Precision Medicine: Can Biomarkers Help Patients Find a Good Fit for Clinical Trials?

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Andrew Schorr:
So, Jim, you know, the president had a big kick-off, HHS Secretary Azar I think just yesterday as we do this program, was before congress and part of it was the discussion of can we lower the cost of drugs ultimately. And one aspect of it is can we speed drug development. So instead of all these trials languishing at the cost of millions of dollars, hundreds of millions of dollars, how do we speed it up.

So one is participation, certainly, but can the process be simplified as well, Jim? What work is going on there so we can try to get these answers and get to the FDA and present the data quicker, and hopefully there's been lower cost in getting to that point?

Jim Omel:
Well, as we're learning more and more about each individual patient, personalized medicine and targeted therapy, we certainly should start relying more on biomarkers. Biomarkers can be a way to select patients that would particularly fit a given treatment.

We need to lower costs. We need to make trials slicker and faster. Single-arm trials are those in which a patient just get—all the patients get the therapy. They all get the same treatment. And FDA has actually approved drugs based on single-arm trials, a much faster and efficient way to get an answer.

The problem is that the costs are going to be there. When I think about Mike and all the work that he does in developing his venetoclax (Venclexta) trial that he mentioned, Mike has put in months or years, and it's all above and beyond his normal time. I mean his day job is to take care of patients, so all of the work that he does to develop a trial is just remarkable in the extra hours it takes and the consistency that Mike gives to doing his work. We need to make the trials more efficient.

We need to use biomarkers. We need to make them shorter. We need biostatisticians to come up with ways to give us an answer without having to approve so many hundreds or thousands of patients to all these potential new treatments.

Andrew Schorr:
So, Mike, let's talk about that. And, Mike, first of all, I want to thank you for yourwell, both of you, but, Mike, certainly in the clinic, thanks for your devotion to this.

But continuing on that, so this was brought up by Jim, biomarkers, and I know in some of the blood cancers now we're talking about more and more minimal residual disease testing, and we're doing genomic testing to see what genes have gone awry, what's our version of lung cancer or a breast cancer or a myelofibrosis or whatever it is.

And then do we qualify for a trial? What's our specific situation? Do you feel that that sort of precision medicine testing and analysis can help refine this so we know which trial is right for which person at which time and also some analysis along the way of how is it going?

Dr. Thompson:
Yeah, so at my site I'm the director for precision medicine, and I gave a talk at ASCO on precision medicine and barriers in the community setting, so I'm very passionate about that. And I think that is one of the ways you can try to get things done with smaller numbers of patients and things done faster. And part of this is alignment, right? So there's different perspectives, a patient perspective, a payer perspective, a pharma sponsor perspective, the physician. There's all these different perspectives, and I think it's trying to get them all aligned and trying to get things done faster.

So, you know, there are some areas where we don't know enough and we can't use biomarkers, but there are other areas where we have a biomarker and there's feasibility, and we can test that quickly. And if we are looking for a large effect size—here I am in jargon mode—but if you're looking for a big, big hit, a home run, is to look for an alteration that is very specific and we think is—a drug can target. So-called targeted therapy. It's a little bit of a misnomer.

So—and lung cancer has been one of the hottest places for this. So there's ALK inhibitors, ROS1 inhibitors, EGFR inhibitors, and now BRAF inhibitors, HER2 targets. So lung cancer has exploded with precision medicine therapy, and the same with melanoma and BRAF. So, you know, I think even skeptics will say you don't really need statistics if the prior therapies, nothing worked and you give something and 80 percent of people respond.

There are issues with precision medicines but the main thing is not response rate but durability. And I think that's going to be the next iteration of the NCI Match study, which is a large precision medicine study, is stop doing just these small groups of people who are showing activity but then they relapse quickly. And I think it's going to look at systems analysis and how do we overcome resistance.