Clinical Trials MythBusters: Are Clinical Trials a Last Resort Treatment Option?

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Andrew Schorr:
Hello and welcome to this Clinical Trials MythBusters program. I'm Andrew Schorr from Patient Power joining you all the way from Barcelona, Spain. We're here for a conference. You're about to meet folks from across the U.S. and wherever you're joining us. Thank you so much for joining us.

Thanks to our wonderful partner, the Patient Empowerment Network, and also the Coalition for Clinical Trial Awareness and the Alliance for Patient Access. And thank you to our sponsors, they all start with A, Astellas, Amgen and AbbVie. They help make this program possible.

We have a lot to talk about in helping debunk the myths about clinical trials and hopefully raising awareness and understanding for you and your family, so you can consider a clinical trial and see whether it's right for you. And I can tell you, in so many areas of cancer now there's exciting research going on. But if you want to get the possibility of tomorrow's medicine today, and it happened for me with chronic lymphocytic leukemia, being in a Phase II trial way back in 2000, 10 years before the drug combination I received was approved. I know it was life-saving for me.

And I want you to meet our first guest. It was life-saving for him, and that is Pat Gavin. Pat joins all the way from Marne, Michigan, which is outside Grand Rapids. Pat, thank you so much for joining us on this program.

Pat Gavin:
Thanks for having me, Andrew. Glad to be here.

Andrew Schorr:
Now, Pat, I want to go over a little background about you. I believe that you've been treated for three cancers, right? Pharyngeal head and neck cancer, in 2007, right?

Pat Gavin:
Right.

Andrew Schorr:
And also you were treated for melanoma 2008, and then in 2014 prostate cancer. Now, you were in a Phase II trial for that pharyngeal head and neck cancer. Do you believe it made a big difference for you?

Pat Gavin:
Well, that trial is absolutely the reason I'm here today. My oncologist described it as we had the experience of witnessing a miracle.

Andrew Schorr:
Let's meet one of our medical specialists who is joining us, who has been on our Patient Power programs before and our lung cancer programs, and that's Dr. Charu Aggarwal. She joins us from Penn Medicine, the Abramson Cancer Center in Philadelphia. She's a lung cancer specialist and also a head and neck cancer specialist, very active in trials. Dr. Aggarwal, thank you for joining us.

Dr. Aggarwal:
Thank you, Andrew. Thank you for having me here. I'm delighted to be part of this program.

Andrew Schorr:
Dr. Aggarwal, you have a lot of research going. It takes patients wanting to participate for us to ever have approved medicines, right?

Dr. Aggarwal:
Absolutely. And I think that's key in clinical trial participation, to get drugs to patients early.

Andrew Schorr:
All right. And certainly in the area of lung cancer and many other cancers now there's a lot happening, and smart researchers like Dr. Aggarwal are trying to prove some things that really seem like they would make a lot of sense, but we patients have to participate, be their partner. I've seen that.

Dr. Aggarwal, lung cancer is a good example, but you're an oncologist, and you see many different areas that are changing fast. What would you say to patients about the opportunity, like I said, did it give me tomorrow's medicine today? Or, Pat, who feels he's alive because of that. You must see that a lot. Doesn't always work out, but it's happening more and more, isn't it?

Dr. Aggarwal:
It is definitely happening more and more. Clinical trials are really accelerating our ability to get patients, like you said, tomorrow's medicine today. In the last five years, we've had upwards of eight to 10 drugs approved for lung cancer alone, and it would not have been possible without patients' participation on clinical trials.

As we understand the biology of diseases better and as more medicines are available to us, the only way to access them and the only way to get FDA approval is through clinical trials. And we've certainly seen that for lung cancer, but we've also seen that for head and neck cancer, and immunotherapy is now possible because of clinical trial participation.

Andrew Schorr:
Right. And I'm living with two cancers, chronic lymphocytic leukemia, where there are many new drugs now, and now we're looking at trials with combinations of new drugs. And then I have another condition, scarring in the bone marrow, myelofibrosis, and I was very grateful that a new drug had been approved for that. And I've taken that drug now four-and-a-half years, a genetic inhibitor, and I've met patient number one, who is in that trial, and I give him a big hug.

Now, Pat, what do you think are some of the myths? You know, you meet people all the time. What do you think are some of the things that people just think are true but really aren't? Maybe you could tick some off.

Pat Gavin:
Well, one of the big myths out there is that there's going to be a placebo arm, and there are not placebo arms to treatment trials, unless the standard of care would be a wait and watch, which is relatively rare. So you're always going to get either standard of care or a combination that includes standard of care and the test drugs or the—test drugs. You're never going to be left out there with just taking a sugar pill.

Andrew Schorr:
Right. So let me go over that with Dr. Aggarwal. People I think are—have heard about trials for other illnesses, but we're talking about cancer now. Your patients don't get just a little white pill with nothing in it, right? They either get quality care, standard of care, or they get something new. Is that correct?

Dr. Aggarwal:
That's correct. So the era of placebo-controlled trials is almost over, and I say almost because in the metastatic setting or in the stage IV setting or incurable setting we almost always never use placebo anymore. We are either randomizing patients to standard of care or meeting standard therapy, the chemotherapy, be it a pill, be it targeted therapy or immunotherapy, and we compare patients to that approach and introduce the experimental approach on the other hand.

Now, if there are patients that have standard of care as observation, then, of course, that observation arm does become our randomized arm.

Andrew Schorr:
Okay. And may I ask what Pat was taking, or like I know in leukemia we have people who are in watch and wait. So we have some people who are in watch and wait, okay. So I get that.

Pat, what's another myth, do you think? So one was where you get a placebo, so we heard no. So what's another myth, do you think?

Pat Gavin:
I think there's always a feeling that I'm going to be just a guinea pig, and that's the one thing I think I hear most often from people is I don't want to be a guinea pig. I want to make sure that I'm getting a treatment and not being exposed to things that are unsafe. Of course, there's always a certain amount of risk with any trial that we participate in, but the chances of some of the things happening that you might see on the comedy TV shows just aren't going to be there.

Andrew Schorr:
Okay. Dr. Aggarwal, let's go over that. So, first of all, you're at a major university center, University of Pennsylvania and Abramson Cancer Center. What sort of panels in decision-making of smart minds are there going to whether you're even going to go ahead with a certain trial? I think you call it an investigational review board. Tell us a little about the process of deciding whether you're going to have a trial at your institution at all.

Dr. Aggarwal:
Yes. So there's a very thorough review of clinical trials, and these are vetted through several committees both in terms of ethical review as well as scientific review. And, you know, when my patients say to me I don't want to be a guinea pig, I

really try and figure out what is it about the trial that they don't want to do? Is it the fact that they don't want to get the investigational drug, or is it the number of tests that are involved in association with receiving that drug?

And I think, you know, most of the time, 80 to 90 percent of the time, I'm able to answer patients' questions and concerns regarding their guinea pig concern, and most of the times actually it's related to the fact that they don't want to undergo extra tests or procedures that they wouldn't have otherwise.

As soon as they hear that this is actually a drug where it may benefit them and they're not just going to get a sugar pill, most patients are actually interested in clinical trial participation, because they're here to really help themselves and to get something that can help their cancer.

Andrew Schorr:
So, Pat, another concern—well, I guess one limitation of people being in trials is people don't even know about them, you know, not only don't understand what a trial is but have not even been told that it's an option, and that's a problem in the U.S. today, isn't it?

Pat Gavin:
Absolutely. I even think it was a problem for me. I didn't know that a trial was going to be available in my home town. If it wouldn't have been for my oncologist recommending it to me, I probably wouldn't have joined. Fortunately, today I think patients are getting more knowledgeable about trials that are out there, and they're hearing more and have the interest in joining a trial, and they'll recommend it to their oncologists and tell them that they are interested. But not knowing about them is a big problem.

Andrew Schorr:
Okay, Pat. So for our viewers today, what question or questions would you urge cancer patients or family members to ask today so that they have the awareness of trials that might be right for them?

Pat Gavin:
Well, the first thing I would do is I would offer to my oncologist that I'm interested in being in a trial. And I would ask what type of trials are available for people with my cancer, and what would you recommend as far as the trials that you see out there that you think is right for what I'm facing today?

Andrew Schorr:
Okay. All right. Well, now joining us I think is Mary Ellen Hand, who has been at the Rush University Medical Center in Chicago for many years and also works with lung cancer patients but has been in oncology for many years. Mary Ellen, first of all, thank you so much for being with us.

Mary Ellen Hand:
You're welcome. Sorry for the technical difficulty.

Andrew Schorr:
It's okay. Thank you for being with us, Mary Ellen. So from your point of view, what's a myth that comes up a lot for people? We've been talking a little bit with our other guests about whether with you get a placebo, no, whether you're a guinea pig, no. Are there other myths that you can think of that you really want to talk about now?

Mary Ellen Hand:
I think that people sometimes come to this thinking I don't want to do something because I don't—as you're saying, a guinea pig or be in uncharted territory as opposed to having an opportunity to have a therapy that may be more impactful in their disease and help control their cancer better.

And, secondly, I will have people who have an out-of-network insurance or something that doesn't allow them the flexibility to maybe even come to our institution or somewhere else for their therapy, and they think cancer trials are free

care, anything you get if you're on a trial is free. And what is true is that ordinary customary charges for things like blood tests and scans and doctor appointments and the medicines that are approved are billed to your insurance, and what the company might provide that's being tested would be the thing that's provided free to you. And so I think that that's a misconception that many people have.

Andrew Schorr:
Okay. Now, can a major medical center like yours help a patient discover the financial issues related to them, maybe even work with their insurance company to see are there options for them related to being in a trial?

Mary Ellen Hand:
I think over the last couple years in particular things have become much more complicated for people. You know, some people sign up for Medicaid or Medicare replacement policies in the different states. There's Medicaid with places—Medicaid policies that don't allow people flexibility. But certainly that's our job is to help people find out where they could go and if they're eligible for a trial to help them get to that trial, and some of that is people who have—fit a particular niche.

And some people need to be well enough to travel, you know, if they need to—if the trial is out of our ZIP code. Here in Chicago we're very collegial in head and neck cancer and lung cancer and, you know, multiple other cancers. You know, if the trial exists five miles from here, we'll facilitate the patients getting on that trial there.

I think that medical records, one of the many—one of the most common medical records systems is available at many institutions across the country, so people can have access to the reports for another hospital. Otherwise, there are coordinators and people who can make sure that all of that gets to the research nurse and gets in the hands of the person who is going to take care of that.

And then at our hospital, and I'm sure across the country, we make sure that they get the imaging so that they have something to compare it to, and then that's uploaded into your chart, you know, at the other facility so that everybody has the right information to take care of the patients.

Andrew Schorr:
Okay. Dr. Aggarwal…

Dr. Aggarwal:
I would just add…

Andrew Schorr:
Go ahead, please.

Dr. Aggarwal:
I would just add that this is a very common concern about the financial responsibility for clinical trials. And here at Penn we are actually trying to make this process very, very transparent so that when I discuss a clinical trial with a patient actually our consent forms reflect what will be the standard of care costs and what will be sponsored by the clinical trial.

And, in fact, we do facilitate meetings with a financial counselor so that if a patient has concerns about what will be covered versus what will not be covered will be discussed at length with a financial counselor. And that actually has gone a long way in allaying some of the concerns that patients have when going on clinical trials. So, you know, it goes hand in hand with what Mary Ellen was saying, that I think once patients hear from the oncologist that there's another level of—from a finance person I think that really goes a long way.

And I would urge patients to actually discuss and ask the facility where they'll be treated if there is such a person who can discuss with them, because most academic cancer centers do have this facility.

Andrew Schorr:
So many people are treated, you know, by a local oncology clinic, but often they can work in partnership with an institution like yours, Chicago, Philadelphia, others around the country. How does that work? How can that work where they can be in your trial but maybe some testing or some other things, or do they have to commute to your institution maybe from a distance all the time? Let's start with you, Dr. Aggarwal. Can there be more partnership, or are more trials available now in the community as well?

Dr. Aggarwal:
So a lot of partnerships exist between community physicians as well as academic physicians, so I see patients for my community oncology colleagues all the time, and the goal really is to make access easier, you know, the access to clinical trials and drugs easier. So while the administration of the drug and the monitoring of the drug may happen at the academic center, there are many tests and imaging procedures that can occur in the community.

And the goal is also to make this easier for patients. So if a patient is 25 miles away, I try not to drag the patients here just for a clinical exam or just for a scan. You know, so I would facilitate them getting scans closer to home with their outpatient oncologist and then ask them to perhaps bring a CD with them for review. They can get their blood work done closer to home.

So there are lots of things and lots of procedures where we work synergistically with their community physician hand in hand to try and facilitate all of these procedures so that they don't have to keep traveling all the time. So we certainly do that.

Andrew Schorr:
In Chicago, too, Mary Ellen?

Mary Ellen Hand:
Certainly. You know, there are some things that the study requires. If an infusion needs to be done onsite, that's what happens. You know, we have patients who travel across the country that might have a genomic mutation. They may be looking for second or third generations of drugs, and so those people may travel. So they have their local oncologist, meaning near, whether that's someone in the community or someone in the academic center.

I think that's another thing, is that patients are concerned that their doctor, whom they've forged this relationship with and the nurse, they think they will be upset if they go somewhere else. And then instead of knowing that it's a great opportunity for us to advance the body of knowledge but it's also—we're always encouraging and hopeful that people can get onto a clinical trial.

And so I think it makes them feel really good that people have these connections. I think they like to know they've talked, they like to know that everybody's on the same page and this many more layers of care take care of that.

Andrew Schorr:
Pat, let's pick up on that. So…

Mary Ellen Hand:
Their problem, their knowledge, all of us together, so.

Andrew Schorr:
Right. Well, Pat, let's talk about that. So people have a doctor, maybe the one who diagnosed them, and they have a close relationship with them, and they're afraid of losing them. What do you say to people? Mary Ellen spoke about that but from your perspective.

Pat Gavin:
Well, I think it depends somewhat on the trial and where they're going to be available. I received all of my care through the clinical trial locally at the Lacks Cancer Center here in Grand Rapids. It was a trial like many others that are in the national clinical trials network, and the NCI-sponsored trials are generally available at the NCORP sites, and there are a lot of those around the country. I was fortunate to have one of the original ones here in Grand Rapids by the Cancer Research Consortium, and those trials are available in academic centers, they're available in community cancer centers like I had. So it depends on the trial.

Now, some of the pharma trials may be a little more isolated and localized to specific hospitals for some of their early-phase trials. Talk to your oncologist again.

Andrew Schorr:
We are getting questions. And so I mentioned I have chronic lymphocytic leukemia. This came in from George, who also has CLL. He said, I've had no treatment, but it's likely that it will be needed very soon, and it seems that Medicare patients are treated somewhat unfairly when it comes to available financial assistance for oral chemo, oral drugs that are now in trials often, Mary Ellen.

And so he says, are we likely to run into problems with clinical trials if you're on Medicare? So, Mary Ellen, any guidance about that, Medicare patients and trials?

Mary Ellen Hand:
No. I think that we're an aging America, so I think that we want to be sure that patients who are on Medicare have access to clinical trials. So I think what he's speaking particularly to is the co-pay of some of these medications is so very high, and co-pay assistance programs are not always built to support and help them in this. But I think that if he's going to be eligible for a clinical trial, he should, you know, number one see if he's eligible. And then hopefully the place that he's at will be able to help navigate that for him so that he can be—you know, be eligible to participate in that trial.

Andrew Schorr:
Okay. One other question, Dr. Aggarwal. This one comes from Stacey. Stacey also has leukemia, and we've had oral drugs being developed a lot now for various leukemias, this is CLL. And so there's a clinical trial underway combining two of these oral agents, ibrutinib (Imbruvica) and venetoclax (Venclexta), is underway at MD Anderson in Houston, and the same trial is supposed to begin at Northwestern in Chicago near where Mary Ellen is, and that's in May.

And she says since there's a four- to five-month period before the introduction of venetoclax following ibrutinib, what would be the chance that I could join the trial at Northwestern in May, or would this be something I would have to direct to the doctor leading the trial? She's wondering about since the drugs get combined but sort of one after the other, can you sort of start, and how does that work?

Dr. Aggarwal:
So I would say that each clinical trial is different in terms of how they're designed and what eligibility criteria are outlined. I would really encourage participation—or I would encourage her to speak with this—speak about this trial with a physician or contact the PI of the clinical trial at MD Anderson…

Andrew Schorr:
Principal investigator.

Dr. Aggarwal:
…principal investigator to try to get some clarification of that. There are some trials that prohibit previous therapies or previous ibrutinib, for example, prior to enrolling in a combination clinical trial, and there are some trials that allow prior participation. In some instances, they may see progression on ibrutinib prior to combination therapy that is ibrutinib and venetoclax. So I think it's a matter of finding what the check boxes on the trial are, and talking to the principal investigator would be the best way to go about it.

Andrew Schorr:
Okay. Here's a question for Pat. So, Pat, one of the things I wonder about is there are people who are on modern therapy now like for instance some of these drugs we mentioned, people are doing well on ibrutinib or people are doing well on venetoclax, or people are doing well on some of the lung cancer drugs. Now, none of us know how long they'll last, so they say, well, why should I even think about trials if it's not broken now? What would you say to them?

Pat Gavin:
We have to as patients look at clinical trials as a form of treatment, and it should be something that should be considered right from the beginning. I hear people saying that, well, I'm going to go with what I've got so far, and if that doesn't work and nothing else is an option, then I'll use it as a last resort. Now, in some cases a clinical trial may be a last resort, but in other cases, like you mention, there may be things about early treatment that would disallow you from participating in a clinical trial later on, so it's best to be talking about clinical trials as an option right from the beginning.

Andrew Schorr:
Well, you know, this is a series we're doing, and so we've covered some ground today, and I just want to recap a couple of things. We talked about the phases of trials. We talked about financial issues, and there are counselors to help you related to that. Pat and I told the story of each of us thinking we wouldn't be alive if we hadn't been in a trial. We talked about genomics. So we've covered a lot in our first one, and we have another program coming up late in June. Dr. Aggarwal, was this a good start?

Dr. Aggarwal:
This was an excellent start. I think we definitely look forward to more patient participation on further trials, further programs like this.

Andrew Schorr:
Okay. And, Mary Ellen, do you think we made a good start today, and hopefully people will now consider trials?

Mary Ellen Hand:
I think that it's always just good to have more education, so whatever venue we can give that to people, whether it's talking one on one with their physician or nurse or whether it's online, to give people permission that they can get more information.

I think the other important thing to know is the criteria we talked about is you can't—if you are truly getting a second opinion, you should get it before you start something, because you don't want to have started a treatment that you get one dose of something that blocks you from access to a clinical trial. Or you don't want to have your genomic testing done yet, and yet you've started chemotherapy. So sometimes it's just educating people that, you know, if they're not very sick, there's time to get more information.

Andrew Schorr:
Good, good point. Pat, what's a final comment from you? What do we want to leave people with, hopefully have more people think about trials, get access to them and have greater participation?

Pat Gavin:
Well, every time I talk I say I'm alive today by the grace of God and the fact that I participated in a cancer clinical trial. They make a difference. They're the reason why you and I are alive today. They need our support. If clinical research is going to advance, we have to have patients in clinical trials.

Andrew Schorr:
Right. So if we want progress and cures for the cancers that we're living with, we've got to work with folks like Mary Ellen, Dr. Aggarwal and the other folks involved in research around the world, really.

Well, I'm over here in Europe, today in Barcelona. This is a worldwide broadcast we're doing. Pat Gavin, I want to thank you so much for joining us from near Grand Rapids and wish you good health, Pat. Thank you for being with us.

Pat Gavin:
Thanks.

Andrew Schorr:
And, Mary Ellen Hand, thanks for joining us again on our programs and in Chicago and 34 years of devotion to us, Mary Ellen, you keep going. Thank you for being with us.

Mary Ellen Hand:
You're welcome.

Andrew Schorr:
Okay. Dr. Charu Aggarwal from Penn Medicine, the Abramson Cancer Center in Philadelphia, thank you for being with us again on one of our programs. And thanks for the research that you're moving forward with and your devotion to patients with cancer. We hope that—well, we know it makes a big difference, and we look forward to you having great discoveries in partnership with patients moving forward.

Dr. Aggarwal:
Thank you for having me.

Andrew Schorr:
Great program. Our next program will be coming up on June 21st, and we're going to discuss are clinical trials a gamble? So how do you decide as a patient, you and your family? We'll talk about that in June. Thank you so much for being with us.