The Leukemia & Lymphoma Society®
Fighting Blood Cancers

Partners in Progress:

An Expert Panel Discussion on Advancing Therapies for Blood Cancers

Stuart L. Goldberg, MD, Gail J. Roboz, MD, Maria Baldo, MS, PA-C, John Hughes and Louis J. DeGennaro, PhD
June 22, 2010 • 5:30-7:30PM ET

LAUREN BERGER: Good afternoon and welcome to all of you onsite at Hackensack University Medical Center in New Jersey and to everyone participating by telephone or web for our program today, *Partners in Progress: An Expert Panel Discussion on Advancing Therapies for Blood Cancers*.

My name is Lauren Berger and I am the Senior Director of Patient Services Programs at The Leukemia & Lymphoma Society.

We have more than 1,700 people participating from across the U.S., Brazil, Canada, Hong Kong, India, the Philippines, United Kingdom and Venezuela.

We are audiotaping, videotaping and transcribing this program for posting on The Leukemia & Lymphoma Society's website at www.LLS.org/survivorship. This provides an opportunity for you to view, listen or review today's program. Especially to follow up on terminology or therapies you may have missed.

We would like to thank Genentech and Biogen Idec for their educational donation and Amgen, for their donation supporting today's program.

And we thank our panelists, Dr. Stuart Goldberg, Dr. Gail Roboz, Ms. Maria Baldo, Mr. John Hughes and Dr. Louis DeGennaro, for sharing their time and expertise with us today.

We would also like to thank the New Jersey Chapter of The Leukemia & Lymphoma Society for helping us to organize today's program.

You should have received program materials by mail or email including an agenda and biographies of our speakers. If you are participating by phone, you can view the program slides on our website at www.LLS.org/survivorship.

Today we will discuss the treatment decision-making process, potential benefits of receiving treatment in a clinical trial, ways to help patients actively participate in their care, financial matters, and the impact of research on the development of new therapies.

Following our presentations, we will have a panel discussion at which time we will answer questions submitted in advance by program registrants and then we will open the program for live questions from our audience in Hackensack as well as those of you on the telephone and on the web.

I now have the pleasure of introducing Dr. Stuart Goldberg. Dr. Goldberg is the Chief of Leukemia at the John Theurer Cancer Center in Hackensack. Thank you so much, Dr. Goldberg.

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DR. STUART GOLDBERG: Thank you. It's a great pleasure for me to be here and to welcome everyone to the John Theurer Cancer Center, and obviously I would like to thank The Leukemia & Lymphoma Society for not only this program, but for the many wonderful programs they run throughout the year.

The title of today's symposium is *Advancing Therapies for Blood Cancers*. And there is good news.

The good news is that there has been dramatic progress and advances in the last decade in treating blood cancers.

Ten years ago a patient with chronic myeloid leukemia would have been facing a bone marrow transplant. Today, a simple pill will put most into remission. Ten years ago a patient with multiple myeloma would have not had access to the immunomodulatory agents, which are changing this disease into a chronic disease. Ten years ago a patient with myelodysplastic syndromes would not have had any therapy other than blood transfusions, and today we have multiple medications that are extending their lives and improving their blood counts and improving their quality. And ten years ago we would not understand the basic biology of lymphoma that has allowed us to understand how important immunotherapy is. Or what cytogenetics can do in helping us define which patients with acute leukemia need transplant and which other patients can get by with chemotherapy alone. So there have been great advances.

But advances do not come by wishful thinking alone. They take hard work, they take bravery on the part of patients and insight from clinical researchers.

Today we're going to talk about how participation in clinical research helps to advance the treatment of blood diseases.

Now the steps to progress are fairly straightforward. We start with the basic science, understanding how the disease operates. The guy who sits in the laboratory late at night looking at a test tube, staring under a microscope, trying to figure out why does that cancer cell do what it does and how do we stop it. And then that sparks the basic researcher to come up with new ideas and early studies to figure out how can I tinker with that cell to make the cancer cell stop doing it. We move into the studies in the mice and the animals, where we learn if these treatments might stop a cancer. And then finally, if we're lucky, and some of those early experiments work, we might move them into the clinic, where the clinical researcher, a doctor, someone who takes care of patients, now takes those ideas and translates them into patient care.

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DR. STUART GOLDBERG: Now to do this on a human being we need to do it safely and we need to do it in a well thought out manner, so that we don't put our patients at risk, but at the same time so that we can learn something, so that we can help the future generations. So we're balancing the risks and the benefits, not only for the current patient, but the future generations.

So how do we do clinical research and clinical trials? Well, we start with the Phase I study. The Phase I study asks one question and that is, is this new treatment safe for a human being. The new therapy may look wonderful for the mouse, but can a human being even tolerate that pill in their mouth, is it safe? So the first patient who goes on to these type of studies gets a small dose and we see what happens. And the second person gets a little bit bigger dose and then the third person a little bit bigger dose, and we work our way up until we see what side effects and what safety profile there is.

Surely, the patients who go on these trials are often the most desperate, but they're also the most brave. So when you're evaluating a trial that's presented to you, if you look at a Phase I study, these are often for patients who have very little other options and who are willing to try something brand new, even though we don't know very much about the drug.

If we're lucky we find a safe dose that looks like it might be promising and then we move into the Phase II trial. The Phase II trial asks the question, is this treatment effective for a particular disease. We figured out the safe dose in Phase I, and now the question is does that drug at that dose help anybody. So we pick a disease and not just any disease, we pick one specific disease with one specific characteristic, so that we can learn whether this drug or therapy help in that particular disease with that particular characteristic.

We might take acute leukemia, but not just acute leukemia, acute myeloid leukemia. And not just acute myeloid leukemia, but acute myeloid leukemia in first remission. And not just first remission, but first remission when you have normal kidney function and normal liver function, but maybe a bad heart function. And we get a whole bunch of characteristics, so that we know a population and then we test the drug or the therapy in that particular set of diseases, that particular population. Because at the end of the day we want to know does that drug or therapy work for that particular disease in that particular population?

Sometimes patients will come to me and say look, I heard you're doing a new study and I want to be part of it. And we say well, you can't be part of it because you don't have that particular characteristic. And patients can become a little

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DR. STUART GOLDBERG: upset at us because we're not allowing them to be part of a new drug or new therapy. But in order for us to learn, we have to pick our characteristics ahead of time and only treat those patients who meet all the eligibility criteria.

The Phase II studies are the most common done in the United States. These are the studies that you can find many times in local hospitals. Because these are the new ideas that look promising, that we want to find out do they work in different disease states. So there may be multiple Phase II trials and these are often the ones that are available.

If we're lucky and the drug really looks like it's promising and has passed its Phase II, we might go to the Food and Drug Administration, go the government and say hey, we had a great success here. And sometimes the government will give us a thumbs-up and say go ahead, start using that drug or that new therapy in that disease, and we've made an advance.

But sometimes the government or the authorities may say nope, close, but do another study, prove to us that it's better than what you're currently doing, and that's the Phase III trial.

The Phase III trial is the granddaddy of them all. It's the, is this new treatment better than the standard treatment. So we take a group of patients with the same disease and the same characteristics and now some of them get the new therapy and some of them get the old standard, what we're currently doing. A head-to-head comparison, to find out does the new therapy beat what we're currently doing. Often it's a comparison of two drugs, one drug we know works, one drug that we think is very promising, such as Gleevec® versus Tasigna®, which just made the news last week. In a head-to-head comparison one drug was better than the other and that may change the standard, but everybody got the drug.

Often patients will say, "I don't want to be in a Phase III comparison because I want the new drug." Or they're concerned because there may be a placebo. Placebos may be ethical, placebos may be ethical when the standard treatment is to do nothing.

If a patient walked into my office today with brand new chronic lymphocytic leukemia or CLL, I might say you're doing fine, yes, you have a disease, but we don't need to treat you yet. And they may say, "Well, there's this new drug, can you see if that's better than doing nothing." There's where we'll do a placebo. We test what we're currently doing versus the new drug to see if it's better.

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DR. STUART GOLDBERG: And by the way, we did that study, 15-20 years ago. The French did that study and found that doing nothing was better than doing something right away. So sometimes a placebo is ethical when doing nothing is what we normally do.

So with this orderly progression of, is it safe, is it effective and is it better than what we currently do, we start to move the field forward.

So with this, everyone hopefully benefits from a clinical trial. And so I want to thank all of the brave patients of mine, one of them is here today, who've been on clinical trials, that help us advance the field not only for themselves, but also for the future generations.

And for those of you who want more information, in addition to checking out The Leukemia & Lymphoma Society's web, you can go to the United States government storehouse of clinical trials, clinicaltrials.gov. Most of the major clinical trials throughout the United States and throughout much of the world are listed by disease. So you type in the name of your disease, where you want to be treated, and see if there's a trial that's available for you in your hometown.

Thank you and I hope everybody enjoys today's program.

LAUREN BERGER: Thank you so much, Dr. Goldberg, for your very clear explanation of clinical trials. I now have the pleasure of introducing Dr. Gail Roboz. Dr. Roboz is the Director of the Leukemia Program at Weill Medical College of Cornell University in New York. Thank you, Doctor.

DR. GAIL ROBOZ: Thank you so much for the invitation. Thank you to The Leukemia & Lymphoma Society for the great work and resources that are provided not only to patients, but also physicians and nurses and everybody on the team on a daily basis. And it's really a pleasure to be here today and to see some familiar faces in the audience, and to hear about some familiar faces who I can't see, but who are on the webcast.

So I have a fun job today of talking about myths and misconceptions about clinical trials. And this is a topic which I think you'll all agree we could spend a long time discussing.

But to open up, so it's 2010, your telephone can find every ice cream store and every McDonald's near you. You can find where your kids are, you can locate your dog, but we can't cure cancer. And actually patients who come into the office with a new diagnosis often just can't believe this, how is it possible that so many other aspects of our life are like Star Trek and yet we still don't have a cure for cancer.

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DR. GAIL ROBOZ: But fewer than 10 percent of patients in the United States participate in clinical trials for new therapies. Why is that? And I can tell you that we spend a good deal of time discussing exactly that. What is the problem? And furthermore, it's a specific problem actually to adults because the children, you know, are doing much better than we are. There's a lot more participation of children in clinical trials. There's also a lot more participation in Europe. So what are the barriers to this and why are we not putting forward a convincing case to get patients who are diagnosed with cancer onto clinical trials?

There have been spectacular successes in blood cancers, in the hematologic malignancies. And I can tell you I get asked all the time, how can you be a leukemia doctor, leukemia is so hard, it's got to be one of the most difficult words in the English language to hear, is leukemia. But there have been phenomenal successes. CML or chronic myeloid leukemia is going to be discussed over and over again today because we actually have several therapies that have come flying forward in the last few years that have really revolutionized this disease. And I can tell you as somebody who participated in the earliest trials of imatinib, which is marketed as Gleevec, we felt like rock stars when this was coming out. It was great. I'm not sure, I'm hoping that I see something like this again in the rest of my career, because there were patients coming in, begging to be on the trial, who were responding. The news was coming out fast and furious. And I can tell you that there are many oncologists older than I am who have said we haven't seen anything like this in our career to date. So now we're greedy, we want more, that was a good feeling. The patients liked it, we liked it. We need more successes like CML.

APL, acute promyelocytic leukemia, and I can wink at somebody in the audience here who knows a little bit about this. This used to be one of the bad ones. This used to be one of the ones that as you were having the conversation, you were worried about the patient not making it to the following week, let alone to the following year. And yet in this disease there are spectacular cure rates. Many patients over 80 and in some patients even over 90 percent. And the amount of chemotherapy that's required is going down over time, not going up. And this is something, one of the few areas of the hematologic malignancies where we actually really understand the basic science as well.

Hodgkin disease. This is a curable disease now. Childhood ALL. I just got off the unit today with several adult ALL patients, who are not happy to hear that their disease is not the one that they keep hearing about that's so curable. The kids are getting cured with this disease. The adults aren't. If you're 9 it's very different from if you're 18. Why is that? Is the disease really that different or are we just

DR. GAIL ROBOZ: doing something differently? Are pediatricians better than we are? I don't think so. There's something that we're missing here. And the question is if you look at the successes in childhood ALL, what's been particularly fascinating about that is that over many years of research it wasn't about putting in a brand new drug. It wasn't about a magic bullet, it wasn't about a Gleevec. It was about changing and updating and changing and updating combinations of older drugs and making them work better. And that's one of the messages that we're really going to talk about today, is that most people think a clinical trial is a new drug. It's not necessarily a new drug. It might be figuring out how to get the other ones to work better.

If you look back to a previous example of acute promyelocytic leukemia, a major, major player in that drug is arsenic. How do you guys feel about that as a patient, when I come into the room and offer you some arsenic, it's a little bit tough to hear. This is a 2,000 year old drug and yet it has absolutely fantastic applications in this subtype of leukemia.

So again, it may not necessarily be brand new, but we have to know how to use it.

Hairy cell leukemia, a rare disease, beautiful argument for clinical trials. This is such a rare disease that if you don't actually treat all patients similarly across the country, or even across the world, you can never learn anything about it because there might only be a few thousand cases total to deal with. And now there are fantastically successful treatments.

So this is meant to be the introductory rah-rah speech to get everybody excited about what can be done, some really, really fantastic results of current ongoing clinical trials. But clinical trials have many objectives. So one of them, and Dr. Goldberg started discussing this a little bit, so one of them is to compare existing treatments. And I didn't put these in any particular order because they're all important, but think about it, everybody wants the new drug. Keep thinking that maybe the new drug isn't the better drug. So let's make sure that with the tools that we have, that we actually know what works and how to use it.

Providing access to novel therapies is also an objective of a clinical trial and it might be the only way to get a drug that is being looked as the new drug. But we also want to compare the new drug to the existing ones because it's not a guarantee that the new one is better.

Sometimes we just want to study the effect of an intervention on a group of patients. Like what if we look at everybody on the stem cell transplant unit and see who does better, those who have an exercise bike versus those who don't. I know it kind of sounds funny, but we're actually doing that. There are very

DR. GAIL ROBOZ: important things that you can do as part of your treatment plan that might make a difference. And wouldn't you like to know if you were the patient, whether it made a difference or not. You want that information in advance that, well, if all I have to do is ten minutes on an exercise bike every day and I would do better, maybe I'd be more motivated to try to do that.

Another thing that we want to do is study characteristics of the patients. What is the quality of life of a group of older patients who maybe are in their 80s and getting therapy? What if we knew that they lived longer and better without the therapy than with it? The patient may make a different decision about their treatment. There's no way to know without studying it.

Now clinical trials give patients the answers they want and they need. So think about it, if you're coming into an office, what are your questions, where do you think we're going to get these answers from? Does X work? That's the first thing you want to know. Well, actually the first thing you probably want to know is, why did I get stuck with this disease? Most people do ask that as their first question. But does this treatment work? I can take a guess, but I have a much better conversation if I can actually point to studies that have been done and tell you about what we've learned from them.

What are my chances of a remission? How long will my remission last? What are the most common side effects? These are really basic questions, right? Anybody who has gone into a doctor's office is going to have these questions and is going to want to know as specifically as possible what the answer is.

Well, unless somebody is on the clinical trial, again, it's guesswork, we can't answer your questions.

What are the long-term side effects? This is one that I put up there because a lot of times if you're doing well and everything is going great, you sure want to forget about us and we understand that. And we're a little sad, we want you to come back and visit, but we want patients to be doing well and forget about us. And yet we want them to let us know how they're doing and stay on the study for the long-term, so that when you ask the question well, what if I do okay, what happens to me in ten years or fifteen years, I'm only 30 now. We want to be able to answer that question. So staying on the clinical trial, even if you're doing well, even if you're no longer worried about the treatment itself, you may be concerned about whether that treatment is relevant for you down the road and what the side effects are. People have lots of concerns about clinical trials and that's why we're here today, to try to spend a couple of hours answering them.

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DR. GAIL ROBOZ: I have to say I don't want to be a guinea pig has to be the number one. And I hear that over and over again. And people certainly worry about that and they worry about well, I really prefer that the guy in the next room get it first before I do, and that's completely reasonable to feel that way. Anybody who's paying attention to our description of the clinical trial is going to be concerned about it. That doesn't mean that you don't get over your concern.

I'm too old, oh, I'm too old, I'm 82, I can't be eligible. Well, maybe you can. There are lots of trials that are targeted specifically at certain age groups and there are many which don't have any targets at all, but there's no such thing as I'm too old or I'm too young. Specific trials may have specific needs, but it's not a blanket statement that can ever be made.

Clinical trials involve experimental drugs. Maybe. But maybe not. The clinical trial might actually not have anything to do with an experimental drug. That doesn't mean it's better or worse, it's just different. And to think about that, that not to put the concept of clinical trials out of your mind just because you don't want to take a new drug.

Clinical trials are for patients with no treatment options is a frequent one that I hear, that oh, you must not think I'm going to do okay. You must think there's nothing for me. Not at all the case. Not at all the case. We, of course, want to develop new things for patients for whom existing therapies are ineffective, but many times we just want to make better or try to improve what's going on with patients who are doing well. And the example of acute promyelocytic leukemia or APL comes up, that what if we can give you less therapy and still do as well. So again, it's not at all true that it's only for patients with no options.

You have to pay to get onto a trial, I don't have enough money. No, absolutely untrue. You get paid to be on a clinical trial. That's also untrue. The economics and finances of clinical trials are worth a long discussion with your doctor, but neither of those statements, which are on my slide, is correct.

My doctor will be mad at me if I participate in a clinical trial. Well, then get a different doctor because that cannot be part of your decision-making. And if there's a trial open somewhere else that isn't open at your institution, doctors should work together and do their very best to be helpful, if that's the right choice for you.

My doctor will get mad at me if I don't participate. That's also ridiculous. This is not a mandatory situation and there is absolutely nothing wrong with listening to

DR. GAIL ROBOZ: the full discussion and deciding that it's not the right thing for you. And again, if your doctor is mad at you, get a new one.

I don't want to get a placebo. I think Dr. Goldberg already addressed that a little bit. That there may be situations where a placebo is absolutely reasonable. And a placebo technically means, quote-unquote, a sugar pill and that's how people refer to it, but there are situations where it makes perfect sense that that's part of the trial. And you should listen to the entire discussion before deciding that you do or you don't want it.

I don't want to stop my other medications. You may not need to. There are many clinical trials in which other medications that you're taking are perfectly reasonable to consider, so don't make that the reason not to look into it.

Clinical trials won't help me, just other patients. Well, we don't know that in most of the cases. And we're very careful with patients in not promising them things that we can't deliver. We can never promise a patient that the trial is going to be good for them, but we hope it will and it might be. And it might be good for you, it might be good only for other patients, but that's fortune-telling and most of us aren't very good at that. The one thing that isn't true is to say right up front that this won't help me because it very well might. And I think the examples that we've been giving you of patients doing phenomenally well on certain clinical trials should ally that concern.

And then privacy. I don't want other people to know my business, I don't want them to know details about me. There are lots of aspects of clinical trials that we really focus on, in trying to make sure that your identity and your privacy are as sacred as we can keep them. And that can be discussed usually in a way that alleviates most people's concern.

My summary statement is whatever your concerns are, ask the doctor about clinical trials and have the full discussion. Don't make a decision without having the discussion of whether you would or would not consider it.

And it is now my pleasure to pass the remote this way and I think Lauren will introduce our next speaker.

LAUREN BERGER: Thank you so much, Dr. Roboz, for your very complete and very clear explanation.

I now have the pleasure of introducing Ms. Maria Baldo, Senior Physician Assistant, Leukemia Service, at Weill Medical College of Cornell University in New York. Thanks so much, Maria.

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MARIA BALDO: Thank you very much and thanks for asking me to participate in this forum.

I was asked to describe the informed consent procedure and how patients enroll in studies and how patients learn about the research protocol. And the research protocol meaning the details of how the research project is going to go forward and what would be expected of them.

For a patient to start making a decision about whether they want to participate in a research study, they need to start understanding what their benefits and what their risks might be. And they also need to understand what their rights are and what their responsibilities are and what would be expected of them in the study. And when patients start thinking about what their risks would be if this is a research project about a new medication, of course, there will always be a risk of, we might not know, investigators might not know what the potential side effect of this medication is.

Another risk might be that this particular medication might not be helpful to you or it might not be helpful at all, and may be less effective than standard therapy.

When thinking about the benefits, which I think that both Dr. Goldberg and Dr. Roboz talked about very well, is that for patients to have access to medications and procedures that might not be available to the public is obviously a benefit.

Many patients, there is a benefit because you are very closely monitored. The research team, there's a lot of people on the research team. There's the PI, there's other doctors, there's nurse practitioners, there's physician assistants, there's other nurses. So you have a pretty large research team.

You're working with physicians who are the top in their field. And that clearly is a benefit.

And lastly, I actually think that many patients are very motivated to participate in research studies, even if they might not individually benefit from the research study, but I think that it's very motivating for them to know that this information is going to be used for other people and it might benefit other people. So I would say that a lot of the patients that I work with are quite motivated in that area.

Patients also need to think about what their responsibilities in the study are going to be and what is expected of them, and to understand their rights. Patients should always understand that this is voluntary. And you would sign an informed consent in a voluntary fashion. And that even after signing an informed consent

MARIA BALDO: you can certainly always leave the study and there will be no consequences to you leaving a study. You can always make the decision that you don't want to participate any more.

And lastly, as the study goes on, some new information might be appropriate for the investigators to share with patients. And it is the research team's responsibility to share that information. And sometimes a patient will actually sign another informed consent with some updated information. I think many times we think about the informed consent as a document that needs to get signed and leave it at that. But in reality the informed consent is actually a pretty dynamic process. And it includes two things. It includes both the document that somebody needs to sign, which is legal consent, that they will actually participate in the study. But it's also about this dynamic conversation between the participant or the patient and the research team. And a lot of times when a patient is first diagnosed, sometimes signing an informed consent and making a decision about a research clinical trial is done very quickly, due to the nature of a particular disease. Other times what happens is that we give a patient an informed consent, they go home, they read through it, they come back to the office, they bring friends and family members, they sit down with the research team, they sit down with the PI and talk about the details of the study and questions are answered. If a patient's native language is not English, we bring interpreter services in. The informed consent is given in their native language. And this should be a very dynamic process and it actually happens throughout the whole clinical trial, not just at that one point when you're sitting down and talking about whether you want to legally participate in the study.

In looking at the actual document that a patient will get, it will include particular sections. And the first thing that will be involved is what is described as the purpose of the study. And this is the basic hypothesis of why investigators are looking at this particular question and what they're hoping to learn.

It will talk about the duration of the study. Different studies have different follow-up periods. Some studies run for a few months, some studies run for years. And it will also talk about, there are particular circumstances where a physician will take a patient off of study for different reasons and it can be possibly because someone's disease has advanced, it can be that another medication is more appropriate. So it will also include both duration and reasons why a participant may later on be removed from the study.

It will talk about the risks and benefits of participating in the study. All informed consents will talk about, what are the alternatives to participation, which generally

MARIA BALDO: includes the standard of treatment, so what would I get if I didn't participate, how would you treat me otherwise. And sometimes, as Dr. Goldberg talked about, sometimes the standard of treatment is actually no treatment at all.

It always includes the details of the study and what is expected of a participant. So a participant will know fully how many bone marrow biopsies they'll need to get, what lab tests they'll need, if they need CT scans and where all these procedures will take place. Sometimes they have to actually take place at the institution where the study is being held. Sometimes these can be done at the home, a phlebotomist can come to the home. Sometimes it's done with a local physician. So it's very dependent on the study itself.

The informed consent will talk about confidentiality. And although a patient's name is never on their records, we code everybody, records need to be shared with quality assurance organizations and also for data analysis. And the patient will be fully aware of who receives these records.

As Dr. Roboz pointed out, if there are any additional costs and who will be charged. So cost and additional expenses is included in the informed consent. How much your insurance company or how much you will be charged or whether if a drug that is being tested on you is all of a sudden approved by the FDA and is available, will you then have to pay for the medication, will your insurance be charged for this medication, would be included.

Contact information. Who to contact if you have questions is included.

And lastly, your signature and your signature is the legal consent that you have decided to participate in the study. And again, if a person changes their mind they always can drop out of the study for any reason with no consequences. Eligibility criteria. So eligibility criteria refers to what patient would be appropriate to participate in a study. And it's a group of characteristics that the principal investigators are looking for in patients. In general, some very common eligibility criteria are disease type. So are we looking at a leukemia, are we looking at myeloproliferative disease. So your disease type is quite important. A patient's age. Sometimes we look at patients who are older than 65, sometimes less than 65. So different protocols have different age criteria. The stage of the disease. In hematological malignancies we usually don't talk about stage, but some studies are made for patients or developed for patients who are newly diagnosed patients. Other studies are developed for people who have already received standard of care. Other studies are developed for patients who have been in remission and maybe have relapsed for the first time and they're no longer in a remission. And so that's what I'm referring to as stage of disease.

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MARIA BALDO: We definitely look at other treatments used by the patient. So how many treatments has the patient received already and what type of treatments have they received. Most studies have a washout period, which means that two treatments cannot be used at the same time and there's a certain period of time that we want a patient to be free of a treatment before starting a new treatment. Other studies actually don't have this and patients can be taking multiple treatments at the same time.

And we look at other illnesses or conditions that the patient might have prior to enrolling in the study. So does somebody have preexisting liver disease or kidney disease or does the patient have any heart conditions. Those would be considered eligibility criteria.

The first process of enrolling in a study is a screening process. And one needs to sign the consent form before they are screened. And the screening process will include most likely a physical exam, some lab tests, maybe some procedures like X-rays, EKGs. So the informed consent needs to be signed before actually screening for the study to see if you can participate in the intervention.

Lastly, I'd like to talk about some questions that I think would be important for patients to ask their research team or ask their physicians before entering a research project. The first is how do the tests or procedures in the study compare to those I would have outside of the trial? And basically a patient is asking what would be different about my treatment if I participate in this clinical trial or if I were not to participate and I would receive the treatment that you would recommend?

Will I be able to take my regular medications? Sometimes there are interactions between the study med and the meds that you are already taking and it's important to know whether you need to switch medications before entering a trial.

Who will be in charge of my care? Most people are used to going to see their physician and seeing them on a regular basis. Once you participate in a clinical trial your team of people who are taking care of you generally grows and there are more and more people, so who will I be seeing on a regular basis, will I be seeing my physician, will I be seeing the PI, will I be seeing a research nurse or a PA?

What type of follow-up care is part of the study? So how long are you going to see me? And if I live far away do I need to travel to the institution and how long will this go on?

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MARIA BALDO: And how could being in the study affect my daily life? And there are many ways that it may benefit or it may actually hinder your daily life. Am I going to be filling out questionnaires on a daily basis, am I going to be getting phone calls from a research person, am I going to be traveling? So there are many different ways that it might actually affect your daily life.

Thank you.

LAUREN BERGER: Thank you so much, Ms. Baldo, for your very comprehensive and clear explanation. I now have the pleasure of introducing Mr. John Hughes. Mr. Hughes is an MDS survivor and a two-time clinical trial participant. Thank you so much for sharing your story with us today.

JOHN HUGHES: Thank you, Lauren.

Initially I'd like to thank The Leukemia & Lymphoma Society for putting this forum together. But I'd also like to thank all of you who are here and participating in this either in person or on the web. Without your participation there wouldn't be a forum and obviously your participation is extremely important to getting the message out.

And I'm here more on your side of the table than I belong on this dais. I'm sort of in awe of the expertise that I'm among here in these chairs. I am here to learn as much as to share my experiences with you because the experiences that I have are really more anecdotal than based upon statistics.

I have participated in two clinical trials. One here in Hackensack and one at St. Vincent's in New York. And those experiences have been interesting, to say the least.

The important thing that I found is that you really have to participate and to help educate yourself. And that source of education is on many levels.

Why did I get involved in the trials? I got involved in the trials because I was asked to participate in the trials. That information was brought to me. Why is it that I did participate, that I agreed to? Well, obviously we have very altruistic reasons and despite what Dr. Goldberg said, I never considered myself brave. My participation was selfish.

Of course, we want to continue the role of science, we want to improve upon the science and how we can treat and understand the diseases that we're confronted with. We also want to have our experience benefit other patients,

JOHN HUGHES: other people who are suffering from the diseases. And all which of has an effect on us in our decision-making process.

But I think most of us make the decision because we really want to see if something will help us and help our condition and that's basically what I considered when I was making my decision, is, is there a chance that participation in the trials are going to improve my quality of life and really is it going to improve the length of my life. And with that chance, obviously I wanted to participate in those trials.

It was important that I educate myself before I participated in the trials and as was pointed out, there's a number of sources of education that we all have to look towards. Obviously, the clinical team is the first level of information that we're going to get. The questions that have been outlined here are questions that we should all be willing to ask everybody on that clinical team. We should make sure that we understand fully what we're getting ourselves involved in. We also have the ability to, especially today, is to do extensive research on our own. There's no reason that we can't go on the web and research all of the information that's being given to us and everything that we're asked to participate in. And the third area of education is, as was just explained, the informed consent document. That's really a great outline of what we're getting ourselves involved in. That informed consent document is not just a consent. The consent is really the end of the document. But in order to make sure that we're informed, that document has to contain some information that's going to be vital to us. The purpose of the study, what the structure of the study is. And of course, what the potential outcome, what the risks and rewards are of the study and our involvement in the study.

And that document really should be carefully read. And I fear that too often people don't really study the document to understand what they're getting involved in. And I would urge that patients who are getting involved in clinical trials make sure that they understand the critical outlines of that.

And of course, lastly, they have to understand that it's their trial. The patient is the one who makes the decision. The clinical team doesn't. Obviously you're making a commitment when you sign that document, but when you feel that it's not in your best interest, it's yours to terminate. And that's something I think was clearly pointed out.

Thank you, Lauren.

LAUREN BERGER: Thank you so much. I now have the pleasure of introducing Dr. Louis DeGennaro, who is Executive Vice President and Chief Mission Officer at The Leukemia & Lymphoma Society. Thank you so much, Dr. DeGennaro.

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DR. LOUIS DEGENNARO: Thank you, Lauren.

I'd also like to thank the audience here with us in the room in Hackensack and the folks on the web and the telephone. And my panelists here, especially John Hughes, for giving us the patient's perspective, because I think this is a critical element in the discussions about clinical trials and how to really advance research progress.

I'm going to start by reminding you of the mission of The Leukemia & Lymphoma Society and to some extent why we organize activities like this.

The mission is a very focused one. Cure leukemia, lymphoma, Hodgkin's disease and myeloma and improve the quality of life of patients and caregivers. It's very focused and we're very directed at that. Our goal is a blood cancer-free world and we're working diligently to get there.

What I'm going to do today is amplify a little bit on some of the comments that Dr. Roboz and Dr. Goldberg made about the advances that have been made in blood cancer treatment. And then tell you a little bit about some of the activities that are underway on the research side, both basic research and clinical research at the Society in our efforts to find cures for these diseases. I want to take us back to first principles. Why does The Leukemia & Lymphoma Society do this? And really this is why for me. Anyone, frankly, anyone can get blood cancer. These diseases don't discriminate on the basis of age or gender. Elderly and young both get them. Males and females both get them. They don't discriminate on the basis of ethnic background or social status.

This year in the United States there will be 30,000 new cases of leukemia, 60,000 new cases of lymphoma and 20,000 new cases of multiple myeloma. It's a significant unmet medical need. Across the United States there are a million Americans, and their families and their loved ones, living with the consequences of a blood cancer diagnosis. This is the challenge for the Society and this is where our work is focused.

I don't want it to sound like a doom and gloom scenario, though, because as has been mentioned several times before, there's been substantial progress. And I really wanted to show you that in this next slide.

This slide tracks the improvement in survival of the blood cancers over the last four decades, from the 1960s right through to the 2000s. And the bottom line is on the left hand side of the slide, survival rates have doubled or tripled and in some cases even quadrupled over that 40 year period. So I call your attention to

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DR. LOUIS DEGENNARO: the right hand side of the slide, where we talk about leukemia. In the 1960s a leukemia diagnosis in general was not a good diagnosis, only 14 percent of patients survived for five years. Today it's nearly 50 percent. And in some cases, as have been mentioned, APL, for example, and in an example I'll elaborate on, chronic myelogenous leukemia, survival rates are near 90 percent. Multiple myeloma has had, on the left hand side of the slide, has had an incredible improvement in survival in patients over the last 40 years. As has non-Hodgkin's lymphoma, going from 30 percent to over 60 percent now. And finally, as has been mentioned, Hodgkin's disease is largely a curable blood cancer today.

Again, the good news is we've made substantial progress and the progress has been made because of research. Research in the basic research laboratory, research in the clinical laboratory and in the clinic in terms of clinical trials.

Now I'm sorry, I apologize for showing you data here, but I think it really makes the point of why it's wise to invest in research. And it's an example that has been talked about here earlier. It's the example of the advent of a new drug called Gleevec to treat chronic myelogenous leukemia.

What you see in front of you is a method that scientists and physicians use to track the success or the efficacy of a new treatment being tested in patients on a clinical trial. It's something called a Kaplan-Meier curve. Across the bottom is time. Time since diagnosis. And that little red triangle at five years is the benchmark that we all use to help judge the success of a new treatment.

On the vertical axis, going up and down, are the number of patients surviving, starting with 100 percent at the top and zero at the bottom. And what you see here is the real data from a clinical trial carried out in the late 1990s, testing the then best available treatment, called interferon, in patients newly diagnosed with chronic myelogenous leukemia, CML. And if you ask how many patients survived at five years, it's 55 percent. And you can see the curve goes down – I should stop and say physicians and scientists look with dispassion at this data, but frankly every tick mark here is a patient losing their life, and that's something we have to remember.

In this study in 1998 only 55 percent of patients survived five years and you can see the prognosis for the rest was not particularly good.

In the year 2001, Gleevec came to the market, came into the healthcare arena, to treat this disease. I'm happy to say that The Leukemia & Lymphoma Society-funded researchers, helped to make that happen. And this is what the data looks

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DR. LOUIS DEGENNARO: like. The line across the top is the data from that clinical trial. And if you carry out the measurement, you see that 90 percent of patients were surviving at five years, 90 percent of patients responded to this drug. And I can tell you this trial was carried out in the range of 2000 and 2001. Most of these patients are still alive. The drug has put their disease into remission for not just five years, but six years, seven years, eight years, going on nine years.

I call this the value proposition for research because this represents lives saved. Over the five year period described here, we would have expected 20,000 Americans to be diagnosed with chronic myelogenous leukemia. Before Gleevec, 4,700 of them would have died during this five year period. With Gleevec, only 700. So 4,000 lives saved by this new drug and counting. Every year there are another 3,000 new diagnoses of CML. Every year Gleevec and the new drugs that have been developed since Gleevec, that are cousins of Gleevec, if you will, have put these patients into remission and have allowed them to survive with good quality of life.

The last estimate I heard was that this drug, since its introduction into the healthcare arena in 2001, has saved 120,000 lives. I think it was a good investment.

I'm going to end by just showing you some of the research programs, both basic research and clinical research programs, that the Society has underway, trying to drive new therapies to patients for the blood cancers.

Across the top here, it's the pathway that Dr. Goldberg described. Start with a bright idea in the laboratory. Reduce that bright idea to practice in what's called discovery research. Carry out the animal studies, that's called preclinical research. And then carry out the three phases of clinical trials and if successful, bring that treatment to patients.

The Society has several programs underway. One to treat a disease called post-transplant lymphoproliferative disorder, and one to treat diffuse large B-cell lymphoma. Where the project is in that preclinical phase, testing in animals, in preparation for asking the Food and Drug Administration whether we can move into a clinical trial setting.

The next four studies are clinical trials in Phase I. These are Phase I trials being supported by the Society and in part being directed by the Society, looking at promising treatments for chronic lymphocytic leukemia and follicular lymphoma, acute leukemias in general, and leukemia and lymphoma. Some of these agents are drugs that were already approved by the FDA, which in a newly discovered

DR. LOUIS DEGENNARO: way, we believe might be active in blood cancer. And we're seeing them through this clinical trial process.

Some of these are drugs that are actually being developed for other diseases like arthritis, but we believe they have great value in treating the blood cancers. And the Society has stepped up to make these clinical trials possible.

This next trial is in Phase II, so again being tested in patients, to ask is it effective in treating acute myelogenous leukemia. I can tell you that two weeks ago at a major meeting of clinical oncologists, a report was made about this clinical trial. The agent being tested here is being tested in older AML patients, newly diagnosed AML patients who are 60 to 75 years old. A very difficult population to treat with the toxic chemotherapy that's typically used in treating AML. In this clinical trial, the report, the trial and the report of that trial showed a significant improvement in the number of patients who achieved a complete response to this drug, and much less toxicity associated with this drug. We have great hope that this will become a first line treatment for older patients newly diagnosed with AML.

And the last trial brings me back to John and his participation in clinical trials for myelodysplastic syndrome. Again, the Society has stepped up to help to support a late stage, Phase III clinical trial of an agent we believe is very promising to treat myelodysplastic syndrome. And if this trial is successful, and as been mentioned before, no one can be sure until the trial is actually done and patients actually participate. But if this trial is successful, the data from the trial will be brought forward to the Food and Drug Administration and we'll ask them for permission to put this into the healthcare arena and make it available for physicians treating patients like John.

I want to just end here with what the Society is doing and how it seeks to partner with patients. Many of you will know about the Patient Services Programs we have. Programs like this one. We have education and support groups available to patients with blood cancer. We have programs which provide financial aid. And we run a clinical trial matching service. We can help you navigate clinicaltrials.gov that was mentioned by Dr. Goldberg, and help you find clinical trials that you might be eligible for.

On the public policy side, we're lobbying daily at the federal level and at the state level for legislation that's important to blood cancer patients. I told you a little bit about our research programs. We're trying to drive these programs forward to develop new treatments for patients.

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DR. LOUIS DEGENNARO: And finally, we bring a patient's sense of urgency. Like John, we want to see these diseases cured and we work very hard every day to make that possible.

With that I'll end and take it back to Lauren.

LAUREN BERGER: Thank you so much, Dr. DeGennaro. It is now time for our panel to discuss questions submitted in advance by registrants of today's program.

I'll start with Dr. Goldberg. Dr. Goldberg, please tell us how you talk to patients about treatment options.

DR. STUART GOLDBERG: I think that when I see a brand new patient for the first time, or a patient I've been taking care of for a long time, I always am trying to figure out if there's something new that we can offer them, something that might change their disease. So we always start with, what is their disease and then we also think about what are the treatment options that are available, that are readily available at our institution or at neighboring institutions. The standard treatments. And I always start with what is standard, what do we know, what can we give the patient right off the bat.

And then I go and do my homework and ask, what is the next step. What are the unanswered questions in that disease and what are the other options that might be available on a clinical trial. And so I always want to give the patient the opportunity of knowing first what is the standard treatment and then say here's where we can build on that, with a question we don't know the answer or a new drug or a therapy, and see if that fits into their mindset of this is something I want to be part of or no, you know what, I want to stay with the standard of therapy. But give them the basic understanding of what is the first treatment they can get without being on a trial.

LAUREN BERGER: Thank you. Dr. Roboz, would you like to add anything?

DR. GAIL ROBOZ: I think I'd just like to add a comment to the group about we have a sense, I think, being on the panel and talking about all of this, I am a little bit worried that we might be overwhelming people by the amount of work that we feel you guys should be doing. And I just have to say that there are a lot of patients who will make the decision from the beginning that maybe they don't want to look on the web right away and look at 9,000 options or look at clinicaltrials.gov or maybe they don't have a computer. And I think we try very hard to be sensitive to what is the patient ready for and what do they want.

And it's perfectly fine to come into an office and say well, hey, you went to school for this, you're supposed to know what you're doing, you pick a treatment

DR. GAIL ROBOZ: for me. And that's okay and I think you need to like your doctor and you need to like your clinical team and you need to sometimes say you know what, pick something. And you seem like you know what you're doing, you'll lead me down the right way. And that's okay. And nobody in the group should feel that they are doing the wrong thing by abdicating a little bit of the responsibility.

Similarly, though, we get all kinds of patients. Patients will come in with 9,000 pages from Google on every single clinical trial in the country. And if we need to, we go through that, because if that makes you feel better and if it makes the decision-making process more helpful, we will do that. I think one has to keep in mind, though, that this is enormously overwhelming. And the one thing I would ask everybody in the audience is to give us enough credit that if the right answer were out there, we're all smart enough to do the right thing, right? That's easy. If the cure were out there, we'd want all of our patients to be on it as quickly as possible. And you do want to have that much confidence in the doctor, that if the best drug would be down the road, if you're not even trusting your doctor to send you down the road for it, you're in the wrong place.

But I think that that initial treatment-making process of deciding how much do you want to learn initially about the disease, take it easy. Don't necessarily leave right away, jump onto the computer, have 50,000 numbers and letters floating through your mind of medications that you can't spell, that may not help anything. And that's okay. We're here, your team is supposed to guide you through that, this is something that people in fellowship take years and years to learn. There's no reason to feel like you need to master it in ten minutes on the computer.

LAUREN BERGER: Thanks, that's great. Does anybody else want to add anything to that?

Okay, Dr. Roboz, I'll ask you the next question. If a doctor does not bring up clinical trials, should at patient take the lead to bring this up to their doctor?

DR. GAIL ROBOZ: There are tremendous variations in terms of patients' sophistication about available treatments. And for some patients it comes as a big surprise to actually learn that the treatments that they're being offered, that there were options. Some patients, just that information alone, that wait a minute, there's more than one way to treat this that actually might be news to the patient. So I think that from the patient's side, the question should not so much be, is there a clinical trial, but is this all that's out there? And open that door. When you're getting treated – and this isn't only for cancer, this is for anything – is this the only way to treat this? And sometimes that can lead into a discussion of well, actually, we could do this, we could do this.

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DR. GAIL ROBOZ: Sometimes when you're getting a sense from the doctor that wait a minute, these numbers don't sound that good or it sounds like the doctor is not that excited about this therapy or not that optimistic that it's going to help, that's the time to say is there something new out there, is there a clinical trial? And I think it's absolutely fine to bring that up. But at the same time you don't have to feel like that's the question for the first visit. You can take a little bit of time to get used to your diagnosis, get used to what you're talking about before you take it to that next step.

LAUREN BERGER: Thanks. Dr. Goldberg, would you like to add anything?

DR. STUART GOLDBERG: I would echo exactly what you said. Feeling very comfortable with your treatment decision, comfortable with your doctor, comfortable with even your diagnosis and what the treatment plan is and what its goals are. I have, unfortunately, some patients who come in, they still don't know the name of their disease, they don't know that this is a cure therapy or this is a palliative therapy. They don't have a sense of really where we're headed. So I think that often if you're not sure, step back a bit, take a deep breath and ask philosophy questions like what is the meaning of life, what do I want out of life, what do I want out of my treatment? Am I only wanting to be cured, am I willing to accept side effects for better life, longer life. And these are philosophy questions. And sometimes doctors don't have those answers. But at least once we have a sense of what you want, then the knowledge that we bring from the training, can start to fit with what you want. So sometimes if you're not sure what you want and you don't feel comfortable, just take a step back, take a deep breath, go home, come back the next day, come back and say I want another opinion.

I think second opinions are something we haven't talked about yet. But I think that's something that doesn't upset doctors. If you have a good doctor who's confident in their own skills, they shouldn't be upset with having you go to see another doctor who will hopefully add to both of our knowledge and actually make you feel more comfortable with the treatment that you picked. I think you heard John, who's my patient, go across the river to see a doctor that I sent him to, to say hey, I don't have anything good at my place, go see somebody else.

So I think that confidence level and feeling that you're comfortable with your treatment decision is probably the most important thing for our patients.

LAUREN BERGER: Thanks. Yes, I think the second opinion is something that people are always concerned about, will my doctor feel like I'm not very confident in his or her skills if I ask to go for a second opinion or just do it. So thanks for bringing that up. Anyone else want to answer?

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DR. LOUIS DEGENNARO: I'd just like to mention that The Leukemia & Lymphoma Society has programs to help patients understand better about clinical trials and sort through the process of speaking with their physician about them as well. So if the patient is uncomfortable with going directly to their physician, they can certainly contact the Society and we can provide some information and help them think through the process.

DR. GAIL ROBOZ: And Dr. Goldberg will let us know the meaning of life at the end of the conference, so you can all stay for that.

LAUREN BERGER: So the next question that we received in advance, I'll ask Maria, and the question is, the subject of clinical trials is not always easy for a patient to bring up. And I know we're talking about that a little bit right now. So how should a patient approach this with their doctor and what are some of those key questions to ask?

MARIA BALDO: I think sometimes people feel a little bit hesitant to ask their doctor because their doctor has been practicing for years and has all the knowledge base. And so they're resistant, they want to be polite, but I think doctors, at least all the doctors that I've worked with, really appreciate when a patient takes an active role in their care. And that initial hesitation really need not be there because physicians really appreciate it.

And I would say that the key question or the key things to think about or to ask your physician is, do you feel that a clinical trial could benefit me in any way, that I would not be benefitted by whatever treatments are out there at this point? So that's really the thing to think about, what are my benefits from going into this trial, what are you thinking would be my benefit?

LAUREN BERGER: Anybody else have anything to add?

JOHN HUGHES: Just in general, with regard to the tenor of this conversation, I found in both of the clinical trials, and even beyond that with the treatment protocols that I've participated in, whether it was here at Hackensack or at St. Vincent's, the clinical teams were more than willing to sit down, all members of the team, and discuss any of the questions that I had. So I found it, not only Dr. Goldberg or Dr. Raza, but also the members of their teams were very happy to sit down and discuss the possibilities and get into discussions about the philosophy of life and other things. But they were happy to share the information and really, as I said before, that was the first source of information and that's the foundation you've got to rely upon in making any decisions that you have.

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LAUREN BERGER: Our next question, actually you discussed it a little bit before, Dr. Roboz, about patients bringing information about clinical trials to you on their own. So does anyone want to add or how do you feel about that?

DR. GAIL ROBOZ: I work in Manhattan, so people come in with a briefcase full of 9,000 documents in several languages. Many people have already been to six different centers. So I'm very, very used to that. But I have to tell you, it's not really a norm. And I think that for some patients – I have, I will tell you, quite a few physician patients who have made a specific point of not doing that type of research. And you have to kind of be true to yourself that sometimes if you want to go in, listen to what you're told, decide if you like the doctor and the plan and not do reams of research, it's fine. There's a lot of stress going on with a new diagnosis and you don't have to do that in the first week or ever frankly. But at the same time I think if your doctor is looking upset or flustered when you come in with a ton of information, that may be a sign of lack of a comfort zone in that area. And rather than getting into an uncomfortable position, it may just be reasonable to wonder whether – I think everybody wants to have a good conversation and a polite and a friendly conversation and I think everybody will be defensive if you come in and say well, you don't know about this and you don't know about this and you don't know about that. And nobody is looking for that kind of an interaction. I think it makes it stressful for everybody.

But at the same time, if you're bringing up something that the doctor doesn't know, you would hope that the reaction is oh, that looks interesting, I need to look that up. And I have to tell you, I do that all the time. I'll say I don't know that one, let's look at that. And sometimes even in the office, if there's an internet connection, you might even pull it up right then and there and try to take a look at its relevance.

So I think the point is that these shouldn't lead to stressful or difficult interactions. And if they are, then you really have to think about it, what's the problem here, why is this stressful, everybody should be on the same page. Nobody should be accusing anybody. It's more that are we all trying to look out for what's out there for me.

Similarly, I think that everybody across the country and across the world needs to be reassured that sometimes it's just not reasonable to participate in a trial. It just isn't feasible. And you can't be sitting at home saying oh, my God, there's no way I'm going to be cured, there's no way I'll be okay unless I get to a trial. Because that's stress, too, and that doesn't help. And actually if it's not feasible or reasonable to participate in a trial, what might be feasible or reasonable is to say well, could

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DR. GAIL ROBOZ: we follow along what's happening in that trial, and see if there might be a time where it opens up some place nearer to me where I could participate. Or is there any program available that might make it reasonable for me to participate in that. Again, the point of this whole forum is not to put pressure on people, that if they can't get access to something, then it's all over. Because there's almost always a way to work things out that improve the access to care, that improve the way that somebody is being followed, but don't make life impossible. Don't bankrupt yourself, don't make it difficult to take care of your grandpa who's at home who needs you there. People have reasonable life circumstances that may prevent them from running out to an academic center hundreds of miles away.

And I think The Leukemia & Lymphoma Society is very sensitive to that also and can be quite helpful in navigating those problems.

LAUREN BERGER: Thanks. And also The Leukemia & Lymphoma Society has a booklet, *Understanding Clinical Trials*. The booklet provides information on how a clinical trial is planned, some of what we talked about today, and also how to evaluate if a clinical trial is right for you. So that is an important resource. And we also have something called TrialCheck®. This is a resource for researching clinical trials. And Dr. Goldberg mentioned that a little while ago, but it's a search tool and it provides listings of blood cancer trials by disease, the patient's diagnosis, stage of disease and also zip code. And The Leukemia & Lymphoma Society can help you search for a trial. So that's not something you have to do on your own. You can just call the Information Resource Center and a trained professional will be happy to discuss that with you, so that's a great resource.

Our next question that was submitted, I'll ask Dr. Goldberg, what do patients need to consider when they're in the decision-making process in terms of going into a clinical trial or not?

DR. STUART GOLDBERG: Well, there's a lot of things the patient has to consider and the doctor has to consider.

To sort of go back to the last question a little bit, though, one thing that I think patients often do need to get out of their first consult or early on is what is the name of their disease. If I just brought home one point to a patient during that first consult, it is what's the name of their disease. And not just the common name, the technical name. Because in all of the things that we talk about, clinicaltrials.gov, TrialCheck, the first thing they question is "this trial is to treat this disease", and if you don't know what your disease is, the technical name you cannot move forward. So if it's acute precursor B-cell, acute lymphoblastic

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DR. STUART GOLDBERG: leukemia, I know it's a long thing, get your doctor to write it down for you, write down the technical name. Because without that opening you can't get into any of these trials. So I think that's a very important thing to get out of your initial consultation or early on, the technical name. And leukemia is many, many diseases and has many different treatments. And the treatments change so fast.

So when a patient does come to me and I say, this is what we might do that's standard, but this is what we can do research-wise, I think the patient then has to start thinking about if I enter the trial, what other things are going to be asked of me? Am I going to have so many more visits that now it's burdening my quality of life. Can I get to the center? Some of my trials, patients can be treated at home and just have to get their labs at home and send them back to me. Some of them have to travel and it can only be done at my site. And that may be a hardship. And we're not trying to cause a hardship. We're trying to advance the field and hopefully help our patients.

So you have to look at what are the extra things that are involved in the trial, above and beyond what would be standard if I didn't go on the trial. I think from a patient standpoint that's often something I want to emphasize to them, is if you're in this trial, this is what's going to be expected of you, and if you're not in a clinical trial this is what's going to be expected of you, because there's a lot that's expected of a patient who's not on a trial.

Often patients will say I don't want to be in a trial because I don't want to come in weekly. Well, if you've got a bad blood cancer, you're going to have to come in weekly anyway. Then the trial may not be that much more burdensome.

On the other hand, if you've got a quiet cancer that doesn't need to be seen except every six months normally, but if you're in the trial you have to come in every month, well, that's five extra visits and maybe five extra visits with your children taking off to drive you to the doctor's. So you have to decide, are these other added burdens burdensome? And then if they are, how much is the altruism and how much does the clinical benefit, that you might think you're going to get, outweigh those?

And then we talk about economics and the cost of it, and I know we're going to talk about that later. So there's those type things. Hopefully the safety questions, as Dr Roboz said, safety, confidentiality, those type of things, if you have those questions, ask the doctor. Because hopefully those have already been thought of by the investigator and should be allayed very quickly by the informed consent process. That's where we're supposed to allay your fears on. But the question of

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DR. STUART GOLDBERG: how much burden by being in the trial is not something we allay, because that's part of the trial.

DR. GAIL ROBOZ: I want to add one more point to the safety issue which I think is one that's worth remembering. The informed consent process, the point is to go through every single thing that could possibly, reasonably, or even not reasonably happen as a safety consideration of the drug.

But what's crazy about this process is that we give you a 19-page informed consent document about a clinical trial, but you don't have a 19-page document necessarily about standard therapy. And I've had a lot of confusion on the part of patients because they'll read the informed consent and go what am I, nuts? I'm not going to have this, this, this. A whole bunch of different problems. I'm going to get the standard.

But we're not giving you a document on the standard. And that's mainly because of the way therapies are designed in this country. We can have a discussion with you of a standard therapy and we can certainly print things out for you from the web in terms of describing side effects and so forth, but a lot of times people walk away from their consultation with the impression that there are 9,000 things that could go wrong on the trial, that wouldn't be going wrong with standard therapy.

And do keep that in mind. Because very frequently the clinical trial might even involve the exact same drugs that you would get if you wouldn't be on the trial. So to keep in mind that in discussing those safety considerations, first of all, you want to say okay, well, what are the safety issues with the standard? But secondly, just ask your doctor, what do you think is going to happen here, which one of these side effects are likely from previous research, which are not likely? Sometimes we don't know. But sometimes we actually have a good sense of that.

And I guess my take-home message is, don't walk away thinking that the 19 pages of side effects on the informed consent document means that the clinical trial has a ton of things that are different from what the standard therapy would be. I think that's a very important separation.

And then I will echo Dr. Goldberg that knowing the name of what you've got actually, even if you just write it down on an index card and keep it somewhere, it helps because often friends and family really want to be helpful and they're trying to do a good thing and they're upset for you and they're bringing you 9,000 buckets of carrot juice to drink and they're telling you don't eat fish or do

DR. GAIL ROBOZ: eat fish or eat a lot of fish. And they're telling you things. But they're also bringing you information based on, half the time, kind of a half or a third of the right diagnosis. And that can actually be very stressful for people, when they're getting recommendations. And I certainly know this is true with, unfortunately, most patients with leukemia, don't actually have chronic myeloid leukemia, so the drug Gleevec doesn't work for their disease. And yet almost every patient who hears they have leukemia wants the magic pill for leukemia. So in order to avoid those types of additional stresses, which you don't need when you're already having enough stress from a new diagnosis, make sure you tell those helpful friends and family that first of all, they can bring expensive presents, that's always appreciated, but more importantly, to not lead you down the wrong path. Don't have you looking up things for the wrong disease.

LAUREN BERGER: John Hughes, would you like to add anything from the patient's perspective?

JOHN HUGHES: Carrot juice. Is that helpful? No, I think it would only be redundant.

LAUREN BERGER: So our next question, I'll ask Dr. Roboz, are there things that you do to help your patients with the decision-making process regarding participation in a clinical trial? And some of that you answered.

DR. GAIL ROBOZ: I think we've gone over some of that. I try, if I can, to let it go home or have a day behind it to think about it. Sometimes in the nature of the diseases that we deal with, the time is not an option. And sometimes patients really are going to just have to make a decision quickly. And considering that most people will ponder which toothbrush to get for 20 minutes in the drugstore, they cannot believe that they're making a decision about a whole bunch of medications that they've never heard of so quickly.

I think that in that sense I would reassure them that if they're leaning toward it and we have to make a decision, we can go forward and get things moving and discuss it every day or several times a day until they get used to it. But that we've got to get moving. And if it seems like the wrong decision down the road, we can back out of it. But to try to reassure people in those rapid decision-making positions that we'll be there with you, you can keep asking questions. But no, we can't actually wait, we've got to get moving.

If there is the luxury of time, then obviously having a chance to have all the family members check in and for people to do research if they want to is very helpful and can sometimes make the process a lot more comfortable for patients and their families.

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DR. GAIL ROBOZ: I do think the most important thing, though, is that second crack at the questions. Because most people say that they forget easily 70 or 80 percent of what's discussed in those first encounters, because you just can't think. To come again, let's re-discuss the whole conversation a day or a couple of days later and take another crack at it.

LAUREN BERGER: Maria, how do you help patients determine when they should bring up issues with treatment, while they're on treatment, and what are some examples of how you help them manage their side effects?

MARIA BALDO: When a patient joins a clinical trial, they are followed very closely and most especially the side effects are followed very, very closely. And each side effect is actually graded on a system of grade 1, 2, 3, 4, 5. So each side effect is graded.

So there are some research drugs that we know a lot about because they're standard of treatment and we prophylax for particular side effects, like GI distress. So there might be nausea involved, there might be diarrhea involved, there might be itching or pain and we can prophylactically treat patients.

Other drugs we know less about, like a Phase I drug, we will know less about. And we are looking very carefully at those side effects.

So we would use the same kind of medications, like Zofran® for nausea and Imodium® for diarrhea, in the same way that how a patient is treated in other ways.

But patients being treated on a clinical trial clearly are in contact with their research nurse or their PA regularly and side effects are documented and graded and shared with the rest of the research team.

And patients should feel very comfortable in talking about their side effects and sometimes patients are asked to write logs of their side effects or computerize their side effects and so the communication is very, very open.

LAUREN BERGER: Thanks. Dr. Goldberg, what kinds of issues come up regarding patient expenses and coverage for a clinical trial?

DR. STUART GOLDBERG: I think as Dr. Roboz started with the common myths, I think, there are a lot of myths about how much it's going to cost to be part of a research trial and also common myths on how it's going to be free to be on a clinical trial. I mean, medicine is not free in general in the United States. We have insurance, Medicare for those who are older and we have commercial insurance. So one of the questions when you're looking at a trial, is what are the costs of the trial?

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DR. GAIL ROBOZ: In general, most trials tend to say that the things that are done standard will be covered standardly. That if typically this is what we charge and this is what's done as part of the standard care, then that's what your insurance company or Medicare is going to pay for.

If we have something that's specific to the trial, that wouldn't be normally done as part of that, then we want to make sure that that part is covered. And in most trials that's what the sponsor of the trial, be it the drug company that's developing the drug, be it the research institution that's sponsoring the trial, the things specific to the experiment, outside of the whole thing. It doesn't mean that every time you come to see me in the doctor's office you're not going to get a bill. You go to the doctor's office normally, you get a bill. But for the part that's dealing with the research, we're going to see if we can do something about that.

Now many trials will put that right up front in the beginning and that's something you should ask right up front. Sometimes we'll make allowances. We know if you have to come extra visits, then maybe there's travel money from the sponsor to get to our visits more often because that's something you would not have normally done. Sometimes that's not part of the trial.

Certainly the experimental drug, you can't buy it on the market, it's experimental. So that obviously has to be provided free. So there's no one right answer. Some studies, patients get their care completely for free and that's the advantage of being in the study. And some, it's just a small portion, but it all depends on what the trial is. So I don't think there's one answer there.

But that's something that should be discussed as part of the consenting process and understand that before you start. But I think the fear that I'm going on a trial, it's not going to be covered, is probably the worst answer, because in all honesty, most trials provide care for their patients at least the standard and maybe even cheaper than if they weren't on the trial.

LAUREN BERGER: Thanks. And The Leukemia & Lymphoma Society has resources not only within the organization, but also some other resources outside the organization they can refer you to.

So feel free to call the Information Resource Center to get information on that.

It's now time for the live question and answer portion of our program. And because we have so many people on our program today, for everyone's benefit, we hope that you will keep you questions general in nature without too many

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LAUREN BERGER: personal details, and the panelists will try to provide an answer to you that's general in nature. And if we're not able to get to all of your questions, once again you can always call our Information Resource Center toll-free. They are open this evening after our program for one hour, as well as during the week from Monday to Friday from 9 AM to 6 PM Eastern Time. And that telephone number is in your packet, but also I'll mention it now, it's 1-800-955-4572.

So will the operator please give instructions for participants to ask a question?

OPERATOR: To participate in the call by asking a question, please dial star-1 on your keypad. If you are joining us by web, simply click on Ask a Question, type your question, and then hit Submit. We will take questions in the order they are received. We can only take one question per person. Once your question has been voiced, the operator will transfer you back into the audience line. Again, to ask a question, please dial star-1 on your keypad or click on Ask a Question, type your question and then hit submit.

LAUREN BERGER: We'll take the first question from our web audience, please.

OPERATOR: Our first question is from Steve who asks can you explain the role of institutional review boards and how they protect patients' rights in clinical trials.

LAUREN BERGER: Good question. Thanks, Steve.

DR. GAIL ROBOZ: I can certainly start with that one. So the institutional review board or the IRB is a committee that convenes specifically for the purposes of reviewing proposed clinical trials at an institution. And generally the members will include a variety of different departments, so not only in the area where the study might be. For example, if it's a study in oncology, there will not only be only representatives from oncology, but there would be representatives from other departments. There are also lay people who are included in the IRB. And the point is to convene a panel of individuals not specifically with expertise in the area of the study, but to provide a broad base of who's going to be listening to and reviewing the study. And the point of the IRB is that the investigators who've come forward with the proposals need to convince the IRB that the study makes sense, that the study sounds safe, that there's protection in place for the participants in the study, and that specifically the study is one that is designed clearly with the best interest of the patients in mind. So this is designed to protect patients.

And the IRBs at different institutions may actually call in sometimes extra panel members if they need something explained or if they need something to have more of a clear explanation or a description, but most of the time what they do

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DR. GAIL ROBOZ: is use both lay language and scientific language to evaluate a protocol, then they come back to the investigators with questions or issues. And those can be questions or issues on any aspect of the protocol. The rationale, how did you come up with this, how does this compare to other therapies, line 472 has the word he instead of the word she. They can question any aspect of it. And they give special attention also to the informed consent to make sure that the document completely represents all elements of the protocol and that the patient would have a very, very comprehensive view of the protocol. So it's really designed as a patient safety protective group.

We cannot run clinical trials without IRB approval. So your documents will have an IRB stamped approval. And any events that happen on the trial are then reported back to the IRB for additional review, to make sure that they're being handled in a way that's consistent with the policies of the institution.

Similarly, data from those trials can't be published unless they are part of an IRB-approved study. And the IRB has the right to, at any point, to ask the investigators to provide explanations or data or follow-ups on what is going on with the trial to make sure that it is proceeding in the way that they thought would be appropriate.

Stuart, anything to add?

DR. STUART GOLDBERG: I think that there's often, as you said, the two parts of the IRB – there's where they look at the study when we propose it and make sure it's safe and that our explanation is correct, that the consent is correct, so that when we give this information to the patient the first time we meet them, that they're signing a document that explains the trial and that the trial is sound, both medically, scientifically and safety. But the other part of the IRB is actually even more difficult and we have to really pay attention as scientists and that is that as the data comes back from the trial, as you're being treated, if there's a side effect, we have to tell the IRB. Hey, there's a side effect. And we have to justify to the IRB that that side effect was worth the risk. And if they start getting calls from all over the country, because many trials are now being done nationwide, internationally, what happens at my hospital may be one out of all the patients who are in the trial, or maybe one out of a lot of patients. Because one at my hospital, one at somebody else's, and the IRBs gather that information and say hey, wait a minute, we've got a problem here. Because one at your hospital, one at your hospital, one at your hospital, that's a problem. And the IRBs gather that information. We have to tell them. And then they start saying wait a minute, there's a problem with the trial in general, stop the trial, it's not safe.

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DR. STUART GOLDBERG: Or go back to the patients and do an amendment to the consent. The trial is having a problem, but we think you can continue if you tell your patients here's the new issue, here's the new information. We just had a success on a trial where the study was doing great, but it was a comparative trial. So the people on the losing arm, they need to be told, they're on the losing arm, but do you want to continue on it? And so we had to go back to the patients and re-consent them because the IRB told us hey, we have a winner and we have a loser on the study, but go back and tell each of your patients what's going on. And the IRB is your ongoing safety monitor.

And in every consent there is that line, if you have concerns you can talk to your doctor, you can call your hospital, or you can call directly to the IRB as an outside source, that is there for your safety. And it's federally mandated. This is not our own institution's idea. This is something that's required by law because we want people to feel safe in the study.

LAUREN BERGER: Dr. DeGennaro, do you have anything to add?

DR. LOUIS DEGENNARO: I thought it was very well covered.

LAUREN BERGER: Thanks, Steve, for your question. We'll take the next question from the telephone audience, please.

OPERATOR: Our first caller is Paul from Ohio.

PAUL: If you have a diagnosis of AML, is Atgam® ever appropriate?

DR. GAIL ROBOZ: Whenever you get the never or ever questions, those of us who take multiple choice exams know about those tricky ones.

Atgam is actually an immunosuppressive therapy, just for the general audience to know. And I would say that the – I'm trying to guess where the question is coming from, but there are potential scenarios in which an immunosuppressive therapy is considered for patients with bone marrow failure diseases. And I would say that it is not part of standard therapy for acute myeloid leukemia, but in trying to guess at where the caller's question is coming from, there are some myelodysplastic syndromes, some myelodysplastic syndromes with certain bone marrow characteristics, where immunosuppressive therapies may play a role. I don't want to ever say without hearing the full clinical scenario, I never want to say never to a question like this, so I think the answer is, is it's not part of standard therapy, but I could imagine scenarios in which it could be included as part of therapy under specific circumstances.

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DR. STUART GOLDBERG: It's not our common; if you pull out The Leukemia & Lymphoma Society's booklet on acute myeloid leukemia and they talk about standard induction therapy and standard consolidation, it would not be mentioned as the standard therapies. But certainly there are research questions of whether immunosuppressive therapies would help in that disease. Certainly in bone marrow transplantation for AML, Atgam is commonly used. So there are instances where with AML, a patient would be receiving immunosuppressive therapies as part of a transplant option or in preparation for something else.

So once again, it would be something that we'd look at the whole picture, but I agree, without knowing the whole treatment plan, it may be appropriate, it may not be appropriate. We'd have to see the whole plan.

LAUREN BERGER: We'll take our next question from our live audience in Hackensack.

AUDIENCE: Hi, this question came because of the panel. You said there's a 19-page document for clinical trials, which is informed consent. But there's nothing for the regular therapies. Do you really think that's true? Because I remember when I went through the chemo, I had to read all the… and sign off on that. So explain that. I had to sign off on regular therapies.

DR. GAIL ROBOZ: So I guess I'd better take that one since I made the comment. They're not always 19 pages, by the way, that was me being me. They could be different numbers.

I'm not exactly sure in your specific circumstance what you were signing off on. So very often when we offer therapies that are considered standard of care, we may in fact provide patient information documents about those drugs and there are lots of different ways to do that. Different institutions sometimes have printed up their own information sheets about the chemo. Sometimes drug companies that are making a certain drug might actually have a booklet or information about it. So I definitely think it is true, and I hope that patients are offered information about the treatments that they're getting.

Typically it's not a requirement that a specific document be signed, though. Just like if you're getting an antibiotic for pneumonia, you don't have to sign a paper about it. Usually if there's a standard chemotherapy, you don't have to sign something about it. And sometimes what ends up happening is that because the doctor is very, very comfortable describing the side effect profile of a standard therapy, sometimes that actually takes place in a conversation rather than with a written document. And I guess that's what I was trying to bring up in my comment, that you can actually have treatment for a blood cancer or for many other types

DR. GAIL ROBOZ: of things without having a written document about it. Nobody might give you a sheet from the computer or a sheet from the institution. And that's perfectly fine. You don't have to sign a paper for it.

What you signed off on exactly I'm not sure, and that may have your own circumstances with it. But that was the point that I was trying to drive home.

AUDIENCE: I read about the documentation. But no, I was given a sheet of paper with the information about that by the pharmacist. And I was asked to sign off on it.

DR. STUART GOLDBERG: Each institution, when we talk about clinical research and we talk about a trial, then we are talking about a formal informed consent form that has certain elements that are required by law in the United States, and that document has been looked at by the external committees, the IRB, and sign off on what the research goals are and what are the side effects, potential side effects I should say, of that research trial.

If a patient is being treated on a standard of care, in other words, what your doctor offers you to do something for anything, whether it's a blood test, whether it's chemotherapy, whether it's a blood transfusion, whether it's surgery, the doctor may have his own hospital-required forms that don't go through that degree of regulatory oversight. In other words, what I write for my patients who are getting standard chemotherapy, is what I've chosen to write. It doesn't have an outside federal monitor looking at that information.

Now some doctors will want to give lots and lots of information, may do it in a conversation. Some may do it by having the pharmacist give you a form about the drugs and saying do you understand this? Some will have nurses go and give their teaching and you'll sign maybe that the nurses taught you. That's what the institution decides by themselves.

But in a research trial we have very specific elements that have to be in that document, that are required by law.

DR. GAIL ROBOZ: And that are the same at different institutions participating in the same trial. And I think maybe that'll help your point, too. That if there are two different centers looking at the same investigational trial, the informed consent, while there might be slightly different language at different institutions, there are basic elements of that that absolutely have to be signed off on in order to participate in the trial. Whereas in standard therapy, people might get different information from different centers. One may have a booklet, the other might have a conversation, the other might have a sheet.

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DR. STUART GOLDBERG: I think that's an important part. One of the major differences between a clinical trial and standard treatment is that in the standard treatment, the doctor is a lot more free to do what he thinks is in your best interest and what the two of you decide to do together. Because it's his therapy, your therapy and you're combining together to come up with an idea.

In a research trial, we're much more by the cookbook. Everyone in the study is going to get the same type of therapy, whether they're treated at my institution or some other institution that's doing the trial. Because at the end of the day we want to be able to group everybody who was on the trial and find out did the drug work or did the therapy work, for everybody. So we all have to follow the same cookbook. We all have to give the same consent, everyone, or similar consent. And we all have to be able to then say we followed the treatment the same way. So therefore the document we give people and the consent we talked about is really what is the recipe, what is the cookbook and how are we going to follow that. And that has its good sides and has its bad sides.

One of the good sides is obviously then there's a whole team. Because the team has to tell me am I allowed to do that or is that something that wouldn't be allowed, because I don't want individuality in a study. I want everybody to be treated the same, if they were at my hospital or somebody else's hospital. You know, if it's 20 different studies at 20 different hospitals, it's not going to be the same as one big study.

LAUREN BERGER: Thanks so much for your question. We'll take the next question from the web, please.

OPERATOR: Our next question from the web is why do we spend money on the results of the control arm of a study, why not keep a very good and complete database on that and only do the experimental arm?

DR. GAIL ROBOZ: There are, depending on what you're treating, there are many different potential controls. And I think that there are some diseases in which I actually really understand the question better because there really is a standard of therapy that is the standard of therapy and anything else could be compared to that.

But especially in the hematologic malignancies, there are actually many controversies about what the control arm would be. And we spend a substantial time in our academic lives arguing and investigators don't necessarily agree what is the control arm.

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DR. GAIL ROBOZ: The other thing is that the control arm differs, depending on the population of patients. So for example, a 65 year old patient with acute myeloid leukemia who is in fantastic shape, who is playing golf all the time, running around, never had another illness, might get a very different control arm as appropriate, versus somebody who has had three heart attacks, in a nursing home, hasn't been out of a wheelchair recently. The control therapy for those patients might be very different.

The other thing is that there is a tremendous bias that can be introduced statistically into looking at what's called historical controls. So for example, the control in 2005 is reflecting what was available in 2005 and supportive care in 2005. So the control arm actually has a very dynamic process over time. The control arm or the, quote-unquote, standard arm, is also a changing arm. And it's for that reason that you actually wouldn't want us to be using one thing that's stuck in time with a standard database because that wouldn't reflect progress.

DR. STUART GOLDBERG: There's a couple of different things. Certainly in a Phase II trial, where we're just treating everybody on a brand new therapy and saying okay, then we're going to look at that, and we might go back and say well, is that better than we expected. Sometimes the Phase II trial is enough to get it approved. I mean, if you have a real winner, if you had nobody being cured with the old therapy and all of a sudden now half the people are being cured, and we had this happen in CML, where two drugs were approved based on Phase II trials, because if you failed Gleevec, if you were one of the rare people that Gleevec didn't work on, you didn't have much. And then all of a sudden two new drugs came out that actually looked very promising and in the Phase II trials, everybody got the brand new drug, they did good. And so the government didn't need a comparison because there wasn't anything to compare it against. So we could say, we don't need a control because we didn't have anything and now all of a sudden we have something that's pretty good.

But if we have a standard that's actually doing okay and we want to build on that, we really need to see what we're comparing it to at the same time frame, in the same era.

The classic example actually happened in lymphoma.. This was actually when I was a fellow. We thought the trial was unethical because in the 60s we had a combination of chemotherapy for lymphoma called CHOP. Four drugs, easy to give, and we cured a few people. And in the 70s we came up with a series of drugs, it was eight or nine drugs, and looked better and people were getting

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DR. STUART GOLDBERG: higher cure rates. And in the 80s we had even another one called ProMACE-CytaBOM. It was like patients were getting chemo almost every week for 12 weeks and it was really tough to get through, but the cure rates had gone up even more. And so we said wow, look at the advances, more drugs, better drugs, and all of sudden the cure rates are going up and up and up and we're getting advances. And then somebody had the bright idea to say wait a minute, are these drugs any better than what we were doing 20 years ago with CHOP. And so they did a head to head comparison of the old drug, which clearly was worse, versus the new ones. Because everybody could read the textbooks and we only cured this many people and now we're curing that many people. But, it turned out in 20 years, we also learned about antibiotics, transfusions, growth factors, our dieticians got smarter, all the things that we didn't think about. And guess what? Head to head comparison, the old treatment was just as good and less side effects.

So if we had just looked at the control arms and said wow, we cured this many people and now we're curing this many people. It wasn't that we got better with the drugs, we got better with the other things. So those are the things you don't know about, so that's why you have to do a control the same time, at the same institutions, with the same type of doctors looking at you. So you have similar things to see, really is it the new treatment or is it all these other intangibles we don't know about.

LAUREN BERGER: Thanks. We'll take the next question from the telephone audience, please.

OPERATOR: The next question comes from Sharon in New Jersey.

SHARON: I'm in New York, thank you. I just want to express, I listen to you often and I want to express my gratitude to everyone. I'm a 33 year survivor of CML. I had a bone marrow transplant in 1991.So I've had amazing opportunity in my young life to watch the field grow, to watch treatments develop. And when I first had leukemia, I was taking what they called experimental protocols. So I understand the concept of so-called guinea pig. And certain things worked and certain things didn't. And there may have been repercussions down the road, but at the time I was very appreciative of being able to be offered opportunities for CML because at that time there was no cure at all and it had a short life expectancy. And then thank God I lived to see the development of bone marrow transplant, where I had a non-related donor in 1991. And I just want to again express my gratitude to not only The Leukemia & Lymphoma Society and supporting research, the programs and doctors, to improve their therapy, but to the doctors, the nurses, the social workers, the PAs, everybody involved in my care, because there's, of course, a

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SHARON: physical component of all of this and then there's the emotional component of handling all of this, and there's the financial component, of course. So again, I just want to really express my gratitude and joy at being able to be here today to listen to new progress and the possibilities that exist for patients across the board with blood cancers. Because in 1977 a whole lot of this was not around. So thank you very much.

LAUREN BERGER: Thanks for sharing. We really appreciate that. We'll take the next question from the live audience.

AUDIENCE: Thank you for taking my question. From your experience, are most patients eligible for trials?

DR. GAIL ROBOZ: That's a great question. It's an unanswerable question, but it's a great question. So I would have to say statistically probably not, because I think what you're getting at is that are most patients who want to be on a trial able to get onto a trial. And I guess that's a hard one, but probably not. So I think in setting people up for whether or not they are eligible, they definitely need to understand that often there are restrictive criteria that are designed both to protect the patients, but also to protect what's being studied. Because if we put patients who are predicted to have the absolutely most difficulty with the new therapy, because let's say the patients have had either multiple other illnesses or major organ system problems, we won't actually be able to figure out whether the drug can help or not.

That said, I think you don't want to make any assumptions up front about eligibility because in my experience most people, the reasons why they think they're not going to be eligible, are absolutely unrelated to what the truth is. So I think it's worth going through the screening process. And I think that most of us can design it in such a way that if there are more difficult parts of the screening process, let's say an extra bone marrow biopsy, which people don't like, or other things which are unpleasant, we can usually take a really good crack at whether or not you're going to be eligible before putting you through those additional elements of the screening. And we do try to do that. But sometimes we really don't know until you go through the entire process.

Don't make any assumptions up front would be my best answer to that question. Because it's never the things that you think are going to be making you ineligible that are the ones that turn out to be the biggest problem.

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DR. STUART GOLDBERG: Some of the problems also are that, you're right, there are a lot of diseases and there's only a few of us who do research or a few trials that can be open. I mean, it takes a lot of time and effort from my whole staff to be able to run a good trial and do it well. And so we have to pick and choose at our institution about which trials we think have the best chance at advancing science and advancing care for the patients. And there may not always be that we have trials for every patient, for every disease.

I think one of the disappointments, that when we do have open trials and we have often patients who we should finish the trials, and that they don't get finished because there aren't enough interest at times from patients.

If you can get on a plane and fly anywhere in the world, you might be able to find it. But once again, we have to look at the practicalities of your local area. But then you have to look outside your own institution and look and see if there are other trials that are within a 100 mile radius and is a 100 mile radius even feasible. If you can drive all the way down to Philadelphia from New York, then maybe you tell your doctor that, then maybe that opens up their options of what might be outside. If you're elderly and have to rely on your children to bring you, you might only have what's open at that one institution. So it often does depend on what your availability is and what the availability in the area is.

Certainly we'd love to have trials for everybody, so we can answer all the questions. But we have to focus our research at times on what we can accomplish.

LAUREN BERGER: Thanks for your question. We'll take the next question from the web audience, please.

OPERATOR: Our next question is from Jeanine. When a patient or caregiver identifies a trial they're interested in, is it advisable for them to contact the study investigator directly or is it better to have the patient's doctor contact the study investigator?

DR. GAIL ROBOZ: That's another great question. For answering that, the problem sometimes is that even for patients and family members who have an excellent, excellent understanding of their disease and who might be able to express themselves very, very well, any good study chair or study coordinator is really going to have difficulty making a commitment with a telephone call, because the last thing we want to do is be promising somebody a clinical trial opportunity without fully seeing the patient. It's almost like doing a telephone consultation, which I think all of us do sometimes if patients are very, very far away, to try to be helpful, but there's really no substitute for seeing somebody.

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DR. GAIL ROBOZ: I think that ideally if a patient is under care of a hematologist/oncologist, if the doctor can contact the study coordinator or a PI, I think that's often the smoothest way of doing things. All of us – Dr. Goldberg and I certainly take calls and our study coordinators absolutely take calls from patients and from family members, but I actually think the process is often smoother and faster when it comes from another physician.

That said, you can also do both. You can have your doctor call and then you can make a follow-up call, at least to get a question of do I need to trek to your institution, are there any slots open, and some of the practical issues that may drive your decision-making about whether you're going to jump into your car and race to an institution versus waiting.

But I have to say that the doctor to doctor call is probably the fastest path.

DR. STUART GOLDBERG: I agree, but that shouldn't discourage a patient who's interested or a family that's interested in making that first phone call. And I think that, as you said, there are some practical questions that you can ask, that is like is the study still open, what type of patients are you looking at, are there any specific things that I should ask my doctor to call about. In other words, if it's only open for this particular disease and you have that particular disease, then obviously you want to find that out. Because studies change, unfortunately, from the time that they show up on the web to the time that we do them. Or the studies may have moved on a little bit longer, we might have some practical information about results that may influence your decision. So if you have a specific question, that's always helpful, to get that first. And then if it sounds interesting, then ask your doctor to make the phone call. And so sometimes that's helpful.

LAUREN BERGER: Thanks. We'll take a question from the telephone audience, please.

OPERATOR: The next question comes from Sylvia in Minnesota.

SYLVIA: Hello, thank you for taking my call. I had the pleasure of listening to your colleague, Dr. Leonard, last week, when he was discussing non-Hodgkin's and Hodgkin's lymphoma. Unfortunately, he wasn't able to take my question because there were so many requests. However, I will generalize. What does a patient do when two top cancer centers offer different advice in terms of when to start a medication? I'm referring to Rituxan®, which has had a wonderful track record in treating B-cell follicular indolent lymphoma, which is what I have. Cancer center number one says take Rituxan right now, even though you are in remission, in order to stave off another reoccurrence. Cancer center number two says wait, you

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SYLVIA: are in remission now, and if and when it does reoccur, and apparently follicular B-cell does have a high reoccurrence rate, at that point you can begin Rituxan. I just wanted to know what your feeling was about a situation like that, which of course, would pertain to any cancer or in fact any disease.

DR. GAIL ROBOZ: I'm sure you are familiar with the joke that if you ask two doctors, you get three opinions. I think that if you ask cancer center three and cancer center four, you're going to get two additional opinions yet. And I think that your question is a perfect one. I'm certainly not going to be able to answer for you personally what to do, but I can tell you that this happens, it happens more often than it doesn't happen. So I would say especially with the diseases that we're talking about, it is very likely actually that if you go for multiple opinions, that you will get differences in opinion. And specifically in some of the earlier clinical trials for patients with more advanced diseases, you might actually, even in the same institution, you might be offered two or three different clinical trials. And I think that is sometimes the most stressful thing for a patient to do, and how do you choose between them.

I have to say that at some point along the way there has to be an element of a coin flip and a trust in one doctor versus another. Because there will in fact be those situations where the science doesn't let us fully answer the question and you have to say to someone what's your gut on this, what's your best guess and you're going to have to go with a decision that the trial data simply are not available.

I think for everybody who's listening to this call, though, to use that as a cautionary note, that if you do go for multiple opinions, you're going to get multiple answers. And sometimes they can be very stressful. We've had plenty of situations where a patient – one center says do a stem cell transplant, the other center says don't do anything. And the patients are flabbergasted, that how could it possibly be the case that either doing nothing or a stem cell transplant might be the right answer here. And yet it could.

So somewhere along the way, unfortunately, you're going to have to put your nickel down on one center or one doctor and say this is what sounds like it's right to me.

DR. STUART GOLDBERG: When everybody agrees, it's always easy. And when we don't agree it's often because it's a controversial area, like follicular lymphoma. Very controversial area. We don't really know the answers. And that's when you get your opinions and go back to the doctor that you trust and sometimes even confront them and say look, I went for another opinion, don't hide that second opinion, I think that's a

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DR. STUART GOLDBERG: stupid answer, go back to the first doctor, the doctor you trust and say hey, I got a second opinion and they disagreed with you and they said this, now come back to me and explain to me why you picked what you picked. And make the doctor justify, just like he explains the research trial, this is what the standard is, this is what the research trial is, and this is why I think this one or this one might be better for you. There may be a reason why he thinks his answer is better or he might say you know what, we don't know the answer, either is good. Or he may say you know what, that other idea might even be better, I hadn't considered that. So go back to the doctor you trust and where you want to get your therapy and challenge them with the new opinion and see what the doctor says. And a good doctor once again should enjoy that. That's what we like to do. We like to help our patients by learning from our colleagues and learning from new research.

LAUREN BERGER: Thanks so much for your question and thanks to all of you for your questions today. Our program has come to a close. Please help me thank all of our panelists for their time and sharing their expertise and their stories. Thanks also to Genentech and Biogen Idec and Amgen for their support of today's program.

We hope that we've answered many of your questions and that the information provided will assist both you and your family in your next steps.

On behalf of The Leukemia & Lymphoma Society and all of our panelists, thank you so much for sharing your time with us today. Good-bye and we wish you well.

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