U.S. Food and Drug Administration
Protecting and Promoting *Your* Health

FDA NEWS RELEASE

For Immediate Release: Sept. 30, 2013
Media Inquiries: Stephanie Yao, 301-796-0394, **stephanie.yao@fda.hhs.gov (mailto:stephanie.yao@fda.hhs.gov)**
Consumer Inquiries: 888-INFO-FDA

En Español (/NewsEvents/Newsroom/ComunicadosdePrensa/ucm375268.htm)

FDA approves Perjeta for neoadjuvant breast cancer treatment

First drug approved for use in preoperative breast cancer

The U.S. Food and Drug Administration today granted accelerated approval to Perjeta (pertuzumab) as part of a complete treatment regimen for patients with early stage breast cancer before surgery (neoadjuvant setting). Perjeta is the first FDA-approved drug for the neoadjuvant treatment of breast cancer.

Perjeta was approved in 2012 for the treatment of patients with advanced or late-stage (metastatic) HER2-positive breast cancer. HER2-positive breast cancers have increased amounts of the HER2 protein that contributes to cancer cell growth and survival.

Perjeta's new use is intended for patients with HER2-positive, locally advanced, inflammatory or early stage breast cancer (tumor greater than 2 cm in diameter or with positive lymph nodes) who are at high risk of having their cancer return or spread (metastasize) or of dying from the disease. It is to be used in combination with trastuzumab and other chemotherapy prior to surgery and, depending upon the treatment regimen used, may be followed by chemotherapy after surgery. Following surgery, patients should continue to receive trastuzumab to complete one year of treatment.

"We are seeing a significant shift in the treatment paradigm for early stage breast cancer," said Richard Pazdur, M.D., director of the Office of Hematology and Oncology Products in the FDA's Center for Drug Evaluation and Research. "By making effective therapies available to high-risk patients in the earliest disease setting, we may delay or prevent cancer recurrences.''

In May 2012, the FDA issued a **draft guidance (/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM305501.pdf)** about the use of pathologic complete response (pCR), defined as the absence of invasive cancer in

the breast and lymph nodes, as an endpoint to support accelerated approval of a drug for neoadjuvant treatment of high-risk, early stage breast cancer. Under the FDA's accelerated approval program, patients are provided access to promising drugs to treat serious or life-threatening conditions while confirmatory clinical trials are conducted.

Perjeta's accelerated approval for neoadjuvant treatment is based on a study designed to measure pCR. In the study, 417 participants were randomly assigned to receive one of four neoadjuvant treatment regimens: trastuzumab plus docetaxel, Perjeta plus trastuzumab and docetaxel, Perjeta plus trastuzumab or Perjeta plus docetaxel. About 39 percent of participants who received Perjeta plus trastuzumab and docetaxel achieved pCR, compared to about 21 percent who received trastuzumab plus docetaxel.

The confirmatory trial for this accelerated approval is being conducted in participants with HER2-positive breast cancer who had prior breast cancer surgery and are at high risk of having their cancer return. More than 4,800 participants are enrolled in this trial, which will provide further data on efficacy, safety and long-term outcomes. Results are expected in 2016.

The most common side effects reported in participants receiving Perjeta plus trastuzumab and docetaxel were hair loss, diarrhea, nausea and a decrease in infection-fighting white blood cells. Other significant side effects included decreased cardiac function, infusion-related reactions, hypersensitivity reactions and anaphylaxis.

The FDA reviewed Perjeta's use for neoadjuvant treatment under the agency's priority review program, which provides for an expedited review of drugs that may offer major advances in treatment.

Breast cancer (http://www.cancer.gov/cancertopics/types/breast) is the second leading cause of cancer-related death among women. An estimated 232,340 women will be diagnosed with breast cancer, and 39,620 will die from the disease in 2013, according to the National Cancer Institute. Almost 20 percent of breast cancers have increased amounts of the HER2 protein.

Perjeta is marketed by Genentech, a member of the Roche Group, based in South San Francisco, Calif.

For more information:

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

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

NCI: Breast Cancer (http://www.cancer.gov/cancertopics/types/breast)

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, 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.

#

Read our Blog: **FDA Voice (https://blogs.fda.gov/fdavoice/)**

Visit the FDA on **Facebook (http://www.facebook.com/FDA)**, **Flickr (http://www.flickr.com/photos/fdaphotos/)**, **YouTube (http://www.youtube.com/user/USFoodandDrugAdmin?blend=23&ob=5)** and **Twitter (http://twitter.com/us_fda)**
(http://www.fda.gov/AboutFDA/AboutThisWebsite/WebsitePolicies/Disclaimers/default.htm)

RSS Feed for **FDA News Releases (http://www.fda.gov/AboutFDA/ContactFDA/StayInformed/RSSFeeds/PressReleases/rss.xml)**