Understanding Targeted Therapy [1]

This section has been reviewed and approved by the Cancer.Net Editorial Board [2], 05/2016

Targeted therapy is a cancer treatment that uses drugs. However, it is different from traditional chemotherapy. The drugs known as targeted therapy help stop cancer from growing and spreading. They work by targeting specific genes or proteins. These genes and proteins are found in cancer cells or in cells related to cancer growth, like blood vessel cells.

Doctors often use targeted therapy with chemotherapy and other treatments. So it might be part of your treatment. The U.S. Food and Drug Administration (FDA) has approved targeted therapies for many types of cancer. Scientists are also testing drugs on new cancer targets.

The “targets” of targeted therapy

Knowing how cancer cells develop helps understand how targeted therapy works. First, cells make up every tissue in your body. There are many different cell types, such as blood cells, brain cells, and skin cells. Each type has a specific function. Cancer begins when specific genes in healthy cells change. Scientists call the change a mutation.

Genes tell cells how to make proteins that keep the cell working. If the genes change, these proteins change, too. This makes cells divide abnormally or live too long. When this happens, the cells grow uncontrollably. The out-of-control cells form a tumor. Learn more about the genetics of cancer [3].

Researchers are learning that certain gene changes happen in specific cancers. So they are developing drugs that target the changes. The drugs can:

- Block or turn off the signals that tell cancer cells to grow and divide,
- Keep cells from living longer than normal, or
- Kill the cancer cells.

Types of targeted therapy

There are two main types of targeted therapy:

- **Monoclonal antibodies.** Drugs called “monoclonal antibodies” block a specific target on the outside of cancer cells. Or the target might be in the area around the cancer. These drugs work like a plastic plug you put in an electric socket. The plug keeps electricity from flowing out of the socket.Monoclonal antibodies can also send toxic substances directly to cancer cells. For example, they can help chemotherapy and radiation get to cancer cells better. You usually get these drugs intravenously (IV).
- **Small-molecule drugs.** Drugs called “small-molecule drugs” can block the process that helps cancer cells multiply and spread. These drugs are usually pills you take. Angiogenesis inhibitors [4] are one example of this type of targeted therapy. These drugs keep tissue around the tumor from making blood vessels. Angiogenesis is the name for making new blood vessels. A tumor needs blood vessels to bring it nutrients. The nutrients help it grow and spread. Anti-angiogenesis therapies starve the tumor by keeping new blood vessels from developing.

Matching a patient to treatment

Studies show that not all tumors have the same targets. So the same targeted treatment does not work for everyone. For example, a gene called KRAS (pronounced kay-rass) controls tumor growth and spread. About 40% of colorectal cancers have this gene mutation. When this happens, the targeted therapies cetuximab (Erbitux) and panitumumab (Vectibix) do not work. The American Society of Clinical Oncology (ASCO) recommends that patients with metastatic colorectal cancer have their tumors tested for KRAS mutations. This helps your doctor give you the most effective treatment. It also protects you from unnecessary side effects. And you do not have to pay for drugs that probably will not help.

Your doctor might order tests to learn about the genes, proteins, and other factors in your tumor. This helps find the most effective treatment. Many targeted therapies cause side effects. Also, they can be expensive. So doctors try to match every tumor to the best possible treatment. Learn more about the importance of molecular testing [5].

Examples of targeted therapies

Below are a few examples of targeted therapies. Ask your doctor or another member of your health care team for more information.

- **Breast cancer.** About 20% to 25% of all breast cancers have too much of a protein called human epidermal growth factor receptor 2 (HER2, pronounced her-too). This protein makes tumor cells grow. ASCO and the College of American Pathologists recommend HER2 testing for everyone with invasive breast cancer [6]. If the cancer is HER2 positive, several targeted therapies are available.

- **Colorectal cancer.** Colorectal cancers often make too much of a protein called epidermal growth factor receptor (EGFR). Drugs that block EGFR may help stop or slow cancer growth. These cancers have no mutation in the KRAS gene. Another option is a drug that blocks vascular endothelial growth factor (VEGF, pronounced vedge-eff). This protein helps make new blood vessels.

- **Lung cancer.** Drugs that block the protein called EGFR may stop or slow down lung cancer. This may be more likely if the EGFR has certain mutations. Targeted therapy is also available for lung cancer with a mutation in the ALK gene. Doctors can also use angiogenesis inhibitors for certain lung cancers.

- **Melanoma.** About half of melanomas have a mutation in the BRAF gene (pronounced bee-raff). Researchers know specific BRAF mutations make good drug targets. So the FDA has approved several BRAF inhibitors. But these drugs can be dangerous if you do not have the BRAF mutation.

The list above does not include every targeted therapy. Researchers are studying many new targets and drugs. You can learn more about specific drugs and targeted therapy in other cancers in the cancer type guides [7]. Look at the Treatment Options and Latest Research pages.

Challenges of targeted therapies

Using a drug that works on your specific cancer may seem simple. But targeted therapy is complicated and not always effective. It is important to remember that:

- A targeted treatment will not work if the tumor does not have the target.

- Having the target does not mean the tumor will respond to the drug.

For example, the target may not be as important as doctors first thought. So the drug may not help much. Or the drug might work at first but then stop working. Finally, targeted therapy drugs may cause serious side effects. These are usually different from traditional chemotherapy effects. For example, patients getting targeted therapy often develop skin, hair, nail, or eye problems [8].

Targeted therapy is an important cancer treatment. But so far, doctors can only get rid of a few cancers with these drugs alone. Most patients also need surgery, chemotherapy, radiation therapy, or hormone therapy. Researchers will develop more targeted drugs as they learn more about specific changes in cancer cells.

More Information

Facts about Personalized Cancer Medicine [9]

Introduction to Cancer Research [10]

Additional Resources

National Cancer Institute: Targeted Cancer Therapies [11]

My Cancer Genome: Overview of Targeted Therapies for Cancer [12]

Links
[1] http://www.cancer.net/navigating-cancer-care/how-cancer-treated/personalized-and-targeted-therapies/understanding-targeted-therapy
[2] http://www.cancer.net/about-us
[3] http://www.cancer.net/node/24897
[4] http://www.cancer.net/node/24376
[5] http://www.cancer.net/node/30536
[6] http://www.cancer.net/node/29831
[7] http://www.cancer.net/cancer-types
[8] http://www.cancer.net/node/25056
[9] http://www.cancer.net/node/24522
[10] http://www.cancer.net/node/30667
[11] http://www.cancer.gov/about-cancer/treatment/types/targeted-therapies/targeted-therapies-fact-sheet
[12] http://www.mycancergenome.org/content/molecular-medicine/overview-of-targeted-therapies-for-cancer