

Speakers

Steven Gore, MD

Jayshree Shah, APN-C, MSN, RN, BSN, BS

Jayshree Shah: So, I'm going to start only because of time constraint over here. Again, my name is Jayshree Shah. I'm a nurse practitioner. I work currently in the solid tumor area and I've been very fortunate to have taken care of working with and taking care of MDS patients along with other types of leukemia and that, my experience, accounts for about over six plus years. Fortunate because I've encountered all different types of MDSs during my practice in regards to having low risk type of patient, intermediate, high risk and possibly them advancing to AML.

I am going to do a quick overview of a slides in regards to just the basic biology of MDS for people that are new to learning about MDS and to understand what it means in relation to your disease. Sandy Kurtin is the nurse practitioner from Arizona that had developed this slide deck and she did a fabulous job in building the slides in regards to *Building Blocks of Hope* and we want to center this topic and discussion to after lunch as well in regards to how we want to be as a resource for patient and caregivers who are living with MDS. So, I am part of that Nursing Leadership Board and that's what I do in regards to going to different places to talk about MDS and educate patients and caregivers. Why? Because MDS, as you heard from Dr. Gore, it's very complicated. You know, every person that's in this room will have a different type of MDS. There will not be one person here that has the same. You know why? It just may be just one little blip in regards to maybe yes, you may be low risk or your mom would be low risk. Yes, you both have that type of disease state, but what makes a difference is that your cytogenetics may be different than your mom's cytogenetics. Hence, you may fall into a different category. So, that's what I mean that MDS is so complicated and it's so different and it's individualized type treatment and options to take care of. This is all in your binder in case you feel like, "Oh, Jayshree. I can't keep up with what you're presenting." In the binder that you have in front of you, it's all included in there. So, feel free to browse through it with it as I'm going through the slides. The colors Sandy picked are Tucson teal, Navajo red and dessert sand. It, again, it's a landscape of where she works at and she wanted to include all those colors because she's from Arizona. Has anybody ever been to Arizona? Sedona? I love that area. Don't you? Beautiful. The red rocks.

So, this is our list of all our international nurse leadership boards. So we not only have nurses and other doctors participating within this MDS Foundation. We have nurses from different countries participating because MDS Foundation, we have all throughout the world. So, which is really nice. So in case you decide to travel and go from one... from the United States to Europe and you need a treatment, you can probably contact the MDS Foundation and we can make some connections to getting a treatment there or whatever you may need there while you're traveling, going on vacation. So, that's an option.

Building Blocks of Hope, again, most common questions that we hear. This list of questions that wrote on this slide that Sandy wrote or typed up is all from you guys meaning patients and caregivers where you've filled out those questionnaires and said, "Jayshree, what is understanding the diagnosis of MDS? I don't understand it. I need more information." So, these

are the most common questions that come up. Again, all of this is included in your binder. Feel free to browse through it later on. I'm going to address some of those things in this slide deck.

Tools and strategies for success. Exploring the *Building Blocks of Hope*, understanding the disease. I think the key part about MDS is 1) as Dr. Gore said you need to, if you can, talk with your physician or your practitioner whoever's taking care of you to getting the necessary information to compile it and make your own binder, which you're going to use hopefully to take with you to your doctor's office and use it to write down the key importance meaning your blood counts, cytogenetics and what... at your diagnosis what level do you fall into with regarding so the IPSS, the revised version. Which category do you fall into? If you identified that then you can understand where you fall into, the treatment options available, what the doctor's going to present to you in regards to options. Overall, scheduling, going over the side effects that treatments that are being offered to you. Consider lifestyle and issues that may come about with change of treatments that are given to you. When you start a treatment like Vidaza initially Dianna was explaining her story a little bit before we started that her mom was newly diagnosed a few months back and she's on a treatment, Vidaza, but upon starting the treatment, she developed febrile neutropenia and that means basically a fever due to low white count. These fancy words that I'm using, they're all included in the binder. Febrile meaning fever. Neutropenia meaning low white count and that could happen. Why? Because you're dealing with a bone marrow that's where we make our stem cells. That's what we as humans are what we made of. Red blood cells, white blood cells and platelets. So, we have to have three different functions and they have to be working properly in order for all of our cells to function and grow. When one doesn't work or two or three, all three of them don't work, it's basically bone marrow that's not working properly. Hence, that is the gist of development of MDS or possibly leukemia and it's important to identify when you start a treatment like Vidaza or Dacogen or any other treatment. What happens? Initially, I'm going to show you in a couple slides when you start the treatment, you're still cleaning out the bone marrow. It's not like a solid tumor type of cancer that people can have meaning a say, for example, lung cancer or colon cancer where you actually physically if need be when you open the patient up for surgery, you can feel the cancer. Right? You can... solid tumor. It's a solid cancer. When you're dealing with blood, it's like you can't feel it. How do you grasp and understand what's going on because it's in your blood. Hence, that's when we deal with numbers and it's hard. It's hard because you're trying to understand how does this happen. All of it, again, originates from the bone marrow. Okay. So knowing that, understanding MDS itself is very, very important and identifying what type you have is very important. That's a beginning step.

Asking for help. That's why you guys are here today to understand about MDS, to ask Deborah Murray who is outside to register to you to connect with MDS Foundation if you should require more additional information.

Building your own MDS plan. As I started saying, every person in here probably has a different type of MDS. Yes, you may be categorized in saying you have low risk type MDS, you have low risk as well, but what makes you different is that your cytogenetics may be one cytogenetic abnormality difference versus this person. Hence you both are different types of MDSs.

Learning to track your progress. Again, this binder is great. It gives you great avenue to write down your blood levels and kind of monitor along with your iron stuff. Iron overload. If you should have that issue going on, that's another way to track it as well.

So, what is MDS? Just to kind of overview it or just about learning about MDS is basically a broken bone marrow. It's dysplastic because it's not working. Think of it as a garden. Okay. When you're starting a garden, you're kind of raking and getting the soil in order. That's your bone marrow. You're planting your seeds, different types of seeds, whether it be tomatoes, cucumbers, peppers. Right? Those seeds have to be perfect. Right? If they're broken, they're not going to grow into the tomatoes and cucumbers or eggplants or whatever you're growing. Hence, we want your stem cells as representing as seeds. I'm making an analogy. When they grow, they're growing into fruition with whatever vegetable fruits that you're wanting to grow, but when you start a garden what else grows in there? Lots of weeds. We don't like those. Always plucking those out. Those weeds are basically mutations, abnormal cells, abnormal things that don't belong or scar tissue. Think of it that way that can happen within the garden as representing your bone marrow. Hence we want to take those out. Sometimes they overtake and overtake the seeds that you're trying to grow into fruition. Simple concept. Simple comparison. I hope you can make that connection. That's what MDS is. The weeds are basically taking over within the bone marrow. It's not one disease. It's a group of diseases originating in the bone marrow.

Again, what I mentioned cells become. They look abnormal. They look dysplastic. They don't look like these cells that we want to see under a microscope and I'm referencing it to whether it be white blood cells, red blood cells or platelets. Those are the three entities that we're always looking for. Again when you go to the doctor's office, you're always looking for the CBC. Those are the three main things that we're looking for. In addition to other stuff which I'll go over what's involved in diagnosing a patient with MDS.

I love this slide because I'm a visual person. So, I like to see what I'm just talking about and representing and having patients understand. This is your bone marrow. This is where your stem cells are. The actual seed. From this seed, it has actual messages. Right? That seed that you're buying, a tomato seed, it already knows to become a tomato. It already has that message within that seed. How amazing is that? We're born with 23 chromosomes from mom, 23 from dad, combine it together that makes you as an individual. Those are your stem cells. So, stem cells know how to differentiate between different types meaning it becomes a multi-potential stem cell into differentiating whether it be a lymphoid type or a myeloid. We're concerned with myeloid at this time with regarding to MDS. From myeloid, it tells you and differentiates into 5 different cells whether it be white blood cells, red blood cells, platelets, eosinophils, basophiles and macrophages. That's what it does. It already has that message in place. But what happens is for people that have developed MDS and I think there was a question about toxins. There was a discussion about what about exposure to different things like toxins, different types whether it be pesticides, herbicide, Agent Orange, benzene, anything. What happens is those are the extrinsic factors that come about. It can create defects within the normal hematopoiesis. Hematopoiesis meaning that message from the original stem cell gets a little mutated inside and says you're not going to become a tomato. You're going to become a pepper and it's not supposed to become a pepper. It's supposed to be a tomato. Hence, causing this change creates the abnormal immature

precursor cells creating a hyper cellular type of bone marrow, creating basically those weeds to overtake that garden instead of growing the tomatoes and peppers and cucumbers that you like.

See this big list over here. This is what it takes for Dr. Gore or other oncologists to do as far as testing-wise to diagnose a patient with MDS. In addition to that, it takes a really good hematopathologist. Who is that? Yes, Dr. Gore can do the bone marrow biopsy on any patient that's here or your mom and say here's the biopsy. We got a couple samples there from the biopsy. We got the core bone chip from that bone that I showed you earlier. We got the blood from the bone marrow. That's sent off and there's one more. Cytogenetics. That's also the liquid part sent over to a special lab and identifying who... Sorry, what was your name. Stanley? Who Stanly is? What are his chromosomes? Does he have mutations? So, that's a separate lab that does the information. So, those two other things that are here that are in this place, say John Hopkins, for example, which is the bone chip and the liquid part of the bone marrow. That information is sent to the pathologist. Hematopathologist is basically a specialized pathologist that does only reading of Stanley's bone marrow and he will identify what is going on within his bone marrow. He only specializes with leukemia type patients or bone marrow failure type patients. He or she has to be very keen in identifying it because, again, MDS is complicated. There's so many different subtypes. You want to make you're not missing something. The important part is identifying does Stanley have scar tissue? I hope you don't mind me using you as an example. It's in the book, Ms. Toby. All of this is in the book if you wanted it. So you don't have to write all of this down. Identifying if Stanley has scar tissue. If he has iron in the bone marrow to make red blood cells which are the retic baby cells. Does have that information? Does he have those stem cells working properly? Does he have dysplastic features? When he or she looks under the microscope, do those red blood cells and white blood cells and platelets, do they look abnormal? Do they look dysplastic? How many are there? Within a slide if there's only two platelets that are floating around that just tells you that there is low platelets issue within that bone marrow. He should be making enough to counteract and make sure that it's plenty around. Those are the things that he's looking for and then he does an analysis of blast count basically looking at the white blood cells that don't look normal. So again, along with this and a good hematopathologist, put that information together makes a diagnosis of MDS. There's a lot involved in it. It's not just simple as oh, let me do a PSA level and identify an abnormal reading to say oh, the patient may have prostate cancer. It's not that easy. You need to do a whole workup. Yes, Toby?

Toby Cohen: Can I ask you a question? What does TSH stand for? Thyroid (inaudible 17:06).

Jayshree Shah: You answered your own question. Yes. The question was what is TSH. TSH is a thyroid analysis.

Toby Cohen: (inaudible 17:14).

Jayshree Shah: Right. So a patient can come in with a basically feeling very tired and fatigued. Patients can come in for all different reasons to a oncologist that maybe be sent over.

Toby Cohen: (inaudible 17:28).

Jayshree Shah: Exactly.

Toby Cohen: There's no correlation.

Jayshree Shah: There's no correlation. No. Correct. When you get a new patient that comes through our doorstep, we do a whole panel and that's included in the panel because sometimes that may be not found as an issue as a sidebar when we identify it as another issue to take care of and monitor. So, we don't want to miss it early on. So, we want to do the whole package together. Testing yup.

This is a nice classification system. Again, these are all the different types of MDS and within it there is different categories or different ways of identifying it, what's included. All of this information is in the book. Again, what is representing in regards to the blast count. Blast counts mean leukemic cells. If people had a question about that what it defines. Blast cells are abnormal white blood cells that don't function to protect you against infections. It could be bacterial. It could be just viral. It could be anything, but it's not protecting you because the numbers are low. You should be making enough white blood cells, neutrophils. Dr. Gore mentioned that, ANC, Absolute Neutrophil Count. It should be protecting you against infections and when it's low, it's not going to protect you.

Again, we use an IPSS scoring system to identify where the patient falls into category. Why? Because it becomes a discussion of treatment options. If the patient has low risk MDS with only... the only problem is the red blood cells, well after we do the EPO level, they may be eligible to get a growth factor such as Procrit or Aranesp. We don't want to start them off and say, "Oh, you need chemo treatment." You got to identify where they're starting and then offer the different treatment options appropriately to that diagnosis. So, that's very important within that category of identifying with MDS, you need to find out the blast count, the cytogenetics and the cytopenias. Cytopenias meaning red blood cells, white blood cells and platelets.

Issue. This is the new revised version that we use. Again, it depends on the physician. It depends on the practice. If you go to Georgetown, if you go to University of Maryland, they may use WPSS scoring system versus IPSS. It's variable. It depends on the facility and depends on preference.

This is a nice overview about just to give you the input which cytogenetic abnormality risk group that patients can fall into. Again, make sure if you're collecting your information and identifying what type of MDS you have, you need to know which category, which cytogenetic abnormality you have and then you can identify do you fall into very good, good, intermediate or poor risk. So, that's a good identification and the risk categories include, again, there's five different things that we look at. We look at cytogenetics. We look at blast count, hemoglobin, platelets and ANC. Again, that's what physicians do. They add up the numbers and to identify which category do they fall into. Yes?

Q1: (inaudible 21:04).

Jayshree Shah: Absolute Neutrophil Count and that is your immune system. Yes.

Q1: (inaudible 21:11)

Jayshree Shah: And after you collect all of that information, people may want to know well what is my survival? How long do I have possibly in survival as far as risk to leukemic transformation. This is a nice overview in that. Again, the facts about MDS. There's an average age of diagnosis is 73 years of age. MDS remains incurable malignancy for the majority of patients. Stem cell transplant is the only cure. The leading cause of death is the disease itself representing about 80 percent and there are risk stratified treatment strategies are available, but again it's individually based. It does not apply that your mom can only get Vidaza or all of the treatments versus the other patient who may have 5Q deletion and they may be eligible for Revlimid. So, it's individually based.

Again, treatment triggers that we look at initiation of disease modifying therapies. We look at transfusion dependence. Where do they start off with diagnosis of MDS? Progressive or symptomatic cytopenias, increasing blasts or high risk disease.

We look at all of the other options as well when we are identifying a new patient with MDS. What is their performance status and this is also kind of included in the package of identifying a patient for possibly transplant as a candidate. We look at performance status. Comorbidities. If you have comorbidities and you're 65 years of age what are we shooting for? Are we shooting for palliative therapy? It's a discussion. Are we shooting for a cure knowing what the risks are involved with going through those steps. Again, identifying the IPSS category where you're starting off. Primary versus secondary MDS. You guys have heard of Robin Roberts. You guys know that she developed MDS and after receiving treatment for breast cancer. So, that is considered secondary MDS and that's because she received chemo and treatment for one cancer and her bone marrow went into failure and we don't know how, we don't know why but we think it's cytogenetic... cytotoxic meaning chemotherapy related that did something to her bone marrow. Again, those stem cells, those seeds that I'm talking about. They became defective. It's not working. So, she needed a transplant to replace those seeds with the whole seeds like tomatoes, cucumbers and perfect seeds. We're also wanting to look at cytogenetic status and lifestyle. So, we look at the whole panel. We want to identify how Stanley is doing. What is his lifestyle? Is he functioning? Is he doing his activities? What is his quality of life? All of those big questions.

What are the current treatment options right now for MDS? We have starting off as supportive care. We definitely do all of this, transfusions, growth factors. There's Revlimid available that Dr. Gore mentioned earlier. Vidaza as well, Dacogen, chemotherapy. There is chemotherapy meaning cytotoxic agents. So, that includes right now we Cytarabine, Clofarabine rarely used, very expensive. Etoposide, bone marrow transplant, investigational agents and that's where we're at right now because we need to come up with more options for MDS patients because we have very few. Unlike breast cancer if you think about how many different options are available? There's more monies going into that research area than MDS. So, MDS needs to have a little bit more of an open voice and awareness definitely made. FDA approved agents are limited. Maximizing each option to its full benefit is critical and I think that's what Dr. Gore was trying to mention earlier. You don't want to switch therapies after 6 cycles if it's stable and saying, oh,

it's stable. No, no. I want it switched over to another therapy. Well, we only have a few options. We don't have 10 options to go with. So, stable is a good thing.

I'm going to stop here because the lunch is here and I'll pick up in about 45 minutes or so. Is that okay with you guys and I actually want to hear after... I have another probably 10 more slides left and then I just want to open up the floor to you and we're going to talk about quick tips as a resource which is included in your binders to help you as patients as caregivers in how to manage MDS. So, I'm going to talk about that in a little bit. Let's eat. Get some food in our belly. Thank you.

Do you guys mind if I take over a little bit of time from your lunch? Yeah? Okay. Alright.

So, I want to just share with you the different types of therapies that are under investigation. I know some of you guys may be on a clinical trial already or maybe considering or wanting to know what's out there. Dr. Gore mentioned a couple things that were out in the field, but other trials that are in the works or on their way to FDA approval. The drugs on the left are the different agents. Entinostat. That's the drug that Dr. Gore was talking about with the Vidaza that he did studies on in 2003. It's still being worked on and carried through. So, that's one of the drug. There's also Onconova. There is Everolimus. These are all oral agents pretty much. There's one IV infusion in there. Again, there's stuff out there for clinical trials. They may take a little bit of time to research it but use MDS Foundation as a guide. If you want to know your situation and whether or not you qualify for a clinical trial or you have a question, MDS Foundation can connect with the Centers of Excellence physician. Again, we have Centers of Excellence throughout United States as well as in the world. Contact them. They can be your middle person. Contact them and find out do they have a trial open that you potentially may be eligible or how you could participate or get information about. So, use the MDS Foundation as a resource. That's what you guys are here for to learn more.

Key principles of therapies in MDS. Again, stem cell transplant remains the only cure. Again most... this is primarily not an option for all of patients that have MDS because of comorbidity issue or insurance issue. It could be multiple different issues. Age alone should not include active therapies and that's definitely a hot topic for discussion because I've gotten to see or meet many patients that come through our doorstep for consultation and they have been diagnosed with MDS and they've been told, "Well, blood transfusions is the only therapy for you and that's it." Then we present to them different active therapies that they may be eligible for or even on trial and they're just amazed. So, I welcome and I encourage patients to be their own advocates, call MDS Foundation and find out what's out there as possible treatment options.

All active therapies for MDS require time to work. Again, you heard from Dr. Gore himself. Again, with MDS it takes a while to clean out those weeds. Things don't, in the bone marrow, clean up automatically with the therapies that are out there except chemotherapy. Vidaza and Dacogen, they're not chemotherapy. They're different agents that work, again, with cleaning out those weeds and it's a slow process. You need all that spray every month for five days or seven days to clean out those weeds. Blood counts often get worse before they get better. Diana's mom has just finished one cycle of Vidaza and she developed a complication. She may have developed possibly a few more complications before her body says, oh, I know what to do now. I have

enough good stem cells that are new that can balance and replace and help her with her energy as well as to fight off infections, but it's going to take time. It takes awhile to clean out those weeds before it gets better.

Proactive management of side effects in early phases is very important. That's why you guys are here for. What can you do if you're currently getting active therapy to help you feel better possibly to life style change you could do? What other resources are out there? What could you do? That's why you have the binder in front of you. Go through it when you have a chance. It has great resources for Quick Tips. We're going to talk about fever, diarrhea, management of foods. There's lots of questions about what can I eat. What can't I eat? Well, people tell me I can't go dig in the soil and to garden. What should I do? All kinds of questions because people think that they need to be in a bubble when you're diagnosed with cancer. You don't. That's the key idea. You don't. We want you to have a quality of life to function because we know when people go into that bubble there's a trickle effect. You get depressed. You become isolated. You don't want to share. It's just a down slope. We don't want that. We want you to function. We want you to do the best you can so you do well with the therapies that are out there. So, this is what I meant by what happens with the cleaning out the bone marrow. Before treatment begins, blood counts are dropping with patients that have MDS. We've identified that. There's a problem. It progresses and normal blood count cells are crowded out because, again, the weeds are taking over the good stem cells and that's what's happening within this bone marrow and it's dipping more. With that the ANC, which is the white blood cells, they go down. Hence, Diana's mom's case, for example, she developed, again, an infection. She had to get IV antibiotics, get over the hump. That's what we call it basically to get over this infection, so she could get number two cycle.

So within that as patient begins to respond, again, it takes awhile. It takes up to four to six, sometimes a little bit longer for the bone marrow to begin recovering allowing it to make healthy blood cells. That stem cell needs a little bit of fixing. Blood cell counts should rise and symptoms of MDS should improve over time. Hence when you see these over here, those are blasts. Those are abnormal white blood cells. As you're cleaning it out, those white blood cells, those bad blasts, they tend to disappear. These little things. Those are platelets. Purple. Purple dots. It's so cool if you ever get to see your own smear of your blood cells under a microscope. It's amazing because you're like, "Wow. These are my cells." It's so cool because you see your red blood cells, your white blood cells and your platelets. It's amazing that we're made of this. We got to fix it. Early toxicities may be difficult and discouraging for the patient because it's problematic. People get like why isn't it fixing? Why isn't it getting better? And I'm sure Diana's mom is probably thinking the same thing. Why am I not getting better? I got treatment. Why isn't it getting better now? Well unfortunately with MDS it takes awhile. Those weeds are just oomph. They're overlapping each other and you got to take one weed layer at a time out to fix it.

Working together is the best response. Again, be patient. Dose modifications and delays, they're okay to do if their patients having struggling while they're getting the therapy. Supportive care. It's very important that you support the patient. Her mom may require maybe a few more blood transfusions or platelets for the first four to six cycles, but guess what. When it starts working

you know what happens. They become transfusion independent. They have a better quality of life. They have a little bit more energy.

And setting expectations. That's what Dr. Gore was trying to say. It's so important when you're first diagnosed that you maybe not the first, maybe a couple meetings after to have that discussion. What are your expectations? Let me tell you what I can do as a practitioner for you. I can definitely give you supportive therapy meaning transfusions, Procrit, Aranesp, Neupogen, whatever the case may be. What are your expectations? What do you want out of this? What do you want as a goal? That's so important to have that discussion with your practitioner or oncologist because if you don't set those expectations that question will linger and linger and linger and you may think, "Well, I want to be cured," while the physician's saying, "I can only do two things – Vidaza or Dacogen and that's all I can and if you progress then I can give you chemo," but again, that's where the discussion needs to happen with your oncologist to be open and honest.

Trilineage. Response following four cycles of Azacitidine. This is actually Sandy's patient, the nurse practitioner from Arizona that has taken care of a patient with MDS that has gotten Azacitidine which is Vidaza and if you look the patient started off with over here hemoglobin of going over at 12 and then going over with the white count going over... I'm sorry. Looking at the numbers over here. The hemoglobin is over there. Platelets down here in the lows and the white blood cell is low over here. So, we're starting off with 2 problems with the hemoglobin still steady. Once you do giving treatment and you're... or diagnosed with MDS after cycle 1, 2 and 3, look what happens to the platelets. It goes straight up as well as the white count. What we're trying to say is that it takes awhile, again, to clean out the bone marrow before it starts working. This is another of Sandy's patient that is on over 10 years of Revlimid that's been diagnosed with MDS. Patient sustained a great moderate to but asymptomatic cytopenias and that's a new normal. Every person's treatment that they receive is a new normal because what you started off was not feeling well and then you started the treatment and that becomes your new norm. What we're trying to say is that every person's experience is going to be different and your body and your mind and your physical activity changes while you're going through the treatment. There's going to be a new normal for you and every person is in their own way considered a normal.

What can you do to stay health? A good balanced diet is very important. Daily activity, exercise, avoiding infection, bleeding. Continue to enjoy things you love to live. That's why you guys are all here to live, to learn. Get enough rest. Again, use and explore the *Building Blocks of Hope* that's included in the booklet, in the binder. Ask for help when needed. That's why you guys are here to ask for resource information. Be an active participant in building hope. I heard from a couple people about support groups. As a starting point if anybody's interested in Baltimore area, is somebody interested in starting something like a small support group for just patient and caregivers to maybe meet once a month or something like that. That's why you guys are here to share. So, I want to learn from each one of you.

Just a quick tabs. Within the binder, tab number one has Understanding of MDS which I kind of briefly synopsisized earlier. Seeking Treatment is tab number two. Quick Tips is great because, again, it gives you nice ideas of what to do if you should develop a cut or bleeding, fever,

diarrhea, nausea, vomiting. It gives you some guidelines what to do. Iron Overload is huge for number tab four. Why is it so important? Because that's part of the package of somebody newly diagnosed with MDS. We do a ferritin level. It's a blood test evaluation to identify patients who are iron overload and we know their survival is going to be longer enough that it may be beneficial for them to start a chelating agent of some kind so they don't develop other comorbidities like diabetes, heart issues or thyroid issues. Did you know iron not only deposits in your liver, but it deposits in other organs. It can develop deposits in your heart, pancreas, kidneys, thyroid. So, you're thinking, "Oh, my God. That's scary, Jay." It is. It is. And that's part of the whole package. We take a look at a patient and we look and develop a plan of action for each individual patient. Diana's mother may not have iron overload, but her survival is going to be 5 years. So, we know that she's going to live potentially 5 years. Why would we not address that now versus waiting 3 years and she's gotten 20 units of blood transfusions. Something to think about and have that discussion with your practitioner and oncologist is good early on. Tab number 5 is Your MDS Plan. Talk about and write down what is your diagnosis. What is your understanding of MDS. Talk it over with your oncologist and your practitioner. This is my understanding. Tell me if I'm reading this correctly. And number 6, MDS Foundation. It gives you a nice synopsis of all of the different things that we offer as an organization online and live support. Somebody asked me earlier, "Jayshree, can I have a copy of today's slide deck and as well as discussion that we're going to have. How can we get a copy of it?" Well, all of this is being recorded to share with you and others that may be interested. All you have to do is click on it. Give us a little bit of time. They're going to be downloading this information onto the computer and you click on it and you can hear it.

Q2: (inaudible 42:02)

Jayshree Shah: They just started doing it again as far as downloading it. They're working on... they just did Chicago, I think. Deb just told me I asked her about it.

Q2: (inaudible 42:14)

Jayshree Shah: They just restarted it. They were still taping it, but I think limited resources and funding didn't allow us to do that. Just recently we restarted the program. I was just talking with Deb outside about it. She said, "Jayshree, we just got some funding to support it," but I will ask Deb exactly what part of the website you can click on it to download it, but that's a good question. I'll ask her after we're done.

Acknowledgements. Again, patient caregivers and contributors, I thank you for coming today and sharing. We're going to share a lot now. MDS Foundation Executive Committee authors Sandy did a phenomenal job in putting all of this together and that's it for me. I am going to close this out. Now, I open the floor because if you can, introduce yourself please and the reason you're here and how long you've been diagnosed with MDS and what treatment are you currently at and then the last question is what is your expectation. What is your expectation? And ask your questions after we introduce if you don't mind. Is that okay? Alright. Go ahead.

Diana: My name's Dianna and my mom has MDS and she was diagnosed a couple of months ago and she has... I always have to look it up because I never remember.

Jayshree Shah: It's complicated.

Diana: (inaudible 43:45) so it's like a medium high risk or so it's pretty aggressive. So, she just started treatment with the (inaudible 43:57) like a month ago and then like (inaudible 44:00) she got... she had a high fever and she was hospitalized for three weeks (inaudible 44:04). Just got out of the hospital, so that's why she's not here. So, I mean, our expectations are we just... the reason I'm here is because I think we're in a place where we just don't know what to expect because there's... it's just...

Jayshree Shah: Too new.

Diana: Yeah and the other thing is that everything we hear is we just kind of have to wait until we know whether the (inaudible 44:24) works, but if it doesn't work, she might... her life expectancy might be less than a year, but if it does work then it could be up to five years. So, it's just hard to deal with that uncertainty and I'm thinking the more I learn about it the more I see that it's a very much a day to day process. We're not really sure what to expect.

Jayshree Shah: I think if you group that information about your mom regarding her status and what she's doing and what's so I think possibly that may guide you in the right direction. Thank you. Go ahead.

Toby Cohen: I'm Toby Cohen. My (inaudible 45:07) in 2001 (inaudible 45:12) red blood cell counts, but I wasn't diagnosed until 2004 and the drop in the blood count was attributed to my (inaudible 45:28).

Jayshree Shah: Interesting. Did you guys hear that?

Toby Cohen: And until... actually, I... just stop me if I'm giving you too much information, but I actually was (inaudible 45:42) for other reasons and... Again, stop me if I'm giving too much information, but I was a patient at Memorial Sloan Kettering for other reasons and they picked up this low drop in my blood count and the internist there just attributed it to my not eating meat and it wasn't until I went to a non-oncologist for a routine colonoscopy that he said to me, "You know, your red blood cell count is low and I said, "Oh, yes. I know. My internist at Memorial says it's because I don't eat meat," and he said, "Nonsense. Your iron levels are fine. This has nothing to do with nutrition," and he called up the doctor at Memorial and told him he was being very cavalier. So, that's when I was sent to a hematologist and they did a bone marrow and diagnosed MDS. I'm receiving blood transfusions, but I have low EPOs and so I'm wondering whether it wouldn't be better for me to have Procrit and there are a whole lot of other questions that I have one of which I answered... asked Dr. Gore and he was not too receptive to it and that's basically my story. I'm finding it very hard to get information that I need for my own specific situation.

Jayshree Shah: You know...

Toby Cohen: And I'm also finding it very hard. My understanding is that Seattle is one of the best places to go if you have MDS or certainly for a consultation and I'm finding it very hard to find out which doctor I should see there, but I clearly need some consultation.

Jayshree Shah: Maybe MDS Foundation could be a good resource to assist you because they've done several, I guess, a reach out programs in regards to contacting the physician with Centers of Excellence and contacted them and letting them know Toby's story by E-mail and saying this is the type of patient, this is what's going on. Is this somebody that you may be able to see for a consultation? If Seattle is the place you want to go to. Again, there is lots of Centers of Excellence. I think you may want to reach out and speak to MDS Foundation a little bit more to seek out what you're exactly seeking. It sounds like you're seeking a second opinion as well as more information which is always valid and great but know that when you seek more opinions, you're going to get more opinions.

Toby Cohen: Thank you so very much.

Jayshree Shah: Go ahead, Stan.

Q3: We found out we were neighbors.

Jayshree Shah: Where are you from, Toby?

Toby Cohen: Manhattan.

Q3: Are you, too? No. I'm live here in Baltimore but my mother is down in Fredericksburg, Virginia.

Stanley Rudick: Very good. Hi, my name is Stan Rudick. I live up in Sparks, Maryland and I went through a lot of what you're going through now. I did have MDS, was diagnosed in January of '09, 3 years ago, almost 4 years ago and in September of 2012 which was just last September, first of all I was under Revlimid because I had the 5Q deletion and the Revlimid was a godsend. It kept me very normal, did all my activities and I thought I'd just live forever with Revlimid and then in August of 2012, my... for some reason my platelets dropped dramatically and I was on bone marrow biopsy said I had a 30 percent blast. So then I was diagnosed with AML last September and so I'm under care now at the University of Maryland over at the Greenbaum Cancer Center which is very, very nice and I'm on Decitabine, 5 days every month outpatient and I'm doing very well. I am blood... I do take blood and platelet transfusions as needed, but my life is, knock on wood, is very normal. I get tired maybe more than I used to when I was 35 years old, but I went through this and as I said I was blessed to have 5Q deletion because Revlimid was researched by Celgene and it... I was doing very, very well. With chemo, I might be on chemo the rest of my life which is... I hope is very long and I was going to say seeking information about MDS. I personally found more information when I had MDS research, just everything. One was MDS Beacon. Is anybody familiar with that? It's wonderful. It keeps you up to date on the latest clinical research and also another one is the Bone Marrow Forum, I think it's called. Excellent and it really discusses a lot of people put in their personal... they don't use their real name, but they put in what they're going through, what they're doing, what drugs

they're on, what this... and it gives you an idea of what's out there and you can add to it on the forums. So, those 2 are excellent.

Jayshree Shah: Thank you.

Stanley Rudick: When I went from MDS to AML last September, I missed having the MDS Beacon E-mail me once or twice a month with information. I called MDS... I called the MDS Beacon, it's a program. I said, "Is there an AML Beacon," and they said, "No." So, I actually... I'm having more difficulty finding resources and people going through this than I did with MDS. So, MDS is doing a great, great job.

Jayshree Shah: Thank you.

Stanley Rudick: I have to say that and I just appreciate great care that I'm getting. I appreciate great research from Celgene and also I am on Exjade for iron overload which comes with blood transfusions. I started that about a month ago and knock on wood very little... no side effects at all. So, I've had either AML and MDS for four years now and I'm doing well thank God.

Q3: How long (inaudible 53:03).

Stanley Rudick: Let's see. I had one in January of '09 to discover MDS and I didn't have another one until late August of 2012 because they don't give you the bone marrow biopsy unless something is happening up or down. If you're cruising along and you're stable there's no reason to put you through a bone marrow biopsy.

Jayshree Shah: Unless you're in a research trial and it automatically sets a guideline and saying cycle one, cycle three, cycle five.

Stanley Rudick: When I started the clinical trial with AML, my hematologist who was taking care of me with MDS for three years as soon as he saw my platelets drop, the bone marrow with 30 percent blast, he called me up. He notified I had AML by the telephone because he didn't want to waste time. He said, "Stan, with your permission I'm going to call down at one of the University of Maryland and get you into a clinical study tomorrow, next day." So, I said, "Please. You got my permission." The University of Maryland called me that night. We expect you at eight o'clock in the morning. Bring this, bring that and within seven days I was on chemo, the following Wednesday. So, and that's... I also had a bone marrow biopsy when I started the chemo program clinical trial and then they did one 1 month later after the clinical... this is September. I had a bone marrow in October and my blast went down to 18 percent after, let's say, 2 months of being on chemo, but it's still above the 10. So other than that, any other questions?

Q4: It sounds like you had a great oncologist. (inaudible 54:51) hematologist an MDS specialist (inaudible 54:54)

Stan: General hematologist at GBMC, Dr. Robert Donnegan. I'll give him credit. Wonderful man, but I have a great doctor, Dr. Marie Bear at University of Maryland and I'll say this. I don't

know if all getting blood transfusions or platelet or chemo. The infusion centers, they are the most lovable people, caring.

Jayshree Shah: Thank you, Stanley. One second. You count very much.

Brian Rudick: My name's Brian Rudick. I'm Stan's son. I'm here for (inaudible 55:37).

Jayshree Shah: Do you want to share anything?

Brian Rudick: No.

Jayshree Shah: He's here. Okay.

Q5: Could you tell us the MDS Beacon (inaudible 55:51).

Stanley Rudick: www.mdsbeacon.com Like Beacon of a light. It's incredible and they'll set you up, get an E-mail when everybody has something. Twice a month (inaudible 56:05) three times a month. It's wonderful.

Bill Greg: Hi. My name's Bill. I'm here with my wife Carrie. She's my number cruncher. She keeps track of all my numbers.

Jayshree Shah: Thank God for caregivers. Right?

Bill Greg: Exactly.

Jayshree Shah: I say thank you. Thank you.

Bill Greg: We're from Springfield, Virginia. In 2008, I was diagnosed with MDS and I guess right now I'm low risk. I haven't had any transfusions, haven't had any treatments. Just as a matter of fact, I had a bone marrow done last week. We're waiting for my results next week. See how my numbers are doing and if I need to start anything. So basically I'm in a wait and see and monitor.

Jayshree Shah: Very good.

Bill Greg: I'm not on any medicine.

Jayshree Shah: He's a wait and watch. I hope it continues.

Bill Greg: Yeah. Me too.

Q6: (inaudible 57:04)

Jayshree Shah: Did you want to speak? No. Okay. Well, thank you for coming.

Gerald Genet: My name is Gerald Genet. I think I've been under MDS for 2004 or '05. I've gone through 5 physicians because my caregiver will not accept the answers some of them have professed and the answer is asking questions, which I don't ask questions. My only illness now is I'm sleepy. I fall asleep at red lights. So, I no longer stop at red lights when I'm driving.

Q7: (inaudible 57:51) question. How is your low platelet count?

Gerald Genet: I'm very lucky. I've been through in New York, Columbia Presbyterian (inaudible 58:05) Center, Mount Sinai and at one time at St. Vincent's Hospital. Each one is a specialist which we didn't want to accept the answers that they were saying and then we ended up at Sloan Kettering with a physician who I think is spectacular. Why is he spectacular? He communicates and the problem we ran into is the doctor who communicates also said, "We're not going to do anything. We're going to monitor you." So, it's 3 years that I'm being monitored instead of being on these medications which every one of them wants to institute except my caregiver and my champion who (inaudible 58:51) start in with me. The most important thing I think I realized that the doctor I was using at Columbia... at Sloan Kettering communicates. He says, "You have any questions, send me an E-mail." I don't do E-mails but Randy's not bashful as you can see and if she sent him an E-mail at eight o'clock in the morning, by 8:30 he would have responded. This made me very happy because there was an open dialog and this is a mystery disease because it's a guessing game today. They're guessing to try and find that magic wand that might help some of the people which will lead to other helping. I guess that's part of discovering all new diseases and trying to discover how to treat it and how to treat the individual. My (inaudible 59:43) is a lot different than most people. I don't think my wife appreciates it, but I'm 80 years old. I've had a good life. Grandchildren and I got a big champion. So, I'm very fortunate and thank you for listening to my story.

Q8: What's your doctor's name at Sloan and Kettering?

Q9: He's not there anymore. So, it doesn't matter.

Gerald Genet: He was offered such a fabulous position in Florida that he couldn't refuse it. I'm using two doctors in Sloan Kettering now.

Q8: But (inaudible 1:00:12)

Gerald Genet: (inaudible 1:00:14)

Jayshree Shah: Where is your physician in Florida?

Randy Genet: I believe he's at the Sylvester Center in Miami.

Jayshree Shah: Steven Niemer, N-I-E-M-E-R. He's one of the original kind of founders of what MDS is and again, this is an evolution in itself. MDS was not like a known type of a cancer if you think about it 15 – 20 years ago. How many times have you heard news or anything regarding MDS. What is that? Yes, Toby.

Toby Cohen: Actually, my father died in 1991 of sideroblastic anemia which is what MDS used to be called and no one, no one ever thought it was a cancer.

Jayshree Shah: Exactly.

Toby Cohen: I think it's treated at Sloan even at Sloan.

Jayshree Shah: But at the bigger cancer centers. I'm talking about in just general. Breast cancer is well known. I'm just giving you guys a comparison. MDS was not known and we've come... we're coming along slowly in regards to awareness of what this is about for other people and general population to be aware of.

Q11: I think (inaudible 1:01:34)

Jayshree Shah: It did. I totally agree with you there. We need a champion spokesperson to speak about MDS. It's similar to, again, I'm giving you another example of breast cancer. You need somebody that had a famous person that had it that went through it to talk about it in a positive light about their experience for people to aware that this exists.

Randy Ganet: Toby, how long did your dad have that diagnosis?

Toby Cohen: Well at first, they couldn't figure out what was wrong with him and we're talking about Cornell and Presbyterian. From 1986 to 1991. He fell ill in 1986 and he died in 1991.

Jayshree Shah: It's becoming one of the bigger kind of like a goal if you want to call it in medical school and for physicians to learn about MDS. Physicians 20 years ago or 30 years ago, they didn't know about it and they knew of it as an anemia.

Randy Ganet: So, how did they handle it then? Did they give him drugs or just monitoring?

Toby Cohen: Well, it started affecting him more so I mean they treated for heart failure and also he died of kidney failure but no one treated the anemia directly. I mean, it wasn't (inaudible 1:03:05) and (inaudible 1:03:10) regarded as a cancer.

Jayshree Shah: At that point. Correct. Again, this is all new. It's all new.

Q12: Is it unusual for family members to have anemia?

Toby Cohen: Well, there's no genetic (inaudible 1:03:25) presumably in my case. According to the doctors it's just coincidence, but it can run in families and it can just like breast cancer with (inaudible 1:03:40) and apparently I don't know the name of it but there is a name.

Jayshree Shah: Yeah. BROCO1 and BROCO2.

Toby Cohen: No, I mean for MDS. There is a gene that is familiar in MDS. (inaudible 1:03:56).

Jayshree Shah: Not that I know of but I think you can have something in relation to an anemia or some kind of issue that can be passed on as a possibility, but it's not very likely. No.

Randy Ganet: I'm Randy. I am chief investigator and cheer leader and our family member Gwen is with us as well who is supportive. Important.

Jayshree Shah: Thank you.

Gwen: And I just wanted to comment on just what a great meeting this has been. Just is a high quality of the presentations and the fact that you all conveyed so much information. It was so understandable. I'm the faculty at Hopkins and we do a lot of events like this in the Alzheimer's disease area and I've really learned a lot about the condition and also about how to organize events like this going forward.

Jayshree Shah: Thank you. I'll thank MDS Foundation for organizing all of this and you guys to come. Yes, sir.

Richard Zorza: Hi, I'm Richard Zorza. I have a diagnosis about last November. I'd had low platelet counts for about for five or six years before then and explained/monitored here at Hopkins and been late last year they moved it to an MDS diagnosis and there are a number of things about the diagnosis that are a little weird. So, I think... anyway. Dr. McDivid (sp? 1:05:22) is my caregiver at this point. The main problem now is low red cell counts leading to complete exhaustion most of the time. I guess actually what I'm interested in hearing about is the thing that I find it actually hardest to get is information about what the various treatments actually feel like because if we all have to make decisions about do we want her bone marrow transplant. It's not only the life expectancy curve, it's what does it feel like. We have to make decisions about what we're going to put ourselves through with chemo, with stem cell and what I would like would be... and again, the clinicians obviously try and tell you, but let's put it this way, I think if I were a doctor, I would find it hard to face how much pain I was imposing on people and so I would tend to minimize not out of a desire to fool them, but out of a desire to fool myself. That's my deal.

Jayshree Shah: Have you reached to your physician about seeking another patient that has gone through a treatment. You can request that because I've often offered my patients who've said, "Jayshree, I want to know or speak to somebody that's been through, say, transplant, an allogeneic transplant or who has received Vidaza." I have tons of patients that have gone through it that would be willing to share their experience and I'm not going to pick just the good ones. I'm going to give them a few, yes or an no and whatever they went through to share. Again, every person's different. They're going to have a different experience depending on their counts and what so but they may be an option.

Joan Zorza: And I know that almost not mentioned is uneasiness about what chemo would be. I'm Joan Zorza, Richard's wife and we live in Maryland just east of the district not quite halfway to Annapolis. Just off Route 50.

Jayshree Shah: Well. Welcome.

Barbara Carr: Barbara Carr and my husband Albert Car has the MDS. He was diagnosed in May... May 29 and this was like the end of my life because we didn't know nothing about it. So, the most research I did after the first thing... after the research I found the MDS Foundation. That's why I'm here today and that's how we found to go over. We have an appointment with him on Monday for a second opinion and so my husband on Procrit once a week, but 30,000 units. What I found out it's probably not enough to take and then he's 3 times a week on Neupogen, but it's somehow he doesn't... It seems like he doesn't feel any better. So, we don't know if maybe it takes a longer time to find out if it really works.

Jayshree Shah: Sure and that's a great question because they've done studies on patients. Again, I would have to... The physician would have to review his information regarding the MDS, but also to review the blood counts and stable is a good thing. I think peoples' expectations is go from 8 to 12 and I think what may happen is 8 to 9 and kind of just waver and hang out there and that will yes, good for us but what for him.

Barbara Carr: But he's also on blood transfusions like every 3, 4 weeks, 5 weeks, he needs a blood transfusion.

Jayshree Shah: I think it's a good review that you're going for a second opinion to seek out what other...

Q13: (inaudible 1:09:09)

Jayshree Shah: Oh, yeah.

Q13: At the same time we have (inaudible 1:09:15).

Jayshree Shah: You can. You can... there's a lot of room for changing around the schedule. The only thing is I would want to know what the percentage of blast count that your husband has and the reason is what Neupogen is just for people that don't know what it is. It's a growth factor. It's a stimulating growth factor. So, that white blood cell that I talked about earlier that is a regular white blood cell that helps you protect against infection. If your blood cells are low and you're stimulating the stem cell saying grow more, but you have a greater than 10 percent blast count. Blast counts are abnormal white blood cells. So if you're stimulating those abnormal white blood cell then you're white blood cell can go up potentially making your MDS go into AML at a little bit faster pace. So, I would want to know that information, have that discussion with your oncologist. What we do at our facility is we wait out about 8 to 10 weeks. If we don't see a response, we usually then talk about what is our next option. What would be tolerable for you? What is your expectation? These are the options we're presenting. I think that would be probably... Again, it's a case to case basis. I would still need to know more information.

Barbara Carr: Thank you.

Jayshree Shah: Yup. Thank you.

Albert Carr: She's answered everything.

Patricia Shimp: Hi. I'm Patricia and what Barbara didn't mention is that she's from Haymarket, Virginia and I'm from Haymarket, Virginia.

Jayshree Shah: It's a small world.

Patricia Shimp: We live down the street from each other. So, I have a new buddy. I was diagnosed a year ago and I got a second opinion from Dr. Gore and my oncologist in Haymarket and Dr. Gore agreed that I would be on a watch and wait probably for a year or two. Well, it was a month or two and I've been on Vidaza for seven months now and it's going to start working next month apparently and I'm on transfusions and I can't honestly say it's changed my lifestyle that much except for the stuff that you read. Don't do this and do that and I haven't been doing some of the stuff, but I've been out finding joy and having a good life, but every once in a while I have that stress of the unknown. The unknown. You're right about the breast cancer thing. I come out of the cancer center and there's all these brochures and classes and everything for breast cancer.

Jayshree Shah: That's just an example and I'm bashing breast cancer.

Patricia Shimp: Oh, no.

Jayshree Shah: I feel there's so much awareness of breast cancer than MDS and...

Q14: Patricia, when you say don't do this, don't do that. What kind of don't do (inaudible 1:12:26).

Patricia Shimp: At the low blood count thing, the one that Dr. Gore and I checked. Did he really say bullshit because my doctor said no fresh foods, no fresh vegetables, no salads.

Jayshree Shah: You can eat everything.

Patricia Shimp: And that's in a lot of books also.

Jayshree Shah: But again...

Patricia Shimp: And he said bullshit.

Jayshree Shah: You can eat anything and everything. Just, again, be cognizant... There's dirt on it, you're not going to be ingesting in that. You want to wash it off.

Q15: Coffee and chocolate.

Jayshree Shah: What's that?

Q15: Coffee and chocolate.

Jayshree Shah: You can have all of that.

Q16: (inaudible 1:13:03) in the books also.

Q17: You want to go back to decaf?

Jayshree Shah: MDS has evolved. It definitely has evolved in information what was started off with to now and the reason it's evolved is because we know patients like yourself that have gone through it or going through it, you're still alive. You're still healthy. If you're eating chocolate and you're drinking coffee, it's not making a difference except making you feel good and high. It's all good.

Patricia Shimp: At the San Diego conference keynote speaker there also had the same answer. He didn't say bullshit, but he said do what your mother taught you. If it looks okay and it smells okay, eat it.

Jayshree Shah: Common sense stuff.

Patricia Shimp: Yeah.

Jayshree Shah: Thank you.

Richard Zorza: I just had to tell you. I tried Googling MDS (inaudible 1:13:54) group. The second one it says never ask an MD. I tried clicking on it. Hopkins security (inaudible 1:14:01).

Jayshree Shah: That's funny. I think you can make your own answer to your own disease what you're going through. It's definitely used as a resource, but again if it doesn't look good or it doesn't smell good then obviously don't approach it, but anything else, again, just common sense stuff. It's okay. There are no restrictions. We don't want to give you 10 list of foods and all of those 10 things are the only things that you eat. No. We're not going to do that.

Q18: I'm sure you're (inaudible 1:14:40)

Jayshree Shah: Yeah. Of course. Common sense stuff. You know, hand washing, staying away from people that have colds and stuff. Again, if they have it make sure you keep your distance and a hello like this is totally okay or even (inaudible 1:14:53). It's all good.

Ted Dorf: I'm Ted Dorf and I'm from Rockville, Maryland and let me start from the beginning. I would do a yearly physical for many years and every time I went in for the physical, I was always told I was slightly anemic and my physician said, "Well, we'll watch that, Ted." Well, they kept watching it for year after year after year after year after year after year and I still stayed slightly anemic until one day the blood numbers dropped a little bit more. So, I went to see a hematologist and they diagnosed with MGUS. What is MGUS? Nobody really can tell me what MGUS is.

Jayshree Shah: It's a pre-bone marrow failure or issue that happens and it's called pre-multiple myeloma. They call it... basically it's like a vague type of a cancer, very low issue within the bone marrow that's not functioning. Go ahead. Finish, Ted. It's complicated because that is such an unknown kind of a territory in approach.

Ted Dorf: That's what was the answer. There's something going inside your body, Ted, but we can't quite tell you what it is at that point in time. Anyhow, and I was told I would come in every three months and they would watch it and take my blood and so forth. Finally, I fainted. Boom. I just dropped and so at that point they went a little further, did some more tests and came up with the potential for myelodysplasia and that they told me that the best place that I could find out further information was if I would come to John Hopkins and if I could see Dr. Steven Gore and trying to see Dr. Steven Gore at that time was like trying to see the President of the United States, but I had a very energetic son, who like that young lady down there, didn't take no for an answer and he managed through some connections to get an appointment. Now, I reminded Dr. Gore today that and this is over... we're talking better than 11 years ago. So, he was meeting people on a Tuesday. That's the only day he was taking new individuals. The rest of the time he's working at the hospital. Well, Mark wouldn't settle for Tuesday. He wanted an immediate examination and so from somewhere, I don't know where, but he was able to get me an appointment on a Friday. When I came to this hospital on that Friday, I fainted as I walked in the door and so they put me in a wheelchair and they wheeled me into Dr. Gore and Dr. Gore after talking to me, he says, "I don't know how you got in to see me on Friday. You must be some kind of a rock star or something." But anyhow, that was the beginning of a great experience and they were at that time conducting a clinical study on Azacitidine and he included me in that clinical study and at that time that study required that you have a 24 hour pump and also you administered the Azacitidine yourself and we went home and we would mix it and my kids would either practice how they would do a better sticking job on me than the next and it seemed to work to some degree and I developed a blood poisoning from the pump because it was put in...

Jayshree Shah: Through a portacath.

Ted Dorf: Yeah and went to the hospital and they took care of the blood poisoning and lo and behold at that point I was running normal numbers. Everything I was doing was running normal...

Jayshree Shah: So, how many treatments did you receive before your numbers became normal?

Ted Dorf: Well, I was in the protocol. So, I was in the protocol for months.

Jayshree Shah: For months. Okay.

Ted Dorf: A number of months. Yeah. And I was running normal numbers. So, we ceased to desist and didn't do anything further except being monitored because at that time they didn't... had not come up with the conclusion that you should continue with the protocol. So, almost a year went by and all of a sudden my numbers started to move down again and so I been on Azacitidine when it was FDA approved it became Vidaza and I've been on it now for 10 years.

Jayshree Shah: Wow.

Ted Dorf: Ten years.

Jayshree Shah: You're probably my last long...

Ted Dorf: I'm told I'm the poster child.

Jayshree Shah: Yeah.

Ted Dorf: Anyhow, I live a normal life. I'm active. I participate. I bowl in a bowling league. I lecture. We go on trips. We do everything under the sun. I eat everything. I drink wine every day. I love dark chocolate. I eat chocolate and I don't know about diets and I'm... I finished my lunch. I don't know if everybody else did, but I eat...

Jayshree Shah: That's great.

Q19: You're never transfusion dependent.

Ted Dorf: I had some transfusions early... early on while I was in the protocol. I had to have some transfusion, but I've only in this whole period, I don't think I've had a half a dozen transfusions. I don't think I've had...

Q20: How long (inaudible 1:21:33) Vidaza. How often (inaudible 1:21:35).

Ted Dorf: Well, the way they were initially looking at it is that you would go off for four weeks and then get seven days.

Jayshree Shah: Yes.

Ted Dorf: Then you go back on four weeks, but I was... I don't... I get normal numbers, but I close to normal... my red blood count will range anywhere from 11 to 11.7 or something and once in awhile I'll get an Annoris (sp? 1:22:05) shot occasionally, not too frequently. My white blood cells usually stay above the normal range. My platelets normally stay in the normal... My hematocrit, 35, somewhere like that.

Jayshree Shah: That's great. So, no more fainting.

Ted Dorf: I do have a couple other challenges. One is vasculitis. Now, there is some thought that there's a connection between vasculitis and myelodysplasia. Are you of that opinion?

Jayshree Shah: I personally have not seen that connection in relation to having MDS and vasculitis. I think that potentially maybe are changes that are happening within the blood vessels that causes the vasculitis. Vasculitis is basically like an inflammation of blood vessels and it

potentially can happen in the lower extremities meaning your lower legs causing maybe burning sensation or pain. What symptom are you having with vasculitis?

Ted Dorf: Well, with vasculitis, I get breakouts. I look like I have chicken pox or something of that nature.

Jayshree Shah: All over?

Ted Dorf: Mostly around the chest and sometimes in the forehead or the face, but I don't... the neuropathy is something... you were talking... Well, you say the lower extremities. Aren't you talking about neuropathy?

Jayshree Shah: No. Neuropathy is more fingertips and your toes.

Ted Dorf: Well, I have neuropathy, too.

Jayshree Shah: You know, it's case to case basis. I think I would know... I would need to dig a little bit more to go through your history to figure out what other things may be happening within your blood and compare your lab values to see what you started off and what you're at now, kind of getting a whole history picture. If I just have a small picture of you then that doesn't apply. Every person doesn't just have MDS. Before you had MDS, you were still functioning. You had other issues potentially maybe resulting into development of other things. So, it's a possibility of one or the other.

Ted Dorf: Well, I'm of the theory that when I was being told I was slightly anemic that something was working that was leading up to this MDS.

Jayshree Shah: It's most likely and I think, again, it takes an astute physician, a practitioner, to know you have an issue. Let's seek another... Let's seek an answer for it and it may mean to pick up the phone and say, "You know what? Ted needs an evaluation. Go to a hematologist that may know what could be going on to figure out the problem." It's almost becoming like a CSI investigator trying to figure out what does Ted have. Yes, Toby.

Toby Cohen: I just wanted to say in reference to your bringing up vasculitis, I have a protein in my blood for (inaudible 1:25:18) which I had for the past (inaudible 1:25:21). I don't have the disease. Could my hematologist at Sloan Kettering sent me to a rheumatologist that special surgery. She said that it was well known. Now, this is not vasculitis. This is (inaudible 1:25:41) vascular disease is where (inaudible 1:25:43), lupus (inaudible 1:25:46) because she said that it was well known that the cause of vascular disorders, (inaudible 1:25:56), lupus could cause a drop in a blood count and it could also cause a bone marrow result that looked like MDS. It was in some ways caused or contributed to my (inaudible 1:26:22) vascular (inaudible 1:26:23). So, I don't know if (inaudible 1:26:27).

Jayshree Shah: Again...

Toby Cohen: ... that category but maybe you might want to see (inaudible 1:26:32).

Ted Dorf: Well, my rheumatologist and my hematologist talk... My rheumatologist do talk to one another and they are on the same wavelength. They believe there is some relationship between the two.

Jayshree Shah: I have seen some of my patients that have MDS and they actually have symptoms of a basically of rash that you're talking about, but more so an inflammation process. Their body just makes this inflammation overgrowth if you want to call it and it can present it in a gout fashion. It can present in a nodule, in different areas in the joints or legs and the only thing that helps them is being on a low dose steroid for on a daily basis.

Ted Dorf: I am on five mgs of Prednisone.

Jayshree Shah: Yes and...

Q21: I was just diagnosed this week with (inaudible 1:27:30) syndrome which is (inaudible 1:27:32) the only cure is...

Jayshree Shah: Steroids. Yeah.

Q21: I think it has something to do with MDS.

Q22: He said 5 mgs. (inaudible 1:27:41) he got a 10 mg. (inaudible 1:27:43)

Jayshree Shah: Every person's different. I think what you'll want to do when any person, any patient, any person is on any steroids, I please welcome you to review what can end up being even on a short term plan to a long term plan especially with having already on top of it MDS or potentially having MDS. It depletes and takes away muscle mass. So, make sure you're exercising, you're doing some kind of resistant type of weight training because you will lose muscle and ultimately you may need a wheelchair or cane or something to help you. You don't want to do that. Address it early on and do the weight resistance therapy.

Q22: From the MDS you mean?

Jayshree Shah: No, steroids, steroid therapy.

Ted Dorf: Interestingly enough when I have attack of vasculitis, they usually heavy up on the Prednisone.

Jayshree Shah: Correct.

Ted Dorf: Now, a number of these cases occur at the same time and I'm going in for my... with the Vidaza...

Jayshree Shah: Can you speak into the mic, sir?

Ted Dorf: With the Vidaza, I opt for it to be infused rather than as a shot, rather than through the needle. Anyhow, when they do the blood counts then when I've been on a heavy... It raises your blood counts. The Prednisone.

Jayshree Shah: It does. That's actually a treatment for MDS believe it or not. Steroids are a treatment. Yes. Because it's a hormone. It's a hormone and there are certain types of MDSs that patients do respond to just hormones meaning steroids and again, MDS is so complicated. That's why, again, it takes an astute physician or a practitioner to say is there a connection? What we can we do? At the end of the day, we as practitioner's we want a solution for you to have that answer to have it resolved and/or to control something meaning your disease. So, it may take a little bit of balancing in trying to figure it out. One doctor may say this, one doctor may say that. You may have to try both answers to figure out which ones works. Medicine is an art. It's not defined.

Ted Dorf: So in my case, I was concerned that it might be a false reading.

Jayshree Shah: In regards to your MDS?

Ted Dorf: Yes.

Jayshree Shah: Well, you may want to go back to Dr. Gore and have him review that. Make another Friday appointment (laughing) without passing out. Yes, go ahead, Toby.

Toby Cohen: Obviously I don't know, but Laura Ephraim died of MDS. She actually also had (inaudible 1:30:53) of a (inaudible 1:30:55) vascular disorder and she... one of her treatments was low doses of cortisone. So, this is just a long winded way of my saying that it seems to me the evidence suggests that perhaps it wasn't a misdiagnosis. The fact that you respond to cortisone because she saw so many renowned physicians including Jerome Grugman (sp? 1:31:24) who concurred that she had MDS and yet one of her most effective treatments was low dose Prednisone.

Ted Dorf: Well, I don't want to monopolize this.

Jayshree Shah: You're not. We have about 10 more minutes or so. I just want to get a few questions in, but I want to finish off with Joan's intro.

Joan Dorf: Well, I guess basically I would be considered a caregiver, but I'm not. Ted has handled this problem from the very beginning. Dr. Gore said something about live and that's what we do and that's what Ted does. He bowls, he lectures on a hobby of ours. We have a good life. So... and he's been on this for a long time. So, it's really a lot of hope out there.

Jayshree Shah: That's good. Thank you. Yes, we'll start here and then I'll go over to you. Go ahead.

Q23: Ted and my question is you've had MDS diagnosed for over 10 years it looks like and you were on Vidaza most of it. What was where you started out with low risk, medium risk or high risk? Was it low risk MDS, medium risk...?

Ted Dorf: (inaudible 1:32:37)

Q23: It was low risk. Okay. That's... May I ask you a question?

Jayshree Shah: Yes.

Q23: When I had MDS, I had the 5Q and I was very fortunate because Revlimid was researched, but it looks like from one of the charts that Dr. Gore put up that Revlimid or the 5Q is not in a long run the best one.

Jayshree Shah: No, it is, but that's without any other mutations. So, I would want to know what your initial cytogenetic result was. Did it have other mutations besides the deletion 5Q? Did it... So maybe your bone marrow potentially over time as a possibility developed a few more mutations.

Q23: It did. I developed a 7.

Jayshree Shah: Monosomy 7?

Q23: Yes. When the bone marrow discovered, I had AML.

Jayshree Shah: Correct. So, what happened was his bone marrow basically evolved into developing a mutation that shouldn't be and hence it progressed into developing AML and that can happen with some patients. Yes, sir, after she's done.

Q24: A quick question. Right before Dr. Gore walked out, I heard him mention someone that there are situations and I wonder if you found this in Hackensack. There are situations when a patient is diagnosed with RARS they can either really have MDS or in few instances they can have a pure refractory anemia and not have it be MDS. So, can you speak to that?

Jayshree Shah: Sure. The few patients that I have taken care of and that have the RARS which is, again, refractory anemia ring sideroblast type of MDS. There are some that do have that. They don't respond anything as far as treatment and their transfusion dependent. There are some that are going to respond to some treatment regarding Vidaza or Dacogen. Your question is I just want to make sure I'm understanding that if they RARS...

Q24: It doesn't mean that they have MDS. Some of them just have a refractory anemia. So, my question is how does one know to really diagnose whether it is one or the other?

Jayshree Shah: The hematopathologist will be the one to diagnose the patient with RA or RARS and to classify it as an MDS. RARS is MDS. Does that answer your question?

Q24: He confused me when he said RARS is the diagnosis, but that doesn't mean the person has. Why didn't he say if the person has refractory anemia it would only be RA. See what I mean?

Toby Cohen: It depends on the bone marrow.

Jayshree Shah: Yeah. That's what I mean. It's a reading.

Toby Cohen: If I can't give you the figures, but if the bone marrow results fail to live up to certain specifications then you don't have MDS. You have this refractory anemia with sideroblasts (inaudible 1:36:28), but if they make the diagnosis based on the bone marrow, in other words, you can have certain abnormalities in the bone marrow, but they don't measure up to what the criteria for MDS is. Now, maybe in five years they'll decide that that also should be included in MDS, but that's how it's made based on the bone marrow.

Jayshree Shah: It's a group. MDS is a group of disorders. So, it falls within that category at the end of the day still.

Q25: How many are attending the first meeting of this group? You're very fortunate. We've attended more than five. Am I right?

Q26: (inaudible 1:37:21).

Q25: Just not quite. This is an unusual group because the lady you are looking is spectacular in explaining the questions that you're asking. Some can communicate a lot better. They all are different because each doctor's approach is different. This is a research doctor that you heard today and he was great. There are other doctors that communicate at a different level and hit different points. So, this is really an unusual and a very exceptionally a good group and I'm not saying because I'm in love with her. I'm in love with my wife, but she's wonderful.

Jayshree Shah: Thank you.

Q25: And you won't run into many like... They're all good because they're devoted to a very difficult subject and they're hungry to help someone else and learn at the same time. Some communicate at a level a little above the average. Some communicate to you that you understand and unlocks the door for understanding and this was a spectacular group and we'll see you next week at the next (inaudible 1:38:27).

Jayshree Shah: That sounds great. There was one question I do want to go over. While we were eating lunch, somebody had asked me about a flu vaccine whether patients are eligible to get the flu vaccine and I say yes. You are eligible to get the flu vaccine, but talk it over with your physician because you want to understand where they're coming from in regards to your treatments, what treatment you're getting because if you're getting hardcore chemotherapy, a flu vaccine is not per se going to work within your immune system at the same time. They may want to break it up. The best thing though I do tell patients if you're not... if the doctor says, "No, I don't want you to get the flu vaccine," what I would suggest is your caregiver and the people that you surround yourself with most of the time, make sure they get it because if they get the flu,

you're bound to get that yourself as a patient. So, Joan is going to protect Ted if Ted... If the doctor said, "Ted, no, don't get the flu vaccine." She's going to protect him. So at the end of the day... Yes?

Q26: When was the last time to get the flu shot because isn't there a (inaudible 1:39:39).

Jayshree Shah: Yes. So, the best time is probably now until February or March actually. You have a good leeway timeframe to get it. The sooner the better. Again, you want to review the allergies and all of that information. Make sure before you get it definitely contact and talk it over with your oncologist or a doctor or a practitioner before you get the flu vaccine. Put that on your list of questions in the binder. Yes?

Ted Dorf: I'm at a... See why I hold it away. I'm not a doctor but I've been given to understand you should get it as soon as you can. It doesn't work... as soon as you get the shot, you're not absorbed from getting the flu. So, it takes a few weeks or even a month before it will get into your system.

Jayshree Shah: It does. It takes awhile depending on your immune system, but the key point is that you get it, you're protecting yourself and you're building up. There are so many different strains of flu that are out there.

Q27: (inaudible 1:40:43).

Jayshree Shah: I don't know for this year. I have to look in to see which ones are out there for... They put in the vaccine itself. Every year CDC comes up with a plan of saying top five strains are going to be the ones that we're going to focus on and build up a factory of something and give people the vaccine.

?: Sure. The best thing is just check with your doctor, but I was on chemo last year at this time and I was given a flu shot. So, thank God I didn't get the flu.

Q28: (inaudible 1:41:17)

Jayshree Shah: So, pneumonia is something that I encourage as well to, again, ask your oncologist. Pneumonia injection should be given about every five years and what I would want to know is every person's different history-wise. Is your history of you developing pneumonia every five years or every year then those patients would be considered high risk. I would definitely have them get it, but talk it over with your oncologist. Yeah?

Q29: One more thing. I remember last year with the flu shot, I have two small children and we're constantly around my dad. They could not get mist. They had to get the regular shots.

Jayshree Shah: Correct.

Q29: So that was one thing to remember.

Jayshree Shah: The live vaccine. So make sure that you talk it over with what the patient, the person, is eligible to get and what kind, what are your cutoffs in regards to exposure. True. Thank you, Brian.

Q29: I don't want to change the subject, but I just wanted to mention to the people who live in Virginia that the Life With Cancer Center at Fairfax has MDS monthly meetings or similar group.

Jayshree Shah: Thank you.

Q30: MDS specific. They're leukemia.

Q29: Yes, yes, yes.

Jayshree Shah: That's great. Thank you. Thank you everybody for coming and contributing and I hope you guys...

Q30: You were great. You were great.

Jayshree Shah: Thank you, thank you, sharing your stories and, again, we welcome any other questions. Reach out to Deb and her colleagues and she will try to hook you up with any resources or help that you guys need. Thank you.