Targeted therapy for cancer

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Driving the change in cancer treatment

An overview of targeted cancer therapies that change or block the effects of cancer cells.

Top target treatments work by targeting the cancer’s specific genes, proteins, or the tissue environment that contributes to cancer growth and survival. These genes and proteins are found in cancer cells or in cells related to cancer growth, like blood vessel cells. Targeted cancer therapies are also called “molecularly targeted therapies,” or “precision medicines.” In contrast to most standard chemotherapies that act on all rapidly dividing normal and cancerous cells, top target treatments act on specific molecular targets that are associated with cancer. Whereas standard chemotherapy agents are cytotoxic (killing tumor cells), targeted therapies are often cytostatic (blocking tumor cell proliferation).

Targeted therapies are a cornerstone of precision health, a concept which represents a real paradigm shift from a “one size fits all” approach to an optimised strategy for diagnosis, treatment and monitoring of disease for each individual person at the right time, based on his or her unique characteristics. Or to put it differently: going from an environment where everyone receives the same care pathway, but only some people have positive results to a world where people follow different care pathways, tailored to their needs, increasing the proportion of people with positive results.

Many targeted cancer therapies have been approved by the Food and Drug Administration (FDA) to treat specific types of cancer. Others are being studied in clinical trials, and many more are in preclinical testing. The development of targeted therapies requires the identification of good targets that play a key role in cancer cell growth and survival. One approach is to compare the amounts of individual proteins in cancer cells with those in normal cells. Proteins that are present in cancer cells but not in normal cells or that are more abundant in cancer cells would be potential targets, especially if they are known to be involved in cell growth or survival. An example of such a differentially expressed target is the human epidermal growth factor receptor 2 protein (HER-
HER-2 is expressed at high levels on the surface of some cancer cells. Several targeted therapies are directed against HER-2, including trastuzumab (Herceptin®), which is approved to treat certain breast and stomach cancers that overexpress HER-2.

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Another approach to identify potential targets is to determine whether cancer cells produce altered proteins that drive cancer progression. A third approach is to look for abnormalities in chromosomes that are present in cancer cells but not in normal cells. Sometimes these chromosome abnormalities result in the creation of a fusion gene whose fused protein may drive cancer development. Such fusion proteins can be potential targets for targeted cancer therapies.

Most targeted therapies are either small molecules or monoclonal antibodies. Small-molecule compounds are typically developed for targets that are located inside the cell because such agents can enter cells relatively easily. Monoclonal antibodies are relatively large and generally cannot enter cells, so they are used only for targets that are outside cells or on the cell surface.

Targeted therapies are an important type of cancer treatment for some cancers, but in most cases targeted drugs are used with other treatments such as chemotherapy, surgery and/or radiation therapy.