Bloomberg

Amgen Wins U.S. Approval for Drug to Treat Rare Leukemia

By Anna Edney - Dec 4, 2014

Amgen Inc. (AMGN) won early U.S. approval for a drug that uses patients' own immune systems to fight a rare form of leukemia as regulators seek to move quicker to provide potential treatments for unmet medical needs.

The Food and Drug Administration cleared the immunotherapy, to be marketed as Blincyto, for patients with a form of acute lymphoblastic leukemia. The medicine was approved five months ahead of schedule after being designated a breakthrough therapy, which allowed Thousand Oaks, California-based Amgen additional access to FDA staff members to guide the drug through its review, the agency said yesterday in a statement.

The medicine is part of a class in one of the hottest areas of cancer research, known as immunotherapy because it stimulates patients' immune systems to work more effectively against the disease. Clinical trials on immunotherapies have shown the drugs may have a significant benefit for a minority of patients. Companies such as Merck & Co. (MRK), Bristol-Myers Squibb Co., AstraZeneca Plc (AZN) and Roche Holding AG began at least 78 clinical trials on such drugs last year.

"Immunotherapies, especially Blincyto with its unique mechanism of action, are particularly promising for patients with leukemia," Richard Pazdur, director of the Office of Hematology and Oncology Products in the FDA's Center for Drug Evaluation and Research, said in the statement. "Recognizing the potential of this novel therapy, the FDA worked proactively with the sponsor under our breakthrough therapy designation program to facilitate the approval of this novel agent."

Melanoma Treatment

The FDA approved another immunotherapy in September, Keytruda from Merck to treat advanced melanoma, two months before the agency was scheduled to make a decision. Keytruda is also designated as a breakthrough therapy.

Blincyto targets Philadelphia chromosome-negative precursor B-cell acute lymphoblastic leukemia, which causes the body to make too many lymphoblasts, an immature type of white blood cell, the FDA

said. The Philadelphia chromosome is an abnormality that sometimes occurs in the bone marrow cells of leukemia patients, according to the agency. The National Cancer Institute estimates 6,020 Americans will be diagnosed with acute lymphoblastic leukemia this year.

'Significant Milestone'

"The approval of Blincyto represents a significant milestone in immunotherapy research, providing clinicians the opportunity to offer a new single-agent therapy to patients fighting this highly aggressive cancer with previously limited options," Anthony Stein, a clinical professor of hematology/oncology at City of Hope, a Duarte, California-based cancer center, said in a statement from Amgen.

The price for Blincyto won't be made public until the drug is available to patients in a few weeks, said Danielle Bertrand, a spokeswoman for Amgen's Onyx Pharmaceuticals subsidiary, which will sell the drug in the U.S.

The drug is part of an agreement between Amgen and Tokyo-based Astellas Pharma Inc. to bring treatments to market in Japan.

Blincyto is approved for patients whose cancer returned after treatment or didn't respond to previous therapy. Clinical trials showed 32 percent of 185 adults with the disease who took the drug were in complete remission for about 6.7 months. Amgen still must prove the medicine will increase survival to keep the drug on the market, the FDA said.

Blincyto carries a boxed warning that some clinical trial participants had problems with low blood pressure and difficulty breathing. Ariad Pharmaceuticals Inc. for several months last year suspended marketing of Iclusig, a drug for a similar form of leukemia, because it was linked to an increased risk of blood clots in clinical trials.

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