MedicalXpress

Study points way to development of drugs for deadly childhood leukemia

December 14 2009

A new study could point the way to the development of better drugs to fight a deadly form of childhood leukemia called mixed-lineage leukemia (MLL).

The study will help researchers in their search for what could be the first highly effective drug for MLL. Such a drug would work by disabling a protein that turns normal blood cells into cancer cells.

Researchers from Loyola University Chicago Stritch School of Medicine and the University of Virginia reported results online Dec. 13 in the journal *Nature Structural & Molecular Biology*.

"This hopefully will lead to an effective therapeutic approach for patients who generally do not do well with current treatments," said second senior author Nancy Zeleznik-Le, Ph.D., a professor in the Department of Medicine at Loyola Stritch.

Acute MLL accounts for about 80 percent of infant leukemias. While survival rates for most types of childhood leukemia are high, only about one-third of patients with MLL live longer than five years. Existing drugs have limited effectiveness and often cause toxic side effects.

MLL is caused by a critical gene that regulates hundreds of other genes in blood cells. The problem occurs when this regulatory gene breaks in half and another gene attaches to it, creating a fusion gene. It's this fusion gene that turns a normal cell into a proliferating cancer cell.

This fusion gene codes for a MLL fusion protein. The MLL fusion protein in turn binds to hundreds of other genes. Consequently, these genes are permanently turned on. So instead of aging and dying like a normal cell, the cell turns cancerous, continually growing and dividing into new cancer cells.

The finding will be a big help in the effort to develop a drug that prevents the MLL fusion protein from binding to other genes, Zeleznik-Le said. The National Institutes of Health has begun screening compounds that might prevent such binding. Zeleznik-Le said researchers likely will be ready to test potential drug compounds on laboratory animals within a year.

Source: Loyola University Health System (news : web)