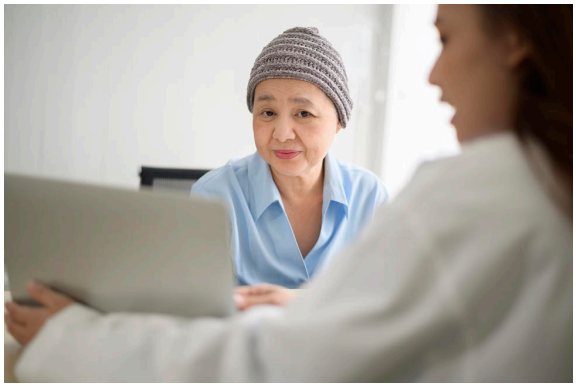
Off-Label Drug Use in Cancer Treatment

What are off-label drugs?



Off-label drugs are often used in cancer treatment.
Credit: iStock

Drugs can be legally sold in the United States only after the Food and Drug Administration (also known as the FDA) has approved them. Drugs are approved after research shows they are safe and effective for a specific use.

Off-label drug use refers to the practice of prescribing a drug for a different purpose than what the FDA approved. This practice is called "off-label" because the drug is being used in a way not described on its package insert. This insert is known as its "label."

The label describes details about the drug, such as

- what the drug is made of
- how it works in the body
- the research studies that led to its approval
- side effects it may cause

The FDA must make sure that a drug is safe and effective for a specific use. However, it does not control the decision doctors make about which drugs to use for their patients. This means that once the FDA approves a drug, doctors can prescribe it for any purpose they think makes sense for the patient.

Off-label uses may include using an approved drug

- for a different type of cancer than the one it is approved to treat
- at a different dose or frequency
- to treat a child when it is approved to treat adults

Off-label uses of a drug can become approved uses if the company that makes it obtains approval from the FDA. To gain the added approvals, the company must conduct research studies to show

Questions?

MERCK EX1206
Merck Sharp & Dohme LLC v. The Johns Hopkins University
IPR2024-00625

that the treatment is safe and effective for the new uses. However, a company may decide not to invest time and money in this research.

The role of off-label drug use in cancer treatment

Research has shown that off-label use of drugs is very common in cancer treatment. Often, usual care for a specific type or stage of cancer includes the off-label use of one or more drugs.

Off-label drug use is common in cancer treatment because

- many cancer drugs are effective against more than one type of cancer
- cancer treatment often involves the use of combination chemotherapy

  Combination chemotherapy (which is treatment using more than one drug) is effective in treating many types of cancer. Examples of combination chemotherapy include:

  - R-CVP to treat non-Hodgkin lymphoma
  - CMF and TAC to treat breast cancer
  - BEACOPP to treat Hodgkin lymphoma
  - FOLFOX to treat colon cancer

  These combinations might include one or more drugs not approved for the type of cancer they are being used to treat.

  The FDA usually does not approve combinations of chemotherapy. There are so many of them that it would not be practical to approve each combination.

- Research studies find new uses for drugs that are already approved. The results of research studies are published in medical journals and shared in the medical community. Doctors then adopt the new use and it may become an accepted and widely-used treatment for a different cancer, even if the FDA has not approved the drug for that use.

Drawbacks to off-label drug use

There are times when off-label drug use may cause harm, such as when

- it has not been shown to be effective against a certain cancer
- there is no reason to believe the drug might be effective
- the possible risks of giving the drug outweigh the possible benefits

However, if your doctor prescribes a drug for an off-label use to treat your cancer, they are basing the decision on knowledge of and experience with the drug, as well as on research that shows it

might be helpful for your stage and type of cancer.

Health insurance coverage of off-label drugs in cancer treatment

Medicare and many insurance companies pay for off-label drugs for cancer treatment, as long as the off-label uses are listed in an approved compendium. A compendium is a collection of drug summaries put together by experts who have reviewed data about the drug's use in patients.

If your doctor prescribes an off-label drug for your treatment, check your plan to make sure the drug is covered. If coverage is denied, it may be helpful for the doctor to provide the insurance company with copies of documents that support the suggested off-label use.

Questions to ask your doctor about off-label drugs

Discussing these questions with your doctor can help you understand why your doctor might prescribe an off-label drug for you.

- Why do you think the off-label use of this drug will help the type of cancer that I have?
- Is the off-label drug likely to work better than an approved drug?
- What are the risks and benefits of treatment with this drug?
- Will my health insurance cover my treatment with this drug?
- If my treatment involves combination chemotherapy and one of the drugs is off-label, will my health insurance cover it?

Related Resources

Learn How Drugs and Devices Get Approved

Reviewed: January 13, 2022