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FDA NEWS RELEASE

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FDA approves Imbruvica for rare blood cancer

Second drug with breakthrough therapy designation to receive FDA approval

The U.S. Food and Drug Administration today approved Imbruvica (ibrutinib) to treat patients with mantle cell lymphoma (MCL), a rare and aggressive type of blood cancer.

MCL is a rare form of non-Hodgkin lymphoma and represents about 6 percent of all non-Hodgkin lymphoma cases in the United States. By the time MCL is diagnosed, it usually has already spread to the lymph nodes, bone marrow and other organs.

Imbruvica is intended for patients with MCL who have received at least one prior therapy. It works by inhibiting the enzyme needed by the cancer to multiply and spread. Imbruvica is the third drug approved to treat MCL. Velcade (2006) and Revlimid (2013) are also approved to treat the disease.

“Imbruvica’s approval demonstrates the FDA’s commitment to making treatments available to patients with rare diseases,” said Richard Pazdur, M.D., director of the Office of Hematology and Oncology Products in the FDA’s Center for Drug Evaluation and Research. “The agency worked cooperatively with the companies to expedite the drug’s development, review and approval, reflecting the promise of the Breakthrough Therapy Designation program.”

Imbruvica is the second drug with breakthrough therapy designation to receive FDA approval. The Food and Drug Administration Safety and Innovation Act, passed in July 2012, gave the FDA the ability to designate a drug a breakthrough therapy at the request of the sponsor if preliminary clinical evidence indicates the drug may offer a substantial improvement over available therapies for patients with serious or life-threatening diseases.

The FDA is approving Imbruvica under the agency's accelerated approval program, which allows the FDA to approve a drug to treat a serious disease based on clinical data showing that the drug has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit to

patients. This program provides earlier patient access to promising new drugs while the company conducts confirmatory clinical trials. The FDA also granted Imbruvica priority review and orphan-product designation because the drug demonstrated the potential to be a significant improvement in safety or effectiveness in the treatment of a serious condition and is intended to treat a rare disease, respectively.

Imbruvica's accelerated approval for MCL is based on a study where 111 participants were given Imbruvica daily until their disease progressed or side effects became intolerable. Results showed nearly 66 percent of participants had their cancer shrink or disappear after treatment (overall response rate). An improvement in survival or disease-related symptoms has not been established.

The most common side effects reported in participants receiving Imbruvica are low levels of platelets in the blood (thrombocytopenia), diarrhea, a decrease in infection-fighting white blood cells (neutropenia), anemia, fatigue, musculoskeletal pain, swelling (edema), upper respiratory infection, nausea, bruising, shortness of breath (dyspnea), constipation, rash, abdominal pain, vomiting, and decreased appetite. Other clinically significant side effects include bleeding, infections, kidney problems and the development of other types of cancers.

Imbruvica is co-marketed by Sunnyvale, Calif.-based Pharmacyclics and Raritan, N.J.-based Janssen Biotech, Inc. Velcade (bortezomib) is marketed by Millennium Pharmaceuticals, based in Cambridge, Mass. Revlimid (lenalidomide) is marketed by Summit, N.J.-based Celgene.

For more information:

FDA: Office of Hematology and Oncology Products (/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm091745.htm)

FDA: Breakthrough Therapies (/RegulatoryInformation/Legislation/FederalFoodDrugandCosmeticActFDCAct/SignificantAmendmentstotheFDCAct/FDASIA/ucm341027.htm)

FDA: Drug Innovation (http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugInnovation/default.htm)

FDA: Approved Drugs: Questions and Answers (/Drugs/ResourcesForYou/Consumers/ucm054420.htm)

NCI: Non-Hodgkin Lymphoma (http://www.cancer.gov/cancertopics/types/non-hodgkin)

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vaccines and other biological products for human use, and medical devices. The agency also is responsible for the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products.

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