Cancer Treatment

Targeted Therapy

Cancer treatment has evolved rapidly in recent years, from surgery, radiation and traditional chemotherapy to targeted therapy that attacks specific types of tumors and immunotherapy that helps the immune system fight cancer.

Targeted therapies work against cancer with specific gene mutations or other unique characteristics that make malignant cells different from normal cells. Some mutations either boost the activity of genes that cause uncontrolled cell growth (oncogenes) or turn off tumor suppressor genes. Targeted treatments often block processes that enable cancer to grow and spread.

Some targeted therapies are monoclonal antibodies administered by IV infusion, while others are small molecules that can be taken as pills. Unlike cytotoxic chemotherapy, which kills both cancer cells and fast-growing normal cells, leading to a wide range of adverse effects, targeted therapy acts specifically against malignant cells. Targeted medications may be better tolerated because they don't harm most types of healthy cells, but some interfere with important biological processes and can cause side effects of their own.

Types of Targeted Therapy

Many targeted therapies interfere with cell communication or signaling pathways that regulate cell multiplication and death. Genetic mutations can cause receptors and other proteins involved in these processes to be overexpressed, or overactive, in cancer cells.

One common type of targeted therapy, kinase inhibitors, blocks the action of enzymes known as protein kinases. These enzymes carry out a chemical reaction called phosphorylation (adding phosphates to molecules), a necessary step in many biological processes. Abnormal kinase activity can lead to uncontrolled cancer cell growth. Many targeted therapies are tyrosine kinase inhibitors, and some block multiple kinases.

Gleevec (imatinib), the first targeted therapy, acts on an abnormal tyrosine kinase present in a certain type of leukemia. EGFR is a receptor tyrosine kinase associated with lung and colorectal cancer. HER2 (human epidermal growth factor receptor 2) is overexpressed on some breast tumors, making them susceptible to HER2 blockers like Herceptin (trastuzumab). Up to half of melanomas have mutations affecting BRAF, a growth-promoting kinase.

Cyclin-dependent kinases (CDKs) bind to cyclins, proteins that regulate the cell division cycle. Some cancers have CDK mutations that trigger uncontrolled cell growth. New targeted therapies

for breast cancer—Ibrance (palbociclib), Kisqali (ribociclib) and Verzenio (abemaciclib)—target both CDK4 and CDK6.

Normal cells have a variety of mechanisms to repair damage to their genetic material—mechanisms that can malfunction in cancer cells. PARP inhibitors, such as Lynparza (olaparib), work by blocking a protein that plays a role in DNA repair. Inhibiting PARP leads to more DNA breaks, which can halt cell division. People with BRCA mutations, which raise the risk of breast and ovarian cancer, do not make proteins that fix this kind of DNA damage, so BRCA-related cancers are particularly susceptible to these drugs.

Some of the first targeted cancer drugs were angiogenesis inhibitors, which prevent the formation of new blood vessels needed to supply a growing tumor. Many tumors produce signals that stimulate angiogenesis, including vascular endothelial growth factor (VEGF). Some cancer drugs, such as Avastin (bevacizumab), bind to VEGF itself, while others, such as Nexavar (sorafenib), target its receptors.

A newer type of treatment, including Lumakras (sotorasib) and Krazati (adagrasib), works against cancers with KRAS mutations—a target once thought to be "undruggable."

Hormone or endocrine therapy, used to treat cancers that grow faster in the presence of sex hormones such as estrogen or testosterone, can be considered a type of targeted therapy. Hormone-blocking drugs, such as the estrogen receptor blocker tamoxifen and the androgen receptor inhibitor Erleada (apalutamide), deprive breast and prostate tumors of hormones that stimulate their growth.

Some targetable mutations occur at a low level in many different types of cancer. These include genetic alterations involving TRK and RET genes. Such mutations occur in only a small proportion of all cancers, but more often in certain rare malignancies. Site-agnostic drugs like Vitrakvi (larotrectinib) target cancer with TRK gene fusions anywhere in the body, while those like Retevmo (selpercatinib) target malignancies with RET gene alterations.

Antibody-drug conjugates (ADCs) combine immunotherapy, targeted therapy and chemotherapy. They consist of an antibody that recognizes a specific target on cancer cells and a cytotoxic drug payload. ADCs allow delivery of potent chemotherapy directly to malignant cells, reducing collateral damage to normal cells.

Making More Matches

Targeted therapies work very well for some people, but they are ineffective for others who may have the same type of cancer but with a different genomic profile. And over time, cancer cells often become resistant to targeted therapies, meaning they eventually stop working.

The increased use of targeted therapy has led to the adoption of genomic testing to help guide treatment. These tests look for targetable mutations in a tumor sample after surgery or a biopsy,

enabling doctors to select which medications are most likely to work.

Scientists have identified hundreds of so-called driver mutations that contribute to the development and progression of cancer, but still, a majority of patients do not have known mutations with matching targeted drugs. Research is ongoing to look for new targetable cancer characteristics in the hope of making precision medicine available to more people. Clinical trials can be a good way to obtain promising experimental treatments.

The following resources offer more information about targeted therapy:

American Cancer Society

National Cancer Institute

My Cancer Genome

Last Reviewed: August 25, 2025

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http://beta.docker.cancerhealth.com/basics/health-basics/targeted-therapy