Is Off-Label Drug Use Safe?

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Andrew Schorr:
So, Dr. Higano, we've talked about standard of care, and we've talked about emerging modes of care.

We've talked about clinical trials. There's one other aspect that is frequently used, and some people wonder what's really going on. Let's talk about off-label use. What is off-label use of medicine, and is it appropriate in prostate cancer treatment?

Dr. Higano:
That's a loaded question.

Jeff Folloder:
Yes, it is.

Dr. Higano:
Of course, I would have to get it.

Dr. Beer:
I could weigh in on it.

Dr. Higano:
Off-label use refers to what the FDA has approved this drug for. So the FDA develops a label for a drug, and it says, "This drug is indicated for" and then, it will say specifically what the drug is supposed to be used for. Off-label use means when the physician decides to use that same drug for something that's not described by the FDA in the label—and the drug company.

And, in fact—so, this is true with cancer medications or other—all medications that we use, and the fact of the matter is that probably, most of the drugs that we use are in the off-label setting, and in prostate cancer in particular, this becomes more of an issue with a lot of the newer drugs because of the cost. So, for example, enzalutamide (Xtandi)—if you were to start using that in a patient with newly diagnosed prostate cancer, most insurers won't pay for that because that is not the indication for which it's been approved, which is in metastatic castration-resistant prostate cancer, not in newly diagnosed prostate cancer.

So, people have—some physicians are using a lot of these agents so-called off-label, and there may be situations where that's okay, and there may be situations where it's not okay. We usually like to look at off-label uses in the context of a clinical trial so that we can determine if that off-label use makes sense, and if it looks like it makes sense, then it can start being incorporated into the everyday use of that drug.

Jeff Folloder:
Anything to add?

Dr. Beer:
Yeah, I'd like to weigh in a little bit more about that. What I look for is the strength of the evidence for a clinical benefit, and that is not the same as an FDA approval. There are areas in oncology where absolutely agreed-upon standards of care are actually not FDA-approved, and that has to do with the fact that companies that own the drugs may not go to the FDA for approval.

That drug may be off-patent, it may be too expensive—a perfect example of that is the use of docetaxel (Taxotere) chemotherapy up front in hormone-responsive prostate cancer. No one disagrees in the field that that is a standard of care. We don't recommend it to every patient. I don't believe that Sanofi has gone to the FDA to gain approval for that, but the evidence is very strong and very high-quality from two large studies.

That's different than some random use of a drug that a single oncologist dreams up with no evidence. We're not in favor of that. We want to see that on a clinical trial. But there are things that we do that are very well-researched, and for one reason or another, the FDA label has not been developed to include that.