



CENTER FOR SARCOMA AND BONE ONCOLOGY

TENOSYNOVIAL GIANT CELL TUMOR (TGCT)

RESEARCH DESCRIPTION

Sometimes, drugs that have been approved by the FDA for one medical condition turn out to be effective for a different condition, as well. Strategically repurposing drugs in this way often expedites investigations into treatments for rare diseases. In one such effort, [Andrew Wagner, MD, PhD](#), collaborated with investigators at peer institutions on a retrospective study of the activity of the drug imatinib in patients with locally advanced or metastatic TGCT, also known as pigmented villonodular synovitis. Imatinib, which is FDA-approved for chronic myelogenous leukemia (CML) and gastrointestinal stromal tumor (GIST), is known to inhibit a protein connected with the growth of TGCT.

For the study, researchers analyzed the response of 27 patients who had received imatinib at one of 12 institutions in the United States, Australia, and Europe. The results, reported in the March 2012 issue of the journal *Cancer*, indicated significant activity, with five patients experiencing full or partial response and 20 achieving stable disease. More than half of the patients reported having less severe symptoms while taking the drug. Importantly, this study provided proof of concept for inhibiting the protein acted on by imatinib as a treatment for TGCT. Investigators are currently pursuing molecular studies to help identify markers that may help predict patient response to imatinib in order to optimize the therapeutic value of this promising option.