The Clinical Trial Process: An Explanation of How Efficacy and Patient Safety Are Monitored

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Andrew Schorr:

So let's talk about clinical trials for a minute. So, we're at a research institution. We're actually here in Colorado in a research building. And if you go to MD Anderson, the same. And many of the major medical centers around the world. So, clinical trials. And I'll mention I've been in two. And I'm just gonna tell my story. So, my original diagnosis years ago, 1996, was chronic lymphocytic leukemia which is the most common adult leukemia. And other patients said, "You should connect with where they're doing research." And I did. It wasn't in my home city of Seattle at that time. Now they do a lot of research there. And I was in a Phase II clinical trial—so not that many patients.

And I had no treatment for 17 years afterwards. I've had treatment again for that condition, but it went 17-year remission which was unheard of. So, I'm very grateful for that. The other thing I'd say about clinical trials, I had mentioned that my original thing that led to an MPN diagnosis was having a blood clot, a DVT that you just mentioned a minute ago, Brandon.

And that being observed in a trial for a blood thinner was what then said, "Hey, as we've looked closer, you have myelofibrosis. You have an MPN." So, there are benefits of being in a clinical trial. Lindsey, when someone's in a clinical trial, you watch them super carefully, don't you? They may have extra visits, they may have extra tests, but you're watching them stem to stern, right?

Lindsey Lyle:

Absolutely. Yes. So, when you're enrolled in a clinical trial, you have a lot more bodies taking care of you. You have a clinical research nurse. And then the visits are often more frequent. And really, being in a clinical trial, a lot of patients are deterred from that or maybe scared about that because they don't know—"What does that mean? Am I even gonna get any therapy? Am I gonna be on nothing?" These sort of things. And so, clinical trials actually are a very great way to get treatment that may be better than the standard of care.

But yeah, being watched on a clinical trial—like Andrew said, perhaps if he wasn't watched so closely on this trial for the blood thinner medication, maybe these other things wouldn't have been picked up. So, we're watching patients very closely on clinical trial number one, to make sure that we're not seeing toxicity that we maybe—maybe the trials are newer in development, and so, we're not exactly sure all of the possibility toxicities.

So, we want to watch people very closely to make sure that they're not developing some bad side effects of these therapies. And then also, of course, to monitor for response or improvement in symptoms or other clinical features.

Andrew Schorr:

Okay. Brandon, let me do the little back and forth with you for a minute. So, first of all, a drug can't be approved unless it's gone through various phases of clinical trials to tell the FDA it's been safe and effective, right?

Dr. McMahon:

Correct.

Andrew Schorr:

The early trial, phase one trial, is it safe?

Dr. McMahon:

Correct.

Andrew Schorr:

Okay. Then you go to Phase II. And you're looking at a broader group of people. And you're looking at dosages, right? Optimal dose, right? And then you go to a phase three trial. How effective is it versus the standards that you—and that can take years, right?

Dr. McMahon:

Years. Yeah, because even before you get to the clinical phase, there's a lot of preclinical work that goes into it with regards to developing the drug. You look to see, "Is this something that potentially could be used in this?" And you also look at use in animal models. We look to see if there is gonna be a problem before you even get to the point where it's used in humans.

Andrew Schorr:

Could be 10 years.

Dr. McMahon:

Easily.

Andrew Schorr:

So, you say, "Well, why do these drugs cost so much?" Well, somebody had to invest in it. Sure, it might have been the government to some degree or private investors, whomever. Takes a long time. And there are very rigorous rules. Now some people say, "Well, I don't wanna be in a clinical trial, because you're asking me to sign these papers, and it has big, black, boldface on it. And I take it home and show it to my husband or wife, and it looks like this huge legal document, and I don't wanna be a guinea pig." So, could you talk about the controls on clinical trials, so you're looking after my safety?

Dr. McMahon:

Right. And that is something that I think that unfortunately, with the safeguards, which are very important, we need them, it can sometimes be a deterrent for some people because, like you said, they look at the consent and they think, "Oh, my gosh. I'm signing away my life. I'm signing away my house, whatever." But it's more just for safeguards. And with regards of being a guinea pig, you are going to be on a trial. It is something that it's an investigation. We're not quite sure if it's going to be as effective as we hope. But there's a reason why we're doing it, because it's not we just sort of pulled it out of the air and said, "Let's just try this and see if it works."

There's a lot of work that went up and to that point to say, "This looks very promising. It looks very safe. It looks very effective." The only way we're gonna be able to know this is by doing a clinical trial. And if anything, oftentimes, as you pointed out earlier and as Lindsey mentioned earlier, you're watched even more carefully in a clinical trial, because we are so aware of being as safe as possible with these drugs.

Andrew Schorr:

Okay. Now you mentioned in MPNs, you looked up on clinicaltrials.gov which can be daunting and saw 1,500 different trials. So, when you meet with an MPN specialist like these folks, and I would urge you to at least have a consultation with one, say, "Okay. I might be open to a clinical trial." And they might say, "In your case, it might give you a chance for tomorrow's medicine today." "Let's sort through among these trials what might apply to me because we already talked about all these myriad of different MPN situations."

And those trials, 1,500 are covering a broad swath, but you're just one situation. So, how does it apply? Okay. And so, how do you get involved? Is it a conversation? Should all of us with our doctors say, "Okay. Tell us what you got that's standard of care now. But let's talk about what's in research and look at it all together?"

Dr. Daver:

Yeah. So, this is something very, very close to my heart and, as you know, most MD Anderson doctors. So, number one, yes. Everybody should look for a clinical trial. Number two, we're doing clinical evaluation. We're not doing experimentation. So, this is very, very important. If you look at almost every clinical trial, you always get—I like your statement—tomorrow's medicine today. So, you can get the standard of care. And often, you get the standard of care plus something else. So, we have no studies at MD Anderson where we're giving people placebo arms when we think they need treatment.

Andrew Schorr:

No sugar pills?

Dr. Daver:

No sugar pills. That's not done. So, I tell my patients, "Look, the easy way to think about this is you came to MD Anderson." A lot of our people come from outside of state. "So, you could get the standard agent and maybe the ruxolitinib (Jakafi), maybe the azacytidine (Vidaza) at home. Or you could get this plus something that I think because of the years of preclinical research and Phase I data is better. So, if you want to go to a good cancer center and get that, I totally think it's worth it. So, I think there's a lot of misinformation about what goes into a clinical trial. So, I would say just like you said, at Anderson and Colorado, the Jakafi was available since 2005.

So, there were patients who were able to get it for seven years before it hit the open market. And I don't know how many people either benefited or got their lives saved by that. So, I would say 100 percent. And any malignancy, you should look for clinical trial options and meet an expert whether it's somebody close to your house, whether it's far away because often, what we see is people are told—I had a patient last week who was 72, MPN. He was told, "There's nothing we can do. He has advanced disease. He has two years." And that is completely not true. There are many things—we had six trials with different JAK inhibitor combinations which have a very good chance of getting him survival.

So, I think you have to at least make that phone call, make that email to your local university center. Say, "I would like to talk to you." A lot of the physicians will reply on a call or email, "We do this every week," or say, "Come down for a visit. Let's review this." This could make a difference between life and death really. It's critical.