

F.D.A. Gives Quick Approval To Two Drugs to Treat AIDS

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In an action long sought by AIDS patients and their advocates, the Food and Drug Administration yesterday approved two new drugs to treat serious complications of AIDS infections.

One of the drugs, ganciclovir (pronounced gan-CY-clo-veer), was approved for full-scale marketing without even undergoing a rigorous clinical trial. Ganciclovir, which is used to treat a viral infection of the eye that blinds many AIDS patients, is only the fourth drug to win full marketing approval for the treatment of AIDS or its complications.

The obstacles to treating AIDS were underscored yesterday with the disclosure that researchers had found several blood samples in New York City infected with a second strain of the AIDS virus, one that has been considered very rare in the United States. [Page B1.] Ganciclovir had been the subject of an intensive campaign for approval, conducted by activists for AIDS patients, their doctors and one of the Government's top officials on AIDS, Dr. Anthony Fauci, director of the National Institute of Allergy and Infectious Diseases.

These groups argued that there were sufficient data from doctors' experience with the drug to demonstrate its effectiveness even without a clinical trial. Although the drug's manufacturer, Syntex Corporation of Palo Alto, Calif., tried to start a clinical trial in January, only 18 people signed up.

The second drug approved yesterday, erythropoietin (pronounced e-rith-roh-POY-e-tin), was not cleared for full-scale marketing but was allowed expanded premarket distribution as an "investigational new drug for treatment." It is the fourth drug to win approval for use while clinical trials continue to determine whether the drug is truly effective. The others are ganciclovir, pentamidine in its aerosol form and trimetrexate. Pentamidine is used to prevent pneumonia, and trimetrexate is used to treat pneumonia.

Erythropoietin, which had been approved previously for use in patients whose kidneys have failed or are in danger of failing, will be used to treat a severe anemia that afflicts many AIDS patients, either because of the AIDS virus itself or as a side effect of treatment with azidothymidine, or AZT. AZT is the only drug approved for

treating the AIDS virus itself. The other drugs with full marketing approval for use in AIDS treatment include pentamidine, which is used only for pneumonia, and alpha interferon, for treating an AIDS-related cancer.

The anemia forces most patients who are afflicted to have regular transfusions. The drug's manufacturer, Ortho Pharmaceuticals of Raritan, N.J., a subsidiary of Johnson & Johnson, said it will not charge for the drug.

The F.D.A.'s decision to approve erythropoietin was based on a preliminary study of about 100 people with acquired immune deficiency syndrome. The F.D.A. plans to continue to study the drug more rigorously at the same time it makes it available to patients. 'This Is a Good Day'

Erythropoietin was also recently approved for marketing for the treatment of anemia associated with severe kidney disease. In theory, this would allow AIDS patients to receive it, because doctors are allowed to prescribe a drug for any disease once it is approved for at least one disease. But AIDS patients may have had difficulty obtaining it because many insurance companies would not pay for the drug, which costs \$8,000 a year, without F.D.A. approval of its use in AIDS patients. Now that the manufacturer plans to provide it without charge for AIDS patients while it is still being tested, the payment issue is moot.

Dr. Frank E. Young, commissioner of the F.D.A., said in a telephone interview that the two approvals demonstrate his agency's determination to release new drugs to desperately ill patients. "This is an example of what we've been trying to do," Dr. Young said. "This is a good day."

Dr. Young added that the approval for use of erythropoietin while it is still being tested will essentially be the new "parallel track" in drug development, a policy that Dr. Fauci advocated over the weekend to allow patients to receive a drug at the same time as it is being studied in clinical trials.

Doctors and advocates for people with AIDS were elated by the F.D.A.'s actions. Dr. Douglas Dieterich, a gastroenterologist at New York University School of Medicine who has a large AIDS patient practice and who helped evaluate ganciclovir, said:

"This is great news. This is the new F.D.A." Judgment Is Withheld

The approval of ganciclovir "is a big victory for the AIDS community," said Mark Harrington of Act-Up, an advocacy group for AIDS patients. Mr. Harrington said his group is withholding judgment on the decision on erythropoietin until it sees how accessible the drug is under the designation that makes it available while tests continue.

The eye infection that is treated by ganciclovir afflicts as many as one AIDS patient in four and, if untreated, leads to blindness. The infection, called CMV retinitis, is caused by the cytomegalovirus, which normally is kept in check by the immune system. In AIDS patients, whose immune systems are devastated, the virus can run rampant. In addition to causing blindness, it can also infect the gastrointestinal tract, causing severe, unrelenting diarrhea, or the lungs, causing pneumonia.

The best evidence that ganciclovir is effective comes from studies of patients with eye infections.

The evidence consists of doctors' clinical experience, showing that when patients receive the drug their eye infections stop progressing across the retina, but when the drug is discontinued the infection creeps over the eye again, destroying cells in its wake.

In addition, Dr. Douglas A. Jabs of Johns Hopkins University presented evidence to the F.D.A. of a preliminary study in which he compared patients who received ganciclovir with those who chose not to. Although the study lacked the customary randomized controls, it played a major role in convincing the F.D.A. to approve the drug, Dr. Young said. Facing a Terrible Choice

Ganciclovir is no panacea, however. It must be taken intravenously, and patients who choose to take it must have a permanent catheter implanted for the drug infusions. In addition, most patients cannot take it and AZT at the same time, and so they are faced with the wrenching choice of their eyesight or a longer life. Most choose their eyesight, doctors say.

Linda Thomas, a spokeswoman for Syntex, said that the cost of the drug to wholesalers will be \$29 a dose. Patients will receive two doses a day for the first two to three weeks and then one dose a day for the rest of their lives.

Erythropoietin is a hormone produced naturally by the kidney that spurs red blood cell production by the bone marrow. The Ortho drug is a genetically engineered replica of the natural hormone.

Both AZT and AIDS virus infections themselves can lead to a deficiency in erythropoietin and a severe anemia. About one-third to one half of the 20,000 patients who currently take AZT are anemic from the drug. In small studies, investigators have shown that AIDS patients that make insufficient quantities of the hormone can be helped if they receive Ortho's drug. Many Require Transfusions

Dr. David Henry, a hematologist at the Graduate Hospital of Philadelphia and an investigator in the study of erythropoietin in AIDS, said that, in his experience, "roughly three-quarters of AIDS patients taking AZT require transfusions." They typically have transfusions, "four, six, eight times a month," Dr. Henry said. Erythropoietin, by relieving their anemia, "will free them from the hospital," Dr. Henry added.

Dr. Seth Rudnick, senior vice president for biotechnology research and development at Ortho's R. W. Johnson Pharmaceutical Research Institute, said that the company will initially supply erythropoietin through the 30 to 50 physicians in major metropolitan areas across the United States who have participated in the company's studies of the drug.

There are enough of these doctors, Dr. Rudnick said, that "most patients will be able to get the drug from the beginning." He said that "as we get more experience, we will expand so that any clinic which specializes in treating AIDS patients will be able to call Ortho and enroll patients."