

Targeted treatment slows progression of rare connective tissue tumor

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In a phase three clinical trial, a drug called sorafenib stopped progression of desmoid tumors for two years in 80 percent of patients who completed treatment, a significant increase in progression-free survival compared with placebo. (Progression-free survival is the length of time a patient lives without worsening of the disease).

Results of the multicenter trial were published in the *New England Journal of Medicine*.

There is no standard of care for [patients](#) with desmoid tumors. "In general, desmoids are locally aggressive and often painful tumors for which there are no [effective therapies](#)," says Gary K. Schwartz, MD, chief of hematology/oncology at NewYork-Presbyterian/Columbia University Irving Medical Center and a senior author of the paper. "Sorafenib is an oral agent that provides a new means to directly target the ability of desmoid tumors to grow."

Desmoid tumors (also called aggressive fibromatosis) are abnormal growths that arise in connective tissue and can occur anywhere in the body. Desmoid tumors are not considered cancerous, because they do not spread to other parts of the body. They do not often cause death, but these tumors can cause significant health problems by invading surrounding tissues, causing pain, limiting mobility, and interfering with organ function. About 1,000 new cases, mostly in young adults, are diagnosed in the U.S. each year.

Desmoid tumors are very complex and display a wide range of behaviors, even in the same patient. Some tumors shrink spontaneously, some remain stable, while still others grow aggressively. Treatment varies from patient to patient. In the past, most patients were treated with surgery, but because there is a [high risk](#) that the [tumor](#) will return, surgery is now mainly used for tumors that are thought to have a low risk of recurrence. Radiation, chemotherapy, and hormonal treatments are sometimes effective in reducing tumor size and alleviating pain.

In this randomized, double-blinded trial, 87 patients with progressive, symptomatic, or recurrent desmoid tumors were given either oral sorafenib or placebo until scans showed disease progression.

Sorafenib is a targeted therapy that acts on tyrosine kinases—enzymes that have been implicated in [cancer development](#)—to inhibit the growth of cancer cells and the formation of new blood vessels that support tumor development. The drug has been approved by the FDA to treat certain types of advanced kidney, liver, and thyroid cancer. It has not yet been approved for the treatment of desmoid tumors.

The study is ongoing, but the researchers estimate that progression-free survival after two years is 81 percent in those taking sorafenib, and only 36 percent in those taking placebo. "This is a truly remarkable outcome," says Schwartz, who is also a professor of oncology at Columbia University Vagelos College of Physicians and Surgeons. "In fact, we have never seen results like this in the treatment of desmoid tumors."

Side effects, including rash, hypertension, and diarrhea, were more common in patients taking sorafenib than in those taking placebo.

The study was designed to measure sorafenib's effect on progression-free survival. It did not address whether the drug led to meaningful improvements in pain or other symptoms.

In addition, 22 percent of patients in the treatment group discontinued therapy due to adverse events, such as fatigue and rash.

Additional [clinical trials](#) are needed to identify the optimal dosage to reduce the risk of treatment-related [adverse events](#) and determine whether an increase in progression-free survival also leads to improvements in pain relief, function, and quality of life.

Researchers are currently analyzing tumor samples obtained in this trial to better understand how sorafenib affects desmoid tumors at the molecular and cellular levels. Understanding [sorafenib](#)'s mechanism of action could lead to the development of additional therapies in the future.

The study is titled, "Sorafenib in Advanced and Refractory Desmoid Tumors."

More information: "Sorafenib in Advanced and Refractory Desmoid Tumors" *New England Journal of Medicine* (2018). [DOI: 10.1056/NEJMoa1805052](#)

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