

**MDS Foundation Educational Patient & Family Forum**  
**UF Health Shands Cancer Hospital**  
**Saturday, February 25, 2017**

Mr. Alderman: Good afternoon, everybody. We are going to go ahead and get started with the discussion session today. Did you get a chance to go over the brief question sheet that we passed out during lunch, about 10 or 12 questions? Get a chance to look over it? Get their thoughts together?

I would like to start with an introduction. My name is Damien Alderman. I am a research coordinator here at the University of Florida. I work with the Department of Health Outcomes and Policy

Ms. Crawford: Hi. My name is Quintina Crawford. I am one of the citizen scientists with the program with the Clinical Translational Science Institute. Pretty much I am on the board to be the laymen's voice for the community so I am pretty much in community engagement, is what I do there.

Mr. Alderman: And that's actually the focus of this session right here. We need to get more community engagement involved in clinical trials. The objective here, like Dr. Cogle said before, was to educate him about things that matter when it comes to research. That's why it's important to have these discussions. As you can see, there is about 10 or 12 questions, and what we hope to gain from this are your insights on the answer to these questions. I like to always start and say that there is no right or wrong answers. All opinions matter, and please take the time to share and let us know what your thoughts are.

So we want to start with the very first question about how long have you had or someone that you know and love has had MDS. Anyone? Raise your hand.

Audience member: 10 years.

Mr. Alderman: 10 years.

Audience member: 16.

Mr. Alderman: 16?

Audience member: 6 years.

Mr. Alderman: Oh, 6 years.

Audience member: 20.

Mr. Alderman: 20.

Audience member: 2.

Audience member 1.

Mr. Alderman: Any others?

Audience member: 2003.

Mr. Alderman: Since 2003 and then in 2005?

Audience member: 2001.

Mr. Alderman: A wide variety of ranges.

Audience member: Eight months.

Mr. Alderman: Eight months. So it ranges from – so I am hearing it ranges from eight months to 13 – was it 13 –

Audience member: 20 years.

Mr. Alderman: 20 years, wow. And that's just a general flow of how it's going to go this afternoon. I would just like to have a question and answer it if I can. If I miss you, please it's important that you stop me because we – like I said before, we want to get everyone's voice heard. So we are going to go ahead and move along. We're going to start with the introduction we just did. Our next topic is going to be about understanding MDS, and then we're going to move on to genetics and gene mutation reports, and then we're going to move to insurance and payment, technology and treatment, and last – and one of the more important topics, the future of research and outcomes that matter to you guys. So first and foremost, let's start with understanding MDS.

Ms. Crawford: Ok. The next question would be, where would you say you get the most information about your MDS, whether that's on the internet, for example, but where do you get your information.

Audience member: The internet

Audience member: MDS Foundation.

Mr. Alderman: MDS Foundation, and you said the internet?

Audience member: The foundation.

Ms. Crawford: Okay. We are going to use the one mic so one person can speak and then the next person. We will be able to hear everyone. So, Thomas, go ahead.

Audience member: Internet.

Ms. Crawford: Okay.

Mr. Alderman: Are there any particular sites on the internet?

Audience member: I look up a lot of clinical trials and things like that.

Mr. Alderman: Have you found any ones that are particularly interesting?

Audience member: Nothing that jumps out, no. The other thing that's informative is I found a good chat board or support group for strictly with my type of MDS.

Mr. Alderman: That's also – in a lot of cases, online resources are very helpful, and support groups are one of the top lines of help. Anyone else? Yes, ma'am.

Audience member: I have learned a great deal from watching the webinars that AAMDS puts on their website. You can actually pick whether it's aplastic anemia MDS as relates to all of us or PNH.

Mr. Alderman: That's kind of amazing.

Audience member: They have experts from all over the country just giving information.

Mr. Alderman: Do you know how often they host those webinars?

Audience member: I think they are quite frequently. I mean, like every couple of months. And then what they do is you can do it live if you happen to be home and want to sit there for a couple of hours. I find that rather difficult. So they archive these things and then you can go back on to their website – just go into MDS and look at whatever topics they have there and look at them.

Mr. Alderman: What was that acronym? AA –

Audience member: AAMDS.

Mr. Alderman: AAMDS.

Audience member: And they just included PNH with their group now, the PNH combination also. So I think they are going to add something on the internet.

Mr. Alderman: Does anyone else have online –

Audience member: I have gone on Amazon and purchased about four textbooks on blood disorders in general and a couple on MDS. They are kind of pricey, that's one thing, and that's a disadvantage and they are – they are pretty high level, but if you read them repeatedly – I think most of my books I have read probably at least 10 or 15 times and every time I pick up something new, and I have asked some of the staff at Shands to explain something, and they are always happy to explain it. So even though it is high level, you will pick up a lot out of the textbooks. To me, what I have learned was well worth what I paid for them. Some of them are – you know, I haven't paid over a hundred dollars for any of the books. Now, that's not saying that there aren't any out there that are over a hundred dollars, but the ones I have bought I tried to keep under a hundred dollars and I have learned a lot out of them. A doctor saw me reading one and he says there's going to be a quiz on that Friday. He understands I'm – you know, and he's glad to explain anything in there that I don't happen to understand.

Mr. Alderman: Going along the lines of textbooks, do you have a particular recommendation?

Audience member: There's one, and I can't think of the exact title, but it's a manual for stem cell transplantation. If you google or if you go on Amazon, look up manual for stem cell

transplantation, it goes through, you know, the pre-treat – the pre-treatment care, the actual transplantation, what to expect in acute TBHD and chronic GBHD. It is very, very comprehensive, and it was one of the more really expensive ones.

Mr. Alderman: Sometimes doing your own personal research does pay off, and it seems like it did in that case for sure.

Audience member: And I'm retired so I've got all the time in the world.

Mr. Alderman: Anyone else have any resources they would like to share?

Audience member: If anybody has any of the blogs or forms that they like, could you –

Ms. Crawford: The resources?

Audience member: A lot of the ones that I have gone to, they're just so removed from what I'm experiencing. I can't seem to get a grasp on –

Ms. Crawford: Some of the language into it?

Audience member: Yeah.

Mr. Alderman: Does anyone have any other blogs or resources?

Audience member: Mail forms.

Audience member: There's been some mail. That's the one I felt like I – they were using a lot of abbreviations and I just couldn't understand what they were trying to say. The abbreviations, like DX, I understand what that is but some of the things like they've been doing, it's all RBT and I'm lost. I wish there was a glossary.

Audience member: Or kind of a note that's like --

Audience member: I don't mean to interrupt but in your packets today is a glossary. I hope you use it. It's very user friendly. I hope that helps.

Mr. Alderman: It might help.

Audience member: Some of that, I'm finding, is in the generational texting with shortening everything.

Audience member: It could be something as simple as –

Mr. Alderman: If you had never seen it in shorthand, good luck deciphering it. Any other resources? (No response)

Mr. Alderman: Next we would like to know – sorry, did someone – how would you explain what gene mutation means with respect to your disease? It's a tough question.

Audience member: Maybe the doctor –

Mr. Alderman: Oh, no, this is our chance to educate him. No calling on him for help. It is a really tough question to answer but basically how well do you feel you understand what gene mutation is.

Audience member: I don't know.

Mr. Alderman: Well, especially if you're saying your situation is new, it's a heavy concept to wrap your head around.

Audience member: You know, again the internet, the more I've gone into it, it is; it's very confusing but what comes out of it is that is the direction where they're finally going to come up with something that can help people permanently besides a bone marrow transplant. The more you read on it the more smarter you get about it and understand it. It's like reading the books, you have to listen to it a gazillion times before it all sinks in.

Audience member: Yeah, true.

Mr. Alderman: Sometimes repetition is the only way to mastery.

Audience member: We heard before from Dr. Cogle's Icare study, you know, if you're involved in that clinical trial, if you get a report from that, it will tell you what gene mutations you have. It doesn't specifically tell you what gene that does, but then you can take those genes that you've got mutated and go research them on the internet and you can probably learn more than you wanted to know.

Mr. Alderman: Well, actually, is a great segue into the next question I had is how well do you trust that your doctor knows all the gene mutations involved?

Ms. Crawford: Go ahead. You were saying something.

Audience member: Our doctor tells us that he doesn't know enough to go back to (inaudible).

Audience member: I feel confident because, again, I am being treated at Moffitt and my doctor is the head of the whole place.

Mr. Alderman: And so that's actually – in preparation for this, Dr. Cogle made an interesting fact, is that there are – and correct me if I get wrong – but between 200 and 1000 different gene mutations, so that's a lot. That's a lot to wrap your head around.

The next segue I would like to go to – the next question is about how much time do you think that your doctor spends looking up specifically your gene mutation. Do you feel like it's enough time? All around yeses, nods.

Audience member: I don't think they have enough time to do that. You know, if they've got like more than, you know, 20 patients, they don't have enough time to spend looking up every mutation. It's unfortunate but it's a fact of life. They've got a limited amount of time. They've got limited hours in their day just like we do.

Mr. Alderman: Time is a commodity that we all desperately need more of when it comes to things, and researching up to a thousand different mutations, it's a large task to ask.

Ms. Crawford: Another question to think about that is if anyone of you have had your report with your doctor discussed and how was that experience for you. Has anyone had that experience in their doctor's office or with their physician?

Audience member: You mean going over the gene?

Ms. Crawford: Yes.

Audience member: Yes, I have had that.

Ms. Crawford: Yes.

Audience member: Yes, I got a full report and sat down with him.

Ms. Crawford: Okay.

Mr. Alderman: So what are your thoughts then about genetic testing? Great? Positive things?

Audience member: Promising.

Mr. Alderman: Promising.

Audience member: Yes.

Audience member: I haven't had it yet, but particularly after listening to Dr. Cogle today, I know it's forthcoming before too long and now I'm even more interested in having it done.

Mr. Alderman: That is a push for the future. There is a lot more people looking to get their genes mapped out sort of.

Audience member: How is that done? Is that just with a blood test?

Mr. Alderman: Oh, that's a question for Dr. Cogle. Oh, he gives the thumbs up. It looks like it's a blood test. So I guess for those who have not had their gene mutation mapped, do you have any concerns or any thoughts about that or anything preventing you in particular?

Audience member: We're kind of back to that glossary thing. There is just so much technical and scientific data there that for the lay person, that's a whole other section of research.

Mr. Alderman: It really is. And, again, getting back to how huge of a concept it is to kind of wrap your head around, getting your full gene mutation, it's a lot to try to understand especially like you were saying without assistance, like you would need that, that extra glossary.

Audience member: I haven't had it done as I just mentioned but listening to the folks here, I wonder if that shouldn't be a suggestion then, that there be some more patient friendly language or an explanation about all of that, you know, in the report that's handed out or at least in the portion that's given to the patient.

Ms. Crawford: Right. Any other suggestions?

Audience member: Just a comment. I think there is a distinction between just having your genes even – your gene mutations tested in Dr. Cogle's iCare study because he goes through just a

massive amount of gene mutations and they detect for those, where I went to Moffitt and they look for very specific ones, one of them being AX01, and they found that one and several other ones. But they don't go through this whole entire spectrum like Dr. Cogle does, and I think it's a matter of cost, you know, to do what Dr. Cogle is doing in his iCare study would be cost prohibitive for every center to be doing. I wish Dr. Cogle was here to either cut (laughter). Is that not true that you go through the whole spectrum where Moffitt may go through a subset that's known to be problematic?

Dr. Cogle: That's true. The cost is plummeting and it will soon be inexpensive so that's why we were asking the questions that we are minus the cost because there will come a point where the physician is going to have all of this genetic information and we want to hear from you on how you would like that delivered to you.

Audience member: One of my experiences, because I have been dealing with treating cancer since 2007, if certain doctors have a certain regimen of meds that they like to do and they stick to those regimens until you start having complications and then they start focusing on maybe looking at other areas after they have exhausted this certain limit, which I would like to see more and more doctors thinking on a broader scale like from what I am hearing you do instead of just sticking to a normal regimen, as I call it, the rut syndrome. So that's my experience.

Audience member: Protocols can be good and restrictive.

Mr. Alderman: Yes, that's very accurate.

Audience member: A full gene study, does Medicare cover that?

Dr. Cogle: That's a complex question because the full gene study that we are doing here at the university, I have cobbled together various sources to pay for that so that it's essentially free to the patients, and then, for example, I have gotten DNA companies who want to test out their product to commit to giving them to patients for free. So it's basically – it's a lot about business deals as it is about science. I will tell you that Medicare does pay is 80% of a gene profile. 20% has to be covered by either your secondary insurance plan or out of pocket.

Audience member: Yes, because – going back to Moffitt, yeah, they didn't do a complete – in fact, in mine they did 53 mutations.

Mr. Alderman: Do you have a comment? Any other questions or concerns you want to voice about getting your gene mutation report?

Audience member: Maybe on the heels of that, another thing that would be helpful is, to the extent that it's possible, sharing with patients what information is known about how it can be paid for depending on where you are with the insurance, whether you are Medicare eligible or not or what. I know that can't be across the board done for everybody but just to give them some sort of a general idea and maybe then it would help them know who to go talk to, who to go ask or whatever.

Ms. Crawford: Right. Okay.

Audience member: This maybe a question for the doctor. When you get a bone marrow biopsy, they check to see, you know, where you stand and what you've got, how many genes do they check? I mean, I didn't even know anything about the gene thing until I came here and I say gosh, that's something I didn't even know about. Do they stop when they find out or do they check for everything when they do the bone marrow study?

Dr. Cogle: When they do a bone marrow biopsy, there are about 50 tests they can send it for, and each physician sends for their own group of tests. It's hard to know unless we look at each patient what their test was sent for. But for genetic testing, it turns out that we can catch the gene mutations in the blood. We don't have to do the bone marrow. If that doesn't answer your question, afterwards I would be happy to answer more of it. If people can let us know, approximately how much time has your doctor spent with you describing the genetic mutations in your chromosomes.

Mr. Alderman: That's an excellent question.

Audience member: I didn't even know it existed until we started talking here.

Mr. Alderman: So the question was about how much time does your doctor spend sort of going over explaining the genetic report with you. All around, not enough time, zeros and nothing?

Audience member: Very little.

Mr. Alderman: Very little, not enough.

Dr. Cogle: If you could ask, what does not enough mean.

Mr. Alderman: What exactly does not enough mean?

Audience member: 5, 10.

Audience member: Yeah, 5 to 10 minutes. I mean, you only get like 15 minutes with the guy and a lot of it is just, you know, chitchatting back and forth. As far as getting into all these numbers and stuff like that, very little.

Audience member: At one time I thought I had a mutation, but I found out my next biopsy that seemed to have disappeared. So I don't know if somebody else had the report and they just didn't pick up on that. I don't know where that stands.

Mr. Alderman: Yes, it seems that for a report of that size you probably would need a little bit more than the 5 to 10 minute time.

Dr. Cogle: Would anybody be open to having like a PhD or molecular oncologist meet with you at the same time you are in clinic to describe the gene mutations?

Audience member: Absolutely.

Audience member: Absolutely.



Mr. Alderman: So again the question was would you be interested in having a PhD or you said a molecular oncologist in the clinic to go over the gene mutation report with you.

Audience members: Yes

Mr. Alderman: Yeses?

Audience members: Yes

Mr. Alderman: That's good to know.

Ms. Crawford: Can you hear back there? I just wanted to make sure.

Mr. Alderman: I'm sorry. Can everyone hear me ok? I have a tendency to talk pretty low when I start talking.

I guess next, for the people who have seen the gene mutation report, we kind of discussed this briefly, but it seems like there are plenty of parts that are really stuff to understand. What were your initial thoughts going through as you were reading it?

Audience member: Just the sheer quantity of gene mutations was scary.

Mr. Alderman: Just the sheer size of the report probably, like you were saying, was intimidating a bit.

Audience member: I was wanting to know more than – they were just focusing on 2 of them. They didn't want to discuss all 5 of them, and I wanted to know the facts on all of it. So that was my thing. I don't want to just know okay, the 2 things that are causing my MDS. I wanted to know what the other genetic chromosome deformities are too, you know, and whether or not they will cause me problems in the future.

Mr. Alderman: So it all comes back again to that issue of time and there never being enough?

Audience member: Yeah, maybe a grade or a sign, a level of severity to each anomaly or, you know, issue, and, you know, that could alleviate some concern. You know, a 5 Q deletion versus, you know, something that makes your toenails a different color, you know, who cares.

Mr. Alderman: And that makes a lot of sense because it's – if that was the case, it would be really easier to say okay, this has a high level of priority, low level; this is like a C grade concern; this is an A level concern. We should really look into this. That makes a lot of sense. You can take that report that is initially intimidating and make it really easy to see which parts you need to really focus and pare down on. That's a great suggestion.

I want to go to the next topic, how would you like to receive the information about gene mutations? Directly in a doctor's office, email, phone call. How would you best like to receive that information?

Audience member: Something written.

Mr. Alderman: Something written?

Audience member: I think a combination. The doctor talking to you and here is the written report.

Audience member: Here is your report presented by the doctor. You know, here it is. Now read it and then ask questions next time we see each other.

Audience member: You can't do it right then because you go home and you read the report and then you've got all kinds of questions you didn't know to even ask .

Audience member: Or they didn't discuss things that were in your report.

Mr. Alderman: All right.

Audience member: I like to see the report 2 weeks before I see the doctor. Even my biopsies, I want to read my biopsies before I see him.

Mr. Alderman: So getting the report and then follow-up in a relative short period of time just to address concerns only about the report. Does that sounds like a pretty good –

Audience member: Yes.

Mr. Alderman: Some pretty good suggestions. Another important question about the gene mutation report, let's say your doctor finds something that's unrelated to your cancer; he finds information about Parkinson's Disease or maybe breast cancer. Would you be interested in having your doctor tell you that information?

Audience members: Yes.

Mr. Alderman: Absolutely yeses?

Audience members: Yes.

Audience members: My personal feeling about that is that should not be a question that is across the board. That should be asked –

Mr. Alderman: On an individual basis?

Audience member: Yes, because I know a lot of folks who would rather not know that.

Mr. Alderman: Okay.

Audience member: If it's related to something else, especially dependent on what it is.

Mr. Alderman: So it seems like individually, if as that is being done, they should ask that person right then and there before the test is even done perhaps, say if we find anything else, would you like us to let you know.

Audience member: Yes.

Mr. Alderman: And how would you like your doctor to sort of relay that information to you? Probably just again face to face, in person like that?

Audience member: Yes.

Mr. Alderman: All right. Sounds great. Next we are going to move to a different topic. We just heard that genetic testing can be relatively expensive. I think we found out that Medicare or Medicaid pays for 80%. How much would you be willing to pay out of pocket for the remaining 20? I guess how much would you be willing to?

Audience member: Well, I guess it all depends but \$10,000. I would have to see but probably \$10,000.

Audience member: Or less. Billing was \$28,000.

Mr. Alderman: 28 was your last billing?

Audience member: Yes. That's what they billed. That's not what they got.

Audience member: That was out of pocket?

Audience member: Well, that's what Moffitt billed Blue Cross.

Mr. Alderman: Okay.

Audience member: So they probably got six or eight.

Mr. Alderman: Okay. Thank you. So the next topic is going to be more about the technology involved in treatment. How do you feel about your doctor using a computer program to sort of design a treatment plan?

Audience member: It seems sort of what we do now.

Mr. Alderman: It's what you do now.

Ms. Crawford: Can you elaborate more on that?

Audience member: Well, I'm not the person that's at the doctor's office; but doing what I do, everything is guided by that.

Mr. Alderman: Sort of putting in, I guess, symptoms and inputs and just seeing what the computer spits back out?

Audience member: At least give a guideline for what the doctor needs to input. I mean, if you depended on your doctor to know everything, you're going to have a problem, I would think. They have got to have some guidelines. And the fellow over there who had four books that were over a hundred dollars, the docs can't know every single word in every single book. So anything that can help would be a good thing.

Mr. Alderman: Anyone else?

Ms. Crawford: I think you had something to say, ma'am?

Audience member: I think as long as it is in conjunction with your doctor's knowledge, and I think there is – I agree, you know, there is just so much information out there, as long as it is programmed properly. That's another issue.

Audience member: I think as medicine evolves, and it has drastically in just the last five or ten years, really what computers are now doing is data mining and data collection, which helps, you know, in the research and the documentation of diseases as long as it doesn't eliminate completely a personal and hands on experience. I mean, we've had some very bad experiences with computer medicine in the hospital because the protocols were so rigid that they didn't allow for – if you don't put it in the computer, the computer doesn't give you the result, and there were some near disastrous results because they were not following the protocols of using the computer and there were some drug interactions that were near life threatening.

Ms. Crawford: So that poses the next question: How safe do you feel that it would be accurate for you if –

Audience member: If they had gone to the computer first and pulled it up, I would not have the issues that I have. But because they reacted instead of researching, I had issues.

Audience member: It came down to the internist in the ICU really didn't know that much about chemotherapy and actually gave drugs that were severe contraindicated with her drugs.

Ms. Crawford: Right. So the data being pretty much accurate to the patient –

Audience member: Yes. But, again, that's maybe a little off from what the original discussion was about but computers are great but they're still has to be – you know, I was in medicine 25 years ago, and it was all hands on. We didn't have computers back then to – you know, to diagnose and tell us what to do.

Ms. Crawford: Right.

Audience member: I think they are very useful but there has to be –

Ms. Crawford: A tool.

Audience member: Yes, it's a tool. It's another tool in the toolbox.

Ms. Crawford: Right.

Audience member: Just don't become so reliant on it that you forget how to touch a patient.

Ms. Crawford: Yes.

Audience member: I was just going to follow up and say that it should just be a tool for the doctor who uses that but then there's got to be a lot of common sense from the doctor based on his experience. If a doctor just takes something off a computer, I would go find another doctor.

Audience member: There's a very old saying with computers – and I'm probably showing my age here – it's garbage in, garbage out. If you don't program the computer right, you can put all the data in the world and it will spit out – you know, if the doctor has got faith in the program

and it has been validated, you know, I'm all for it, but there's got to be a common sense factor in there with the doctor looking at it and saying, "this doesn't make sense." There's still got to be a human element in there right now. Some day we may get to the point where the doctor, you know, the doctor spits out, you know, call time of death and they do it, but right now I think you still need a doctor using the device saying, "no, this doesn't make sense."

Mr. Alderman: You bring up a really good point about, I guess, validation, about – I guess, what would make you feel that this computer or algorithm isn't properly validated? I guess how many doctors would it have had to have gone through. To you, what does that validation mean?

Audience member: Is the ultimate outcome better than the alternative, you know, what measurability.

Mr. Alderman: Any comments? Questions?

So one thing that was definitely a comment in everyone talking about this, there still has to be a human component to all of this. No one is comfortable with just letting a computer sort of spit out a diagnosis or I guess a prescription on its own without at least having been seen by your doctor just to confirm and make sure everything is right.

Audience member: One of the good reasons for having a human component is last year in California where the computers were high jacked and nobody was able to access their patient charts. That's why you need to have a human component and knowing the history because we don't know – there's too many hackers out there.

Audience member: Well, it's kind of scary how much the health care sector is affected by malware that the public isn't really made aware of because it's scary, down to high jacking the IV pumps.

Ms, Crawford: So that's a concern.

Mr. Alderman: On the flip side of that, is there anywhere else you would like to see technology contribute in further? No where in particular?

Audience member: More accurate survival charts. (Laughter)

Mr. Alderman: So next we want to move on to a little bit more about research. How would you feel – what do you think personally about a meeting where a group of doctors and scientists would discuss your case? How do you feel about that concept or that meeting?

Audience member: You mean do I want a group of doctors and scientists to get together and discuss my case?

Mr. Alderman: I dream big.

Audience member: You're doing good to get a few minutes with a doctor much less –

Audience member: He said would you like it.

Audience member: The next question is how much do you want to pay for it.

Audience member: I think anytime you get a group of any higher knowledge people together to discuss your diagnosis, you're going to benefit in the long run because they are going to be brainstorming possible new treatments, and any time we can get new treatments out there, the better we're going to be.

Audience member: I think that's the difference between a teaching facility and your every day general practice doctor, even if he's, you know, specializing in hematology, if he's in an office setting versus a clinical or teaching, you know, that's why Shands is a little bit different than –

Ms. Crawford: Than other facilities.

Audience member: Yes.

Audience member: I would like to see something like that, like an annual review that way, you know, once a year.

Ms. Crawford: Like a progress report?

Audience member: Yes.

Ms. Crawford: Okay.

Mr. Alderman: I guess alongside the next question -- are there any other comments? Any other thoughts about such a meeting? (no response).

Well, my next question is how would you personally feel about attending that meeting? Would you like to be there for that discussion?

Audience member: Absolutely.

Mr. Alderman: Absolutely. Yeses all around.

Is there anyone in particular you would like to take with you to that meeting? Like can you think of – I see people reaching over and looking to their friends.

Audience member: I would hopefully like my doctor to be there.

Mr. Alderman: Well, hopefully your doctor would be at that meeting.

Audience member: Well, I don't know because he's in Fort Myers and we're here.

Ms. Crawford: So distance – so that's another disparity. Okay.

Mr. Alderman: So in a meeting like that, would you feel comfortable, let's say, having your doctor appear by webinar or Skype or with technology like that?

Audience member: Sure.

Mr. Alderman: Okay.

Audience member: My doctor I have right now, I would feel comfortable but he is closing his clinic as of April so now I have to look for another doctor, so now that's the other problem. So,

yes, I would like to have any doctors that would be available to go – you know, from Fort Myers to discuss my treatments rather than me having to do all the research.

Audience member: The spouse, I would like to have my spouse in that meeting with me and doctors and no lawyers. (laughter)

Ms. Crawford: Go ahead ma'am .

Audience member: I haven't thought about this until you just brought it up, but I'm sure a lot of us in this room see other physicians and I mean – primary care, but maybe others too. Now that I think about it, there's very often – something relates to the MDS that comes up as part of your other care, and the point is they may suggest something that you know shouldn't happen because you know something else about – you know, and a lot of other doctors are not real knowledgeable – they're beginning to know MDS, I'm finding. I also have PNH and boy, you can say that word out there and very few people know what that means. And so if they start suggesting something – anyway, where I am going with that is it might be interesting, whether it's by Skype or otherwise, to have the folks, you know, that you're seeing for other purposes involved in that. I know Dr. Cogle had told me ten years ago to be real careful from now on with limiting my sunshine and burning – and just all that kind of thing, and I started seeing a dermatologist, and to her credit, she immediately went and researched this herself and found out that long-term use of cyclosporine can – because of the suppression aspects of that can cause skin cancer to a much greater degree than otherwise. But, you know, if I hadn't said something or if she hadn't taken it on her own to do that – that's just an example of stuff that just happens out there, you know. It would be nice if they could all kind of get a feeling for what it – how it relates to what they are treating you for.

Ms. Crawford: So what I'm hearing is basically kind of like the doctors being educated about other things that are going on with you.

Audience member: Yes. And if it can't be done specifically with the patient, maybe there still needs to be just more education of other doctors about our group's illnesses.

Ms. Crawford: And how it connects, right?

Audience member: Right.

Ms. Crawford: Okay.

Mr. Alderman: It seems to me you would have to be sort of like a primary care network would need to be in attendance – need a little bit more cross talk for everyone involved to learn more about all the different aspects of everything else.

Anyone else you can think of who you would want to attend the meeting? I like the idea of having more than just the physician – having your entire network of physicians sounds like a very smart idea.

Anyone else? Any other suggestions?

So next I want to ask which questions in regards to MDS do you think researchers need to focus on? You heard about a couple clinical trials that are being conducted, or are being applied for, grants. Are there any questions that you would like to have answered or any trials that you would like to see researchers conduct?

Audience member: I received a phone call probably from the MDS Foundation that the drug (inaudible) was going up for approval. I'm recommending to you, if any of you ever get in that situation, do what I did. I said, "I'll be on the next airplane," because I wanted to talk to the panel of doctors before they start approving or disapproving the drug. There was 15 of us that actually got up and spoke to this panel. I highly recommend that. Personally I feel like it made a difference. If the doctors are just sitting there and nobody explaining what their condition is and why you should approve or not approve the drug.

Ms. Crawford: Okay. So being at the front table when these medicines are being approved is pretty much what he is saying. And to having your voice heard there.

Audience member: Yes.

Ms. Crawford: Okay. Anybody else?

Audience member: Is there work being done on a cure or just to treat?

Dr. Cogle: We do intend – a lot of the research is geared towards a cure.

Audience member: Okay.

Dr. Cogle: But it's helpful to hear that from you because there's no – we only have a certain amount of money and a certain amount of time. Do you focus on reducing transfusions initially or do you go for a cure which actually make transfusions worse. So as a physician and researcher, you've got to make choices and it's helpful to hear from you, the patients, about where the priorities are. Hearing that you want a cure, it's helpful to us too.

Ms. Crawford: I think you had something to say.

Audience member: The point Dr. Cogle just made about priorities comes into what I'm going to say and maybe I'm the only one that feels this way, and this would not be what I suggest to get top priority obviously. But it's still of great interest to me why, where it came from. I mean, I've heard what we know at the time. I had something happen just a few months ago that has caused me to wonder if they are indeed finding some other things that might be underlying MDS, and that has not been proven or whatever yet. And, as I say, that's not a top priority thing. Obviously curing and helping our illness is. But for me personally, it's just one of those things, why. I mean so many illnesses you know where they come from, and we don't really know what – where this came from.

Ms. Crawford: So the origin of –

Audience member: Yes.

Ms. Crawford: Okay. Where it comes from.



Audience member: Peggy's MDS is secondary. It's because of years of other treatment with chemo and radiation, so it would be interesting to see if there's any research to determine what drugs are more likely to cause it, and then is there any way to build – I can't believe I'm about to say this – another drug to help prevent that or some way to figure out how to stop secondary MDS from occurring.

Ms. Crawford: That's a great question. Anybody else on that?

Mr. Alderman: A lot of researchers, from personal experience is why we ask this question, partly why the citizen scientist program was started in the first place – a lot of researchers that I've worked with a lot in the past – like Dr. Cogle was saying, they have a limited amount of funding and they do research that they feel is important to the community. A lot of it was done before even talking to the community about it. So part of the reason why we designed this program was so that we could actually talk to the communities and see what research questions they – like what matters to them so we can better design research to answer their questions. That's what the intent was in the first place. So that's why we really hope you take the time to think about the answers to these questions, like what research questions matter to you so that we can hopefully get things geared in answer to questions that are actually pertinent to you and your situation.

Any other research ideas from you guys or any other topics that would matter to you? Research questions that you want to see answered. (no response)

So along those lines, what life outcomes are most important to you, things such as survival time, disease relapse, quality of life, and anything else we didn't mention, which of these outcomes are the most important to you?

Audience member: Survival.

Mr. Alderman: Survival.

Audience member: Survival and quality.

Mr. Alderman: Survival and quality of life.

Audience member: Yes, both.

Mr. Alderman: Both. All around survival and quality of life?

Audience members: Yes.

Audience members: Quality of life.

Mr. Alderman: And what exactly does quality of life mean to you?

Audience member: As close to be free of MDS as possible.

Audience member: Yes, not feeling like being hooked up to machines and being able to get out and go walking, being able to fly if you wanted to travel.

Mr. Alderman: So freedom from machines, not being hooked up, being able to move around on your own accord.

Audience member: Yes, I don't want to be affected like a lot of patients who have cancer in the end are hooked up to machines, and that's not a good quality of life. I don't want that. I want to be able to function on my own, have independence.

Mr. Alderman: Having independence.

Audience member: Yes.

Mr. Alderman: Any other takers? What does quality of life mean to you?

Audience member: Not being so tired all the time and you are able to do what you want to do frankly.

Audience member: Yes, as close to normal as you can get.

Mr. Alderman: Well, you know, that's the next question I was going to ask is, how do you define normal.

Audience member: To do the things that are normal for you before.

Audience member: Yes, normal function.

Mr. Alderman: So to be able to continue with the hobbies that you had before?

Audience member: Yes.

Mr. Alderman: I understand. Any other takers?

Dr. Cogle: Can you ask a question. In defining quality, how about being able to work again?  
(Laughter)

Mr. Alderman: I think we can scratch that question.

Audience member: Wrong age group.

Mr. Alderman: It is important to get an accurate description on how you feel about quality of life, what that term is, because to different people it means a different variety of things, even just from the answers you have heard here. So thank you for answering that. Anyone –

Audience member: One other thing on quality. To me, so many things remind me of quality of life. One of the things that stick out in my head is the children, grandchildren, and great grandchildren, to be able to spend that time with them. It's frustrating not to be able to go out there and play baseball or kick a soccer ball around. You can't do those things anymore. I try to keep a positive attitude going forward – I try to keep that up – but it is sometimes hard not to think about it. You just don't have the energy to do it.

Ms. Crawford: You had something you wanted to say?

Audience member: One other thing about quality of life, I would say is not being dependent on drugs. I can attest to that. I despise it. I mean, I will live with something, have a headache or something and take Tylenol before I take anything for the headache. I just despise the meds. That's a personal problem with me that I think for me a good quality of life would be not being tired and not being dependent on machines or drugs.

Ms. Crawford: Okay. Dependency. Anybody else?

Mr. Alderman: And one of the last major questions we had then, what can researchers now and future researchers do to make you want to, I guess, better participate in clinical trials? Like is there anything in particular that we could do shaping research in the future to help or convince more people to join in these clinical trials?

Audience member: Give more feedback to the person that is participating. If I'm going to participate in a trial, I mean, I may sound selfish, but I want something out of it too. I want to know what my input, how it – you know, I want to know how my bone marrow looks. You know, I think giving more feedback to the individual participants who is doing the clinical trial would encourage me more personally.

Ms. Crawford: Right. Did you have something to say? No, okay.

Audience member: No, I was agreeing with him.

Ms. Crawford: Okay.

Audience member: We're about to start a trial. We're just – I don't know how you get around this, but one of the values is that you don't know if you're getting the real drug or not. So we're going through all this testing and everything, and she maybe getting a placebo or something. She might not be getting the actual drug that they're testing rather than some saline or something,

Ms. Crawford: Okay.

Mr. Alderman: I do know that for a lot of trials they do try to increase the ratio of people who are actually getting the real treatment, if that's any consolation. Again, you probably won't know which one you are getting but they do try to tip the odds in your favor, if that helps.

Audience member: That defeats the clinical trial, double blind aspect of it.

Audience member: That's a key point in a clinical trial, it's not double blind anymore.

Mr. Alderman: Yes, you don't know who is getting what. You kind a lose a little bit of validation.

Audience member: How do they go about picking the person who is put on the real trial or not? That's the question.

Audience member: It depends on the trial.

Audience member: Yeah.

Mr. Alderman: More often than not it's randomized, so no one has the –

Audience member: That's the thing that scares me about being on a trial is, okay -- what is the guidelines of saying this person gets the treatment and – actually I feel like they are playing God. Who is the person who is going to benefit more from the treatment and am I going to say you're not going to benefit so you don't get to be part of the treatment. You get to be part of the placebo group. And that's the one thing that scares me about being in a clinical trial is who holds that over my head and who controls whether or not I live possibly longer or not.

Audience member: Nobody, not one person. It's just a --

Audience member: I know but it's still scary.

Audience member: If you let a doctor decide that we're going to give the real medicine to the people that are more likely to respond, then you've defeated the purpose of the trial because it's not a trial anymore. It's a – you know, it's a biased trial.

Audience member: But it's still scary.

Audience member: Oh, yes, I would never argue that.

Audience member: It's like the people who shoot. There's one person who has the bullet and then there's four others who don't have the bullet so they don't know if they actually killed the person or not.

Mr. Alderman: So would – I guess, with the trial aspect being that's it's completely random who gets what, and even if they tipped the odds in your favor, say 60 to 40 percent or 70 to 30, does that make you feel a little less nervous or still the same amount of anxiety?

Audience member: I don't know that there's a way to change that but the dissemination of the data would be of benefit. And not having to wait five years to receive the data.

Audience member: Okay.

Audience member: Maybe that's why they don't do it, the ones on the placebo, so they know if you see some improvement.

Audience member: Okay.

Dr. Cogle: May I ask a specific question related to this?

Mr. Alderman: Of course.

Dr. Cogle: Some of the physicians and nurses concern is that there isn't enough time to talk to the patient when you guys come to the clinic – you have blood, marrow visit, infusion, it's not – what we're wondering is would you be willing to sit with a research nurse so that when you come to the clinic, on your itinerary, would it be ok to have a slot where you have a research nurse visit and you go over a trial, what are new trials, maybe even a follow up to a trial that you're on, would you be okay with having that itinerary visit?

Audience members: Sure, absolutely.

Dr. Cogle: Anyone not like that? You're hesitant?

Audience member: No. How many years ago was it when we went to Moffitt the first time? Five years ago?

Audience member: That was not MDS related.

Audience member: I know, but we had a research person come in and talk to us and ask me if I would be interested in a clinical trial. Of course, I'm willing to do whatever it takes. I get my hopes up. And then I go in and see the doctor and have that doctor tell me — deny me the chance of even being on a clinical trial.

Dr. Cogle: Okay.

Audience member: And that's the concern that you have to think about too.

Dr. Cogle: So the plight was I don't want someone to come in and then hype up this opportunity and then have the doctor come and say sorry, you can't do that. Okay. That's good to know. Sorry that happened to you.

Audience member: One other suggestion. I would love to see a much more in-depth medical history done early, early on. More than just the typical medical history. I'm talking about stuff that relates to perhaps parents, other family members, maybe places where people have lived, exposure other people had to things. It would just seem to me that there are a lot of reasons down the pipe because there's so much that isn't known yet and it's going to be found in the future that — a lot of that information might just spring something up if there was a collective amount of it. It could be put in a computer, and all of a sudden it shows well, there's a proportion of folks that had such and such happen. What happened with this. Whatever. You know, I don't know what it might prove but — I have a social work background. I just can't help but think that that might be helpful for a lot of research perspectives if it was done by a person who really knew how to do that. It would be much more in-depth than just, you know, what are your allergies and, you know —

Mr. Alderman: So a comprehensive medical history.

Audience member: More like what the source of this might be. Not the personal stuff.

Ms. Crawford: Geographical area type —

Audience member: Yes, and talk with family members. Still relate to some concerns or significance or something.

Ms. Crawford: Right.

Mr. Alderman: Sir.

Audience member: Something that — and this is really not clinical by any means but it's something that just drives me crazy with technology and electronic medical records, there needs

to be some way to pull all of that together. I have nine different logins for electronic medical records and none of them talk to each other. So just as you're talking about paper versus, you know, the computer way, if everybody has a different electronic medical record system and they don't communicate, that's impeding progress.

Mr. Alderman: This is going to be a tad bit off topic, but there's an institute, the Health Outcomes and Policy, where they are making a push for exactly that, where big data sort of connects to each other, and that's one of the priorities I am working on. It's called the One Florida CDRN, One Florida Clinical Research Network. They are actually looking to work with doctors across Florida to sort of link all the medical records. So patient 1, if he was seen at a clinic in like Daytona and then another clinic in Miami and then another one in Orlando, they would be able to link those records through each site. So that kind of addresses what you are talking about. There is a push for that. So hopefully that helps.

Audience member: That helps with over medication.

Mr. Alderman: It will help with a lot.

Audience member: There might be some pushback with that from the privacy aspect people because, you know, if one doctor has got access to like Shands information, that's fine, but if he's got access to all my information, some people – I don't object because I don't care if people know what's wrong with me, but some people might object to that. And another fact that my wife just mentioned, you know, if you put all that data into one database, everything that man made man can have –

Audience member: I would rather it be in one than in nine. I would rather it be in one place than in nine places.

Audience member: If somebody is going to have programs, what do they most likely have? Microsoft or Windows. Because it's the most predominate program out there. If somebody is just going to hack a medical record facility – if health medical records are in, you know, San Antonio, what organization do you think they're going to concentrate on, and then you're going to have all the hackers concentrating on that one database. The chances of one person getting into the database, you know, is ex percent. The chances that thousands of these idiots out there hacking from their bedroom hacking into this database –

Audience member: That used to keep me awake at night and then I finally realized it's going to happen.

Audience member: It is.

Audience member: So just live with it. It's going to happen.

Audience member: Just because it's going to happen doesn't necessarily make it –

Audience member: That you have to make it easy.

Audience member: -- you have to make it easier.

Audience member: Yeah, I know.

Audience member: There have to be safeguards.

Mr. Alderman: Well, I guess, since this is actually my area, I could talk your ear off about that. But just to set your minds at ease a little, in this database there are no identifying records of – no identifying names. There is a very, very complicated algorithm used to sort of hash any identifying patient number. It's called SHAA-256, and I think there's over like nine quintillion –

Audience member: So it's like a block chain?

Mr. Alderman: Yes, it is. It's a very complicated algorithm. It's almost, almost hack proof, but there's always issues around it. Are we running out of time?

Audience member: Yes, we are.

Mr. Alderman: I will stop talking about that.

(Applause)

Dr. Cogle: This has been fantastic. All of this information is going to be used for designing our studies and also to changing our system, and we're also going to be asking the MDS Foundation to disseminate this information to other MDS centers. There will be no identifying information on the transcript. This has gone well.