmenopausal women with osteoporosis who are at high risk of fracture | Treatment of glucocorticoid-induced osteoporosis | SC injection | Manufacturer reported receiving an approvable letter April 2008 |
| Relcast (zolefronic acid) | Treatment of Paget's disease and postmenopausal osteoporosis | Treatment of glucocorticoid-induced osteoporosis | IV infusion | Supplemental New Drug Application filed 2008 |

SPECIALTY PIPELINE UPDATE

New Dosage Forms in the Pipeline:

| Drug Name | Indication | Current Route of Administration | Investigational Route of Administration | Comments |
|-------------------------------|--|---------------------------------|---|--|
| Cayston (aztreonamlyssine) | Treatment of patients with cystic fibrosis who have pulmonary pseudomonas aeruginosa | IV injection | Inhalation | Designated as an orphan drug. FDA has notified the manufacturer that an additional study will be required. Drug is available through an expanded access program. |
| TBM100 (tobramycin) | Treatment of patients with cystic fibrosis who have pulmonary pseudomonas aeruginosa | Solution for inhalation | Powder for inhalation | Expected to provide more rapid and convenient administration. New Drug Application filing planned for 2009. |
| Mylinax (cladribine) | Treatment of relapsing forms of MS | IV infusion | Iral | Fast track status. |
| Viveta (treprostinil) | Treatment of pulmonary arterial hypertension | SC or IV infusion | Inhalation | Studied in combination with Tracleer or Revatio. Response to New Drug Application is expected April 2009 |

2008 4TH QUARTER SPECIALTY PIPELINE UPDATE

Medications in Phase III Trials

| Drug Name | Indication | Route of Administration | Comments |
|------------------------------|--|-------------------------|--|
| Kiacta (eprodissate) | Treatment of amyloid A amyloidosis | Oral | Phase III Trial expected to begin 4QTR of 2008 |
| Human fibrinogen concentrate | Treatment of congenital fibrinogen deficiency | IV infusion | Orphan drug designation |
| Bosatria (mepolizumab) | Treatment of hypereosinophilic syndrome | IV infusion | Orphan drug designation |
| ATryn (antithrombin alfa) | Prophylactic treatment of thromboembolisms in patients with hereditary antithrombin deficiency who are undergoing high-risk surgical and childbirth procedures | SC injection | Orphan drug with fast track designation |

Medications in Phase III Trials, continued:

| | | | |
|---|--|--------------|--|
| Denufosal | Treatment of cystic fibrosis | Inhalation | Orphan drug with fast track designation. Primary endpoint was achieved in first phase III trial in June 2008 |
| prGCD (plant cell expressed recombinant glucocerebrosidase) | Treatment of Gaucher disease | IV infusion | Phase III trial being conducted under FDA Special Protocol Assessment |
| Velaglucerase alfa | Treatment for type 1 Gaucher disease | IV infusion | Worldwide enrollment completed for phase III clinical program in July 2008 |
| Albuferon (albinterferon alfa-2b) | In combination with ribavirin for treatment of Hepatitis C | Injection | Phase III data expected by spring 2009 |
| Viramidine (taribavirin) | Treatment of hepatitis C in combination with pegylated interferon alfa-2b | Oral | Enrollment for phase II trial using a weight-based dose was initiated in March 2007. Based on early review of the study, the manufacturer will determine whether to begin a third phase III study. |
| Berinert P (C1 inhibitor) | Treatment of acute attacks in patients with hereditary angioedema | IV infusion | Orphan drug designation |
| DX-88 (ecallantide) | Treatment of moderate to severe acute hereditary angioedema attacks | SC injection | Orphan drug designation with fast track status |
| Rhucin (C1 inhibitor) | Treatment of acute attacks in patients with hereditary angioedema | IV infusion | Biologic License Application expected for the end of 2008 |
| Vicriviroc | Treatment of R5-type HIV infection in combination with other antiretroviral agents | Oral | Two large phase III trials initiated in September 2007 |
| Corifollitropin alfa | Infertility | SC injection | Primary endpoints in phase III trial were met July 2008 |
| Golimumab | Treatment of rheumatoid arthritis (RA), psoriatic arthritis and ankylosing spondylitis | SC injection | Response from FDA to the Biologic License Application is expected April 2009 |
| Dirucotide (MBP8298) | Treatment of secondary progressive MS | IV infusion | Fast track designation. Patient recruitment for phase III trial completed August 2008 |
| Fingolimod, formerly FTY720 | Treatment of relapsing-remitting MS | Oral | New Drug Application planned for the end of 2009 |
| Teriflunomide | Treatment of relapsing forms of MS | Oral | Also being studied in combination with interferon-beta and with Copaxone |
| Pasireotide | Treatment of Cushing's disease and acromegaly | SC injection | New Drug Application expected in 2010 |
| Zactima (vandetanib) | Second line treatment of non-small cell lung cancer | Oral | New Drug Application expected first half of 2009 |
| Opaxio (paclitaxel poliglumex) | Treatment of advanced non-small cell lung cancer | IV infusion | FDA granted fast track status |
| Xerecept (corticotropin-releasing hormone receptor type 1 antagonist) | Treatment of peritumoral brain edema | SC injection | Orphan drug designation |
| Lestaurtinib | Treatment of acute myeloid leukemia | Oral | Orphan drug designation. New Drug Application expected in 2009 |
| Provenge (sipuleucel-T) | Treatment of metastatic hormone-refractory prostate cancer | IV infusion | Final analysis of the Biologic License Application is expected mid-2009 |

| <i>Medications in Phase III Trials, continued:</i> | | | |
|--|--|-------------------------|---|
| HuMax-CD20 (ofatumumab) | Treatment of refractory chronic lymphocytic leukemia | IV infusion | Potential Biologic License Application filing in 2008 |
| Genasense (oblimersen) | Treatment of relapsed or refractory chronic lymphocytic leukemia in combination with chemotherapy | IV infusion | FDA response to the New Drug Filing is expected December 2008 |
| Virulizin | First-line treatment of advanced pancreatic cancer in combination with Gemzar | Intramuscular injection | Orphan drug designation with fast track status |
| Phenoxodiol | Treatment of hormone refractory prostate cancer in Taxotere non-responders and recurrent chemo-resistant late-stage ovarian cancer | IV injection/Oral | Fast track status |
| Afinitor (everolimus, RAD0001) | Treatment of advanced Renal Cell Carcinoma and neuroendocrine tumors | Oral | FDA granted priority review status September 2008 |
| Voraxaze (glucarpidase) | Adjunctive therapy for cancer patients undergoing chemo who are at risk for methotrexate toxicity | IV injection | Designated orphan drug with fast track status |
| Larotaxel | Second-line treatment of pancreatic cancer | IV infusion | New Drug Application planned for 4QTR 2009 |
| Denosumab | Treatment of postmenopausal osteoporosis and cancer-related bone loss | SC injection | Primary endpoints achieved in phase III studies. |
| Thelin (sitaxsentan) | Treatment of pulmonary arterial hypertension | Oral | Orphan drug designation |
| Numax (motavizumab) | Prevention of RSV in high-risk pediatric populations | IM injection | Expected to be more potent than Synagis. |
| Mozobil (plerixafor) | For the mobilization of stem cells for collection and subsequent autologous transplantation in patients with lymphoma and multiple myeloma | SC injection | FDA granted marketing approval December 2008 |
| Certican (everolimus) | Prevention of solid organ transplant rejection in combination with Neoral | Oral | Clinical trials ongoing |

AIDS/HIV

Daunoxome
Doxil
Intron A
Roferon-A
Serostim
Taxol
Fuzeon

Alpha1 - Proteinase Inhibitor Deficiency

Aralast
Prolastin
Zemaira

Antihemophilic Agents

Antihemophilic Factor
Advate
Alphanate
Bioclote
Helixate FS
Hemofil M
Humate P
Hyate C
Koate DVI
Kogenate FS
Monarc M
Monoclote P
Recombinate
ReFacto

Anti-nausea

Aloxi
Anzemet
Emend
Kytril
Zofran

Asthma

Xolair

Cancer/Related

Adriamycin
Adrucil
Alkeran
Aredia
Avastin
BiCNU
Blenoxane
Busulfex
Campath
Camptosar
Cerubidine
Cosmegen
Cytarabine
Cytosan
Depocyt

Doxil
DTIC-Dome
Eligard
Ellence
Erbitux
Ethyol
Faslodex
Fludara
Gemzar
Gleevec
Herceptin
Hycamtin
Idamycin
IFEX
Intron A
Leucovorin
Leukine
Leustatin
Lupron Depot
Lupron Depot-Ped
Mesnex
Mustargen
Mutamycin
Mylotarg
Navelbine
Nexavar
Nipent
Novantrone
Oncaspar
Ontak
Paraplatin
Platinol AQ
Proleukin
Rituxan
Roferon-A
Sutent
Tarceva
Taxol
Taxotere
Temodar
Thyrogen
Toposar
Trelstar Depot
Trelstar LA
Trisenox
VePesid
Vinblastine
Vincasar
Vumon
Xeloda
Zanosar
Zoladex
Zometa

Contraceptives

Depo-Provera

Crohn's Disease

Remicade

Dystonia

Botox
Myobloc

Factor IX Concentrates

Alphanine SD
Benefix
Mononine
Profilnine SD
Proplex T
Bebulin VH

Gaucher's Disease

Ceredase
Cerezyme
Zavesca

Growth Hormone Deficiency

Genotropin
Humatrope
Norditropin
Nutropin
Nutropin AQ
Saizen

Hematologics

Arixtra
Aranesp
Epogen
Fragmin
Innohep
Lovenox
Neulasta
Neumega
Neupogen
Procrit

Hepatitis C

Copegus
Infergen
Intron A
Pegasys
Peg-Intron
Rebetron
Roferon-A

Hormone Deficiency

Delatestryl
Delestrogen
Depo-Estradiol
Depo-Testosterone

Hunter Syndrome

Elaprase

Primary Immunodeficiency

Carimune NF
Gamimune N
Gammagard S/D
Gammar-P
Gamunex
Iveegam EN
Panglobulin
Panglobulin NF
Polygam S/D
Venoglobulin-S

Miscellaneous

Alferon-N
Milrinone
Zinecard

Multiple Sclerosis

Avonex
Betaseron
Copaxone
Novantrone
Rebif
Tysabri

Osteo/ Rheumatoid/ Psoriatic Arthritis

Enbrel
Humira
Hyalgan
Kineret
Orencia
Remicade
Supartz
Synvisc

Osteoporosis

Forteo
Miacalcin

Psoriasis

Amevive
Enbrel
Raptiva
Remicade

Respiratory Syncytial Virus

Synagis

Rh Hemolytic Disease

BayRho-D
Micro-Rhogam
Rhogam
WinRho-SDF