

FDA approves first drug for bone marrow disorder

16 November 2011, By MATTHEW PERRONE , AP Health Writer

The first drug to treat a rare disorder that causes red blood cells to build up inside bone marrow was cleared Wednesday by the Food and Drug Administration

The twice-a-day pill Jakafi from Incyte Corp. was approved to treat [myelofibrosis](#), which causes anemia, fatigue, pain and swelling of the spleen. The disease spurs abnormal blood cells to build up in bone marrow, forming thick [scar tissue](#) that slows the production of healthy blood cells. To make up for the shortage, other [internal organs](#) including the liver and the spleen begin producing blood cells.

Incyte estimates the disease affects between 16,000 and 18,500 people in the U.S., though precise figures are not available. The disease is currently treated with chemotherapy or [bone marrow transplant](#), though some patients are not eligible for the procedure.

The FDA approved the drug based on two studies including 528 patients with the disease. Patients were randomly assigned to receive a placebo or Jakafi. More patients in the drug group saw a significant reduction in the size of their spleen as well as a 50 percent decrease in symptoms, including pain, discomfort and night sweats.

The drug, known as ruxolitinib, works by blocking two enzymes associated with the disease.

The FDA reviewed Jakafi under its priority review program for important new therapies, which aims to clear drugs in six months instead of the usual 10.

Side effects reported in patients taking the drug included diarrhea, headache, dizziness and nausea.

Jakafi is the first drug to reach the market from Wilmington, Del.-based Incyte. The company has

partnered with Swiss drugmaker Novartis, which holds foreign marketing rights to the drug.

Incyte plans to launch Jakafi next week through specialty pharmacies throughout the U.S., according to a company statement.

Company shares rose \$1.29, or 10.2 percent, to \$13.89 in midday trading.

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