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stable disease. The mean tumor size reduction among all 14 patients was 61 percent.

The drug, PLX3397, is a tyrosine kinase inhibitor that potently inhibits the colony stimulating factor 1 (CSF1) receptor kinase, a driving force in the development and growth of PVNS.

“By taking this drug that potently inhibits a single genetic process, several patients with advanced PVNS appeared to experience, in a relatively short amount of time, relief from pain and stiffness as well as marked improvement in joint function, all with minimal side effects,” says [William D. Tap, MD](#), lead author on the study and Chief of the [Sarcoma Medical Oncology Service](#) at Memorial Sloan Kettering. “This is a proof-of-principle study that demonstrates the powerful clinical benefit of matching the right drug to the right molecular abnormality and further spurs excitement over the potential of precision medicine.”

PVNS is a rare and destructive joint disorder that affects approximately 600 young and middle-aged adults in the United States each year. Patients with PVNS experience an inflammation and overgrowth of the synovium, or joint lining, which results in swelling, pain, and reduced mobility in the affected joint. PVNS is not considered a cancer because it usually remains within a single joint rather than metastasizing, and is not known to cause death. The knee is the most common site affected, followed by the hip.

PVNS tumors express high levels of CSF1, a protein that continually attracts an abundance of cells with CSF1 receptors, such as macrophages, to the joint. This influx of cells causes the inflammation and overgrowth of the joint lining, which can destroy the joint over time.

There are no drugs approved to treat advanced PVNS. Surgery to remove as much of the tumor as possible is the standard of care, with patients sometimes receiving local radiation. However, because these tumors are locally invasive — wrapping around bone, tendons, ligaments, and other parts of the joint — the disease may recur, requiring additional surgery or joint replacements and eventually advancing to the point where it is no longer operable. These patients have few treatment options, among them

amputation.

In this study, 23 patients with advanced PVNS in a single joint received 1000 mg of PLX3397 orally each day. Patients underwent an MRI every two months to measure tumor volume using a novel tumor volume score developed for PVNS. Among the 14 patients with evaluable MRI scans, the mean tumor size reduction was 61 percent. Eleven patients experienced a partial response (at least a 50 percent decrease in tumor volume compared to baseline screenings) and three had stable disease. Patients remained on the drug until disease progression or intolerability.

The most common treatment-related side effects were hair-color changes, fatigue, nausea, swelling around the eyes, abnormal taste, diarrhea, vomiting, and decreased appetite.

Dr. Tap will present data from the trial on Sunday, June 1, at the ASCO Annual Meeting.

PLX3397, which received orphan drug designation by the FDA in February 2014, was discovered and is being developed by Plexxikon, who funded the phase I trial. The drug is moving to an international phase III clinical trial, which Dr. Tap is helping to organize.

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