

# Friday Focus

## 'Off-Label' and Investigational Use of FDA-Approved Drugs

The FDA approval process is designed to ensure that any given drug meets minimum standards for both safety and effectiveness for its approved use, but the FDA does not control which drugs providers choose to use for their patients. "Once the FDA approves a drug, doctors can prescribe it for any purpose they think makes sense for the patient" (NIH, 2014, para 4).

When a drug is approved by the FDA, the package insert, "label," lists the diagnoses/diseases for which it has been approved. Sometimes, when a drug hits the market, it is found to be effective at treating another condition; a good example of this is Wellbutrin (bupropion). While it was FDA approved for treating major depressive disorder (MDD) and seasonal affective disorder (SAD), in clinical use it was found to be effective as both an adjunct for tobacco cessation and a treatment for bipolar disorder. Ideally, when this occurs, the manufacturer would submit a request to the FDA for the investigational use of the drug to treat or diagnose the additional disease. Unfortunately, due to the expense involved in the FDA approval process, most manufacturers will not go back and seek additional FDA approval. Instead, "off-label" use may be indicated in a drug compendia or in a significant body of peer reviewed medical literature.

If physicians use a product for an indication not in the approved labeling, they have the responsibility to be well-informed about the product, meaning they should base its use on firm scientific rationale and on sound medical evidence and maintain records of the product's use and effects. Use of a marketed product in this manner *when the intent is the "practice of medicine"* does not require the submission of an Investigational New Drug Application (IND), Investigational Device Exemption (IDE) or review by an Institutional Review Board (IRB). However, the institution at which the product will be used may, under its own authority, require IRB review or other institutional oversight" (FDA, 2016, para 1).

To be covered by insurance, "off-label" or investigation use must be documented as both "medically necessary" and recognized as effective in a drug compendia or well-established as a standard of care in a significant body of peer-reviewed medical literature. Even then, it is always a good idea to verify if health insurance will cover specific "off-label" use, particularly if it is not FDA approved for the indication or in a drug compendia.

As always, we appreciate your ideas and feedback. Thank you for the quality work you do. All editions of the Friday Focus are available on the SWHP website: <https://swhp.org/en-us/prov/news/providers-friday-focus>.



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#### References

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