

Speakers

(Selina Luger, MD)

(Emmanuel C. Besa, MD)

Sandy Kurtin, RN, MS, AOCN, ANP-C

Sandy Kurtin: ... Philadelphia and I wanted to just take a minute to remember Bob. Some of you may have known Bob. Very sweet, dear man, husband, father, friend, colleague, MDS advocate. I had a great privilege of working with him over the last several years in the MDS Foundation and he was on our executive committee and just an amazing advocate and worked very hard and worked very hard with me in developing the *Building Blocks of Hope* and actually contributed a couple of testimonials in there. So, he will be all withheld in fond memory. This is his lovely wife, Rochelle, and you have on your table a flyer. She started a group here in Philly for spouses of MDS patients and I understand just had one meeting and was planning the second meeting. This is on October 26<sup>th</sup>. So, if there's any interest amongst the group to take part in that, there's flyers kind of spread out over the table. He was diagnosed in 1998 and so he lived a very long time with MDS and that can happen. He was also on Exjade for probably 10 years. So, just... and one of his quotes out of his testimonials that I just took out. This is Milkshake, one of his favorite St. Bernards. Milkshake died at some point and I was trying to find the picture of Cognac which is the new St. Bernard who's really quite cute but now quite large also, but one of Bob's quote was, "I think that my sense of helping someone else took away my anxieties," and so he very much was a part of this and embraced it and really just wanted to take a minute to remember him.

This is the Nurse Leadership Board. So, we have in the MDS Foundation, we have a board of experts throughout the world. Physicians, scientists, oh here, I'm no the wrong slide. Sorry. Should have learned. But we're very fortunate to have an international representation. This group of people also helped me a great deal in putting the together the *Building Blocks of Hope*. They are now helping to translate that into multiple languages. So, we have Spanish, French, German so far. We have... not quite done but pretty close. We're working on Armenian and many other languages going forward so that this will be available to people all over the world. So, we're excited about that. So, I was in Boston. This is in Boston and I was at a meeting and I was with my daughter on the trolley and this is a building commission by Bill Gates to MIT to say come up with some unique design and this was the award winner and I'm on the trolley and we're driving by this building and I said, "It looks like MDS," and it's because it is... Can you see it? Because things are a little too crowded. Things are not quite right in terms of size and shape. So, dysplastic and we have white blood cells, red blood cells and platelets. Right? My daughter said, "Mom, you're crazy." I've been doing this a long time and it kind of gets to you after awhile.

?: Also, it's a very expensive building.

Sandy Kurtin: It is a very expensive... and it's really amazing that it's just a really striking building, but I took a picture of it and I thought, "Gosh. It looks like MDS," but part of the challenge is, I think, that it is something that sometimes is hard to find and it isn't the most common kind of cancer. So if you're in a practice in a community setting, for instance, they're going to see way more breast cancer, lung cancer, colon cancer than MDS and you may not have

someone who's as informed or up to date with the latest science and so it's good that you're here because you can bring that information to your physician and often that's what spurs them to read something or go to a website or find out more information and we do have a lot of resources for clinicians on the MDS Foundation website. I'm going to show you just a couple of examples. But in the *Building Blocks of Hope*, our goal is to help you understand the disease, how is it diagnosed, what are the treatment options, what are the side effects of those treatments, what's on the horizon, what's the consequence of blood transfusion, beyond iron overload, there are others. Should you be considered for chelation therapy? That's the treatment to remove iron. If you are a candidate for a bone marrow transplant, how do you choose a center and then really how can you stay healthy? So, we're going to go through some of these, but I encourage you to explore what I call explore the *Building Blocks of Hope*. Ask for help, be a partner in your journey and we're going to show you a couple of tools in there that can help you to do that. I'm going to skip over this because I think Dr. Luger did a great job and unless there's any question about the disease itself. Does anybody have another question that wasn't clear to you just about MDS before we skip over this? Anybody have anything that wasn't clear? I think she did a really good job.

This is a tool that's there for the physicians and it's listed in the *Building Blocks of Hope* and it really... it will take you right to an IPSS calculator. So if you're curious about your IPSS score, what you need to be able to score that are a couple of things. Can you click on that hyperlink? Are you able to do that? So, it'll take you... if you... and then can we scroll down a little bit? So, it'll allow you plug in your values and then it basically gives you a score and then it gives you the information about low risk, intermediate risk or high risk and that's really how we as clinicians figure out how best to recommend what particular treatment is good for you. Yes?

Q1: Isn't there a new IPSS scoring system?

Sandy Kurtin: This is it. It's the IPSSR which is the revised IPSS. IPSS stands for International Prognostic Scoring System and Dr. Peter Greenberg who is one of our executive committee members at Stanford is the one that really did... lead this work the first time and the second time. This revised scoring system is based on almost 8,000 MDS cases throughout the world, 11 countries. Many, many, many scientists, statisticians, clinicians that came together hammering out the data and saying here's how we're going to figure out where people fit and there used to just be 4 categories and now there's 5 categories and they correlate with how much do we worry about transforming to leukemia. So, the question earlier and there's a percentage of people that never develop leukemia from their MDS ever in the lower risk categories. If you're in a higher risk category, the chance of that happening is actually quite high and we don't wait long to start treatment because we worry about that and this is really what drives it. So, it's good to know kind of where you fit and what should... and that will help guide basically the recommendations for treatment. Okay. Can we go back?

We talked about the current treatment options. So does anybody have any specific questions about any of the treatments or the treatment that you're on? So what we currently have are what we call supportive care. So, transfusions and growth factors. Those are things that don't really change the underlying disease, but they help with symptoms. We have the Revlimid or Lenalidomide, Vidaza, Dacogen. Both of those are chelating... or excuse me, hypomethylating

agents. We still use some standard chemotherapy not as often as we did, but we can still use some of that. Bone marrow transplant you heard about and then the clinical trials emphasis. Does anybody have any questions specifically about any of the individual treatments or something that maybe has been posed to you as a possible option or concerns about any of it? Yes?

Q2: Do the various cancer centers around the country tend to take different approaches because of the research they're doing?

Sandy Kurtin: They can. You know, it's much more common in diseases like myeloma, for instance, where you have so many choices that if you're on the East Coast what they start with is different than what they start with on the West Coast because we really only have three active agents currently, hopefully more soon, there's less of a discrepancy and I think that people are getting better about how best to treat individual subtypes. I think there still is some hesitancy to get started in some cases, but it's true... That's very true for probably other diseases more so. Yes?

Q3: I'm not sure about the pronunciation, but is there a drug called Pronotica (sp? 10:45)?

Sandy Kurtin: Yes and so that is a drug to help stimulate platelet production and so that would be appropriate for you, obviously. It is not currently FDA approved for MDS. It is approved for a disorder called ITP, Idiopathic Thrombocytopenia Purpura, which is why we say ITP. Right? We could practice that later, but it can help people and it has been used in trials. There is some concern because of the way it stimulates the bone marrow. You know, your red blood cells, white blood cells and platelets all start in the same place. They come from the same factory and so if you do anything that manipulates that environment, there's always a worry that there's some off target effect meaning it can effect something else in the meantime along with producing the desired effect and so there was some concern in those trials about it promoting leukemia and very small numbers and so in order to really definitively say no, it doesn't, you need much larger numbers. So, there are still studies going on trying to clarify that, but right now, it's not FDA approved for MDS. We have been able to get it through certain avenues if we work hard at it for selected patients. Yes?

Q4: My mom recently was in a clinical study for Revlimid. She is... my mom here. She has reactive anemia with 5Q deletion. So, she took the Revlimid for three days, broke out in hives.

Sandy Kurtin: Yeah. That happens.

Q4: So, she had to stop. So, what do you think...?

Sandy Kurtin: I would rechallenge here. So, I was very fortunate work... I've been very fortunate to work with some remarkable people over my career, but I worked for 16 years with Alan List. We were the only site in the world who started that trial and we... and because it's an immunomodulatory agent, it works by... it works in many ways, but one of the things that it does is it help regulates T lymphocytes and T lymphocytes are part of your immune system that have a lot to do with things like rashes and hives and they're very rich in the skin and so we had a number of people who had patchy... what I call urticarial dermatitis because then people don't

get too freaked out about it because it doesn't sound like it's dangerous. I mean, it's different if you're covered from head to toe in a red rash that's burning and itching. Was it just hives?

Q4: Well, when I had radiation two years ago, it was cancer. My whole body broke out in hives and now when I took the Revlimid just for 3 days, 10 mg. each day it was mostly my head (inaudible 13:40).

Sandy Kurtin: And people look like they have goose eggs and they can go away.

Q4: Well, they did. They put me on Prednisone stuff and then it (inaudible 13:49). Today, I have to get a transfusion because my hemoglobin is down to 8.4, but I guess she'll start the medicine again maybe next week.

Sandy Kurtin: Yes and so we have... I'll show you a couple... Let me get... see if I can get to that. So, let me... let me explain because I heard some of you say you did it for 2 months and it didn't work. So, I want to just cover this because this is a really important element in treating MDS and we speak of it a little bit in the *Building Blocks of Hope*, but before start treatment with MDS, we know that our factory isn't working right. It's crowded, it has immature cells, they're not... they're dysplastic. They look that building in Boston. It's crowded, they're all morphed, they're not organized and they don't work very well because of that. So, we know that there are problems. When we start treatment, we're actually going to probably make your blood worse before it gets better. So, you may say, "You know what? I get blood every 18 days," and most people know exactly the timeframe between transfusions. Right? You got... You're tracking it, you're logging it and now you might need a transfusion in 12 days. So when we start treating, we're going to, what I say, make it worse before it gets better. This graph up here on the right is secondary analysis of the Lenalidomide or the Revlimid trials. This is Dr. Michael Secress who's at the Cleveland clinic and he works with us on the trials and it showed this early dip and then coming back up later on. So, we expect this. It doesn't mean everything's going terribly. It means that you're... We have to clean out the bad cells in the marrow and get to the other side so some good cells recover and that's basically what you end up doing and it may mean that when you get to the other side, your platelets aren't completely normal. Your white count might run a little low for awhile. Hopefully, the hemoglobin recovers and you no longer need transfusions. If you're on Revlimid, this is what this graph was, but the same thing can happen with Vidaza or Decitabine or any of the drugs, really. So, we expect this early cytopenia and most of these therapies require at least 4 months of treatment before you can say they have failed and what ends up happening, unfortunately, is it gets a little tricky here because then your counts are low and people get nervous and then we stop and if you stop when you're here, then all we've really done is exposed you to side effects and not the potential for benefit. So, you got to just commit and hang in there and give it a good 4 months and in some cases it might even take 6. So, you get to the other side and now you have a marrow with more normal cells. So, this gets tricky. People get nervous. We see people discontinue therapy most often at 2 months treatment and that means they haven't had enough to even respond. So, that's one thing that I think is really important to take away because you get nervous. You think, "Well, heck. Everything's getting worse. Why on earth would I want to keep doing this thing?" Right? Yes?

Q5: In my case with Vidaza, I was warned in the beginning that that sort of a thing would happen and lo and behold it's exactly what happened and it came back up and it's been steady ever since.

Sandy Kurtin: And so you got to get through... This is what I call the ravine. You got to get through the ravine in order to really get the benefit and if you give up too soon, you don't get to the other side and then you run out of treatment options. So, we have to make a plan. There are ways to strategize this. Dose modifications, little bits of breaks in treatment for short terms. There's ways to get through that, but when I meet with a patient when we're first starting, I have this laminated and I show them the picture so that when they come in see me in 4 weeks, I'll say, "Remember? We talked about this," because they're like, "Well, wait a minute. I thought the idea was less transfusion and now I'm getting it in 12 days instead of 18," and then we remind, "Okay. We got to get to the other side here."

So, I'll share with you a couple of things. This is a patient that I had and these are actually are her counts and just to demonstrate this, so the gold is platelets. Right? The dark purple or blue is white cells and this is the hemoglobin and you can see... Well, there's 2 different... So, one of my patients is a truly rocket scientist and he's way, way smarter than me for sure, probably most of us in this room, builds rocket engines for NASA. So, he's like really, really smart, but a very lovely person and so he made this grid so there could be 2 different axes here because I couldn't figure out how to do that in Excel. So if you look to the right, those are platelets. Right? So here, her platelets when we started were 52. We actually started treatment here. She had 36,000 platelets. A lot of people would say, "Well, we're going to wait a little while and just kind of let that get better." Well, it isn't going to get better because the factory is not normal. Right? And her white count was low. So, somewhere around 2,000 and her... You can see we transfused. Every time it bumps up really quickly like that, we transfused. So, we have cycle 1, cycle 2, cycle 3, cycle 4 of Vidaza and until the cycle 4, really there's nothing happening and all of a sudden boop, up they go and all 3 cell lines improved and she was young enough to get a transplant. So, she went onto have a bone marrow transplant, but if people aren't patient you don't get there. You have to commit long enough to even... to give it really a good enough trial to see a response. Now, here's my rocket scientist. He's been on Lenalidomide longer than anybody in the entire world. So, he... and he sends me this graph on a regular basis and I haven't updated the slide, but he just recently sent me one. So, his first dose was on April 2, 2002 and his last transfusion was on April 22, 2002 and so he is now... what does that make it? Eleven plus years transfusion independent and that can happen. He's a 5Q deletion. It's not everybody, but it can happen, but again big drop in platelets, got worse before it get better and his platelet count has been normal twice in 11 years. So, his platelets now run 80,000 – 90,000. They're not normal, but they're not... it's not a problem for him. Never been hospitalized. He finally agreed to retire because all these young kids are... just they just don't know how to do this and so he's still very committed to his work and... but you have to get through this early phase of treatment in order for something like this to happen. So, it's just something to say people can do well. We were able... He was on our original trial. So, he participated in a clinical trial. So, I would echo Dr. Besa and Dr. Luger's point of we do need more drugs. We need to find out more about the disease and this is how we do it is doing through clinical trials.

So, the last thing and then we'll... It looks like they're setting up the food, so we'll get out to lunch here. A little bit about how to stay healthy and so I think this is to me really important. You know, we... If you're otherwise healthy, you're just going to do better. So, taking care of yourself as a whole person is really important. So, we talk about diet, staying active. Very, very important to fight fatigue. So in all the research that we've done, we've tried pills, we've tried all kinds of things. The only thing that shows us scientifically to improve fatigue is exercise and it doesn't mean you have to go to the gym and spend two hours at a time. It means just simple little strengthening exercises while sitting in a chair, walking if you can, but... and I know it's hard when you're anemic and your blood is low you get tired, but just trying to find some way to build in a little bit of something to keep your muscle strength up is really important and I always tell people get those little elastic bands. I'll probably fall if I do this while I'm trying to stand up, but I'm getting too old to be that coordinated, but if you just sit in a chair, nice steady chair and you get those little elastic bands that they sell, you know, the flat ones, not the rope, but the flat bands. All you have to do is if you stick... Grab it in both hands, stick one right under your foot like this. Right? And in one motion, you're just doing this and it strengthens your quads here and these muscles here and those are the things you need to wash your hair, get dressed, get up and down out of a chair, get in and out of the car, huge, huge difference and you can do it while you're watching TV. Very, very simple. It's not going to... you don't have to worry about falling because you're sitting in a chair and they're very inexpensive and unless you have really bad knees or rotator cuff tear or something where you shouldn't be doing that, it's a great way to just keep some muscle strength and then if you walk that's just a little bit... even up and down the hallway or whatever it is that's going to really help you kind of keep active.

Avoiding infections, you know, MDS is not like leukemia or other diseases where you're getting chemotherapy. So, there's really not a reason to not be out and about and we don't tell people that they have to stay out of crowds. There are a few exceptions and when people's white blood cells count get very low if that happens, but most of the time it's a matter of taking care of yourself in other ways, staying hydrated, washing your hands, avoiding people who are obviously ill. No kissing and hugging. So, you can go to church, but don't let everybody kiss you like they do. Right? They all want to kiss you and hug you and just say, "Nope. That's okay," and nod from afar and say, "Amen." There you go and because that's an important part of life is doing the things you love to do normally and so you shouldn't be shut away and I think sometimes I know nurses that I work with have the same story line for all the patients regardless of their true level of risk. So, there isn't that level of risk. You're more at risk for things like that are in your own body and getting infections in your own body which is why it's so important to do the other things that we just talked about. Getting enough rest and so that can be a problem. Sometimes too much rest is... you got to balance this with the first couple. Asking for help. I think is and we'll talk more this afternoon a little bit about strategies for that, but that can be hard but you... you got to... this is the time to call people in and get organized with it and so I try get patients to make a list and so if somebody calls and says, "You know, Frank, what can I do to help you?" You can say, "Well, you know what? I need somebody to go here on Tuesday at 3:00." Give them specifics. Make a list and check there that's done. Otherwise, everybody's going to bring you lasagna. Right? And you can only eat so much lasagna. So, organize your thoughts. Think about what are the things that I can do that are going to take a little of the load off of me and put them to work. There's a lot of people out there that want to help and they just need a specific task and that's going to make it easier for you if you make that list and then being

an active participant. So, being here today is a good first start. There's actually a really nice program that's relatively new that we are collaborating with called "Healthy Body Healthy Mind" and can I get you to click that hyperlink for me? And so this is something that runs regularly on TV and it's available if you go our website you can click on the link and it'll take you to this nice spinning wheel here and maybe the link's not working right, but it really just talks... It gives you a nice quick synopses of MDS and then it talks about maybe I pasted the hyperlink wrong or something and then it'll give you a quick synopses of how to stay well. So, summarizing a little bit of what we just talked about. Let's try... can we try the second one on there? Maybe... I might have pasted...

?: Not a chromosome there...

Sandy Kurtin: It could be a chromosome or it could put you to sleep like in a trance. No, it's down. More, more, more. Let's see. Go up. There. Let's try that second one see what happens. No, that's just the... that's the TV schedule but it airs regularly on TV. So, unfortunately, that didn't work out. Okay. Let's go back to the slides.

And then this is the *Building Blocks of Hope* and we'll explore a couple pages later on as we have our discussions and go through questions. So, can you click on that hyperlink? So if you go to the website, the MDS Foundation website, and if you click on For Patients and Visitors.

(audio playing)

*This is a video introduction (inaudible 29:09) Blocks of Hope program (inaudible 29:11) MDS Foundation for patients and caregivers living with MDS. At the end of this video will be instructions for accessing resources available to you. You or someone you know has been diagnosed with MDS. Hearing the words 'Myelodysplastic Syndrome' or 'MDS' can be frightening. The diagnosis of MDS is often unexpected and filled with both immediate and long term challenges. You probably have many questions in your mind. The Building Blocks of Hope program is designed to help you get the information you're looking for. There's several components to the Building Blocks of Hope including printed materials, digital materials, videos, brief educational slide sets, links to online resources and a number of very practical tools. All of these components are intended to provide you and your caregivers with strategies for living with MDS. The best place to start will be our Building Blocks of Hope handbook. This will provide you with information, resources and tools to help you through your journey. This continuously updated document includes the following sections:*

- *Understanding MDS, which will give you a complete description of the disease process of MDS and answers to common questions.*
- *Seeking Treatment. The treatment of MDS will vary based on the type of MDS you have and loss of (inaudible 30:42). This section will provide details about the various approaches to treatment.*
- *Quick Tips. The quick tips offered in this section include guidelines for monitoring your symptoms and reporting them to your healthcare provider when necessary.*

- *Iron Overload.* Iron overload is a possible outcome of receiving repeated red blood cell transfusions and this section answers common questions.
- *My MDS Plan.* Understanding the diagnosis of MDS will help you and your caregiver take an active part of your individual treatment life. My MDS Plan provides several tools to allow you to track and manage your journey.
- *The MDS Foundation.* The MDS Foundation is an international publicly supported organization dedicated to serving the MDS patient, their caregivers and the professionals that are working to improve the lives of patients living with MDS.

*The MDS Foundation provides a number of resources which support the Building Blocks of Hope program. You can access the handbook in a variety of ways. Through our website, you'll be able to answer a series of questions and download a customized PDF that you can review and print with your computer. You can view the complete handbook in a beautiful page turning format. This includes a search feature and (inaudible 32:04) views that will help you quickly find the information you're looking for. This is also a great way to share information with others. You can also contact the MDS Foundation directly and we can mail you a (inaudible 32:17) version of the handbook. Allow yourself some time to adjust the diagnosis of MDS. Take time to explore the Building Blocks of Hope. We wish you the best in your journey and hope that the Building Blocks of Hope program will provide you and your caregivers with tools and strategies for living with MDS.*

Sandy Kurtin: Okay. Can we go back to the slides? Thank you. So the digital version is actually quite versatile and there are many embedded videos in there that give you short little lectures or slides about specific information. So if you go to the website, you can view those easily. The next phase of this will be... There's a section in here called My MDS Plan and that allows you to keep track of your counts, your treatments, your medications, all of those things and this is something else that we're developing in digital format, so you can if you want to put it in the computer and print it out for yourself and then you can change it as things change and it allows you to take it with you if you're seeing various providers because you're going to be able to tell them something much faster than they can find it themselves. Trust me. Even in our electronic health record era, finding the right information is sometimes challenging and so what I encourage patients to do, honestly, is to take that section, the MDS Plan, out and create their own little folder and with it there's a cardholder in the back of the book and then they add their lab sheets. So when you're there seeing the physician or the nurse practitioner or whoever it is you're going to say can I get a copy of my labs? And you can just add it or an x-ray or whatever else it is and then you have your own little book that tells your story and so if you're going to a cardiologist or a pulmonologist or an oncologist or a specialist for another opinion, you have it all together and it's easy to track of it and these sheets can be Xeroxed and filled out so you can build it as you go either in print or by hand if you prefer that or eventually this will be all in digital format on the computer so that you can... if that's the way you like to do it do it in that way.



Q5: Is there an app for that?

Sandy Kurtin: It's coming. Apps are all... It's all about apps. Apps are way more expensive than you think, but we're trying... It will be a feature that will be... you can put on your smartphone or your iPad or whatever it is eventually, but a nice way to really be able to create... maybe not what I call the Chet Chart because this patient's name is Chet, but some way to be able to visually see how you're doing and that's really important to get a sense of the progress that you've made and sometimes it's very subtle where you're used to get the transfusions every 18 days and now it's every 23 days and that's the first clue and just being able to see those milestones and a trend format is a very good way to get a sense of how things are going. This is the PDF format which you can download on an iPad and read. I'll skip over that. Most of you have met Dee who you registered with. Audrey Hassan is our patient liaison and her information is in the booklet under the MDS Foundation tab in the back and she's a great resource and you can call in with questions. She will field that out to members of the board if it's something that is not... something that they can answer right away. So, you can call in with specific questions and we'll find a way to get back to you either by E-mail or by phone through Audrey.

So with that, the food is here and then we're going to come back and talk about you. So, think of questions. We're going to talk a little bit... have people share their strategies, concerns, fears and we'll go through that. Yes?

Q6: I have that.

Sandy Kurtin: You have that.

Q6: That is the best thing. Whatever you want to know, you'll find it. Really. Only I didn't get this. (inaudible 37:19) add the papers.

Sandy Kurtin: When we mail them out they come in a format where you can buy a binder and slip in the covers just because of... and these binders are the wrong size. They're too big. The next phase will be smaller, but the beauty of this and the reason that I designed it this way is that science changes, we can change individual pages and not... produce the entire booklet, those little booklets that we use to create our out of date very quickly and this makes it a very versatile document.