

Speakers:

Suman Kambhampati, MD

Jean Ridgeway, DNP, APN, NP-C, AOCN

**Suman Kambhampati, MD:** Happy to take questions if there are any. Yes, ma'am.

**Q1:** How many MDS patients does KU treat in a year? I mean, are there a lot? I mean, there aren't a lot of MDS patients anyway.

**Suman Kambhampati, MD:** That's a good question. In fact, we looked at that data just last week or two weeks ago and we looked at the whole data within this shop here and the other satellite shops. I think within the last quarter or so the average number was about close to 80 to 100 patients spread across all the satellite offices. We are still teasing out that data to see if just regular anemic patients were called as MDS. There is a new ICD10 diagnostic coding. That's very laborious. So, I think some of the patients were just presenting with anemia or low blood counts are probably being labeled as MDS. We are looking at that data, but that's just the quick snapshot we got a few weeks ago.

**Q1:** And was that 80 to 100?

**Suman Kambhampati, MD:** Yeah. Again, we have not looked at the demographic data yet, but that's just the average number we have seen.

**Q1:** Okay and the 'to' is a T-O.

**Suman Kambhampati, MD:** What's that?

**Q1:** It's 80 to 100, not 8,200.

**Suman Kambhampati, MD:** Yeah.

**Q2:** My question is regarding Revlimid. Is there any... how long should a patient take it or is there any... What's the longest? Should it be taken forever? Should it be stopped? When should it be stopped?

**Suman Kambhampati, MD:** Right. As long as the clinical benefits are being met, the clinical benefit would be transfusion independence or in 5Q, the cytogenetic benefit, but as long as they're doing okay with it staying transfusion independent.

**Q2:** How about okay what if you're not transfusion independent. Should you still be taking the Revlimid?

**Suman Kambhampati, MD:** So again, the study looked at patients who were transfusion dependent. So, if you're not transfusion dependent then I usually try the erythropoietin stimulating agents.

**Q2:** Yeah. But that didn't work.

**Suman Kambhampati, MD:** So, if that didn't work then I think...

**Q2:** I've taken it for about eight years now. I mean, is that...

**Suman Kambhampati, MD:** That's fine.

**Q2:** It's not hurting anything. Okay.

**Suman Kambhampati, MD:** Congratulations.

**Q2:** That was my question.

**Suman Kambhampati, MD:** So, we keep going as long as there's clinical benefit.

**Q2:** Okay. That's kept me going I guess.

**Q3:** If you're on Vidaza and it's been working, but say it stops working for you so you need a transplant. What if there's not a donor at that point? Do you go onto a transfusion type thing I mean till you can get a donor or...?

**Suman Kambhampati, MD:** We're always on the lookout for clinical trials. As I said, clinical trials definitely offer better survival benefit than just transfusions. So hence, we look for clinical trials and then there is also this alternative donor, cord bloods, haplo transplants that could also be considered. Definitely more risky, less data as compared to sibling or unrelated donor match, but definitely feasible in the right fit... right patient population.

**Q3:** And I noticed you said on that one when it didn't seem to be a benefit like if you do go for a transplant to do that radical chemotherapy before you do your transplant.

**Suman Kambhampati, MD:** Yeah.

**Q3:** Are they getting away from the chemotherapy type thing?

**Suman Kambhampati, MD:** Yeah. We are actually getting away. In fact, I've not used the complex chemotherapy in several years now. I usually stick with Azacitidine or Decitabine and beyond that we just look for clinical trials.

**Q3:** I mean, if you were like Vidaza didn't work and you had to go have a transplant. Did they still do that chemotherapy there before the transplant?

**Suman Kambhampati, MD:** Yes. Yes.

**Q3:** Okay. I misunderstood. Okay.

**Suman Kambhampati, MD:** The purpose of chemotherapy there is to 1) is to reduce the leukemia burden so that it doesn't come back immediately after transplant and secondly to suppress the immune system just enough so that it can accept the donor cells. Good question. Great question.

**Q4:** How long is Procrit normally good for for a patient? Two years? Three years? Four years?

**Suman Kambhampati, MD:** Yeah. It's variable. Actually, it's variable. In some patients... in some of my patients have been on it for several years now. So, it varies and generally if they stop responding to Procrit I add the white cell growth factor, GCSF, to see if it would restore the response.

**Q4:** What quantity of Procrit is too much?

**Suman Kambhampati, MD:** Right. So, too much would be if your hemoglobin is exceeding 12 grams.

**Q4:** I guess too much to... if it's not working like mine it started out at 20. It's now at 40.

**Suman Kambhampati, MD:** You can go up to 80,000.

**Q4:** Eighty. So, there's two more steps up. Okay.

**Suman Kambhampati, MD:** Yeah, but generally at 40,000 if it's not producing the desirable results, I add that Filgrastim, the GCSF or Neupogen to see if we can get some boost or better boost from Procrit. Sometimes you can double the responses by adding the GCSF.

**Q4:** When the time comes that none of this stuff is working, what's your last three months of life like?

**Suman Kambhampati, MD:** Well, I mean it all depends on infections. Infections still remain the most common cause of death in MDS patients. Regardless of low risk or high risk. So, infections pose significant problems and the burden of anemia, if your transfusion burden is high then those last three months are likely going to be difficult. Transportations, wait times in the

clinics, so on and so forth and the platelets, the low platelets are something that we see it as a problem, but patients often are not very symptomatic from it, but the anemia and the low white blood cell count pose the biggest threats to quality of life and also to lifespan.

**Q4:** I mean, are you mobile?

**Suman Kambhampati, MD:** Yeah. Yeah. Again, depending on the (inaudible 7:18).

**Q4:** And your brain is still working? Okay. So, what's the... Aside from the inconveniences, what's the symptoms that you wind up with?

**Suman Kambhampati, MD:** The fatigue is the predominant symptom. I call it the sun downing fatigue. Patients are usually bright and awake, more awake in the morning and then as the evening hours or night time they usually slow down and the fatigue also correlates with the degree of anemia. So fatigue, activities of daily living, nutrition. Appetite goes down. So, I usually tell my patients to have their most caloric intake in the morning. Don't keep your... don't wait for the dinner for your good calories. Have calories, more calories, in the morning and lunch. You might not have the same degree of appetite late in the day because the digestive system slows down late in the day in patients who are anemic and you just have to know to conserve your energy. You kind of have to time your day such that you get most of your work done early in the day and save very little for the later half of the day. Patients who are unable to do their activities of daily living should be supported with transfusions and that can vary from below seven to below eight. In patients who have preexisting heart disease we often are a little bit more liberal for transfusions. So, quality of life, impact on activities of daily living helps us with the transfusion thresholds.

**Q4:** What would be a normal number of transfusions you'd get in a month or a week or...?

**Suman Kambhampati, MD:** Well, the less the better as I showed you.

**Q4:** I saw that.

**Suman Kambhampati, MD:** So, every unit has to be clinically justified. Clinical justification is done by the degree of anemia and the symptoms. So patients who are symptomatic, very symptomatic, we usually have a liberal transfusion prescription. Patients who are doing okay with seven of hemoglobin, we let it go down to seven or below before we transfuse them. So, there's no fixed number for any patient. It's variable.

**Q5:** That was going to be my question. I had transfusions now for 9 – 10 years, but I had a hard time at some time the doctors didn't want to transfuse me in the eights because they said, "No, the hospitals won't do it."

**Suman Kambhampati, MD:** No, that's not true.

**Q5:** And that's what I've been told and I had to fight to get... I had to point out that it differs from different patients and what you can tolerate. My head was just totally dizzy and fainting.

**Suman Kambhampati, MD:** To be fair to the doctors, I think they're practicing the data that I showed you based on that data less the better, but we always have to keep in mind that patients still need to be supported whenever they get symptomatic. So, in our practice we do not have a fixated number. It varies from patient to patient and it's dependent on their symptoms.

**Q4:** If your number goes to seven then you got a real problem at that point.

**Suman Kambhampati, MD:** Yeah. Generally, we transfuse below seven, yeah, or at seven.

**Q4:** Your opinion, any chance of getting a cure for this disease in the next twenty years?

**Suman Kambhampati, MD:** So, some of the data that I presented I know doesn't look like a cure, but it's still a massive advancement in this field. I see it as a very big advancement especially in certain types of MDS. The cure is transplant, but the barrier to transplant is age. Majority of patients present it 70 or beyond, 75 or beyond. The Medicaid cutoff...

**Q4:** I was told I'm not a candidate for transplant.

**Suman Kambhampati, MD:** Right because the Medicare cutoff is 65. The bone marrow transplant trial that I cited allows up to 75. So, the trials give us some leniency in doing transplants, but the age alone is not a factor. We look at multiple other variables.

**Q4:** What's the success rate of a transplant?

**Suman Kambhampati, MD:** It's, again, variable anywhere from 20 percent to 30 percent to 40 percent depending on the preexisting factors, donor availability, transfusion burden before transplant, chromosomes. So, it's very variable.

**Q6:** I'm very impressed with the science behind this that people like you have come up with.

**Q5:** Come a long way in 10 years.

**Suman Kambhampati, MD:** Again, the patients and their families they need to be commended for this. I mean, without their unselfish commitment, I mean, I wouldn't be standing here giving you a one hour presentation. I mean, I remember doing my fellowship and Jean, you'd remember this. Azacitidine. That was it. Vidaza and transfusions. Our didactics used to be 10 minutes or 15

minutes at the most and now here we are looking at different types of MDS, cytogenetics, molecules and it's going to get better and better.

So going back to your question again, sir, about cure. We don't have a cure yet other than transplant, but we have certainly made advancements in improving the quality of life by addressing the anemia and by addressing the chemotherapy related side effects. Again, one of the key points I want to make today is patients do not derive the full benefit from their treatments, okay, if treatments are stopped early due to the drop in blood counts. That's going to happen. Eighty percent, 90 percent, I showed you data. It's going to happen, but we should not stop those treatments because if you are patient enough then the best results, best outcomes would be seen and I've seen several patients who... where treatments were discontinued because their counts dropped very low early on in the disease or the treatment course. So, that's a given. That has to happen.

**Q7:** Is there any age where they wouldn't do a... I mean, age-wise not and I know it depends on your health at the time that they wouldn't do a transplant? I know at one time, I mean after a certain age they say we wouldn't want to risk a transplant, but is there a certain age now that they don't really do that?

**Suman Kambhampati, MD:** Well, the Medicare age and Jean can correct me if I'm wrong is 65 and VA we have gone up to 70 in very fit patients. The BMTCTN study that we are doing here and across the United States is also a little bit more liberal with the age cutoff. So, 65 is sort of the de facto cutoff. However, it's variable.

**Jean Ridgeway:** We're doing allogeneic transplants where the donor is not this... it's an outside donor up to age 74 and we saw someone yesterday in clinic where he screened for... with MDS who's screening for a transplant. He's 74. Fit. That's part of it. It's not just how old you are and there are other considerations that work into the equation of is this really... does the benefit... are you really going to benefit from this or not.

**Q7:** And why is it that like the bone marrow registry, I mean, why do they cut off the age at a certain... I mean...

**Jean Ridgeway:** To be a donor?

**Q7:** To be a donor.

**Jean Ridgeway:** There's some theories that we'd like... The theory is that we'd like the younger stem cells. Some people ascribe to a theory of senescence that we only have so many and that the younger ones potentially can be beneficial. They'll also choose a male over a female.

**Q7:** Because we've had a lot of people that we know, of course, at our age and now everybody's too old. I mean, I'd love to do it, but they won't take...

**Jean Ridgeway:** It's 50 unless you're a sibling. Then sibling the age (inaudible 16:38) is removed.

**Q7:** Okay.

**Q6:** Something else that has impressed me over the last couple of years is I've been referred to four different places including KU. I saw Dr. Kay at the VA. He referred me to Nashville. I hear the same thing from every expert which tells me how widespread this knowledge is. It's impressed the heck out of me.

**Suman Kambhampati, MD:** I think, again, I have to commend MDS Foundation and the advocates, the patient advocates. They have improved awareness. The educational programs are not at Ash. I think it's a half day symposium is it, Audrey?

**Audrey Hassan:** Yes. We have a symposium every year where we educate the local community based hematologists and it's so important for them to learn and we have Dr. Peter Greenberg usually is on the faculty and the top thought leaders in MDS, but just like Dr. K was saying when I started with the Foundation over 14 years ago, we just had growth factors for supportive care, transfusions and we've come a very long way. Now, we have drugs approved by the FDA, but back then all we could offer was supportive care like transfusions or growth factors like Procrit. So, we are making a lot of headway.

**Suman Kambhampati, MD:** You had a question?

**Q8:** It's okay. In the early '50s, my grandfather passed away from what they called bone marrow cancer. Could that have been MDS?

**Suman Kambhampati, MD:** It could very well...

**Q8:** And is it hereditary?

**Suman Kambhampati, MD:** No, it's not. I mean, there are some patients I've seen who have had family history, but that's still very rare. It's still very rare.

**Q8:** My mother was diagnosed with it. Myelodysplasia. They didn't call it MDS. Just that myelodysplasia and she had bone marrow biopsies for the diagnosis.

**Suman Kambhampati, MD:** So, age... and I said age.

**Q8:** Well, she was younger. She was like being anemic almost our entire lives.

**Q9:** But is there and should we at least let our children know that if they come up with that they should ask...

**Suman Kambhampati, MD:** Yes, indeed.

**Q9:** Are you doing a CBC on me because...

**Suman Kambhampati, MD:** Absolutely.

**Q6:** I've done that.

**Suman Kambhampati, MD:** I mean, that should be part of the annual physical anyways. A CBC has to be done as part of the annual physical.

**Jean Ridgeway:** I work with some of my colleagues at the University of Chicago who are looking at familial tendencies. Does it really run in families and like Dr. K talked about the molecular markers. They're looking at different molecular markers within families, RUNX1 mutations and to see if are they expressed and is this person at a higher risk. So, Dr. Lucy Godley is really spearheading that movement at University of Chicago and has identified some familial genes. It's very cutting edge and some of those patients at younger ages. So, the parents were maybe in their 50s or 60s and had transplants and the children have had transplants and in their 20s. Almost like a prophylactic mastectomy with BRAKA genes. So, there's a lot more science being done, but there's just so much information it's hard to put your finger on it.

**Q9:** Because my father died an anemia. He just became anemic and I can remember that he got shots and those didn't work and then he became transfusion dependent and then one day he decided he didn't wasn't going to do that anymore.

**Q6:** How old was he?

**Q9:** You know, when he died, he was in his 80s. So, I mean, he lived quite a long time with it then he just got more and more tired and got more infections, but I just want to make sure my kids know that if the doctor says oh, you're a little anemic, you maybe want to...

**Suman Kambhampati, MD:** Right. Should be tested.

**Q9:** Push that (inaudible 21:12).



**Jean Ridgeway:** And in this day and age you can always go on the Internet and look up familial blood cancers and see where there's a center with academic centers close by to see a very specialized subspecialist in hematology and have it explored.

**Suman Kambhampati, MD:** Thank you very much. I know I don't want to eat up Jean's time because Jean has an outstanding presentation also. So, I appreciate everyone being here. Again, thank you and look forward to any questions. Please send them to me and thank you, Audrey and Deborah for all the support. Appreciate that.

**Jean Ridgeway:** Thank you for coming. Good to see you.

**Suman Kambhampati, MD:** It was great to see you.

**Jean Ridgeway:** If you see (inaudible 21:55) tell him I said hello.

**Suman Kambhampati, MD:** I will. Take care.

(Applause)

**Jean Ridgeway:** Alright. Well, we're going to switch gears and do a lot of information that's very similar. I think there's when as a patient when you hear talks like this this kind of talk is right up my alley. No problem. Very medical. Very academic, but I think in a lot of ways it gives you good information, but perhaps it gives you information at a level that you need a just a little decompressing for and how you own the information and can you kind of sort it out a bit to make it your own. So, that's what we're going to do in the next little bit. So, I just need to check with Dee about timetable. Lunch is coming at?

**Dee Murray:** Eleven forty-five, but we'll have lunch at 12:00 to 1:00.

**Jean Ridgeway:** Very good. How about if we start out with a little housekeeping. There's a restroom directly if you follow this little hallway here right across the way there's a restroom right there. So, feel free to get up at any time and do what you have to do, but I think it might be helpful for all of us if we went around the table and introduced ourselves. It would certainly be helpful to me to understand who you are, why you're here, what brought you here and what you hope to gain from today's presentation. The slides are actually... We'll work with them, but not so much. We'll see.

Anyway, so I'll introduce myself and then we'll start with (Attendee) across the table and we'll go around and so my name is Jean Ridgeway and I'm actually a doctorally prepared nurse practitioner and I work at the University of Chicago. I know Dr. K when he was a fellow. So, he had finished medical school and residency and then he was doing a three year subspecialty training in hematology oncology and I used to be at the University of Illinois. I was there for

over a decade. Many of the doctors I worked with transplanted. It's like business. It's a small community. People move. So, people move to U of C, we all move together. So, I find myself at U of C for 13 years and work in malignant hematology. So, lots with MDS, lots with leukemia and then stem cell transplants. So like Audrey, I can remember back in the '90s when MDS was kind of... it was a questionable diagnosis and the physician scientists at that point in time who were putting their efforts into trying to understand what the disease was about were almost shunned by their colleagues because they felt like it was probably not a real disease and it was not worth studying and so it's been very interesting to see the metamorphosis that's taken place in the last couple decades. So, it's very interesting.

So, what brings me here? I do volunteer work with the Myelodysplastic Foundation. There's a group of nurses, internationally actually and so since you see your doctors, but oftentimes if you're a transfusion patient the person you spend a lot of time with are the nurses and so just like doctors, he got a 10 minute lecture when he was a fellow. Nurses really don't get a lot of focused education about blood disorders and especially Myelodysplastic Syndrome if they don't see it on a regular basis. So, I work at an academic institution. I work at a university. So, what do we see? We see rare things. So, the rare diseases that are out there people oftentimes will come to these academic centers to get an expert opinion, but the majority of people may be seen by their local oncologist and then they hear comments like this, "You're my only patient with MDS," which doesn't exactly make you very confident, but it's reality because it is a rare disorder. So, people come to academic centers for specialized care. How many patients do we see in MDS? We see hundreds of patients a year. We see continuing patients. We see new patients. We see referral patients and if it's care that can be given jointly with the referring oncologist, we certainly do that. There's nothing magic about 5-Azacitidine that can't be done closer to your house. You don't need to drive to university settings to do so. So, but my passion is to really help my colleagues understand the impact... first of all to understand the disease, the treatment options and then how to be a good communicator with patients giving them the right information and helping them make the best treatment decisions and options for them as they go through their journey with the disease. So, I'm one of the nurses who does that trying to help my colleagues and then we come and... we all take turns and we facilitate these meetings. So, that's what I'm doing here, but I'm going to tell you if I wasn't here I'd be taking a really long walk outside because I love fall. So, but I live in Chicago and I'm married. I have four children. My one daughter is a nurse. So, that's kind of fun, but she works with pediatric patients.

So, that's who I am and why don't we go around the table. Tell me who you are, if you're a patient or a caregiver and then what brings you here, what would you like to accomplish and if you weren't here, where would you be?

**Q10:** (Attendee). We live up in Perry, Kansas and we farm and going to be eventually be a caregiver with my wife, (Attendee).

**Jean Ridgeway:** And so you'd be doing... are you actively farming?

**Q10:** Yes. Somewhat. We farm with two of our sons and they do the important work.

**Jean Ridgeway:** That's big work. Good fall. Nice and dry. Crops are in.

**Q11:** Pretty much. Not quite.

**Jean Ridgeway:** Not quite. (Attendee), you're next.

**Q11:** I'm (Attendee) and I was diagnosed with MDS somewhere in the neighborhood of February of 2014 and I had never heard of it before and about sometime earlier this year I said to my nurse, "Well, is there a support group that you know of?" and she says, "Well, not here in Lawrence." I go to Lawrence Memorial and she said, "but maybe Topeka or I could get on the Internet," and I said, "Well, I can get on the Internet" and that's when I found MDS Foundation. So, I'm very grateful for the information they've given me and to be able to be here today to find out if other people get people get cranky when they don't feel good. That's the word he told me to use.

**Jean Ridgeway:** That's good. Where would you be if you weren't here today?

**Q11:** At home.

**Jean Ridgeway:** (Attendee) is next.

**Q12:** (Attendee). I'm (Attendee)'s husband. So... (Laughing)

**Jean Ridgeway:** Currently.

**Q12:** Yeah. You can explain.

**Jean Ridgeway:** And are you the caregiver or...?

**Q12:** Yes. Well, I will be I expect.

**Jean Ridgeway:** Alright and if you weren't here where would you be?

**Q12:** Probably out for a walk.

**Jean Ridgeway:** You'll see us in a few minutes.

**Q13:** So, I'm (Attendee) and I was diagnosed two years ago this last summer. So, 20... close to that. So... and I just went in for a physical and I said to my doctor... and I'm a boring patient.

So... because I don't have anything wrong with me. So, I said... and she said, "Well, anything going on?" and I said, "Well, I'm a little more tired. I'm finding it harder to do my exercise class and everything," and she picked up on that and she said, "Let's look at that a little bit more," and so she did and when they call you back and say, "Doctor would like for you to come in for another blood draw," and then when the doctor calls back and says, "You're going to have see an oncologist on this date and they're going to schedule a bone marrow test and if you don't get it by this date this is my personal phone number and you call me." So... and I had no idea what MDS was, but I knew that dad had had... I mean, he died of anemia and he died fully aware and doing what he wanted to do and not doing what he wanted to do but just being more and more tired, but certainly able to talk to people and visit and tell jokes and that sort of thing. So, it wasn't scary and oh, my gosh this is going to be painful because it wasn't for him.

**Jean Ridgeway:** And are you from the Kansas City area?

**Q13:** I'm from Kansas City and we live on the Plaza. So, this is handy and where would I be if I weren't here. I'd probably be putzing around at home, but looking outside...we just moved into an apartment. So, there are lots of things to do like trying to find things that other people unpacked.

**Jean Ridgeway:** (Attendee)'s next.

**Q14:** Okay. I'm (Attendee). They discovered the... I was anemic and I had low blood doctor at St. Luke's and I had enlarged blood cells, too big, and she followed it and she left and I went to another doctor and a year later... my hemoglobin started dropping, stayed fairly good in the 9s and I had been living with it in the 10s which I didn't know. I paid no attention because I felt good. So, it started dropping and it hit 7.9 or 8.1 and I was just wiped out. So, they did another bone marrow transplant and...

**Jean Ridgeway:** Exam.

**Q14:** Huh?

**Jean Ridgeway:** An exam.

**Q14:** Exam. Took another bone marrow and they compared the two and he says it's mild and they put me on a subcutaneous injection, A-R-A-N-E-S-P.

**Jean Ridgeway:** Aranesp. That's that erythropoietin. It's the red cell growth factor stimulator.

**Q14:** That's what it does. So, and I... and it has gone up. I have to feel better, but it goes up and it goes down.

**Jean Ridgeway:** And when did you say you got diagnosed?

**Q14:** Probably or about six months ago. I'm trying to think.

**Jean Ridgeway:** It's hard to think when you're anemic.

**Q14:** No, I'm not anemic.

**Jean Ridgeway:** It's true. It is.

**Q14:** But I mean I just don't remember because they... he says it's been a year. He says he we need to this over because you're so...

**Jean Ridgeway:** Anemic.

**Q14:** Anemic and I still walked. I still do my exercises. Everybody says keep your exercise up. So... and I noticed that was getting... when it was eight, it was really hard.

**Jean Ridgeway:** Our bodies are wonderfully made in that we can accommodate or we get used to a new normal, a normal hemoglobin for men and women is over 13 grams per deciliter, but as you begin to become slowly anemic, your body has time to adapt. Now if somebody got in a car accident and they had a big bleed and they were profoundly anemic, it would be a whole different situation, but things kind of recalibrate.

**Q14:** I know. I had a bleeding ulcer when they did my...

**Jean Ridgeway:** But there's a point of bad quality of life. You start getting short of breath, you get headache, all that kind of stuff.

**Q14:** I just was tired when I walked. That's when we decided treatment had to start and he says, "But it's very, very minimal," he says, "when you compare what was done in the fall of 2014," or was it 2013 to what they...

**Jean Ridgeway:** Do you have... do you know your subtype? Do you know your diagnosis?

**Q14:** No, no. He says it's minimal.

**Jean Ridgeway:** I have just a small piece of advice for everyone, but if you want to understand what's going on with your disease get your information. So, you own that information. You ask the doctor for a copy of your bone marrow report and then you ask them to help you understand what it says.

**Q14:** Well, I don't have a doctor right now.

**Jean Ridgeway:** Where somewhere in the archives of medical records, you can go to medical records actually and just say this is... I'd like a copy of my medical records and they'll give it to you.

**Q14:** I'm going to get it.

**Jean Ridgeway:** They kind of treat you like a criminal, but it is your information and so you just go on...

**Q14:** So, I can get a second opinion.

**Jean Ridgeway:** If you seek a second opinion, they will ask you most definitely probably for not only the reports from your bone marrows, but they may ask you for the actual slides which are kept in a pathology library at the institution where the exam was done. It's very common for a pathology departments to get a requisition and they say I'm seeking a second opinion and then you just have to have the referral.

**Q14:** I just know they sent the pathology report from St. Luke's to Children's of Mercy because he was amazed. He didn't know why they sent them down there. They took like three weeks to...

**Jean Ridgeway:** It does take a while. It does and there's some very good reasons for that. When the exam is done... So, I'm somebody who... I do the exam. I teach others how to do it and I do it because I saw doctors doing a very bad job and I thought oh there has to be something better and so one of my projects is... that's a side project. So when the exam is done, there's a few components to it. They always look at your blood on the day and they can do that either with a finger stick or if you had a CBC done, but then there's the liquid component and then there's the biopsy and so from those three components, the liquid part of the test, it will be sent off... they make the slides and what does it look like under the microscope, right, and when we look at somebody's pictures we'll see pictures of peripheral blood smears. That just means your blood and then we'll look at the aspirations. That's just the liquid part of the (inaudible 36:59) and they stain them so that the pathologist can look at them and be able to describe and identify what those cells are and they use different stains to give them the opportunity to see that and some cells have distinguishing signatures and that will respond to a certain dye and so they use all different types of dyes and then that's also sent for cytogenetics, the DNA analysis, right, and that test it takes them about 100 hours to actually grow your cells because when they look at your DNA what they need is chromosomes. So, chromosomes are present when the cells are ready to divide. Way back in biology perhaps you learned that before the cell divides into two sister cells the chromatids line up on the middle and those are the DNA and then it splits off and so they have to actually incubate your specimen in a petri dish and encourage those cells to grow, grow, grow and so they do that for a number of days and then they put a fixative in it and then the cells

stop and they're able to then drop them and look at them under the microscope, the DNA. They extracted out. There's a whole big process that they do, but it takes a really long time and then it has to be analyzed. So just like at the bakery you get in line, so there are some other people who've had the test done before you. So, those are being done and so the actual delay in getting the information, yes, it takes time to prepare it, but then the pathologists also have other cases. Now, sometimes they do another test. It's a little more sophisticated and it's called FISH, fluorescent in situ hybridization. That looks at genetic material called RNA, but that test you have to have the specialized probe in order to identify it. So if someone has 5Q-, there's actually a probe that when they put it into the milieu of the cells it highlights and it's a different color and so then they can see it and count it, but all of it takes a long time and then when all the information is ingathered then the hematopathologist reviews your case. So, that's the delay in getting the written reports is because of the process. Usually what practices do is that after the specimens are stained, in our institution we have hematopathology on site. We're an academic institution. Not everybody does that, but we're able to then go downstairs and sit with the teaching pathologist and look through the microscope with them and get a preliminary look, but it's preliminary and they will guardedly tell us that as well, but then those slides are... they're put in a library so that if you were our patient and you came January five years ago and then January three years ago and then next week, they would take all of that information and they pull all of your slides from the libraries and they actually compare one to the other to the other so they can say to you there's not much change and that's how they make those judgements because they actually physically look, but when you go to see another hematopathologist or you're going to see an oncologist for second opinion, you're going to have to obtain that material. I would say give yourself two to three weeks to do so, but it can be done.

**Q14:** Okay. Thank you.

**Jean Ridgeway:** (Attendee)'s next.

**Q15:** I'm (Attendee) and I'm a caregiver.

**Jean Ridgeway:** And where are you from?

**Q15:** We're from St. Louis, Missouri, but we drove here from St. Petersburg, Florida and I wish I was back.

**Jean Ridgeway:** You do? You miss the sun?

**Q15:** It's beautiful weather there. Nice and warm.

**Jean Ridgeway:** It's very warm down there.

**Q15:** Yeah. In the 80s.

**Jean Ridgeway:** In the 80s. It's November.

**Q15:** A little bit warmer than normal.

**Jean Ridgeway:** It's stone crab season opened up last week, right?

**Q15:** Yeah.

**Jean Ridgeway:** See that. I'm a woman of the world and (Attendee) is with you.

**Q16:** I'm (Attendee) with my great caregiver here, (Attendee). We discovered this a disease when I needed a heart operation and the hemoglobin at that point was 9.2 and the surgeon wouldn't operate until it got above 10. So, we had a transfusion at that point and it raises it to 11.something and the operation was successful. About six weeks after the operation, I went in for a bone marrow test and came back with yes, you have this wonderful disease. Well when they told me that, it didn't register very much. It was like, okay, what do we do to cure it and well you go for these shots. They didn't tell me that those shots didn't cure anything. They just helped. They also didn't tell me it was a life ending disease. So, I had to do a whole bunch of research on my own and found out that this is not really something that's a nice thing to have and I feel sorry for everybody here that's got it. We did go to Wash. U for a consult. We now have a doctor at Wash. U who we see once every six months as well as a hematologist in our local hospital. That is very nice. She will give me the appropriate shots anytime I want them as often as I want as many as I want and any degree that I want and I try to keep cutting back on them and she keeps raising them. I started at 20 every two weeks. Now, I'm at 40 every week. I don't feel tired in the afternoon or the evening. My last hemoglobin deal was 8.7 probably because I missed the one in between because I was out hunting in Wyoming and wasn't able to get a shot. Apparently, Procrit needs to be refrigerated.

**Jean Ridgeway:** If you read the box it does say please refrigerate.

**Q16:** So, I couldn't take it with me kind of to do this. At least that's what she said. So when I went back it was 8.7 from a 9.6. So hopefully, I got a shot that day and hopefully I'll get another one Tuesday and that will bring it back up of nine which I'm told is the true guideline that you want. I've had a whole hard time getting any information on it that was newer than 2008 which is all the stuff that these nice people sent me and lots of stuff I got off the computer. It looked like nothing was done since 2008. We got on one about two weeks ago. It was a doctor. The headline was "This Is How I Treat my MS Patients," and above it in the tiniest little print you could ever see this article was written in 2008, but the headline was this big, so I didn't notice that first time and I started reading it and it came up with a whole bunch of the same stuff that they had and he's trying to sell a book for \$30, which I was going to buy until I figured that out.



**Jean Ridgeway:** Did you get the... did you all get the book from Dee?

**Q16:** Please some of this stuff is now more current.

**Dee Murray:** (inaudible 45:32) questions and answers. Yeah. Everyone got one.

**Q16:** So and hopefully that the gal at Wash. U said that they were trying to get a study together that she would like to include me in and that's supposed to happen in February. I haven't heard back from her since.

**Jean Ridgeway:** Anybody here enrolled in a clinical trial? So, when we talk about studies or clinical trials it's relatively complicated on many levels. First, a physician scientist basically has to write the grant and there's a lot of research that's done like in the laboratory before it comes to "the human environment." So, you have to hopefully identify a treatment that could make a difference and that's the kind of studies we're looking for because there's many different types of clinical trials and then for an organization like Wash. U to open up the study, it's very lengthy process. All clinical trials are held responsible to the federal government through the National Cancer Institute and the FDA, and the Food and Drug Administration. So before the documents can even come and centers be offered the opportunity to "participate" in the site, all this other legwork is done. So, it's many years to get these documents and everything validated, etc. to get it to the investigational review board and then if it's a pharmaceutical company then contracts are made with the hospital or university and it really takes a great amount of effort and dedication of the sponsoring institution to offer clinical trials because you need personnel because one of the things that goes on is if there's going to be any effective measurement that drug A is better than drug B is that somebody's got to do the paperwork and there's just reams of paperwork that go along with it. So, there's all these layers of clinical trials that... and the work that needs to be done in order for a clinical trial to be offered. However when a clinical trial is then available, so if your doctor says, "We have a clinical trial for people with this type of MDS." So, there's usually two major sets of information. One of them is called the inclusion criteria. So, you must have everything on the tick box. If the study is looking at Myelodysplastic Syndrome for patients who have had exposure to erythropoietin, you have to have both of those things. If you don't have one or the other then automatically there's this other set of information called the exclusion criteria and if you have only one little box checked off in the exclusion criteria then you're out of the box.

**Q16:** Well apparently, we went through all of that and there were no boxes checked off that would throw me out of the study.

**Jean Ridgeway:** They're still working through the intricacies of trying to get this study open.

**Q15:** She also said the fund was not there, too.

**Jean Ridgeway:** And there's funding of course because... but it takes a long time, but clinical trials are great. I mean some clinical trials are done on very large... on a very large setting and those are called cooperative groups where there are centers all over the country. So, he has a colleague. He actually was in fellowship together, Dr. Verma, who's at Eisenhower and so sometimes if you have a collaborating physician friend it's kind of like well, we're going to open up the study. Would you like to join us? And so it's quite... It really is a huge endeavor on the part of both and so it just takes a long time and sometimes the... with clinical trials they may ask for a whole set of information like different types of blood sets and then sometimes bone marrows are done a little bit more frequently in clinical trials than they would be in standard of care because they really want to look at what's the response. So, like when the drug 5-Azacitidine came to be approved that drug got approved in 2004. So, that study was a cooperative group done across the United States. It was not an international study and the study showed that patients definitely benefited from the drug. I mean, there was an improvement and people went into remission, but when they did that study since the compound was relatively new, patients were... they were randomized like a flip of a coin. You get randomized to drug A, you get the standard of care, but in that clinical trial they had something called a crossover design. So if you got standard of care which was not the drug, after so many weeks you could crossover to the drug. So, that was a crossover design. Those are usually... Statisticians hate crossover designs. I love crossover designs because everybody wants the drug. Right? I mean that's... sometimes the drugs are not good. Sometimes they're detrimental. They cause bad side effects, but in this case... but what they did was when patients began to have a response they could receive the drug for about three to four cycles beyond having a complete response and then they stopped it. So, what one of the learning points from that study has been that patients can continue to benefit and sustain their responses after four or six cycles and so that's why patients are... that's why it's recommended that people stay on treatment as long as they continue to benefit, but that was a very large study. That was like 200 patients which doesn't sound like a lot, but to meet the eligibility criteria and patients wanted to do it and interestingly if anybody in this room is on Azacitidine, patients were given the drug and the reconstitute. So, they gave the shots at home. So, it was kind of interesting because... nobody does that anymore, but they did it back then in '90s.

**Q16:** And I would probably be in St. Petersburg now.

**Jean Ridgeway:** Well, what made you come home?

**Q16:** This thing.

**Jean Ridgeway:** Okay.

**Q15:** Seventeen hours drive.

**Jean Ridgeway:** Oh, lordie. Oh, gosh. How about we'll go with you next.

**Q17:** I have...

**Jean Ridgeway:** Your name is? I can't see your name.

**Q17:** Oh, (Attendee). (Attendee). My name's (Attendee). I was diagnosed with MDS with the 5Q deletion 10 years ago and reading all the information about the scores and longevity of life scared me to death. So, I don't pay attention to that anymore because I lived for 10 years now and I was transfusion... I was getting transfusions every three weeks approximately. The first time I was scared to death of a blood transfusion as well. Had to get down 5.7 and a visit to the emergency room before I said I would have a transfusion. Of course over the period of time, too many transfusions your iron level also increases dramatically. So, I was taking... I got in touch with a doctor... Is it Raza? R-A-Z-A. She was at Massachusetts at the time and she recommended Revlimid and of course my doctor wasn't treating anyone with Revlimid at the time locally here. So, I started on it and was transfusion dependent for only 11 months, but I continued taking it until last December I have been, again, transfusion independent since then and I'll tell you later this strange thing I want to ask you about what happened to me when my transfusion on December 16, but that's my story. It's been... I mean, I've had two hospitalizations and four emergency room events, but mainly it's because of not so much the fatigue, but it also anemia can affect your heart I found and that's why I've had rapid heartbeats or just dizzy, so dizzy you couldn't... and that's what put me in the hospital for studies, but just two days at each time. So, that's my take care... I've taken care of my grandson who's now five. I've taken care of him every Friday since he was born. I have a granddaughter who's going into... going to be a nurse. She's a junior in high school. She's taking all the courses. My grandson just graduated, is going into premed at MU. That's University of Columbia, obviously, and so I've had my ups and downs. I get really, really tired and it does cause pain. They say anemia... one doctor told me anemia doesn't cause pain, but it does cause bone pain that I found, but anyhow I'm still... I don't pay any attention to the longevity of life or the scores anymore and so where I'd rather be today right now is at Columbia where my granddaughter is competing in the state champion... cheerleading championship.

**Jean Ridgeway:** Is it far away?

**Q17:** It's two hours. It's halfway between St. Louis and Kansas City right on I70.

**Jean Ridgeway:** Which I'm sure you guys go that way.

**Q17:** And Independence where I live is right on the way, too. Right close. It's a suburb of Kansas City.

**Jean Ridgeway:** Alright. We'll switch to the back row. (Attendee).

**Q18:** My name's (Attendee). (Attendee) and I, my wife, live in Olathe just 20 some miles south of... I was diagnosed two years ago actually by our family physician. He did the blood work during regular physical. He called me and said, "This is what I think you have and you've got an appointment on date X at a certain time with a hematologist because I think this is what you have," and he was spot on. He said, "I think you have MDS. It may not be, but I think you do." Saw the hematologist at Olathe Med. He scheduled me for a biopsy, bone marrow biopsy. He came back, he said, "This is what you definitely have. I'm going to send you to KU lab with the results of the blood workup and the biopsy." He said, "I'm positive this is what you have, but," he said, "I want another opinion." I've seen three hematologists shortly after here at KE Med. Every one of them said the same thing. A few months later, I decided to get in touch with the VA because MDS is one of the things that they think was caused by the bad water at Camp Lejeune, North Carolina. Well, I was stationed at Camp Lejeune for two years during the period of time when the water was so bad. That's when I saw Dr. K. They assigned me to Dr. K at the VA. He confirmed everything that I had already learned from everybody else and considering my health at the time and my age he said, "I want to do all of the background work for at transplant. So, we're going to run that gamut and see how far it takes us," and Linda even had to go through some tests as my caregiver.

**Q17:** Not physical

**Q18:** No, not physical but... That's one of the things you got to do is find out if they think you can handle the transplant mentally. We went through all the tests here in Kansas City at the VA hospital and by the way the VA treated us wonderfully. Wonderfully. We passed all the tests here in town and we had our choice of three VA transplant centers – San Antonio, Nashville or Seattle. We chose Nashville because we liked Nashville and it's closer...

**Q17:** It was closer to the relatives here in Kansas.

**Q18:** The VA hospital in Nashville is like KU Med. KU Med does a lot of work with the VA hospital here. The VA hospital in Nashville is on the campus of Vanderbilt University. The doctor I saw at the VA hospital also teaches at Vanderbilt and works in their cancer center. How could I find any better expertise than what I found at Olathe, KU, the VA here, the VA in Nashville. Everybody that we saw was just like Dr. K, just full of information, very pleasant to work with. I don't think either one of us consider my diagnosis as a death sentence because it wasn't. It was a notice to get on with your life and keep living which we've done. We travel.

**Jean Ridgeway:** Did you get a transplant?

**Q17:** Not yet. He's on a list.

**Q18:** No. They did an international search for a donor. The best they could come up with was a 9 out of 10 which isn't too bad.

**Jean Ridgeway:** It depends on where the mismatch is.

**Q18:** Yeah. It depends on where the mismatch was. It was recommended that we don't proceed with the transplant at this point because I'm getting along so well with the Vidaza. Been on the Vidaza two years now and my blood work has been very stable every month ever since we started this process. I got the same symptoms that all of you folks have. I'm tired.

**Q17:** We just thought it was old age.

**Q18:** I honestly thought it was old age.

**Jean Ridgeway:** Do you get crabby?

**Q18:** You'd have to ask her. I think I do.

**Q17:** Yeah.

**Q18:** Especially during the treatments.

**Q17:** He's not so crabby, but... I mean, just listen (inaudible 1:01:30) and stuff.

**Q18:** Now that I found that the way to get rid of headaches and upset stomachs a lot during the treatments is I get on the motorcycle and by the time I... Yeah. Like I'm serious. By the time I'm at the end of our short driveway they're gone and it's because my mind's off of it.

**Q19:** Do you mind telling us your age? Is that a secret now?

**Q18:** I'll be 68 next month.

**Q19:** So, you're still within the transplant.

**Q17:** Right now, we're just hoping that the Vidaza keeps on working. When he started out, he was doing the five days, skip the weekend and do two days and then after that Dr. Korman (sp? 1:02:10), our hematologists said if you want to try to the five days then that's also... He's been doing that for quite a while. The last time we went to the doctor which we go every month he said some people choose maybe... because he goes every month and he said some choose to go six weeks or eight weeks. We said no.

**Q18:** It's working.

**Q17:** As long as it's working, we're not messing with it. So, we're just kind of hoping before the Vidaza quits working for him that maybe something else will come along.

**Jean Ridgeway:** Many times they can rerun that search. They do rerun the search because new people enter the registry on a daily basis.

**Q17:** The doctor, the VA doctor in Nashville, he said as good as health as he is in other than the myelodysplasia he didn't want to go ahead with the transplant because it's, you know, I mean it really drags you down. I mean, it's...

**Jean Ridgeway:** It has its own set of complications.

**Q17:** It has a whole set of things, so he said if something quits working then we're going to continue. (inaudible 1:03:14) I'm the caregiver so I get to talk, too.

**Jean Ridgeway:** You passed the test.

**Q19:** I might add I have no caretaker... caregiver. I'm taking care of my own self.