LA JOLLA, Oct. 14 -- Supported by a newly awarded orphan drug grant from the U.S. Food and Drug Administration, Scripps Clinic and Research Foundation is conducting a human trial of a promising new anti-lymphoid cancer drug.

The agent, 2-chlorodeoxyadenosine, or 2-CdA, has given "spectacular results" in leukemia patients and is considered "one of the most promising new agents of the decade," said Dr. Ernest Beutler, chairman of the Research Institute's basic and clinical research department.

Collectively, the types of tumors receptive to 2-CdA therapy are common; individually, they are not. FDA orphan drug grants are designed for such projects - those that show promise but, since they have little commercial potential, do not attract industry support. Scripps Clinic received a three-year, $250,000 grant.

Undergoing trials only at Scripps Clinic, the single institution with FDA approval to use the drug, 2-CdA was synthesized by Dr. Dennis Carson, head of the clinical immunology division. So far, it has been tested on approximately 100 patients suffering various lymphomas, acute leukemia, chronic lymphocytic leukemia, hairy-cell leukemia, autoimmune hemolytic anemia and chronic immune thrombocytopenic purpura, a disease in which the body produces antibodies against its own blood platelets.

(More)
The two patients with advanced hairy-cell leukemia have had complete remission, one of them for a year and a half following a single seven-day course of continuous infusion; these two cases were reported in the Oct. 1. New England Journal of Medicine. In 60 percent of the patients with chronic lymphocytic leukemia resistant to all other treatments, there was marked improvement.

"In some cases, the response is spectacular," said Beutler. "We still have a lot to learn about this therapy, but we're very confident. This may be the most promising anti-lymphoid cancer agent to emerge this decade."

The drug has other advantages, including low toxicity.

"None of the patients treated has suffered hair loss, nausea, liver failure or central nervous system effects," said Dr. Lawrence Piro, a principal clinician in the trial.

Other uncommon features of 2-CdA, which causes chromosome breaks in cancerous cells, making replication impossible, are that it works on resting cells as well as those dividing, it is highly selective for white blood cells, and is particularly active against immune system cells.

Patients in the study are treated at Scripps Clinic's General Clinical Research Center, a National Institutes of Health-designated center for the development and evaluation of experimental therapies.

Other principal investigators in the clinical trial are Drs. Carlos Carrera and William Miller.

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