

# Site-Agnostic Therapies: A New Approach to Cancer Treatments

A new class of targeted therapies is changing the cancer treatment game. Instead of only treating one type of cancer, they can treat many.



As long as cancer drugs have been researched and approved, the process has worked essentially the same way. The drug is tested for a cancer that originates in one area in the body such as lung cancer, and, if proven effective in multiple clinical trials, is eventually approved by the FDA to treat that form of cancer. If the drug manufacturers want it to be tested for another type of cancer, such as prostate cancer, it has to go through the process all over again.

“We have always treated cancers by their name: breast, sarcoma, etc.,” says [Dr. Gina D’Amato](#), a sarcoma medical oncologist and assistant director of clinical research at the Sylvester Comprehensive Cancer Center. “That has always been the standard before we understood the molecular basis of cancer.”

## **Targeted therapies are ... well ... more targeted at cancer cells.**

While chemotherapy kills cells that are growing and dividing, [targeted therapies](#) stop specific molecules involved in the growth and spread of the cancer cells. “In general, targeted cancer agents and immunotherapies have fewer adverse events than older chemotherapy agents,” says [Dr. Gilberto Lopes](#), associate director for global oncology at Sylvester Comprehensive Cancer Center.

As research with targeted therapies has progressed, it's become clear that different cancers that express the same genetic mutations will produce the same molecular targets. Thus, a targeted therapy that works for lung cancer with a specific molecular



target will also work against a prostate cancer with the same molecular target. The result is “site-agnostic” cancer therapies — therapies that are highly effective in cancers that produce the same molecular target.

“The concept of site-agnostic research and approvals might change the landscape of how we treat patients with cancer,” says Dr. Lopes.

*Hopefully this will make drug development quicker and more effective.*

## What's the hold-up?

While site-agnostic therapies may represent a major cancer treatment breakthrough, the problem has been that the FDA has been slow to change their traditional approval process. “Researchers and pharmaceutical companies have been excited about these therapies for years,” says Dr. D’Amato. “We’ve just been waiting for the FDA to catch up.”

In recent years, however, the FDA has started to change the approval process.

The first site-agnostic approval was for [Keytruda \(pembrolizumab\)](#), an immune-

oncology therapy that uses the immune system to fight the cancer. It has been shown to be effective and is FDA-approved for multiple cancers such as melanoma, lung, head and neck cancer, Hodgkin's lymphoma, bladder cancer and kidney cancer.

The second major breakthrough came in November 2018, with the approval of [Vitrakvi \(larotrectinib\)](#). This drug is effective against tumors with specific mutations in certain NTRK genes. Even though this mutation is rare, if the tumor expresses it, larotrectinib is highly effective. Patients in the trial that led to the FDA approval had a variety of cancers such as thyroid, lung, and salivary gland cancers, as well as soft tissue sarcoma. Now that it is FDA-approved, patients who have tumors that express this mutation are eligible to receive the drug regardless of what type of cancer they have.

## Looking to the future

Dr. D'Amato says that site-agnostic therapies represent a significant and important cancer breakthrough. "The challenge is finding enough patients with the tumor gene mutation to perform a clinical trial," she says. "But once you identify them, you can potentially treat many different cancers with one drug."

Now that the FDA has embraced this new way of researching and approving these cancer drugs, Dr. D'Amato is optimistic that many more site-agnostic targeted therapies could be on the way in the future. In fact, many site-agnostic therapy trials are currently ongoing at the Sylvester Comprehensive Cancer Center.

Learn more about [clinical trials at Sylvester](#).

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*Wyatt Myers is a contributing writer for UMiami Health News.*

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