

Speakers

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Jean Ridgeway: So, Dee was talking a little book. It's called *One Hundred Questions and Answers About MDS - Everything You've Always Wanted to Know*. Some physician offices carry it. Some carry it in their resource center. Anyway, the MDS Foundation, there is a second edition being written and I'm not quite sure when the publication date of that is, but it's coming out. So, alright. This time has been reserved for question and answers. Probably lots more questions than there are answers and that's perfectly fine, but just kind of wondering where... who's got the most burning question? Oh, the other thing is my IT helpers are bringing in those sandwiches and stuff and it's going to be on the back, so when you do leave, please grab stuff to take home, okay, because it's out there. Questions. One thing... Let's talk about this. I'm going to borrow your book for a minute. Dr. Shammo made a reference to doing the IPSS score. Okay. And I'll see if I can find it in this book *Understanding MDS*. I think... cytogenetic testing class. Probably page 13. Yes.

?: (inaudible 1:18).

Jean Ridgeway: Oh, one more question. These two gentlemen who are here actually are from a company called Celgene and Celgene is the pharmaceutical company who provides Lenalidomide as well as Thalomid, Thalidomide and a bunch of other drugs, but Sam is a reimbursement specialist. So if you are on an oral therapy or even in an injectable therapy, (inaudible 1:48). Didn't you guys still hold the patent on that? I don't know. He's the reimbursement specialist. So if you have questions about reimbursement... For those of you who aren't on therapy, Lenalidomide is an oral agent depending on your prescription package and provider, it is how you're going to obtain the drug. It's a specialty drug. It's considered an oral chemotherapy drug. Sometimes it gets shipped from specialty pharmacies or it gets special ordered from Walgreen's, CVS, wherever you're picking up your medicines, but co-pays... Now, you can tell me. I'm to trying to like embarrass you but the cost of the drug is per month?

?: (inaudible)

Jean Ridgeway: Right.

?: The drug could be very costly.

Jean Ridgeway: Like \$5,000 a month?

?: (inaudible)

Jean Ridgeway: \$10,000. Okay. I guess I didn't get the update. So, if nobody affords that, maybe Prince Charles, but he doesn't need it. So, reimbursement specialists are on staff with all the pharmaceutical companies to help us navigate the financial burden of healthcare and so that's Sam's job to help do that. So if there's anybody here who has questions, comments, problems,

he's your man. If you want to just raise your hand, he can give you his card and then you have his number. Feel free to raise your hand, but if not Sam's going to go because he would really like to go outside and play if we don't need him, he's out of here. Good enough?

?: He has to go meet his future in-laws.

Jean Ridgeway: He's meeting his future in-laws.

(Group talking)

Jean Ridgeway: They rode up in the elevator. That's funny. Okay. IPSS scores. I should know this like the back of my hand. Okay. So, the IPSS score is only... is done when you get diagnosed. Okay? You don't rescore yourself. That's not how it's done. So when you initially are diagnosed with your MDS, there are 3 components. There is the number of cytopenias. So, we look at cytopenias at white cells, red cells and platelets. So for white cells, it's if your absolute neutrophil count is under... I thank you very much... under 1,000, hemoglobin under 10. This is the standard that was done with and then let's see, and platelets under 100,000. So, those are cytopenias. If you have none of those, it gives you that component. So, there's 3 components: blood counts, cytogenetics and number blasts. Okay. So... and then cytogenetics. Remember there was a very complicated slide that showed all the different possible cytogenetics. Some are good. That scores 0. Some are intermediate. They give you a score of .5 and some... if you have 3 or more, it's considered high risk and that gives you a higher score.

?: Blasts are 100,000?

Jean Ridgeway: Blasts are 100,000. No, no, no. That can't be. No, no. It's platelets. Platelets. One hundred thousand for platelets. Yes sir?

Mr. Henzel: I have a question. Is there a cure?

Jean Ridgeway: For?

Mr. Henzel: My particular case.

Jean Ridgeway: Do you mind telling everybody what your particular case is, so we can kind of speak apples to apples?

Mr. Henzel: MDS with 5Q-.

Jean Ridgeway: So, a 5Q- only.

Mr. Henzel: Yes.

Jean Ridgeway: Yes. Just to make that clear. Can I give a little snip? So, 5Q... When you have MDS and your only cytogenetic abnormality is 5Q- that means the fifth chromosome... Chromosomes are very interesting. They have arms. They have P arms on the top and they have

Q arms on the bottom. So when someone says they have 5Q-, to me that says it's your only cytogenetic abnormality and your only problem is that on the fifth chromosome, the Q arm is either totally gone or there's an interstitial deletion or there's a component missing and that's what Mr. Henzel has, correct? 5Q- and we know that Lenalidomide has terrific success for people who have 5Q-.

Mr. Henzel: So, I've been on for years with the same blood analysis.

Jean Ridgeway: You mean CBC counts?

Mr. Henzel: Yes.

Jean Ridgeway: So now, your blood counts are stable.

Mr. Henzel: It's worked up to I'm about normal.

Jean Ridgeway: When was the last time you had a transfusion?

Mr. Henzel: Pardon me?

Jean Ridgeway: When was the last time transfused?

?: Before he started (inaudible 6:12)

Jean Ridgeway: (inaudible 6:14)

?: Eleven years.

Jean Ridgeway: Eleven years.

Mr. Henzel: So, what that hasn't changed in years all of my (inaudible 6:21) counts and they're all normal and am I cured?

Jean Ridgeway: Are you cured. Here's a question I have for you.

Mr. Henzel: (inaudible 6:29) say we don't know.

Jean Ridgeway: We don't know and that's right. I'm going to like sneak down that little cubby hole also and say I don't know, but it's an excellent question. When you have a bone marrow done, do you still have the 5Q- deletion? Is it there or no?

Mr. Henzel: You know, I don't know.

Jean Ridgeway: Okay. You don't know either. So, it's interesting because these sophisticated smart drugs that are finding usage in very unique situations, you know, your stem cells... Here's how I conceptualize it. Your stem cell is probably still abnormal and the drug continues to keep

it in remission and keep your blood counts normal. What would happen if we stopped your Revlimid? I don't know. There really are two possible answers. You could be cured and your blood counts would remain normal. The other answer you would relapse. How long would that take? Good question. Maybe not so long or maybe a long time and if you relapsed would you respond again and some... a lot of these questions are unique with some of the other drugs that we have out there that specifically are for a very small individual group of patients like CML patients take a drug called Imatinib. The same question is being asked. Am I on this drug forever? Now, I'm normal. Am I cured? Can we stop therapy? So...

Mr. Henzel: Well, I guess we're looking for... We're taking this medication. Now, we've stabilized. It's like an aspirin taking care of your headache...

Jean Ridgeway: Exactly.

Mr. Henzel: ... and when your headache is gone, you can stop taking aspirin.

Jean Ridgeway: Is that true. Right. I don't... The current thinking in hematology is that if you're on the medicine and it's corrected your blood counts, you need to continue to take it because if it's not then causing your blood counts to go in the other direction, it's working physiologically and so it continues to keep you in remission. Like a diabetic with their insulin. Their blood sugars are high. They take the insulin. I'm not a diabetic anymore. What happens if I don't...? So, it's that analogy and there's really not a good answer because...

Sylvia: I have a question, too. Rich was... As I say, it's over 5 years now.

Jean Ridgeway: Are these microphones hot? No.

?: They need to be turned and (inaudible 8:57).

Jean Ridgeway: So when we can use them so we can hear one another? That's good. We'll try to use the microphones. Alright, Sylvia. Start again.

Sylvia: He was...

Jean Ridgeway: Hang on.

Sylvia: I would say about a year ago...

Jean Ridgeway: Don't be shy.

Sylvia: The doctor said let's try and take the Vidaza maybe five weeks and six weeks. He did that for about two or three infusions and all of a sudden it started and right away he said you don't want to lose it because you may... if you relapse you may never get back to where you were. So, he's back to every four weeks and things have little by little stabilized. He's still not up where he was a year or so ago, but at least he's at that point where it's better. So...

Jean Ridgeway: It's very individual and hard to say.

Sylvia: Very much so.

Jean Ridgeway: I'm not sure how many cycles you had. So, I don't know if you'd heard...

Sylvia: Sixty. He's had 60 cycles.

Jean Ridgeway: Sixty, 6 – 0 as in 5 year's worth.

Sylvia: Correct.

Sylvia: Are these what they're talking about is this a low impact?

Jean Ridgeway: So, do you know... Rich, do you know... were you an intermediate or a...

Sylvia: He's intermediate.

Jean Ridgeway: Let's go back to this IPSS scoring. So, I'm going to pretend that... We'll give a scenario. How do you score it? I'm sorry I don't have this computer behind me, but that's okay. So, the score goes from zero and then .1, 1, 1.5 or 2 or greater. Okay? So 0 is in the IPSS. This is not the revised. Zero is low risk; .5 to 1 is low risk; 1 to 1.5 is intermediate risk and then when you... high risk is 2.5 and above. So, each of those 3 components had a score. Correct? So, if I go into the doctor and I'm getting diagnosed with MDS and they do all my workup. So, they draw my blood and I have normal platelets. I have a normal white count, but my hemoglobin is 7. So, I only have 1 cytopenia. So, that gives me a 0 score in the blood count part and they do my bone marrow biopsy and I have a very nice nurse practitioner who does it and she does a great job. So, but they draw back my DNA and they get it back and they say, "You have a 5Q- as your only cytogenetic abnormality." I know that 5Q- is a good risk cytogenetic. This is like kind of what I remember. So, that gives me a score of 0 and when they looked at the number of blasts in my bone marrow, they said, "You had 4 percent blasts." So, that also gives me a 0. So when you add up all my categories, 0, 0, 0. I come up with a grand score of 0, which puts me in a low risk category of MDS. So, that's how you score it. Now, a high risk MDS patient, so say I come in and I get the diagnosis and I have all of my cells are low. I'm neutropenic, I'm anemic, I'm thrombocytopenic. So, all 3 cells lines are down and my cytogenetics are what we'd consider a complex cerotype. It looks like alphabet soup if you're reading about my DNA and they tell me I have 10 percent blasts in my bone marrow. So, how does that score and what does that mean? So for the low risk person who has a score of 0, remember without treatment we know that those people have a average life expectancy from 5 to 7 years... 4 to 7 years. Okay. Now, I'm a high risk person, right, because you add up all my cytopenias, my cytogenetics and the number of blasts and you give me a score that now is greater than 2.5. When you add those up, I'm considered a high risk person. My life expectancy without any treatment is less than a year. That's how you make out... That's what it means to score yourself with the IPSS kit... scoring system and what that does for the clinician is it gives them insight into the tempo of your disease, kind of what's going on, what can be expected and insight into what's an appropriate therapy for

you because if I'm a 0, you're going to treat me 1 way and if I'm a high risk, you're going to treat me totally differently. Does that make sense?

Group: (Agreement sound)

Jean Ridgeway: So in the book and even if you just Google, I'm assuming that people like to go on the Internet, but if you Google IPSS there's a lot of web pages that you can plug your stuff in. Remember, you don't rescore yourself. You do it from your initial workup and that's where that information comes from.

?: Why don't they rescore? What if you get better on your own?

Jean Ridgeway: So the data looked at because when you analyze how was the data done, so the original IPSS score looked at when people were diagnosed. What did they use...?

?: That could change, right?

Jean Ridgeway: What could change?

?: This doesn't apply. (inaudible 13:59)

Jean Ridgeway: So, why is it scored that way? When the original data was done about the IPSS scoring system, what the reason it was done was back in the '90s, so that paper came out and was published in '97. Why it was done was people who didn't have a good understanding of what MDS was and they tried to identify what were the characteristics of patients that said this is the characteristic of the disease and this is what we know about it. Is it age? Is it gender? Was it thyroid? They looked at a myriad of... and those were the 3 things that really helped prognosticate what was going to happen with the disease and identify it. So, they are looking retrospective in the data. So, they had 900 different patients that had those workups and so they teased out from all those patients what their score was and they looked over time how people did because at that point in time there were no approved treatments, what the longevity was and what was the survival and so that's where those numbers came from.

Sylvia: Because he was diagnosed was a 5 when was originally...

Jean Ridgeway: So Philly is saying that Richard was a 5. Now remember, one of the... and what you hear about is that people... that you have to understand the context of the IPSS scoring system. It prognosticates survival without treatment. Okay. And that's a drawback because as people get treated and we have good therapies, it changes the outcome. Right? Because... I mean, even for a low risk person like Mr. Hentzel, I get to pick on him. So, you know, he's 11 years on this drug and there is no 11 years on even for a low risk person, but we have to change with the available therapies because now we have therapies that really do help change that and then someone truly with a 5, I mean, you can get a number of cycles. Your blood counts get normal. Right? So, then you're thinking about how many cytopenias I have. So, the tool is not designed to be workable with your changing blood counts and with your changing disease status. It's just a tool.

?: Each drop is a 5, but you won't stay a 5.

Jean Ridgeway: That's absolutely true and the goal is that you may be a 5 and yes, you need treatment, but you're not going to "stay a 5." It's one of the shortcomings of the tool. That's all it is. It's a tool.

?: So the drugs that you're talking about, are those the low intensity chemotherapy? Is that what those are?

Jean Ridgeway: So, Revlimid is an oral medicine and it's indicated for people who are 5Q- so... and 5Q- folks tend to have a lower risk score, right, because when we score it I pretended to be a 5Q- patient. I had a very low score and in general, that's how people present. They're anemic. I don't know how you presented, but it can fit that picture. Now, these other drugs that people have talked about is Azacitidine or Vidaza or Decitabine or Dacogen. Those are... They both fit under the category of being hypomethylators. That's how they work. They work like big demethylate things and those are really used for people who have intermediate to high risk disease.

?: What I'm trying to understand though is that a low dose chemotherapy? Is that what that is? Is that was those are?

Jean Ridgeway: They're both considered chemotherapy.

?: And is there... if you're on it for a long time, can it build a toxicity in your system over time?

Jean Ridgeway: What do you think, Richard? You've been on it 6 years. Do you get kind of toxic being on the drug? Pass the microphone, Sylvia. No. He's saying no. How about somebody else? Who else in here in this room has been treated with a hypomethylator? Anybody else? What do you think?

?: I'm on 21 months.

Jean Ridgeway: Twenty-one months on?

?: Azacitidine.

Jean Ridgeway: Azacitidine or Vidaza. Shot or infusion?

?: Infusion port.

Jean Ridgeway: Through the port. So, it's a drug that can be given as a shot or it can be given... A lot of people have portacasts (sp? 18:03), which they can for lots of different things and so they get it as an infused drug.

Sylvia: Then also the port is the way to go because otherwise the veins collapse.

Jean Ridgeway: Your veins kind of, you know, they get fatigued.

?: So I mean, what I'm asking is can you be on it for years and it doesn't build toxicity or (inaudible 18:22).

Jean Ridgeway: Correct. Yeah.

?: Average lifecycle is 18 months. There are the exceptions that I'm sitting next to (inaudible 18:31) others right across from me that...

?: I quit working at 13 months.

?: The average cycle is 18 months.

Jean Ridgeway: Average life expectancy or...

?: And then you would go off it?

?: And average lifecycle of the effectiveness of the drug.

Jean Ridgeway: Of the drug. So, some people and I will tell you, I mean, honestly, these group of drugs work for some people. Sometimes they don't and there's no way to predict who's going to do really well and who's not going to do well, but there are therapies that are relatively easy to tolerate. They don't make you lose your hair. Sorry. He came with that. They don't cause a lot of... You chime in. I would say in my experience some people have some nausea. Other peoples really don't. Some people have a little bit of GI complaint like dyspepsia, constipation, diarrhea. There's a mixed bag, but people generally do pretty well with the therapies compared to paralleling someone who's getting acute leukemia treatment which causes your blood counts to drop really low and they have higher intensity therapies. They have side affects. Absolutely they have side affects. How well you deal with them, I think... some of it mindset and some of it just how do you do that.

Sylvia: Your body chemistry, too. I think... Now, he's diabetic also. He has a pump, but changes every three days.

Jean Ridgeway: So you have insulin...

Sylvia: I don't think there's an answer. It just... What doesn't work for him may work for somebody else vice versa.

Jean Ridgeway: So, go back to your question again. Ask your question because now I can't remember it.

?: Well, I was just wondering about how long can you be on it and then if you do have to go off it what happens to you if your levels are good? Does it mean you'll or are you in remission or...?

Jean Ridgeway: It sounds like that's what... It really depends. As you work with your oncologist about what your plan is. If you're an intermediate or a high risk patient, it's not unreasonable to start with a hypomethylator with the Vidaza or with Decitabine. That's reality common and because of what we know how these drugs work, we know it's going to take 4 to 6 cycles. That means 4 to 6 months of treatment before we can tell if they're really, really working for you and remember we talked about like things will get worse before they get better because and one of the reasons is when you have a diseased bone marrow with MDS, as you get rid of the malignancy and the bad cells go away, sometimes the bone marrow itself is recalibrating to work up to its full potential because sometimes those malignant cells actually do benefit your blood counts and when you begin to take them away then the blood counts drop and then as the normal healthy bone marrow continues to regenerate then your blood counts can get better.

Sylvia: And that's one thing I didn't realize because I noticed after he had been on Vidaza, I said gee his counts are dropping and he said that's normal.

Jean Ridgeway: And that's totally expected. Totally, totally expected. Now the second part of your question how long can you do it? It depends what the plan of care is. Now, I don't know how old you are, Richard.

Sylvia: He's 81.

Jean Ridgeway: You're 81. Okay, so you know we don't transplant people who are 81 years old because stem cell therapy... To undergo a stem cell transplant is exceptionally rigorous on your body systems and so there's been a line in the sand that's been drawn that says 70... early 70s. Seventy-one, 72 years old is kind of the extent of people's comfort level of when they will do an allogeneic stem cell transplant because it's a difficult therapy. So knowing that that's not an option for Richard, it makes sense to me that he continues on other therapies, but it does get to a point in time 6... 5 years you've been on it. It's like hey let's kind of... let's space it out and see what happens.

Sylvia: And that's what he did.

Jean Ridgeway: And your blood count started to slide. So somehow that malignant clone began to re-grow and repopulate and come back and sometimes it can be sensitive, again, to the hypomethylator. Sometimes it doesn't. I had a patient who... he was a professor and he developed... He developed actually a therapy related MDS. He was the gentleman who had prostate cancer and when men have prostate cancer, they get their pelvises radiated. So, this is the pelvic field and as adults this is where a lot of our hematopoiesis takes place in the pelvis. So, they call it pelvic field and so he had external damage from radiation to his bone marrow trauma and he developed a therapy related MDS and he went on therapy with Azacitidine, Vidaza and he did it and he had a great response and he became transfusion independent and he said, "I'm done. I'm going to Arizona." So, he went to Arizona and he's on that same track... Okay, we gave him all kind of instructions, whatever. He came back and his blood counts were okay and then they started to drift slowly and in the summer he said, "Okay, I'm ready to do this again," and so we did this cycle with him where he would get treated. He'd say, "I'm now going to

Europe. I've always wanted to go to Europe. I'm going to Europe." So, he'd go to Europe and he came back and the we... and we did that a couple of times, but there did come a time where he didn't respond for whatever reason. I mean, the biology of these diseases is very sophisticated and we just don't know enough. I mean, we're learning more and more but for this gentleman, his quality of life and what he wanted to do to live life was to bucket list, going to Europe, doing whatever and he was able to do that and said, "I'm willing to take the risk that this might not work again," but now if you're a younger person and your doctor is looking at a stem cell transplant, they may give you a number of cycles and then in the process of you getting treated, maybe begin to look for a donor, asking siblings to come in and be typed, etc. So, it really depend... that individualized therapy...

?: How would you know at what point you're at that you would need... that you would consider that transplant?

Jean Ridgeway: A transplant? So if you're under the age of 72, your doctor has already looked at you saying transplant is the cure. That's the lens that you get looked at from an oncology perspective honestly because one of the first things that gets asked are do you have brothers and sisters because why? Because they're looking for a genetic match. Question.

?: Haven't they recently dropped the age for transplants?

Jean Ridgeway: What do you mean dropped the age? Like made it lower?

?: Yes to 65 and even less than that.

Jean Ridgeway: So, there are some transplant centers around the country who will only transplant people until a certain age. The transplant center that I work at we're transplanting people... our oldest has been 72. We've done a good handful of people who have been in their early 70s. Now, they're robust 70 year olds. They're not somebody who's... that performance status of being in bed all the time, that's not them. They're really active kind of folks, but if you go to the Fred Hutch Center in Seattle which is like the largest transplant center. If you're over 55, I think they turn you away. So, there are some criteria. Better outcomes. It just... It really depends and the biggest gatekeeper of all within that process is your insurance company. Written into the very fine print of all of our insurance policies is if you need a stem cell transplant which none of us ever bothered to read, you will go to A, B or C institution. Go ahead.

?: Only they look at they say well this person is 75. His life expectancy is fairly narrow. Let's give it to a person who's got more life expectant because that happened... Who's that baseball player?

Jean Ridgeway: That was a liver transplant.

?: Yeah, well...

Jean Ridgeway: That's different. We're not talking about that.

?: (inaudible)

Jean Ridgeway: But that organ had to be secured from a donor. Right? You get your... You can get stem cells from your siblings. Now if you're 70, there's a good chance that your siblings are 70 plus and to get their stem cells and to collect them is more of a medical challenge just because of the biology of aging with their normal stem cells. So, it can be, but we've had patients in their 70s donate to their siblings in their 50s and they mobilize. There's a couple new medicines out there too that help succeed. We have better success with that, but age somewhat. It's just the way it is. I will tell you that the mortality risk with a stem cell transplant even if you're 50 for the first 30 days is still 20 percent period. Just across the board. Things can happen unexpectedly with transplant people get bad infections, etc. So, I mean, it is part of the thinking process with transplant because it's appropriate, but for some people it's not appropriate. If you're 82, it's not appropriate. Just isn't, but it's not just... So, there is an age piece, but you just got to look around and see. Question.

?: (inaudible 28:35).

Jean Ridgeway: So, what's the criteria to choose a donor? So, I'm the patient again. Now, I have a mother and I had a father and so genetically if you remember back to biology now you get half of your genetic material from your mother and you get half of it from your father. So, my brothers and sisters also had the same opportunity to get from mother and father and just because of how gene shuffling occurs, you literally have a 1 in 4 chance of identically matching your sibling. So, the first piece that's done from either patient is to get my biologically related siblings, full siblings. The same mom and same dad to get them typed. Now if they're, say, I don't have any matches. What's the next step? The next step is to go to something called the National Marrow Donor Bank and this is all done via numbers. So when I am typed, they draw a couple vials of blood and they do an HLA typing on one of my chromosome. It's the sixth chromosome and that becomes the whole series in grouping of numbers and we have that set aside. So now, it's matching. Does anybody match and it has everything to do with ethnic origin. So if you're half Portuguese and half Eastern European, finding a match for you is probably going to be pretty different because there's really not a lot of people who are Portuguese and Eastern European descent that are going to share that genetic material. You may be really lucky. Minority groups have a more difficult time, but it is an international search. So, it all depends. So, that's the next step. Now, sometimes you get a match and sometimes you don't. If you don't get a match, the other option that's more... It is investigational, something called a half match or haplo match. So if my brothers and sisters don't match me 100 percent, they're for sure going to match me half, right, because we have the same parents, wink, wink, right? And so hopefully they're going to be half a match unless my mom was just being kind of rogue. So you can do a haplo and then a cord and there a lot of people nowadays after they deliver their babies, they donate the umbilical cord blood at the delivery and that's automatically taken, frozen and put in a bank and that's pretty easy and there are companies that that's what they do for a living. They secure these cords and it's frozen and you go ahead and you look for a haplo cord. So, that's another option if you can't secure a donor.

?: Those are stem cell rich.

Jean Ridgeway: Those are very stem cell rich. So, those little babies little cord is very dense. Now the only potential problem is if you're a person whose 120 pounds that's good. If you're somebody who's 420 pounds, that little cord and all those stem cells... I mean, you do need a certain percentage to repopulate your bone marrow. So, it gets a little tricky. Size matters.

?: Do they ever look at relatives like cousins and...

Jean Ridgeway: So, do they ever look at relatives?

?: Things like that.

Jean Ridgeway: Let's go back to biology. Here's my mom and here's my dad. Here's my cousin. How much genetic material do I share with my cousin? Well, my cousin's mother is my father's sister. So, the genetic likelihood of you matching with a relative statistically is less than if we look in the International Bone Marrow Registry.

?: How about children?

Jean Ridgeway: Children. So, children good. Mom and dad and now I have children. My DNA and my husbands. How much DNA am I giving to my kids? Half. What's the greatest likelihood... how much am I going to match with my children? Half. So, a haplo. There are these uper rare people who do match with their kids, but it's infinitely rare. Infinitely rare. So, we do do that. I mean, at my institution happens to do something these haplo cords. They are investigational. We've had good results, but it's not the standard of care. So, you can look for children, but only after those other pieces are done. Look at my siblings, look at the National Marrow Donor Registry and then you look at children. Question.

?: Why would get constipated every time we get chemo?

Jean Ridgeway: Do you take a drug Odansetron?

?: Yup.

Jean Ridgeway: Zofran?

?: I'm Odansetron.

Jean Ridgeway: He asked how come he can't go little boy's room because Odansetron's biggest side effect is constipation. It slows the motility. So, I would say if you're taking and on Odansetron, take a Senokot S or a pericolate softener and mild laxative.

Sylvia: Well, we have this... it's like... I can't think of what it's called now.

Jean Ridgeway: Is it tea? Is it called Smooth Move? There's some people who've heard about that.

Sylvia: No. This is actually from...

?: MiraLAX.

Jean Ridgeway: MiraLAX is now over the counter.

?: (inaudible 33:41)

Jean Ridgeway: So, I would think, Richard, that's why. The Vidaza doesn't make you constipated, but Zofran does or the Odansetron does. Question.

?: It took me five months with (inaudible 33:59) both in Wisconsin and (inaudible 34:01) to figure out a formula so that number one, I drop my Zofran down the formula brand, but then I start drinking Smooth Move tea.

Jean Ridgeway: He drinks the Smooth Move tea. You guys think I was making this stuff up. It's true.

?: Then day number three, I augment that with one... So, I go to... like next Wednesday, I start drinking day one, two, three, the tea alone and then I go to bed and drink the tea with one Senokot S and but it's... I guess what I'm trying to say is everybody is different.

Jean Ridgeway: Everybody's different.

?: And what you have to do is push your oncologist team and the pharmacist and everyone you can talk to because the Zofran can really make it very uncomfortable (inaudible 34:50).

Jean Ridgeway: It can make it just miserable. Where do you buy Smooth Move tea? We all want to know.

?: You can buy it any grocery store in the organic area (inaudible 35:00).

Jean Ridgeway: Did you get that? Buy Smooth Move... It's rich and some... and Senokot is a mild laxative. It really...

?: It's a combination the first three days (inaudible 35:11) then tea and (inaudible 35:13).

Jean Ridgeway: So, he's figured out his magic recipe and you have to figure out your own, but there are some other options out there.

?: MiraLAX.

Jean Ridgeway: MiraLAX. Sandy's got a question.

Sandy: Regarding stem cells.

Jean Ridgeway: Stem cells. Okay. It's a hot topic today, you guys.

Sandy: When I was first diagnosed which was three years ago and I have (inaudible 35:37).

Jean Ridgeway: We're going to ask you to use the microphone because they can't hear you back there. Right?

Sandy: I asked my oncologist at that time well what about a stem cell transplant after I read up about the whole thing because I had no clue and it was, "Oh, no. Don't even think about that," and my reasoning was I'm younger. I'm healthy, relatively healthy, except for this one blood cell count that was low. Why would you not do that?

Jean Ridgeway: I can't speak for your oncologist. I can say that getting a second opinion is often really good medicine. This is a rare disorder and there are a lot of people who don't see a lot of MDS... Rush... Dr. Shammo sees a lot of patients. We see a lot of patients, but you could be someplace where your physician doesn't see a lot and I think there is great validity to getting a second opinion. I'm not trying...

Sandy: I did.

Jean Ridgeway: And?

Sandy: I did do that, but I didn't even get the opportunity to ask that question because it's like, "Oh, no. You are intermediate."

Jean Ridgeway: Intermediate risk.

Sandy: And it was like don't even think about it and they really pushed it off.

Jean Ridgeway: They pushed it off. Okay. And anybody else want to speak to their comment talking to a second... getting a second opinion that kind of you're uncomfortable with? Doctors are your employees.

?: I think they don't want to put you in harm's way too soon if you can survive without any symptoms and then the problem is that's where I'm at. I have no symptom. I work all day. I go to the barn and ride my horse everyday. I don't feel sick. They were telling me I'm sick. I'm not buying it because I don't feel (inaudible 37:27).

Jean Ridgeway: Who wants to raise their hand put their stamp on that? Who else was in that group when they got diagnosed? Feeling good, blood count's great.

?: When I first was diagnosed.

Jean Ridgeway: When you're first diagnosed.

?: You're first diagnosed.

?: My blood count isn't great. My red and my white are low.

Jean Ridgeway: Your red and your white are kind of low.

?: No, they're low. Yeah. And I have 11 percent blast, but I'm not sick.

Jean Ridgeway: You feel well. You have a bone marrow...

?: And I've got no infections or anything. My whole family, my kids, my husband all had flu and colds and I work with children. I teach.

Jean Ridgeway: That's good.

?: Yeah, but what's going on? And then they tell me I'm sick.

Jean Ridgeway: You have a disease that's... it's not going to go away and you need to be monitored. We talked about triggers for treatment, right? Are you transfusion dependent? And their answer is no.

?: I never had...

Jean Ridgeway: Are you getting more infections? The answer is no. I don't know what your blast count is doing. Maybe there's unfortunately you only check it with the exam and if you're continuing to feel well, I don't know what your cytogenetics are.

?: None.

Jean Ridgeway: You have no cytogenetic abnormalities.

?: Normal.

Jean Ridgeway: So, I mean that intermediary group is for clinicians it's kind of like this fuzzy group. People are doing well. They have a great quality of life. Things don't really seem to be mattering. I mean, there's really nothing to do except I would encourage you to remain vigilant. If they're asking you to come in once a month for a blood count, I would definitely do that regardless of how you felt.

?: I go every three months.

Jean Ridgeway: Every three months. Yeah and have you had a second opinion?

?: Yeah. I just went and had one. She was pushing the clinical trial. I said no clinical trial and then she started talking about a transplant. So now, she's talking about me going (inaudible 39:24). The insurance company has approved me to have the testing done.

Jean Ridgeway: For the transplant?

?: (Agreement sound) and they've typed me. So, they haven't got the results back yet.

Jean Ridgeway: It takes about three weeks.

?: So, I mean I feel like I could do it, but now I definitely do, but as I read about it I realize that you're not officially cured. I mean, it can come back.

Jean Ridgeway: That's true. There is a risk for that.

?: It can come back as a secondary cancer, too. You might end up with a different cancer. So, it seems like it's not the total answer either.

Jean Ridgeway: It's not a simple illness and there are lots of variabilities within everyone's illness.

?: (Agreement sound), but I mean that's when I asked the doctor originally about a stem cell transplant and basically he said, "Well, why would we do that to you right now? You're having no symptoms. Why would we do that? I could kill you." That's basically what he said, "I could kill you with the procedure."

Jean Ridgeway: Well, you don't just jump into therapy for a transplant. The first piece is to really get your MDS under control. Eleven percent blast. So, 11 percent blast... You should have 5 or less in the classification systems when we even look at what's the difference between MDS and AML. So, there was the old classification that was the FAB and the WHO. So... and the FAB classification, if you have 30 percent blast, you have acute leukemia. In the WHO, it's 20. So, I mean, that really is... It doesn't sound like a big difference, but it is a huge difference and so I would...

?: There's a lot of old thinking and new thinking going on at the same time.

Jean Ridgeway: Well, there's just revision. It's kind of like if you have 20 percent, in probably a short amount of time you're going to have 30 percent and what's been found through the years is that when people have an intermediate or a high risk disease, you do need to be treated because the disease continues to progress. So, I would say keep following it. It'd be interesting... You need to find a practitioner that you trust. When that person says to you I recommend you go this way. You say I'm on board. You could go to this guy over here and you go, I don't really like that and you get this uncomfortable feeling. I think you really do need to look for someone that you feel exceptionally confident with. You're putting your hands in this practitioner's life. It's a big deal and if you haven't found that somebody then feel free to ask the people in the room. Do you like your doctor? Is he great... a great guy? A great gal? Do they know what they're talking about? It's a big deal. It's a big, big deal. You have to find the right person and I'm kind of hearing that you haven't really found that person. Other questions. Twenty-five after.

?: You were talking about the umbilical cord. How do you go about doing that?

Jean Ridgeway: So if you're pregnant...

?: I have a daughter who is pregnant.

Jean Ridgeway: You contact the International Bone Marrow Registry and you work through them. So you can even Google 'umbilical cord donations' and it has to be set up and organized before the delivery. Usually, a bit of time beforehand like a good four to six weeks beforehand to donate.

?: (inaudible 43:05)

?: I'm going to ask her about that.

?: (inaudible 43:10)

?: Oh, really. Okay.

?: There's a fee, but...

Jean Ridgeway: There's a fee and oftentimes there's a fee for the donor which seems ridiculous.

?: Right.

Jean Ridgeway: But there is a difference between a public donation and a private donation and so there's some... Question again.

Paula: My mother is not living here. She's not (inaudible 43:44). So in the case (inaudible 43:49) more (inaudible 43:51) do that here in the United States without insurance because she (inaudible 43:58).

Jean Ridgeway: I would suggest two things. So, your comment is your mom lives in Venezuela. Just so for people who can't hear you which I think most people are unable to hear what you're saying. So, your mom's from Venezuela and you're looking for a second opinion. Could she do something in the United States? You can. It's a fee for service and if you want to get a second opinion of someone here you can. I mean, I would honestly in the back of the MDS Foundation, there're Centers of Excellence and that means these centers see a lot of people with MDS and you do need to see somebody who is experienced with the disorder. I would say look through there and match up where you live and make a phone call and say we want to come for a second opinion and you just say it's a fee for service. It's an out of pocket expense that your mom has no insurance. You can do that.

Paula: Well, my question is in the case she has to (inaudible 44:53)

Jean Ridgeway: Treatment.

Paula: Treatment (inaudible 44:55) and in my country it's not possible because the cure rate has (inaudible 45:05) her sister and (inaudible 45:10).

Jean Ridgeway: I'm really not familiar with health systems outside of the United States. I do know that in the back of this booklet it does give you some international references to look for, but again if people aren't a match, the type of service that you can get from any 1 transplant center is limited by what they offer. If they put the age limit at 60 and say if you're over 60 and you don't have a sibling donor we don't do the transplants then that's the situation. Like you were saying, it's not a therapy that's benign. It's a rigorous therapy with quite a few side effects and you need to be robust enough and the center has to be expert enough to be able to handle the difficulties that are going to arise and they're going to arise. So, I would look in the back of this book or go online and get some recommendations, but it's a bit of consumer shopping of where can you go and get the service that she needs. Other questions.

Sandy: Can you explain my blasts are under five percent and yet my blood count started with just low platelets and I had low white count. Now, I have low red count. Now, all three are low. Why is it that keep going down and yet...

Jean Ridgeway: So, Sandy's comment is her blasts are low in her bone marrow, but her blood counts are starting to drift. Have you had treatment?

Sandy: I was on the clinical trial.

Jean Ridgeway: You were on the clinical trial of?

Sandy: It was the combo... It was Dr. Shammo's trial with the combo drugs, Vidaza and Revlimid which I flunked because...

Jean Ridgeway: The drugs failed you.

Sandy: Well, my count kept going so low, they couldn't keep doing it. So, she just had to stop.

Jean Ridgeway: So, she stopped and now you're on a bit of a holiday?

Sandy: And since I stopped it which was in February, my red... hemoglobin was like in the 11s and lowest was like 10.5 and now 9.3.

Jean Ridgeway: So, Sandy's question is she was on these treatments and her blood counts got low. Please take some of the stuff in the back as you guys leave. I would think that initially she removed you from the therapy because your blood counts were decreasing and I would think that she hoped that by removing the therapies you'd have some recovery.

Sandy: Right.

Jean Ridgeway: And has she done another bone marrow biopsy?

Sandy: I've had five.

Jean Ridgeway: You've had five of them.

Sandy: And the blasts don't... they're not increasing. That's why I don't... It's like why is the disease progressing if the blasts...

Jean Ridgeway: Because the blasts are only one...

?: Element.

Jean Ridgeway: One element of the disorder. The whole bone marrow matrix and there's lots of other things that you don't see when you see a slide of this. There are all these cytokines and molecular markers and there's lots of other biology happening in your bone marrow other than just your blast. If she's telling you that your disease is continuing to...

Sandy: No, but she was very happy that my blasts weren't (inaudible 48:45). She was more like oh that's good.

Jean Ridgeway: That's good but for whatever reason your bone marrow is not coming back and it's hard to say. Sometimes people who get these therapies have prolonged cytopenias. So when they talk about adjusting therapies like pulling back and seeing if people can recover is a common strategy. Not always effective because maybe it's not the drugs that are doing it. Maybe it's something else that your disease has decided that these drugs are no longer going to be effective.

Sandy: Okay, which is like that's the other option is to do nothing with this disease.

Jean Ridgeway: Well, it is an option. It's always an option.

Sandy: Sometimes it's...

Jean Ridgeway: Sometimes you have to wait and watch. You have to see what's going to happen. Other questions?

?: The continuing education is (inaudible 49:40).

Jean Ridgeway: Continuing education. You got it.

?: It's a hard thing to deal with though because...

Jean Ridgeway: It is a hard thing.

?: Once you know you have it, it kind of... It's ruined my life for the last year because I know that that it's there (inaudible 49:55) and that's very annoying. Like, I mean it keeps me from

sleeping at night sometimes because it's on my mind and I can't let it go. The doctor will be like you need to just let it go. How can you? I would rather not know. I would have had a good year.

Jean Ridgeway: How about some of the other survivors in this room. How do you deal with that?

?: One day at a time.

Jean Ridgeway: One day at a time. How do you sleep? How do you deal with the anxiety? What do you do?

?: Like the doctor told me (inaudible 50:29) first diagnosis don't worry about (inaudible 50:31) nothing you can do. He says try to make (inaudible 50:36) one day at a time. If you do what you want to do one at day at a time you'll worry about it but you won't...

?: As soon as you get too smart.

Jean Ridgeway: Getting too smart. Richard, how about you? What do you think? What kind of advice would you give?

Richard: You live with it. Yeah. Live today and tomorrow.

Jean Ridgeway: Words from the back row?

?: I think you just learn to deal with it and get up every morning and do what you feel like doing.

Jean Ridgeway: Stay positive. Stay positive. How do you stay positive when like you're fretting and then you read the stuff on the Internet and you go, oop. How do you stay positive?

?: Go to a hospital and volunteer and see people.

Jean Ridgeway: Do something outside yourself.

?: See somebody that's really, really in trouble.

Jean Ridgeway: Exercise.

?: Somebody that's worse off than you are.

Jean Ridgeway: Your mother told you that, didn't she?

?: She did.

? Go to Michigan (inaudible 51:51).

?: Go to the casino.

Jean Ridgeway: Go to the casino. Yeah. I mean, coping with a chronic illness is difficult and somehow we're all unique people. We've got very different backgrounds and family situations and going to a counselor is a good idea. Maybe you need... You might be able to benefit from someone external saying giving you some inputs to help you retool your perspective.

?: One thing we thought of is nobody's promised tomorrow.

Jean Ridgeway: No one's promised tomorrow.

?: And we can be killed in a car accident today or and nobody knows what their timeframe is here on Earth.

Jean Ridgeway: It's very difficult. It's very difficult. You had a comment in the back.

?: We were asking (inaudible 52:54) regular (inaudible 52:56).

?: Well, yeah. I don't sit around the house fretting. I mean, I go to work everyday. I do all the things I normally do, but it's never... I can't get away from it no matter what I do. It's always there. It's always biting at me. I feel like I'm always trying to run away from it. That's annoying.

Jean Ridgeway: We have some input from the front.

?: I think the one thing that was not said put your faith in God. The power of prayer works. God has a plan for every one of us here and as long as you put your faith into the Lord, He will help you and you've all lived a good long life. I'll be honest with you and hopefully will live a lot longer. Think of the people that 10 years old or 20 years old. Be thankful for what you have. Be positive and say a prayer. That's the biggest remedy.

Jean Ridgeway: And it sounds like you're doing all the right things. You're looking for... You sought out different opinions and you need to take care of yourself and do things to make yourself do better and staying positive.

?: How long ago were you diagnosed?

?: Like May of last year.

?: It takes awhile I think. I think it takes quite awhile to get over that hump. You accept that you have the disease and then you just kind of go from there, but it takes longer than a year. That's for sure.

?: Nobody said life was easy.

Jean Ridgeway: Nobody said life was easy.

?: It's nice, too, knowing that there are other people that are dealing with this. I never ever heard of another person having a blast until we came to the first symposium and I was shocked at how

many people were there and that gives you a little bit of hope too that there are other people dealing with the same thing.

Jean Ridgeway: Another comment from the back.

?: Try not to let this be an interruption in your life.

Jean Ridgeway: Try not to let it... Try.

?: Normally, get up every morning and (inaudible 55:24) and that should be your goal for (inaudible 55:35)...

Jean Ridgeway: Yeah.

?: To get up and do the thing that gives you pleasure (inaudible 55:39). While it is an interruption in your life in many cases, don't let it be.

Jean Ridgeway: Other questions.

?: You try to do that. However, I had to give up tennis. So, there's also limitations which is what you have to do. Accept and deal with also.

Jean Ridgeway: Who funds clinical trials? So, there are different types of clinical trials. There can be investigator initiatives. So say, once again, I'm using myself as an example, but say I'm a doctor and I say I know that substance A and substance B work together. There's a whole process of getting that through. So, a lot of times those are "unsupported." It's just the physician saying let's try to mix this drug and that drug. If there's a new drug under development, you're getting the drug from the company and oftentimes the companies who are like involved in drug development, they know who the movers and shakers are, say, in MDS and so they'll go to those physicians and say like the Onconova trial. You have MDS patients. Would you be willing and it's very rigorous. So, some of it is private funded and some of it is government funding. You know, the National Cancer Institute.

?: Insurance doesn't cover (inaudible 57:09)

Jean Ridgeway: If you enroll in a clinical trial, insurance companies cannot not allow you to participate in it and most of the times if it's a drug then the drug is provided free like when you first went on the clinical trial, the drug wasn't available and he received drug free of charge for...

?: He still does.

?: It was only the drug. All the other follow up stuff is out of your pocket. Your insurance will not pay.

?: When I was in the clinical trial, everything was covered. It cost me nothing.

?: I think it depends.

Jean Ridgeway: Yeah, but it really depends.

?: The drug company pays for most of it.

Jean Ridgeway: It depends on how the protocol is written and what's allowed at the various institutions. Sometimes it could be that the drug alone is covered and sometimes drug companies will say whatever patients enroll on the trial will continue to provide them drug until they start pushing up the daisies. They give drug forever which is great. There are other studies that say we'll provide until... If the drug gets approved then that person will just have to get it through regular means. Sometimes blood like if there are additional blood tests that are needed, if there are bone marrows that are needed, those will be covered because they're not considered a normal follow up of a hematological problem. So, there's really...

?: His insurance company covered those. It was the drug we got for free, but that was...

?: The only thing that was free was the drug itself. Everything else was a fee.

Jean Ridgeway: Right and it was considered standard of care.

?: The insurance paid it.

Jean Ridgeway: Right and so the insurance company would say you were seeing the physician and you had CBCs or you had a blood transfusion. That gets covered, however, the insurance that you have but then there are other clinical trials that have these huge umbrellas and there's just so much variability and we have all of those trials where I work. We have investigator led that are it's just we have the drug but everything else is standard of care. Now, we've got examples like yours that say it covers everything, but usually things that are considered standard of care don't get covered like the visit or the CBC. There's so much variability. Questions? Go ahead.

Paula: Could a non (inaudible 59:43).

Jean Ridgeway: Can a non US citizen be part... So when clinical trials are written, they're very explicit on who's allowed and who's not and many, many, many if not all the time it's being hosted in the United States. They exclude people because they want to be able to continue to follow people and so they'll say like you can't have HIV, you can't be pregnant. It has to be 18 and older, US... They will put that restriction sometimes, not all the time, but sometimes. Every protocol is different, but that can happen.

?: What do you see in the future with Obama Care coming into (inaudible 1:00:28)

Jean Ridgeway: What do I see? You really want to know that?

?: All these problems. I mean, is our level of care going to go down, the medications.

Jean Ridgeway: I mean, funding has already been cut for lots of... There's been funding cuts across the board to the National Cancer Institute and they really fund investigators and drug development. I mean, the government does do a lot of drug development through the NCI and through various other mechanisms.

?: Well, the sequestration has resulted in cuts across...

Jean Ridgeway: Across... The sequestration... It's kind of like lopped it off.

?: It's not the President. It's congress.

Jean Ridgeway: But there are entrepreneurial people who fund private funds and will still invest in pharmaceutical companies. So, I think that that will continue. It's just going to look different and then over the three decades that I've been in practice, it's looked different every 10 years. So, I think it will continue. It's just going to look different. I would hope that... It will be very interesting to see. I mean socialized... If you take a look at socialized medicine, there are many countries in the world that practice socialized medicine and they say if you have disease X, you get treatment Y. Period. In Europe, in Italy, for example, the government says if you have MDS, you can have EPO therapy or injections. You can have a transplant, but we don't cover Azacitidine. However, if my private employer or if I have money I can still get it. So, there will...I think continue to be that type of subset. England is a pretty good example of restricted healthcare as well as The Netherlands. So, how it gets implemented in our country as it rolls out, we'll all find out. We just don't know. Anything else? One more.

?: My past experience (inaudible 1:02:31) since been diagnosed. For instance, I've lost weight and I have not been able to regain it even though I eat. I eat three meals a day.

Jean Ridgeway: That's interesting.

?: I also have the swollen ankles periodically. I was wondering if other people had other symptoms like that or (inaudible 1:02:49).

Jean Ridgeway: Other symptoms for folks. Weight loss.

?: When I first found out, I lost 10 pounds.

Jean Ridgeway: You lost 10 pounds?

?: Yeah. When I first found about (inaudible 1:03:01)

Jean Ridgeway: When you first found out.

?: I'm back up.

Jean Ridgeway: It's unusual for people with MDS to have that. I don't know what's going on concurrently. Usually, weight loss is not something, but...

?: It further proves that I no longer have MDS. (inaudible 1:03:24).

Jean Ridgeway: I don't have a good answer for you. Other than it's unusual. Do you see a good doctor? Check your thyroid?

?: Do you ever drink Ensure? The milkshake, the Ensure.

?: Yeah.

?: That's the second time (inaudible 1:03:54) my thyroid. What does the thyroid have to do?

Jean Ridgeway: Oh, the thyroid... Your thyroid can... it affects your bone marrow function.

?: Oh, does it?

Jean Ridgeway: So if you're severely hypothyroid, you can be anemic. That's why and it's very common. So, common things occur commonly. So, you kind of look for the what could be a possible easy fix.

?: The only reason why I even see that is what I... Well, part of the reason they claim that I got MDS is their radiation treatments...

Jean Ridgeway: To?

?: In my forehead.

Jean Ridgeway: Your forehead.

?: Because that was a... (inaudible 1:04:35) had it on 60 Minutes, how I got on it. They... In all radiation accumulates in your thyroid.

Jean Ridgeway: It has an effect on that. And years ago, this is before my time, they used to treat... They would radiate people's thyroids and they also used to radiate people's adenoids when they had repeated infections. So, I mean things that were done a long time ago seem to make sense and it was the current state of medicine but now here we are decades later and the repercussions are unpredictable.

?: Like the doctor told me when I was talking to him about it back in (inaudible 1:05:12) he said that half the people that work, actually work on radiation, die from it anyway.

Jean Ridgeway: The guy who actually invented I think was Rogan and he said repeatedly to his hand and the guy ended up with some type osteocarcinoma of the bone, of the hand, that he x-rayed a kabillion times. How did he know?

?: Would radiation (inaudible 1:05:36)

Jean Ridgeway: An x-ray, a chest x-ray has an equivalent amount of radiation as a half a day of sunshine.