An experimental drug derived from cottonseeds shows promise in treating the recurrence of glioblastoma multiforme, widely considered the most lethal brain cancer, said researchers at the UAB Comprehensive Cancer Center.

The new results are from a Phase II clinical trial of AT-101, a pill manufactured from a potent compound in cottonseeds that overcomes the abnormal growth patterns of tumor cells. This cottonseed-based agent must be properly dosed and monitored by physicians.

In clinical tests, AT-101 halted the cancer's progression in many of the 56 patients, said John Fiveash, M.D., an associate professor in the UAB Department of Radiation Oncology and the lead researcher on the new study. Despite undergoing other treatments, including surgery, chemotherapy and radiation, the trial patients' brain cancer had begun to grow again prior to starting AT-101 treatments. The trial-monitored patients took only AT-101 daily for three out of four weeks.

Glioblastomas are more common in adults and are considered fast-growing brain tumors that are very difficult to treat, Fiveash said. "After getting this drug some of these patients went many months without any new growth in their tumors," Dr. Fiveash said. "We are able to do that with a well-tolerated oral medication, and that is a major benefit."

His initial results will be presented May 30 during the poster discussion of central nervous system tumors at the American Society for Clinical Oncology annual meeting in Orlando, Fla.

Dr. Fiveash said the drug would likely work best in combination with radiation and chemotherapy to boost the cancer-fighting properties of those treatments. Also, investigators are trying to learn which patients are most likely to benefit from AT-101.

The AT-101 trial is a partnership that includes Dr. Fiveash, the UAB Comprehensive Cancer Center, Massachusetts General Hospital in Boston, Johns Hopkins University in Baltimore, The Cleveland Clinic in Ohio, Henry Ford Hospital in Detroit, Emory University in Atlanta, Moffit Cancer Center in Tampa, the University of Pennsylvania in Philadelphia, Wake Forest University in Winston-Salem, N.C., and the National Cancer Institute's Cancer Therapeutics Evaluation Program.

AT-101 is manufactured by Ascenta Therapeutics Inc., based in Malvern, Penn. Multiple preclinical and clinical trials with AT-101 are ongoing in several tumor types, including prostate, lung, B-cell malignancies and other forms of cancer.

The UAB Comprehensive Cancer Center is the only one in a five-state region to have the National Cancer Institute's comprehensive designation. The center collaborates with the UAB Department of Radiation Oncology and other divisions to remain a world leader in groundbreaking translational research and leading-edge patient care.

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