LA JOLLA, Oct. 19 -- The U.S. Food and Drug Administration today announced approval of a highly purified concentrate of Factor VIII, a blood coagulation protein used to treat hemophilia. The purification process, developed at Scripps Clinic and Research Foundation, allows treatment of hemophiliacs with a greatly reduced risk of AIDS, hepatitis and other viral infection.

Until now, hemophiliacs have had to risk treatment with concentrates of Factor VIII contaminated with viruses present in the blood of the original donor - and they almost invariably develop non-A, non-B hepatitis, which cannot be screened from the blood supply. In recent years, up to 90 percent of hemophiliacs who received concentrates in the early 1980s have shown evidence of infection with the AIDS virus, and between 1 percent and 2 percent have developed acquired immune deficiency syndrome or have died from the disease.

The process of purifying the Factor VIII protein from normal blood plasma was developed by Drs. Theodore Zimmerman and Carol Fulcher of the Research Institute of Scripps Clinic basic and clinical research department.
Commercial development and clinical trials of the new concentrate, known commercially as Monoclate, were funded by Armour Pharmaceutical Co., which will undertake its worldwide marketing and distribution. Armour is a division of Rorer Group Inc. of Fort Washington, Penn.

The specific activity, or units per milligram, of Monoclate is at least 1,000 times greater than Factor VIII concentrates previously available. The purity is 99.9 percent, compared to other commercial Factor VIII products, which have a purity of less than 1 percent, according to Rorer officials.

In clinical trials of Monoclate, conducted at various institutions throughout the country, the concentrate thus far has not transmitted viral infection. Monoclate, self-administered intravenously, also solves another common problem of crude concentrates: It does not depress the immune system. In fact, in some hemophiliacs treated with Monoclate, including those exposed to the AIDS virus, depression of some aspects of the immune system was stopped or reversed.

"The possibility of widespread application of a purified Factor VIII concentrate only became apparent when we realized how relatively simple the process was, and that it had an excellent yield," said Zimmerman. "And, of course, the AIDS epidemic makes very clear the importance of such a technique."

The method of purifying Factor VIII involves exposing plasma or a Factor VIII-containing fraction of plasma to monoclonal antibodies attached to a bed of inert material. These antibodies bind and retain the Factor VIII complex while all other materials, including viruses, are washed away. Pure Factor VIII is then stripped from the antibodies, collected, heated, frozen and dried to ensure stability.
Zimmerman and Fulcher's work, funded in part by the National Institutes of Health and Armour, began in 1980 as a research project to characterize Factor VIII, a coagulation factor essential for the formation of fibrin, an insoluble protein that binds platelets together into a tough, wound-sealing mass - a blood clot.

Hemophilia, one of more than 1,400 diseases now known to result from the inheritance of a single mutant gene, is a blood-clotting disorder affecting one in 10,000 people worldwide. About 80 percent of hemophiliacs are deficient in Factor VIII, the most severely affected having less than 1 percent of normal levels in their blood.

Although hemophilia is incurable, for the past 30 years victims undergoing bleeding episodes have taken supplements of Factor VIII extracted from normal blood plasma. In most cases, this extra Factor VIII is sufficient to allow blood clotting, but has carried the risk of viral infection.

Zimmerman, a graduate of Harvard Medical School, Boston, has been at Scripps Clinic and Research Foundation for 17 years. Chief of the Scripps Clinic Coagulation Laboratory, he is head of the experimental hemostasis division in the Basic and Clinical Research Department. In July 1985, he was appointed chairman of the National Hemophilia Foundation's Medical and Scientific Advisory Council.

Fulcher, a graduate of the University of Virginia, Charlottesville, has been at Scripps Clinic and Research Foundation since 1979.

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