

Prescriptions for Off-Label Drugs: What You Should Know

What are “off-label” drugs?

About one in five prescriptions in the US is for “off-label” use. This means that the use was not approved by the FDA (Food and Drug Administration).

It is legal to prescribe off-label and it can be useful. But there is growing concern that off-label use:

- Is on the rise.
- Can be harmful.
- Can be a waste of money.

Why do we need off-label prescriptions?

Drug companies do studies to prove that a medicine is safe and works for one main use. But after the FDA approves a drug, people often find new ways to use it. These off-label uses may be for:

- A similar condition.
- A very different condition.
- A different group of people, like children.

Off-label use of drugs can save lives. For example, there was some evidence that aspirin could help prevent a second heart attack. Doctors started prescribing it off-label. But it took many years for large studies to take place and for the FDA to approve its use.

What are the risks?

Doctors often prescribe a drug off-label without being aware that the FDA did not approve it for that use. Many of these uses are not supported by scientific testing.

For example, the newer antipsychotics are approved to treat people with schizophrenia, bipolar disorder, and depression. But they are used off-label for many other conditions, including substance abuse, eating disorders, and insomnia. Research supports few of these uses. And the drugs can have serious side effects.

In the hospital, children are often prescribed drugs off-label, such as painkillers. Off-label prescriptions for mental health issues in children are especially worrisome.

Sometimes the benefits outweigh the risks.

There is good scientific evidence to support some off-label uses of drugs:

- Some cancer drugs can be used to treat several types of cancer. Many states require insurance companies to cover off-label use of cancer drugs.
- **Topiramate**, an anti-seizure drug, is used for the off-label treatment of alcohol dependence.
- **Amitriptyline**, an older antidepressant, is used for short-term pain relief for people with fibromyalgia. It costs much less than approved treatments.

Our advice:

Ask your doctor if your prescription is for an approved use. Or check with your pharmacist.

If a doctor prescribes an off-label drug for you or your child:

- Ask if there are studies that show it helps people with your condition.
- Ask why it is better than drugs that are approved for your condition.
- Find out if your health insurance pays for the off-label use.
- You can look up approved uses of a drug at **DailyMed** (dailymed.nlm.nih.gov). Once you find your drug, go to “Indications & Usage.”



If your doctor prescribes an off-label drug, ask about the evidence to support it.

Drugs commonly prescribed off-label

The chart below lists some drugs that are often prescribed off-label. Some uses may be appropriate and may benefit health. However, some uses may not be appropriate.

Specific Drug, Type of Drug*	Examples of Off-Label Use**
Albuterol (AccuNeb), <i>for asthma</i>	Bad coughs
Amitriptyline (Elavil), <i>antidepressant</i>	Fibromyalgia, migraines, eating disorders, pain after shingles infection
Aripiprazole (Abilify), <i>antipsychotic</i>	Dementia, Alzheimer's disease
Bevacizumab (Avastin), <i>some cancers</i>	Wet age-related macular degeneration (eye disease)
Clonidine (Catapres), <i>high blood pressure</i>	Smoking cessation, hot flashes, attention deficit/hyperactivity disorder (ADHD), Tourette's syndrome, restless legs syndrome
Gabapentin (Neurontin), <i>antiseizure</i>	Nerve pain caused by diabetes, migraines, hot flashes
Lamotrigine (Lamictal), <i>antiepileptic, antiseizure</i>	Depression, bipolar disorder, mood stabilization
Lidoderm (Lidocaine), <i>skin patch for shingles</i>	Lower back pain, sore muscles, tennis elbow
Modafinil (Provigil), <i>for excessive sleepiness</i>	To enhance wakefulness and alertness
Prazosin (Minipress), <i>high blood pressure</i>	Post-traumatic stress disorder (PTSD)
Propranolol (Inderal), <i>high blood pressure, heart disease</i>	Insomnia, anxiety, bipolar disorder, stage fright
Quetiapine (Seroquel), <i>antipsychotic</i>	Dementia, Alzheimer's disease, obsessive-compulsive disorder, anxiety, PTSD
Risperidone (Risperdal), <i>antipsychotic</i>	Alzheimer's disease, dementia, eating disorders, PTSD
Sildenafil (Viagra), <i>erectile dysfunction</i>	To enhance sexual performance for those not diagnosed with erectile dysfunction, to improve sexual function in women taking certain antidepressants
SSRI antidepressants: Paroxetine (Paxil) & Sertraline (Zoloft), <i>antidepressants</i>	Premature ejaculation, hot flashes, tinnitus (ringing in the ears)
Statins: Atorvastatin (Lipitor) & Simvastatin (Zocor), <i>high cholesterol in adults, children with inherited cholesterol condition</i>	Rheumatoid arthritis, and to lower cholesterol in children who lack the inherited condition
Tiagabine (Gabitril), <i>antiseizure</i>	Depression
Topiramate (Topamax), <i>antiseizure, in combination with phenteramine for weight loss</i>	Migraines, bipolar disorder, depression, nerve pain, alcohol dependence, eating disorders
Trazodone (Desyrel), <i>antidepressant</i>	As a sleep aid and for insomnia

*These examples are not meant to be a comprehensive list. Many of the drugs listed here are also available as generics.

**Does not imply that use is clinically appropriate or inappropriate, or beneficial or not.