# FDA Approves Pexidartinib, a Targeted Therapy for a Tumor of the Joints

By Julie Grisham, **Monday, August 5, 2019**

Medical oncologist and sarcoma expert William Tap led the clinical trials for pexidartinib.

## Summary

> Pexidartinib is the first drug approved specifically to treat the rare joint tumor tenosynovial giant cell tumor, which is also known as pigmented villonodular synovitis.

On August 2, the US Food and Drug Administration announced that it had approved pexidartinib (Turalio™) for certain people with tenosynovial giant cell tumor (TGCT). It is the first drug approved specifically to treat this rare tumor of the joints.

Memorial Sloan Kettering medical oncologist and **sarcoma** expert **William Tap** led the clinical trials for this drug. The results of a phase III **study were published** in June 2019 in *The Lancet*.

"For the right patient, this is a drug that can really help," Dr. Tap says. "However, because of the potentially serious side effects, it's important to consult with doctors who understand this disease and this drug."

## A Valuable Drug for a Debilitating Condition

TGCT, also called pigmented villonodular synovitis (PVNS), is not considered a cancer because it doesn't spread to other parts of the body. But it is a condition that can be painful and debilitating. It most often affects the knees. The disease is most often treated with surgery. If it continues to come back, people with the condition may run out of treatment options.

---

> Tenosynovial giant cell tumor (TGCT) is also called pigmented villonodular synovitis (PVNS).

---

In the phase III study, which enrolled patients in the United States, Europe, and Australia, 120 people were randomized to receive either the drug or a placebo. After nearly six months, 39% of people who got the drug had a measurable response, meaning that their tumors got smaller. Many of those who responded to the drug had noticeable improvements in range of motion and a reduction in pain in the affected joint. No one who received a placebo had any measurable response.

The drug is a targeted therapy that works by blocking a protein called colony-stimulating factor 1 kinase. This protein drives the development and growth of these tumors.

Pexidartinib is approved for people who can no longer have surgery for their tumor, or who are trying to avoid amputation. Because the drug can cause liver damage, the FDA did not approve pexidartinib for people who can be treated surgically or if the tumor is not seriously affecting a person's quality of life.

> "For the right patient, this is a drug that can really help."

**William D. Tap**
Medical oncologist

"Unfortunately, this drug can cause a specific type of liver toxicity called cholestatic hepatotoxicity," Dr. Tap explains. "It's exceedingly rare, but when it occurs, it can be very dangerous. It's important that people who get the drug are treated somewhere where they can be closely monitored for liver problems." He explains that for this reason, only certain pharmacies will be able to dispense the drug, and doctors will have to go through a certification process before they can prescribe it.

## Meaningful Improvements for a Neglected Condition

Despite the warnings, Dr. Tap says the approval of pexidartinib is an important breakthrough that can lead to meaningful improvements in many people's lives.

"TGCT has been neglected by much of the medical community and the pharmaceutical industry for a long period of time," he notes. "Even though it's rare, it has a relatively high prevalence. This is because it tends to first affect people when they are in their 20s and 30s. If it can't be successfully treated with surgery, they have to live with it for the rest of their lives. So there are a lot of people out there who are coping with this disease."

The phase III study and other research studies on pexidartinib were funded by Daiichi Sankyo, the company that developed the drug. Dr. Tap has received personal fees from the company.

PREVIOUS
‹ In the News

NEXT
Experimental Drug Targets Rare Joint Disorder ›