Emerging Therapies Workgroup
October 18, 2019

Mike Bonetto: Welcome, and thank you for coming in on a wet early Friday morning. I know we've got some folks on the phone. We'll do some introductions in just a second. I want to make sure everybody has info in front of them, so we can go through the agenda in just a minute. We have some new folks who couldn't make it at the last meeting. So, if we could just take a . . . and we have some guests here today. It would be great if we could just take a, a couple minutes to go around the room like we did last time, just a quick round of introductions, who you are, who you're representing, just to kind of have a lay of the land. Then, we'll kind of [inaudible]. So, just so everybody knows, I'm Mike Bonetto. I'm with OHSU. I'm helping facilitate the core session. It's a pleasure to be among this group and learn a lot more.

Judy Zerzan: I'm the Chief Medical Officer of the Health Care Authority.

Donna Sullivan: I'm the Chief Pharmacy Officer at the Health Care Authority.

Robyn Williams: I'm the senior [inaudible] to the Governor for Health and Human Services at [inaudible].

Tom May: I'm a bioethicist with Washington State University.

Rebecca Owen: I'm a pharmacy expert at [inaudible] of Oregon.

Carly Rodriguez: I'm [inaudible].

Petra Eichelsdoerfer: I'm a [inaudible] Center.

Yusuf Rashid: I'm [inaudible] pharmacy and [inaudible] relations at [inaudible].

Bruce Wilson: I'm the [inaudible] of the [inaudible] of Washington [inaudible].

Stephanie Simpson: I'm the executive director of the Bleeding Disorder Foundation of Washington.

Ken West: President of Metropolitan Seattle Sickle Cell Task Force.

Melissa Tribelhorn: Executive Director with the NW Parkinson's Foundation.

Marco Mielcarek: I'm a medical [inaudible].

Monica Thakar: Pediatric bone marrow transplant doctor.

Emily Transue: Associate medical director here at Health Care Authority.

Leta Evaskus: Prescription Drug Program and Health Care Authority.

Mary Fliss: [inaudible]

Joe Schmick: [inaudible]

Christian [inaudible]: Fourth year pharmacy student on rotation with [inaudible] with Optima RX.

John Espenschied: [inaudible] State University [inaudible] College of Medicine.

Cody Gillenwater: Medical Director with Regence.

Armen Khatchatourian: Senior director of industry relations and [inaudible]. Thanks, Christian. I'm glad you made the call.

Donna Sullivan: And I see that Kerri Fowler and Sean Sullivan are on, but they haven't entered their audio pin. So, they can't speak yet. So, if you guys can, uh, try either calling back and then entering your audio pin, which should be on the webinar when you log in.

Mike Bonetto: So, yeah. If you log in, feel free just to introduce yourself. OK. Can we jump in? Judy, you've got slides here. First, if everybody has an agenda in front of them, let's make sure we can go through that quickly so we know what our [inaudible]. Do you want to make just one small correction? If you go through this agenda, Judy and I are gonna swap number 2 and 3. So, after we get closer to the introductions, I'm just going to give a quick recap of the last meeting, the June 18th meeting.

Then, Judy will kind of go through that survey that everybody did after that meeting. Then, we got from the deck of presenters on the patient advocate position and from a patient perspective. Then, we got right now from the provider perspective. Then, we wrap all this up. Kind of a more in-depth discussion of, OK. So, what are the ethical situations and conundrums that we need to be considering? So, I think it just lays out well and really with everything that we were trying to tie together, it's more on the ground of understanding of really what's happening at the patient provider levels.

So, I just wanted to spend a few minutes, 'cuz I know not everybody was at the June 18th meeting, including people waiting to review some of the notes that were put out there, but I think this is helpful in this context. So, I think we'll overview some [inaudible]. So, why are we doing this? I think Judy is going to even try to touch on this again, but it really was Health Care Authority coming together and kind of asking kind of a panel of experts around. OK. We know this is coming. We know we've got this pipeline of emergent care therapies at high costs. How Health Care Authority should be looking at this. So, in this scope, what are some longterm funding mechanisms? What's the quality and oversight that we should be looking at? How should we be managing patients all the way through? So, at least it starts to give them an understanding of how they want to start to look at administering and having their own internal policy [inaudible].

So, this is, I think it helps to probably clarify, when we started the first meeting, this was very much just a context setting and laying the groundwork. Now, we're really starting to get into more of that kind of in-depth understanding today much more at that patient level. What are those patient aids? How are providers communicating with doctors and communicating with patients. What are some of those best practices?

We then have February and April to get into the more in-depth discussion on OK. How are we going to finance>? What are some of those obstacles? Then, in April get into, so what are those metrics? What are those quality outcomes that we could or should be looking at? Then, we'll start to tie that all together, so we'll then have a package [inaudible]

up here with some of those final recommendations that we would have for Health Care Authority. Any questions or comments on that?

OK. So, again, just based on the background, this is what we kind of touched on at a high level back in June. Not surprisingly, many of you guys already know this. The idea is that we've got a big [inaudible] on our hands with the specialty medicines basically doubling over the last ten years. The bigger issue is the trend forward. So, we know that we already have the significant increase, but then we start looking out to those next five or ten years. It certainly doesn't get any better from the economic angle. We spent some time in June just starting to go through that laundry list of those pipeline drugs. They just keep coming. Again, one of the big points was headed towards a smaller and smaller portion of the population, which actually makes it that much more difficult [inaudible]. Again, more staff in terms of what we're looking at in terms of the pipeline being more specialized, being more focused on specialty drugs and [inaudible] drugs, which we know are going to be that much more costly. So, again, what can we do to get ahead of that curve and be as proactive, as possible?

So, we also spent a bit on just hearing from the commercial factor and then hearing from Health Care Authority, in terms of what did that look like from a managed care standpoint. There were certainly some differences, but what we had on this slide, we're really just trying to kind of overlap those similarities. So, you really had those four buckets that whether you're in the private sector or public sector, you're going through this type of process to really understand, you know, what is going to be covered. So, there's the evaluation of the evidence, which again, I think presents some challenges with these emerging therapies, just because there isn't a lot of evidence out there right now. So, that has been . . . could be a challenge. Regarding financial independent analysis, development of critical criteria of when it's going to be kind of required of the providers [inaudible]. What's going to be required of the clinical criteria for patients? Then, tracking down [inaudible]. So, all four of those buckets, really we saw them as parallel between [inaudible]. That's something to keep in mind, as we start to look at this, what changes for some of these emerging therapies.

Donna Sullivan: Can you speak into the microphone, just to make sure the people can hear you?

Mike Bonetto: I'm hoping that's better. So, this slide here was one that Donna brought up. I think we're going to actually circle back to it even again today when we have the ethics discussion. This really hits this common theme. When you start looking at how we're going to be dealing with this, it really is from these multiple buckets, but it's not just . . . they're not [inaudible]. Right? We have a lot of overlapping. So, we're going to hear kind of more today from that patient and provider perspective, but we also have kind of the society and payer perspective from the Health Care Authority aside from a taxpayer and an operations side. Then, we obviously have the supplier side, as well. So, all of those pieces . . . this group right here is trying to make some sense out of. I think this is really where you start to get to the heart of it, and how do you begin to set policies when you have all of those overlapping issues?

This last piece, we're not really going to dive into right now, but this was at the end of this meeting where you guys did some quick brainstorming. You had some of this huge download of information. It was, like, what are some initial reactions. These were just capturing some of your initial thoughts that we'll be diving into even more today [inaudible]. If you look at those bottom pieces, those really start to get in deep in a lot of today's discussion. Hopefully, that can [inaudible] a lot of those [inaudible] today.

I'm going to turn this over to Judy now. So, I'm just, for context, after this meeting, we have put together a survey that went out. It was really kind of asking for some direct feedback around how that meeting went, what were some areas of focus. Judy is going to kind of walk through that at a high level. Thank you.

Judy Zerzan: So, we wanted to just sort of briefly go over your reactions, and a few questions came up on the survey at the end of last session. So, we thought it would be helpful to go over. So, most people liked the meeting in Spokane. There were some comments about a better breakdown on the growth of specialty drugs and more information on how pharma sets prices. Then, a few different questions on the

understanding of sort of the end goal. Limiting participating to one representative from each organization, which we have done. So, I think one piece where I wanted to spend a little bit of time is helping on the focus. So, what is Emerging Therapies? From the Health Care Authority's perspective, it could be very broad, but one thing that we had done specifically, and I think that this might help with some of that, there is probably a spectrum of super expensive drugs and moderately expensive drugs, and less expensive drugs, but we have carved out for sort of a variety of reasons, drugs that cost over $100,000 a year for an individual. So, that may be a drug that costs $10,000 a month for a person. Or it may be a one-time drug that costs a million dollars. So, we have carved those out as a managed care organization starting, I think, in January 1st? Or did that already happen? It did already happen. So, I think Emerging Therapies could sort of be in all of that. We carve them out so that we could have a common management approach to that and figuring out how to pay for them without making the managed care rate sort of crazy. So, for our purposes, I think over $100,000 a year is the bare minimum, and it could be more than that.

We also wanted to chat a bit about the end goal of this. So, our hope is that by the end of the fourth meeting, we'll have some recommendations from you on sort of how we should approach the new very high cost drugs. So, what sort of criteria should we take into account, not what are the actual criteria, but more sort of down the things that we should think about? I also want to be clear that this has come up in the interim, that these are not open public meetings, because we are not making any concrete set policy decisions. We're coming up with options that leads to a public meeting. Having some conversation as well with that. So, we want you to help us identify concerns, maybe ethics [inaudible] today, perspective around the new high cost drugs that we can use to help build some proposals that then are taken to our normal open public meeting process.

A little bit of an update on that. [inaudible] and from the outside, you will note that there are no pharmaceutical manufacturers present in these meetings. That was purposeful in some ways, but we have decided to invite them to the next meeting, which is going to be focused on financing. I think there have been some people that have been wanting

to ask questions [inaudible] spoke at the meeting yesterday and said this is totally not sustainable how these prices are set. How do we have a conversation about that? So, I think it'll be useful to bring them into that meeting to have some conversation. So, expect that. Then, after this is over, we've also set a meeting that you are invited to, but you don't have to come to, on June 9th. It's slightly shorter. It'll be here from 12:30 to 3:30. I'm not sure the invitations have gone out for that yet. The goal is that meeting to have it be an open public meeting so that anyone that wants to comment on the process. We'll sort of give a summary of what the workgroup does and a summary of some of the slides with information that we've gone over to get additional stakeholder questions and feedback. If there are policies set about any specific drug or about the approach in general, that will go through our usual process, which is the pharmacy and therapeutic committee. It just met earlier this week. It, in general, meets on a quarterly basis at SeaTac. We'll actually be able to hitch our wagon to those meetings. So, the space is already reserved for the P&T Committee meeting. We'll be there. So, if you're interested, at the next meeting, I think we're meeting, I don't have that, in the morning. Then, you're in the afternoon. Is that right?

Donna Sullivan: The other way around.

Judy Zerzan: So, if you want to come early and listen to the P&T Committee, you can. You don't have to. Then, we'll have a meeting afterwards in the afternoon. So, I think that is all. The topic that pertained to the next meeting, the state budgetary administration and [inaudible], the next meeting will all be about finance. So, like, where we'll talk about money. Do people have questions, comments? Maybe I'll start with the phone?

Sean Sullivan: I just want to introduce myself to everybody. I'm at the University of Washington School of Pharmacy. I'm a health economist focusing on pharmaceutical economics, and I'm dean of the school.

Judy Zerzan: Excellent. Good morning. In the room, any questions, more questions?

Mike Bonetto: Other housekeeping, I apologize I didn't announce this earlier. I think everybody had some coffee, but coffee and pastries are in the back. The

bathrooms are right around the hall. We will be taking a break, oh, at maybe another you know 45 or so, but please help yourself.

So, next up, we'd like to get into hearing from the patient advocate perspective. We just did a little rejiggering. I think Stephanie was going to go last. So, if I can ask Melissa, am I going first?

Melissa Tribelhorn: Good morning, everyone. I am the executive director with Northwest Parkinson's Foundation. Our organization aims to be the lifeline of hope for the Parkinson's community throughout the Northwest. We serve people in Washington, Alaska, Montana, and Idaho with [inaudible]. We have a huge service area. We're very [inaudible]. Our goal is really [inaudible] with individuals and families to navigate [inaudible] whether you have Parkinson's, your family member has Parkinson's, or any other resources, such as for prescriptions. We have a [inaudible] social work team that works directly with patients and caregivers and other family members. We have offices based out of Mercer Island near Seattle and in Spokane.

To give an overview of Parkinson's, about 1% of the population over the age of 65 have Parkinson's. So, Parkinson's disease is not rare. I'm sure you all have heard of Parkinson's. It's not a rare disease. That's about a million people in the United States, and 18,000 estimated in Washington State. We believe that the 18,000 number is a pretty conservative estimate of how many people actually have Parkinson's in Washington. Our database has 33,000 people in it, but that does include partners and family members, as well; 13-20% of people with Parkinson's are diagnosed before age 65, and unfortunately, we don't have great data on Parkinson's in the United States to really look at, does that 1 million number really encompass all of those [inaudible]. Depending on what study you look at, it could be 13% or 20%. So, it's a big range there. Sometimes treatment [inaudible] individual, and this is something that I think can be hard for people to understand, but if you have Parkinson's, your progression of the disease, your [inaudible] probably anyone else with Parkinson's. So, these are really, really hard to [inaudible]. I think when you think of Parkinson's, most people will tell me [inaudible] tremors. The full of one-third of people with Parkinson's do not experience tremors at all. A lot of people also will tell me, oh, well

Michael J. Fox, I know [inaudible] very dyskinetic. That's actually a side effect of medication and not a symptom of Parkinson's itself. Not everyone experiences that either. That is an off period. The medications try to treat the major medications [inaudible] treat currently. Carbidopa and levodopa are the major medications for Parkinson's. We have been using those [inaudible]. There have been some dopamine agonists, another add-on medication that have been added to people's medication regimen [inaudible] in the last seven years we've made huge upticks in medications available for Parkinson's. However, the major medications are still carbidopa/levodopa. That has not changed, since 1950. We have looked at ways [inaudible] protein, but the major medication is the same.

Endstage progression is really where Parkinson's [inaudible]. Medications start to wear off after about, depending on the person again, after about 7 to 10 years. Then, [inaudible] you start to see tremors [inaudible] but both sides of the body. Symptoms move into the trunk, they start with the issue of swallowing, with breathing, and other certain things.

So, our organization helps patients self advocate in a number of ways. On the individual patient level, we really look at education around comprehensive care and palliative care, what is your disease progression going to look like. What do you need to plan for? We help people make the most of their position with it. So, looking at medication management, what type of questions you should ask your provider, bringing a friend or a family member to your appointment to help you track your symptoms, and help track what the doctor is actually telling you to do. Tracking symptoms, tracking medication side effects, and we help people understand the medical coverage [inaudible]. So, we really help people understand what are their coverage options, what they came in for, what the doctors [inaudible], etc.

On the group level, we do a lot of educational programming for patients and caregivers, specifically under these [inaudible] treatment options and [inaudible]. We also have trouble [inaudible] payer [inaudible] to increase access to specialty care. So, our belief is that people with Parkinson's are [inaudible] like a neurologist who has an extra two year fellowship [inaudible] Parkinson's. We know the outcomes are much

better for patients with Parkinson's if they're under the care of a specialist. However, most of the [inaudible] are located in the Seattle area. We have about 15 in the state right now, and I think 10 of them are in Seattle. So, if you live in an outlying area, even if you live in Spokane, there's only two there. Each physician is seeing 400, 500 patients. We have less than half of the Parkinson's population in Washington being [inaudible] specialists. Especially in the outlying areas, many people are not even seeing a neurologist. They're only seeing they're [inaudible].

We do a lot of world provider support. We're working with [inaudible] University or Washington, University of Idaho to help increase education for physicians and what they can do to help their patients if they don't have [inaudible] specialist, or [inaudible] neurologist [inaudible], and we also help them access to TeleHealth and TeleMed programs.

On the [inaudible] level, we use state and federal level policy advocacy. That's why I'm here. We collaborate with national and regional organizations, and we are trying to get a lot better at utilizing data to demonstrate what the need for [inaudible] specifically in our service area.

Alright, so what is the experience of a Parkinson's person? Over one year in an average Parkinson's person's life, 54% are chronic, and that is on an outpatient basis. However, 11% will spend a year in an institution. So, in Washington State, you're looking at that 18,000 number, about 2000 people who are going to be institutionalized, specifically due to Parkinson's. About 23% are chronic, not institutionalized, and will experience an acute event. So, that's about 4000 people in Washington that will experience an acute event, and 12% are going to die. So, mortality for Parkinson's is about three times higher than the general population. Younger onset people tend to have more severe symptoms. The consent [inaudible] is relying on [inaudible].

Alright, so 60% of people with Parkinson's will report at least one fall, and recurrent fallers report 4 to 67 falls per year. So, as you can imagine, this is definitely going to increase medical costs if you're falling a lot. The risk of suffering a fracture is twice as high in people with Parkinson's. The risk of a hip fracture is 4 times higher. If we were to do the onset of

Parkinson's or the diagnosis of Parkinson's or [inaudible] or the variations of Parkinson's, we can review the [inaudible]. Dementia is almost inevitable for people who have Parkinson's, maybe not right away, but especially when we start looking at that 20-year mark, we see more than 80% of the people will start to experience signs of dementia and other cognitive issues.

Parkinson's places a huge economic burden on individual patients, but also families, and certainly on the healthcare system. The total annual cost of Parkinson's in the United States is 51.9 billion dollars. Direct medical costs are about 25.4 billion, and indirect are 26.5 billion. You can see our divided costs into direct and indirect. On the direct side, we have hospital inpatient, outpatient care, physician office visits, durable medical equipment which people with Parkinson's will need at some point during their diagnosis, prescription costs, which become more and more expensive [inaudible] Parkinson's, and non-acute institutional care. The indirect medical costs, we have disability income, attributable death, reduced employment, particular for our young onset population. This one is huge when you cannot work anymore because of your Parkinson's symptoms, what do you do, especially if you've got children at home. Absenteeism, we see a huge productivity loss [inaudible] to think about and talk about more [inaudible], and other nonmedical costs. So, the economic burden of Parkinson's in the United States is quite high.

This leads us to why innovation is important. The value of innovation in Parkinson's cannot be understated. We have a lot of exciting drug therapies coming down the [inaudible]. The three that are the most likely to come to market in the near future I listed here. We have two gene therapies [inaudible]. I'm not a medical doctor, so I'm not going to be able to speak to the specific mechanism right here, but you can see we have one gene therapy, well basically it's an enzyme allowing better communication between cells in the nervous system. In early stage clinical trials, it reduces the amount of levodopa that the patient needed to take, up to 42% needed less levodopa in the higher dose group. So, that type of therapy would allow drug costs for carbidopa levodopa to be reduced. People could take that and reduce that therapy. So, the second therapy is what I'm really a lot more excited about, because this one was in code for three clinical [inaudible] production. It produces a 42%

improvement in [inaudible] of the progression of the disease, and improvement in activities of daily living in only three months after treatment. So, when we were reducing these major symptoms of Parkinson's by 42% after only a three-month treatment, you have not [inaudible] how much that [inaudible] prescription cost, but also the other related medical costs and indirect costs, as well. The third is a stem cell therapy. It found improved motor function in Parkinson's subjects with mild to moderate disease. So, those are just the three that are some likely to come to market in the near future. There are a ton more drugs in the pipeline, but I won't go into all of them, but we think that this is a pretty exciting time for Parkinson's. We're exited to see these come to market.

Again, value of innovation. If we are able to slow Parkinson's by 20%, we would have a savings of almost $76,000 per person, per year. It's [inaudible] the math for 18,000 people in Washington, that is over 1 billion dollars in Washington State alone. If we were to completely halt Parkinson's disease, we would have a savings of $442,000 per person, which is almost 8 billion dollars in Washington State alone. One thing that I do want to say here is that I think when we're looking at, how does this define Emerging Therapies, for Parkinson's, a cure is probably not the right end [inaudible], because what we're really looking at is how can we help just the symptoms. How can we treat the symptoms better? So, we haven't had very many innovations, since the 60's. We really want to look at, if we even are able to reduce them and reduce the progression. How can we help folks? How do we improve quality of life for people with Parkinson's and their families? When we look at indirect medical costs, medical costs especially, it's not [inaudible] these things. It's also [inaudible]. It's also adult children with family members with Parkinson's. The whole family system ends up spending a lot of money. I think that there's something that is [inaudible] to make a recommendation to [inaudible] and not just the [inaudible]. So, thank you, so much.

Mike Bonetto: Questions for Melissa right now?

Female: Is there [inaudible] timeline on the [inaudible] therapy?

Melissa Tribelhorn: Yes. [inaudible] presentation. I can find those for you. [inaudible].

Leta Evaskus: I just want to make a comment. Since we're recording this, and it will be transcribed, please state your name before you speak. So, that would [inaudible].

Rebecca Owen: Melissa, do you have, on your expected savings, have you quantified how much is direct medical costs versus social costs?

Melissa Tribelhorn: I didn't break out all of those. I did, I think, on one of the slides, I did medical costs savings of almost 38,000.

Rebecca Owen: So, on your total numbers?

Melissa Tribelhorn: Yeah. So, it looks, yeah. Right there. So, this is for the whole [inaudible].

Rebecca Owen: Yeah. That's the total cost, but I'm, on the [inaudible] that you go forward a little bit, one more. So, you have the medical cost savings of 37,927. So, it would be really interesting to see the true versus the treatment if that medical cost savings is net of the [inaudible] increased cost of the drugs just it's, just so it's really clear to people who are trying to figure out what pocket their savings go in, what that actually looks like.

Melissa Tribelhorn: Yes. I can try to [crosstalk] really fast. I'm taking this from a very [inaudible]. So, I can try to extrapolate that for those categories. Then do the breakdown.

Rebecca Owen: Yeah. I think it'd be really helpful for people if they don't . . . because we do think about [inaudible].

Thomas May: It would be helpful, because when you look at these numbers, it's striking, because I tried to Google the entire Washington state legend was [inaudible]. So, [inaudible] a huge [inaudible] just for one . . . that sort of savings just for one condition that [inaudible].

Melissa Tribelhorn: Except, this is not the whole [inaudible] that this is impacting. It's broader than that [inaudible]. It's only a slice of this that would impact [crosstalk].

Thomas May: This is why I say it would be . . .

Melissa Tribelhorn: But not all of it.

Thomas May: And all of those 18,000 would all be covered by [inaudible].

Rebecca Owen: And Melissa, if I can help you with that, I'd be happy to.

Mike Bonetto: Judy, you've got a question?

Judy Zerzan: On the phone, [inaudible] has a question. I can read it for him. Or if he wants to unmute, he can read it himself. OK. Does the economic [crosstalk].

Male: I was muted, but if you could read it that would be great. Thank you.

Judy Zerzan: OK. Does the economic burden of PD include the economic burden of relative families, how much research investment would be needed to slow down by 20% and to save . . .

Male: And to save the 1.366 billion dollars in Washington State?

Melissa Tribelhorn: That's kind of a difficult question to answer, [inaudible]. The first part, I'm sorry. Can you repeat the first part?

Judy Zerzan: Yeah. How much research investment would be needed to slow down by 20%?

Melissa Tribelhorn: That's a great question. In terms of [inaudible], you mean clinical medical research? Or do you mean . . .

Male: All types of the research. I wonder if . . . to save 1.3 billion, or 1.4 billion, maybe even there's a need to invest the same amount of money in research to make it happen. Right? I was just curious [inaudible].

Melissa Tribelhorn: Yeah, probably at least that much. So, the Michael J. Fox Foundation, [inaudible], they are the [inaudible] organization that is completely focused on medical and clinical research. They are investing, I don't know, probably at least 20 million dollars a year into research or into

therapies here and in other [inaudible]. However, I think we often need a lot more research extrapolating the data for Parkinson's. So, we know exactly [inaudible] in Washington State. Our organization is actually partnering with the University of Washington and the VA this year to put together a study for who [inaudible] Washington State, and how can we make sure that they all get [inaudible] specialist or [inaudible]. We're hoping that through that data we can better figure out who [inaudible]. Who was diagnosed after 65, and just doing that, we'll be able to have a lot better data on where all do we need to do research? Where do we need to invest more money in infrastructure, for example, like we were talking about [inaudible]? So, this is really where the state come in with funding, but there is also a lot of other avenues where the costs. So, longterm and institutional care, assisted living, and all those other indirect medical costs, as well.

Male: OK, and the first question, was there an economic dollar burden put in one of your slides earlier slides. I was wondering, I saw medical costs and everything for the patients, but do those costs include the economic burden to the family who care for the patients?

Melissa Tribelhorn: So, yes, the indirect medical costs, that does include economic burden to caregiver and family members. Yes.

Male: Alright. Thank you.

Male: [inaudible] Thank you for this and for the work on the foundation. I see so much hope that comes [inaudible] presentations [inaudible] foundation. I'm just wondering, the Parkinson's Foundation, what level of coordination or collaboration is given between Foundation and pharma companies?

Melissa Tribelhorn: We do [inaudible] pharmaceutical companies for our [inaudible] programming. They're not involved in the [inaudible] or what the actual contents are, but they're definitely involved in exhibiting our conferences and educational programs. I would say that's about it. We do work with pharmaceutical companies [inaudible] understanding what drugs are coming down the pipeline. We have meetings with all of the reps and [inaudible].

Mike Bonetto: Any other questions?

Bruce Wilson: I think it's wonderful that you're trying to monetize these other costs. [inaudible] we always like it when we can justify a drug offsetting other medical costs. That's the evidence [inaudible] for that reason. With that said, also the cost calculations [inaudible] of the other [inaudible] costs sometimes is a little tricky. We look at organizations like ICE or [inaudible] utilize them exactly. They rarely [inaudible] the value part of the challenge. So, I think, as an advocacy group, the better you can [inaudible] matter. These costs demonstrate [inaudible]. Really, that [inaudible] the payers, and it helps government have even more direct relationship to these other costs.

Melissa Tribelhorn: I actually have a ton of [inaudible] that I'd love to share with you, but I don't feel like I have enough time. These studies that I put into my slides here, they have a lot more data about a lot more [inaudible].

Mike Bonetto: Keep in mind from our point of view there's the evidence and then there's actual . . . there are different levels of proven lengths to these costs. So, that's . . . [inaudible] study would be defined to say how much did this cost to society [inaudible] a randomized study by pharma.

Sean Sullivan: Can I make a comment?

Mike Bonetto: Yes, please.

Sean Sullivan: Thank you. So, over the last three decades, there have been many drugs introduced with the promise that they would reduce costs. We have very few examples where that's actually the case. In large part, because of the way the manufacturers price the treatment. So, if they price the treatment to actually absorb and pull in those cost savings, or what would be cost savings for their revenue stream. So, I just want to be cautious about sort of having an expectation that just because a disease costs a lot of money that a new treatment that is going to delay progression is actually going to save resources for the healthcare system.

Mike Bonetto: Thanks, Sean. I know we're going to have the whole [inaudible] in February, and I'm gonna be looking to at the profit levels there, too. When you start to look at that, [inaudible] discussion on the [inaudible] cost, that means that that could be coming out of the different agency buckets. Right? Then, how do you actually then quantify that and then take that money out of that agency bucket with those expected savings. So, those are not always easy issues to grapple with, but [inaudible] that was great. Thank you, very much. Ken, I'm going to ask you to come up.

Ken West: Good morning everyone. I am president of the Metropolitan Seattle Sickle Cell Taskforce. What I'd like to talk about just a little today is sickle cell, starting with the background of sickle cell. Sickle cell in the United States affects about 2000, and that's the sickle cell disease versus the sickle cell trait, which is a lot more evil.

Basically, sickle cell is a disease of the red blood cell where the red blood cells are sickle shaped, as opposed to rounded, smooth. This causes a myriad of different symptoms. One is joint erosion or avascular necrosis. There could be stroke, [inaudible] syndrome, and a lot of other issues in the body.

Despite knowing about sickle cell since I think 1908, there have been very few drugs developed around sickle cell, and very little research, more now, around sickle cell. So, one of the drugs that was develop, and I was in the clinical study for, was hydroxyurea, and that has had a huge impact on my life and other's lives. However, that is pretty much the only drug that has been brought forth that really treats sickle cell disease. The latest drug is [inaudible] so just treats symptoms. It doesn't really . . . it's not a cure. It just treats some of the symptoms. Unfortunately, we have not had enough research, and research dollars attributed to sickle cell so that we can have more therapy and drugs developed. Fortunately, we are at almost a new age in research that is really exciting, at least for sickle cell and other therapies, just like Parkinson's and bleeding disorders, as well. That is gene therapy.

We, in the sickle cell community are struggling, have been struggling for a long time, to one, understand how clinical trials are developed, how we can contribute to getting more information out about sickle cell, how we

can be more of a force and raise awareness of sickle cell to the community and include the medical community. In the Northwest and other places, there is very little knowledge about sickle cell.

So, there are . . . we rely on our local group, the Northwest Sickle Cell Collaborative, FDA advice, and clinical trial advice to give us information about what is going on within the sickle cell community, and what's going on around . . . research around sickle cell, what clinical trials are out there, what drugs and emergent therapies are there. There are some conferences that have more information that [inaudible] purveyor of that kind of information for us. We're lucky to be associated with them. Still, there is a lot of information that we are not getting out there that we are still trying to make sense of. That's where we rely on our medical providers and our associates to help us with understanding what clinical trials are out there, whether emerging therapies are out there, because it's about hope for us, hope for us for our kids to grow up in a world without sickle cell.

What defines a cure for sickle cell? For us, we have, of course, the bone marrow transplant, but that only effects 10% of our population. You have to have exact matches or now these days, not exact matches, of course, of bone marrow but still, that's a small percentage of the sickle cell population. We are looking for a cure that will help the majority of us. The majority of the kids. I'm not too worried about it for myself. My job is to speak out about sickle cell, raise awareness, create that sense of community, and pass that on to our younger generation so that they know that they can advocate for themselves and to have hope.

What does the Metropolitan Seattle Sickle Cell Taskforce do? We provide education advocacy and, of course, partnership with other organizations, so that we can raise awareness about sickle cell. The way that we do that is through education. We do lunch and learn sessions. We have a sickle cell camp, which is for ages of kids from 7 to 15 over on Vashon Island, beautiful spot. What that does, it creates a sense of community. So, a lot of the kids start out going to camp when they're 7. They are now getting to be young adults, and they come back to the camp as counselors. That's an awesome thing, because that really reinforces our community. Fortunately, we are a growing community. With others coming from

other countries, we are really trying to reach out to those communities, as well. Our struggle is to continue to raise awareness, and continue to raise interest, continue to reach into those other communities so that they know that there are people that are advocating for them. We do have a website, www.mssctf.org. I encourage everyone to go out there and take a look at what my organization is doing. We are partnering with [inaudible] organization, as well, the bleeding disorders organization, and other organizations, to raise more awareness about sickle cell, because I think that in today's world there are a lot of things that are out there, and sickle cell kind of gets missed. That's been our struggle over quite a few years is that once in a while you hear something about sickle cell, but people don't know enough about it. People are struggling with sickle cell every day. There are lots of people that, unfortunately, are still dying of sickle cell every day, and we need to change that.

My hope is that through this group, that we raise awareness. We create a way that our families with sickle cell have a way to have hope and understand that sickle cell does not have to be a death sentence. There are things that are being done to advocate for them to help them in their daily lives to help with the struggle of having sickle cell disease. Questions, comments?

Female: This is an indirect question. So, one of the things that I've heard is that access to pain medications for people with sickle cell has always been kind of iffy. Is it worse now with the new focus on trying to eliminate the opioid epidemic? I mean, are we cheering for people well?

Ken West: That's a huge can of worms there. We have been significantly impacted. One of the things with sickle cell is that there is pain, pervasive pain, and we have been treated with narcotics, opioids, over time. Now, of course, this, it's, like, oh, now you've got to change that now. We are not encouraging you to use oral opioids. It's, like, OK. So, what are the alternatives? What alternatives do we have? It's a big struggle. Our young adults are struggling with finding alternatives. The doctors are struggling with finding alternatives. So, it is a huge kerfuffle right now on how do we go forward. Those are the questions that need to be asked. I mean, we are continually trying to find alternatives that actually work. When you're in pain, you don't want to hear, well, you know what, we

can't give that to you. This has worked for you before, but now, you know what, we're not doing that now. How would you feel if you had this drug that was actually working for you? Then they said, you know what, we can't do that now. That is a struggle.

Male: How does the emerging therapeutic drugs, and again it comes down to [inaudible] more [inaudible] and things that are studied in a way that allow us to believe in them.

Ken West: Absolutely.

Male: [inaudible] Washington State is not [inaudible] were not always treated appropriately. Every one of us who see patients are frustrated with that [inaudible] control [inaudible] and the opioid situation.

Ken West: Absolutely. [inaudible] to continue to address those issues and shed some light. That's really what needs to be happening every day, shed some light.

Leta Evaskus: I'm a [inaudible] has a question. Do you have an idea about how much funding is spent on sickle cell research per year and over the past five or ten years?

Ken West: I do not have those stats with me, but I can certainly investigate that.

Male: Can I just add one statement? [inaudible], can they go to the sickle cell coalition and . . . what information, what kind of guidance is provided?

Ken West: So, fortunately, in Washington State, we have a newborn screening program, which identifies kids with sickle cell and other diseases early at infancy. So, we engage those families and those kids early on in their lives. So, [inaudible], we have had some contact with their parents. We've encouraged their parents to send their kids to camp. At camp, they meet other kids with sickle cell. Then, of course, we try to speak at and educate [inaudible] so that they, as kids, get to know more about sickle cell, as well. Then, we, of course, continually try to reach out to the parents so that they understand that there is a community and a connection with all of us.

Stephanie Simpson: [inaudible] So, we don't . . . in our program, we don't treat very many people with sickle cell disease. So, our referrals are very low. Oftentimes, if you do need them, they are family referrals or supplement referrals, but there is a [inaudible] hesitancy if you don't have a well matched donor, which is very rarely highlighted, because as a disease, it's often hard to find a sibling who is not also affected, but there are now strategies that we use for alternative donors where you can use a parent who has a trait or unrelated donors. I was just curious, with your work with families, how that idea of transplantation is used, because in our world when we talk with our . . . it's all colleagues. We know the detrimental effects of longterm sickle cell disease, even ones that are on hydroxyurea, even if they are compliant, and our adult colleagues oftentimes say, wow. If we could have transplanted this child as a 10-year-old or an 8-year-old or a 5-year-old we could have potentially prevented [inaudible].

Ken West: Absolutely. Well, it is a great thing that we do have active transplant options; however, I think that in the community, there is some hesitancy, simply because it's an arduous protocol. Some people would rather live with the complications that they have, rather than actually doing something different that could possibly be life-threatening to them. It also goes back to education. Right? Explaining what is completely involved in transplant working, what the positive is, of course. We have someone [inaudible] in May who went through the transplant protocol and is now free of sickle cell, and that is a grid model for us to show other families that there is a positive in the transplant routine; however, I think that there is still some hesitancy simply because it is a scary thing. I mean, unless you know someone that has gone through that transplant routine and come out good on the other side, it's a scary protocol to talk about and to actually go through. So, more education would be helpful. I'd love to partner with your group, just so that we can really maybe have a lunch and learn so that we can explain more about that to our families.

Leta Evaskus: So, John Bramhall Roullet sent a link, but I can't open it from the, oh maybe I can. Let me see. No. I can't open it from the chat. JB, do you want to . . .

JB Roullet: Don't worry. That's just for your information. There is a link. It goes to your publication that compares [inaudible] for sickle cell disease and cystic fibrosis. So, I suggest you follow up a little bit about my question to the speaker about how much research is going on in terms of dollars for sickle cell disease. So, it's just to keep in mind and maybe to share with others.

Ken West: Absolutely. Cystic Fibrosis really is, compared to sickle cell, is raking in the dollars, as far as research, and as far as [inaudible] new drugs and those type of things, and the dollars coming in for sickle cell are a fraction of that. Cystic Fibrosis affects less people than sickle cell. So, [inaudible].

Stephanie Simpson: So, the other thing I was thinking about, you know, there's all this excitement with gene therapy for sickle cell and the [inaudible] technology and gene editing, and it's very exciting. The ironic thing is to get all of [inaudible] gene therapy, you need high dose chemotherapy. I don't think families really get that oftentimes they think chemotherapy you get for a transplant [inaudible]. The dose of chemotherapy can cause problems, as well, you know? So, it's the toll of the chemotherapy, which is incredibly exciting, but to get those cells [inaudible] over, you have to give that patient chemo. So, I think the education piece would be great, because we also don't have any understanding what the trajectory is, how long will those cells persist? You know, oftentimes, in other gene therapy trials, you will see that it persists in sickle cell will kind of diminish over time. Then, in order to [inaudible] going back to old school bone marrow transplantation, but it's just an exciting time. A lot of people are more interested in sickle cell research, but it's . . . I think the education piece would be great to help families, because I just can think of this one young adult who was having a lot of complications with pain. She was always in the Emergency Room. It was really a leap of faith that she said, you know, I just want to have this transplant and go through it. She knew that the consequence would be infertility, which is a big thing. People don't want to hear that they are likely to be infertile. She has turned into this huge advocate where now she is pain free. She has been to an Emergency Room. She has had limited complications. So, it's hard to think about the longterm when you're so . . . you have to get through a lot in that short-term, but she's a huge advocate. So, I think you're right. The people who have done well going back and talking about [inaudible].

Ken West: Education is key.

Marco Mielcarek: I think it's important to develop some kind of a strategy to identify high-risk sickle cell patients early before they have developed all these complications that happen all the time. [inaudible] transplantation safer, because the patient comes to transplantation [inaudible] disease, toxicity of the transplant is exponentially increased and [inaudible] to do the transplant, but [inaudible] just above the [inaudible] transplantation [inaudible] treatment, and [inaudible] from a different donor to treat patients with sickle cell disease, and you probably are facing a mortality risk because of the transplant in the 10-20% range [inaudible] within the first two years after a transplant. Some patients choose to live with a chronic disease despite the complications of wanting to accept these early mortality rates, which I think is totally reasonable. Education is important in the context.

Ken West: Absolutely. That's why we really need to partner more and educate our families, our communities, about the differences between the therapies and the similarities. Thank you.

Mike Bonetto: We're going to take a break. One of the things we're talking through, if you saw kind of those opening slides, we had a laundry list [inaudible]. So, these are just to kind of give you kind of real life examples. Then, we can begin to kind of extract later [inaudible], as well. Alright.

Stephanie Simpson: This is super exciting, because I love [inaudible] presenting more data and getting [inaudible] information reminding us that the amount of [inaudible] that are going to be eligible [inaudible] are so diverse. Some of them have been so underserved and misrepresented. It was our policy [inaudible] need to embrace all of them. That was really something that was important to me and [inaudible] love to talk about money, and it has so many treatment options, it's a little embarrassing to stand up here after these individuals, but it also provides so many stories and examples for us to learn from, but I don't want gene therapy and emerging therapies [inaudible] hemophilia. I want to make sure that we don't forget [inaudible] at all. [inaudible] policies for them and for our larger populations, like, Parkinson's and so many other [inaudible] conditions.

So, I kind of hope, I do not have as many beautiful numbers as [inaudible], but I wanted to talk about [inaudible] therapy and [inaudible]. I have a hand out board. I can set it up here. There are so many [inaudible] for hemophilia, you can't even read it. [inaudible]. That's for a number of reasons. I think [inaudible] explain hemophilia, because all of you guys are ones to know exactly what it does and what it costs, because it's been on your radar for years and years. Anyone who doesn't know, right now, hemophilia can range in cost for a young child anywhere from $100,000, to a couple years ago [inaudible] a million dollar patient in the state of Washington. So, the important part [inaudible] treatment for hemophilia are really [inaudible] the quality of life. They are looking at an average lifespan. A lot of these advancements came out of these [inaudible]. So, when HIV presented, it presented in four groups, hemophilia being one of them. The [inaudible] that I represent [inaudible] that there was something like 10,000 who [inaudible] the treatment and that [inaudible] in 9/11, and nobody knows that. So, they have a [inaudible], and it's one of the reasons why, I think, that the drug companies are probably [inaudible] in them is [inaudible], but [inaudible] for us not to forget, not because of hemophilia, but because of the [inaudible] of the treatment. We need to make sure we have nothing [inaudible] in place to monitor patients to make sure that we know it's something we're preventing that is not supposed to be. I think that that needs to be in the back of our minds, as well.

Hemophilia is looking at having both A and B [inaudible] FDA in 2020. They are both fast-tracked. So, they are both [inaudible] likely approval by 2020. [inaudible] that's going to be interesting. They are not looking to [inaudible] children. So, they will be over that, and you have to have a systemic antibody that is not going to [inaudible]. There is also the [inaudible] for the [inaudible] last couple of years. We're also looking at . . . we have been monitoring other gene therapies that have come down the pipeline, because we knew that we were [inaudible]. One of the things that [inaudible]. So, what does that mean for payers? We also understand that for hemophilia, still you're looking [inaudible] one-time [inaudible] transfer that cost throughout the plan for other [inaudible] for so many years. The State doesn't budget for savings. So, we are looking at those different [inaudible] on how they did it. I am very vocal. So, those [inaudible] to say I feel like they had not done a good job working

on the policy of gene therapy. [inaudible] lot of money. I said, well what are you doing to make sure that this is paid for? Do you know [inaudible] to figure out how 50 states and the federal government would pay for this? [inaudible] gene therapy. So, let's work on it. So, I do feel a lot of pressure on them on, you know, what is going on with it. So, we're trying to understand [inaudible] the patients, especially [inaudible] roll out with gene therapy, the reaction of the patient, lessons learned. I [inaudible] I know you guys talk about [inaudible] a lot and [inaudible] where there is a lot of concern for [inaudible], because they are not really collaborating with the [inaudible]. My understanding is, they are trying to figure out how to do that a little bit better. Other people might be able to. So, I [inaudible]. So, a couple of years ago, actually [inaudible], there was a family foundation that came out with [inaudible] would like to kind of create a mechanism to see how we can evaluate them differently. [inaudible] a lot of the insurance companies came in and helped fund it. ICER evaluated the potential costs of the drug versus the benefit, like, the actual, like, durability and the actual rate of return on the drug. Then, they give their evaluation, usually after the FDA. [inaudible] research.

There's a [inaudible] challenging with ICER. It's, like, so hemophilia [inaudible] drugs that came out [inaudible]. [inaudible] population also [inaudible] for cost savings for our Medicare patients, going from a patient who is costing 3 million a year to [inaudible], increased level of productivity for sure on the patient and the families. So, ICER can be really, really [inaudible]. Then, making sure it was the right patient [inaudible]. I think when you look at patient [inaudible] in any disease, you have, like, [inaudible] patient [inaudible]. Maybe [inaudible] years on what it really is like to be a patient on the ground level. It's [inaudible] is that you're working with Washington nonprofits and not just [inaudible] and not national nonprofits. I can tell you the names of the [inaudible]. I'm sure you guys [inaudible]. Those are the same patients that you know, because you're serving them, as well, in Washington.

Like [inaudible] challenges of patients after treatment and [inaudible]. There's just a lot when you're potentially at [inaudible], and we'll look at what that means. Then, educational needs for patients and their families [inaudible] treatment.

This is why we're monitoring these treatment. For many, these treatments provide quality of life. [inaudible] treatments for hemophilia. These are often the [inaudible]. It's very difficult to raise money now, because I don't have any [inaudible] patients. Right? Because you guys have [inaudible] to invest in [inaudible] treatment that is allowing you to no longer be attached to a pump. I have moms that are now starting their own businesses, willing to go and try other jobs, because that's [inaudible] intense treatment that was for that [inaudible] patient [inaudible] alleviated. Then, the families who do not have an [inaudible] attached, which [inaudible] treatment are shocked by the level of freedom this is providing [inaudible]. They did not now . . . because you can't, as a parent. Right? When you're a parent with your kid, you can't admit the burden, because you're full of heart. Right? And it would be horrible to your child and [inaudible] minute. I was talking to a mom this morning, and she said, I could never cry around this one child. [inaudible]. They're providing that. So, I think that we have to find a way to capture that, and if we can find a way in Washington maybe with the Health Care Authority, because [inaudible]. I think it's important. One other thing [inaudible], and I'm hearing it [inaudible], is the patients really understanding what we need [inaudible]. [inaudible] treatment that we're talking about. Anything we talk about, hemophilia, [inaudible], and the gene therapy, too. Gene therapy was at the beginning of the conversation 20 years ago with hemophilia, and the patient population is inherently comfortable with the thought process now that it's almost here. There are just so many questions that people don't know. How do we help them really understand those questions [inaudible]?

So, how patients define a cure. I think that I've [inaudible] when I started here about nine years ago. We [inaudible]. So, patients thought they were going to have a lifetime of poking. So, I've watched the evolution of a cure be redefined almost [inaudible] hemophilia. [inaudible] treatment for sure, whether you're looking at your [inaudible], just going from 150 [inaudible] to [inaudible] some severity and that for some of the gene therapy the treatment every year or that I don't have . . . every ten years [inaudible] condition. [inaudible]. I think that with [inaudible] for the payers, but what does it mean for a family if there is one less [inaudible] they have to [inaudible] their child a week [inaudible]. That's 50 times in

a year, and that's more times that the parent arrives [inaudible]. I don't know. [inaudible]. [inaudible] treatment options [inaudible].

What do patients need? Education on what gene therapy and emerging therapies are. I've been talking with a lot of different folks on how to do that. I think that it's going to have to be in a real collaborative way. Patient advocacy leaders are trying to figure out how to educate, but how do, I'm trying to figure out how to say it [inaudible]. Right? I think we need to rely also on the people that are in the room. When I started working in hemophilia, I [inaudible] the doctor [inaudible] program. At the time, he was [inaudible]. You guys are already [inaudible], and you have [inaudible], like, you need to help the patient advocacy group, [inaudible] too. [inaudible] easy for us to get information from the drug companies. So, what are we supposed to do? Right? When it comes to new treatment and consequences [inaudible], [inaudible] payer models but also looking at the patient model. I think if you want to talk about the elephant in the room. I'm really good at that. [inaudible]. I think that that is something that we should look at in a lot of detail. So, we provide really [inaudible] sickle cell [inaudible]. We do have a chance for a [inaudible]. Then [inaudible] 8 to 12, teach them how to cope with the needle. Now, we are teaching them about how to [inaudible], because [inaudible]. We [inaudible] conversation with a provider, how to work with your insurance company, advocacy, and how to work . . . how to talk to your buddy that you have a bleeding disorder. How do you talk to your [inaudible]? So, we do a lot of education with our families. We [inaudible]. I think that's really helpful. For any of us who have had a sick kid, whether it's the flu or something really traumatic, like sickle cell or hemophilia or cancer, it's vital to have people to help you figure out what to do. That's so hard. I think that there's different ways. I think we've tried to work on that through the years, like, with the insurance companies having individuals you could talk to. [inaudible]. So, that's, I think, part of the same [inaudible]. So, here . . . one of the things that I've been thinking about, since the last meeting, I've had that opportunity to go to a conference and talk to different [inaudible] leaders and all of this kind of stuff is, I heard you, the payers on both sides, say patients understand [inaudible]. They understand the magnitude of their purchase and all of those different components. So, one of the things [inaudible] treatment, especially if there's a little time, [inaudible]

patients need to know how much time do they have to decide. I mean, this is a really big decision. With a transplant for people donating, to understand more [inaudible] timeframe [inaudible]. Gene therapy [inaudible], how many weeks do you have to make this decision? Hemophilia, you have so many options. Gene therapy is one of many. For the one of many, or the one you can wait for doing, [inaudible] look at mechanisms to develop [inaudible]. So, pretty much [inaudible]. If you have gene therapy, you're no longer [inaudible]. So, [inaudible]. Change a lifestyle. So, do we need to screen folks beforehand for [inaudible]? Especially, if you've had a disease that had chronic pain, other things [inaudible]. [inaudible] before you're ready for different kinds of transplants to make sure you're keeping these [inaudible]. So, maybe those are some other things to look at. Maybe we [inaudible] mental health evaluation to understand what it means to not have this condition anymore. [inaudible] changing so that holistically they are able to accept it, and that they really understand their choices. I think that's something to look at. [inaudible] to say that, but I think it would be [inaudible] for [inaudible] other conditions, and [inaudible] really don't know the longterm impact for [inaudible]. I don't know what to say about that, but patients really need to understand that. I think that patients need to understand, I heard this when I was chatting [inaudible] with sickle cell, hemophilia has been exposed to gene therapy for 20 years. Right? [inaudible], cancer, but not everybody knows how long or how many are being tested on gene therapy and all the different mechanisms. How do we make sure that nobody feels like they're the ones that are [inaudible] tested on, and again, [inaudible] a lot of change modeled [inaudible] educate [inaudible].

Mike Bonetto: Questions?

Female: [inaudible] Then there are also the cost implications with that. [Inaudible] That's something that I always like to have a conversation around, how do we educate a realistic expectation amongst patient providers, payers, and other stakeholders [inaudible].

Stephanie Simpson: [inaudible] time [inaudible] going to the ER. Now, they have a [inaudible]. So, now you're looking at more ER visits, [inaudible]. I think there is [inaudible] conversations are [inaudible]. I think that's what I

was hoping [inaudible] conversations [inaudible], because the payers and the provider and the patient don't necessarily all get together [inaudible]. But what are all these other components, and how do they all work together. [Inaudible] I think is a really big deal. I think for gene therapy and hemophilia, you guys are also going to have to look at [inaudible] for hemophilia [inaudible] 50% [inaudible] gene therapy [inaudible] 45. [inaudible] on the severity of the disease. It's always [inaudible], and for [inaudible]. I think on that sickle cell, I'm really excited to hear that [inaudible]. Hemophilia [inaudible] long time. So, I don't have the answer [inaudible]. That's why I say there are so many wonderful stories [inaudible]. It's just a crash course lesson and how we're going to do that [inaudible]. We have more safeguards for patients, and we're getting that patients, even if they have gene therapy, they may have to go to their doctor more regularly. Some conditions are more used to that than others.

Mike Bonetto: Any other thoughts?

Female: One thing that I'm hearing [inaudible] more education, and one thing to advocate for what we do [inaudible] for things like that is an incredibly personalized [inaudible]. So, you, when you're talking about the percentage, sometimes their eligibility for trial and [inaudible]. When you're going through your algorithm of [inaudible] appropriate for the patient [inaudible] discussion, [inaudible] take one to two hours of time. It's hard to [inaudible] and apply the [inaudible] at the same time. I think from a pediatric [inaudible] performing gene therapy would be [inaudible], because you're also making [inaudible] for those that are [inaudible] bone marrow transplant [inaudible] children [inaudible] gene therapy. So, research is always FDA approved, it would be having all of those individuals [inaudible] with the doctor, because what I'm worried about is sometimes some of the, I don't want to say folklore, because there is experience, but some [inaudible] communication in the interpretation of what things mean. Even when, for example, when we have an oncology patient [inaudible], our oncologists who are board trained and have bone marrow transplants, we don't allow them to talk about bone marrow transplant. We have them come to use [inaudible] chemo side. I think there's a lot of misunderstanding about what [inaudible] go through. Even though it's scary, the percentage that

[inaudible] I don't get that same number in the pediatric [inaudible]. So, it's very different. You have to [inaudible] when you talk with your people. If you're interested, I would just say make an appointment and [inaudible] covered by insured that initial consultation.

Stephanie Simpson: [inaudible]. They have to figure out, like, what [inaudible]. So, [inaudible] hemophilia mom, sickle cell mom [inaudible]. How do I talk to the patient? So, we have to leave their training [inaudible]. How to, you know, what the questions that you should ask are. How [inaudible] intimidating [inaudible]. [inaudible] very intimidating, because you have [inaudible]. All [inaudible] and all [inaudible]. Kid keeps getting an infection in his port. The mom [inaudible] interpreter. [inaudible]. That patient [inaudible], she hasn't even, she can't even, like [inaudible]. [inaudible] really understand that. I think that [inaudible]. I [inaudible] challenges [inaudible] let alone to act. [inaudible]

Female: Anybody on the phone? Last comment?

Mike Bonetto: Thank you. We're going to take a break. [inaudible] put you on the spot. I saw you come in late. [inaudible] introduce?

Female: [inaudible]

Mike Bonetto: We're repeating all the stuff for you later this morning. We're running a little bit behind. So, how about five minutes. [inaudible] We'll be back here at 10:15.

Judy Zerzan: I think Sean had [inaudible] trying to get a word in [inaudible]. Then, we'll shift gears.

Sean Sullivan: Thanks, Judy. Can you hear me? Can you hear me?

Donna Sullivan: It's not on mute.

Sean Sullivan: Hello? Can you hear me?

Group: [inaudible]

Sean Sullivan: Can you guys . . . I think my colleagues on the phone can hear me.

Donna Sullivan: He's not there.

Mike Bonetto: Well, thanks for the quick [inaudible]. Melissa, Ken, and Stephanie, thank you, again. That was really [inaudible] really having a better understanding at the patient level of what you guys are doing and working on. So, now maybe shifting gears a bit and hearing more [inaudible]. Just would love to [inaudible], then Emily, you, too. You've already talked through some of the [inaudible] of [inaudible]. I think it would be great to get your perspective [inaudible].

Female: [inaudible]

Sean Sullivan: How about now. Can you guys hear me?

Donna Sullivan: I don't know. I'm trying to. I don't know what's . . . how we got muted.

Male: I think we lost the main room.

Donna Sullivan: It says they're not muted.

Mike Bonetto: Well, hopefully you can hear us, but maybe not have any questions just yet. If you have anything, we're gonna get going with Monica and Marco.

Sean Sullivan: How about now. Can you hear?

Marco Mielcarek: [inaudible] stem cell transplantation [inaudible]. [inaudible] monitoring very close [inaudible]. [inaudible] underlying cancer [inaudible]. [inaudible] for the transplant and they get the transplant [inaudible] after the transplant. [inaudible] other patients [inaudible] insurance [inaudible]. However, this is not always the case, because we sometimes are [inaudible] insurance companies, they were [inaudible]. [inaudible] benefit from it [inaudible]. [inaudible] involve [inaudible]. [inaudible] very widely published and available [inaudible]. [inaudible] evidence [inaudible]. [inaudible] transplant and very likely [inaudible]. [inaudible] representing the insurance company [inaudible] transplant, but it [inaudible] where we get involved [inaudible]. [inaudible] somewhat not

so [inaudible] treatment modality [inaudible] centered on [inaudible] can be used. [inaudible] patients where we are dealing [inaudible] transplant with stem cell medications. They [inaudible] kind of try to get coverage for the patient, and I think that's kind of challenging sometimes. I will be [inaudible] financial discussions with patients [inaudible]. I am a physician. I am not a financial counselor [inaudible]. [inaudible] could consider [inaudible]. [inaudible] conversation. So, I have to beware about the financial impact of treatment [inaudible]. [inaudible] insurance companies don't cover [inaudible]. Therefore, insurance companies have [inaudible]. [inaudible] patient that [inaudible]. That becomes tricky and [inaudible]. [inaudible] evidence available to actually [inaudible]. [inaudible] there might be something [inaudible]. So, [inaudible], but [inaudible].

Male: [inaudible]

Marco Mielcarek: Yeah. The [inaudible] consultation all the time. Before patients come to Seattle and [inaudible] consult [inaudible] like a week ahead of time before [inaudible], chart, medical record, or medical data from [inaudible]. [inaudible] us with [inaudible]. [inaudible] medicine to somehow [inaudible] communication [inaudible]. [inaudible] consultation [inaudible] no idea what you're talking about. [inaudible] to [inaudible]. I'm not saying that I can do it all, but it's very challenging, and that is probably the best you can do and [inaudible]. [inaudible] discussions. [inaudible] we do have consultations, and we try to warn patients [inaudible] also the treatment [inaudible]. [inaudible] the right thing for all patients who come for a consult. [inaudible] transplant, [inaudible] risks [inaudible] conversation [inaudible] because that's the [inaudible] treatment [inaudible] potential toxicities, and [inaudible] treatment and thus motivated to [inaudible]. It's just [inaudible] process.

Male: Do you think there's any difference in the folks that you're treating that are on Medicaid versus [inaudible]? You said you [inaudible] medical reference. So, is that, you know [inaudible] commercial insurer [inaudible]?

Marco Mielcarek: No. I don't expect that [inaudible] Medicare is [inaudible] patients [inaudible] patients [inaudible]. [inaudible] syndrome [inaudible] not

treatable [inaudible]. [inaudible] clinical trial are patients treated with [inaudible]. [inaudible] indications [inaudible] Medicare would [inaudible]. [inaudible] Medicare indication, if you had made a strong case [inaudible]. [inaudible] transplant patient [inaudible]. [inaudible] initially insurance companies [inaudible]. So, then [inaudible] Medicare [inaudible] private insurer.

Male: [inaudible] Monica?

Monica Thakar: [inaudible] having this conversation [inaudible] has shown [inaudible]. So, we have accepted that those are [inaudible] straightforward. So, typically [inaudible], but one of my [inaudible]. So, of course, [inaudible]. They were [inaudible]. [inaudible] for probably an hour to two hours making sure it [inaudible]. [inaudible] one more time after having a group session and [inaudible] group session with probably five or six of them, which [inaudible] separate [inaudible] five or six [inaudible]. [inaudible] and none of this is paid for. The consult [inaudible], but all the add on time and the [inaudible] after hours, trying to fit it in [inaudible]. [inaudible] most appropriate plan [inaudible]. So, I'm thinking more of an [inaudible], but this is not an [inaudible] thing that happens. I think this idea of [inaudible] questions [inaudible] group, [inaudible]. [inaudible] but I agree with Marco. The families who are maybe more, they're coming from a [inaudible]. [inaudible] are the ones that are more clearcut. The underlying treatment is probably the standard of care. There is always a choice. It always [inaudible]. [inaudible] although [inaudible] child and a parent [inaudible]. So, it's just [inaudible]. [inaudible] make sure that [inaudible] understand why [inaudible].

Male: [inaudible]

Monica Thakar: [inaudible]

Judy Zerzan: Not only Medicaid but also public employees and [inaudible]. So, because we're a big chunk of the population. Whenever [inaudible] I would think [inaudible] both of those. Emily is [inaudible], so.

Monica Thakar: [inaudible] we understand the amount of patients that are in that more kind of a private plan and [inaudible]. [inaudible] because [inaudible] the impact [inaudible] social [inaudible] is really [inaudible]. So, I'm [inaudible].

Marco Mielcarek: [inaudible]

Mike Bonetto: We only get involved after a [inaudible].

Marco Mielcarek: [inaudible] I don't really know [inaudible], because [inaudible] patient [inaudible] biology [inaudible] family history [inaudible]. I'm not trying to push your buttons, but [inaudible], but I wouldn't [inaudible] all this information.

Male: [inaudible]

Female: I'm not sure Stephanie [inaudible] cover or who is covering it, but also understanding the background and the resources and the [inaudible].

Stephanie Simpson: Yeah, and [inaudible]. We were not talking about the [inaudible] and how they are going to get access [inaudible].

Male: [inaudible]

Stephanie Simpson: [inaudible] So, I just wanted to kind of clarify just [inaudible] the conversation [inaudible].

Male: But in terms of those two [inaudible].

Male: 1.8 million on Medicaid. Then [inaudible] are . . .

Female: 155 on [inaudible]. It's almost [inaudible]. Yeah.

Male: [inaudible] Again, I just want to make sure that it [inaudible] you know, we got [inaudible] on.

Female: The numbers that are uncovered are heavily biased towards Medicaid, although many private insurers in the state [inaudible]. I think this really should be part of a statewide [inaudible].

Stephanie Simpson: I, I do think it's [inaudible] for them to [inaudible]. Different challenges. Right? And we can't forget the challenges that families [inaudible].

Female: I [inaudible] Stephanie [inaudible]. [inaudible] is that she [inaudible]. [inaudible] are now starting to break us into our pricing model [inaudible] so significant on an actuary basis. Then, [inaudible] also is that for the insurers, [inaudible] one of the really hard things is that we have to have all the material that we send out to our numbers written at a level that is appropriate to people who are reading it, and I'm wondering if some of this transplant information that you [inaudible].

Male: [inaudible]

Female: Yeah. [inaudible]. Boy, I think that would be a real challenge to find out [inaudible] about [inaudible]. [inaudible] investigation of treatment [inaudible].

Male: Now, we can wordsmith it, [inaudible] isn't just before we get there. [inaudible]. I think it probably is Bruce.

Judy Zerzan: Is anyone on the phone? I don't know. [inaudible]

Male: Hello.

Judy Zerzan: Hooray.

Male: Hey. We were having our own good conversation on this side. Sorry you guys missed all the fun. Well, so, Judy, this is Sean. And my comment here doesn't relate to the most conversation, but to the one just before break on hemophilia. Is it still OK that I make a comment?

Judy Zerzan: Sure.

Sean Sullivan: Great. What I want to comment on is that the speaker referenced ICER in her remarks. I want to talk about that for a second, because I think it's something that we should all be considering, as perhaps part of a potential solution. So, on April 16th, 2018, ICER issued its final report on the new Gentech drug for hemophilia A. In that report, ICER goes to great length to talk about how much work they did with the patient groups and incorporating the patient voice, along with incorporating input from clinical experts. So, I want to point that out, because ICER has made very important steps to include the patient's view on a lot of their work. Then, the second is to say that Gentech drug is one rare example where after the clinical trial is reported, and more became known about the drug's benefit, it was clear that this treatment was superior to what patients with hemophilia A were using beforehand, which is a whole host of things and transfusions, etc., that were costing over 4 million dollars a year. So, when Gentech priced its drug at $480,000 per year, a lot of people were worried about that price, but the fact is that relative to 4.4 million dollars a year, it actually saved the healthcare system money. In this particular case, the company and its approach to pricing the drug was very responsible. So, I highlight that as an example of both good corporation of patient voices, second a drug that had substantial benefit that even though it had a high price it actually returned money to the healthcare system, and thirdly a company that in this instance actually made a pretty reasonable pricing decision.

Mike Bonetto: That's helpful, Sean. Thank you. Any other examples like that? None. OK.

Female: [inaudible] So, it is a very [inaudible] social workers that [inaudible]. So, we try our best, but we [inaudible], but it is [inaudible]. [inaudible] happened to be there [inaudible]. [inaudible] where we try to have our [inaudible] available for child [inaudible], but if [inaudible] is not available, [inaudible] family [inaudible], and they were all floating around the room and [inaudible]. [inaudible] I mean, I was [inaudible] here where there is not as much [inaudible]. [inaudible] visitors who come from international [inaudible]. [inaudible] around disability [inaudible].

Male: [inaudible] What if the patient has a 24/7 caregiver? Who can take that four months off work [inaudible]? [inaudible]

Female: [inaudible]

Male: [inaudible] life-saving treatment from a patient, that's because your [inaudible] economically disadvantaged, and because [inaudible].

Male: [inaudible]

Female: Yeah. I don't know [inaudible]. [inaudible] population, there are [inaudible]. So, [inaudible] issues [inaudible] and [inaudible]. I mean, I had mentioned that we were getting [inaudible] part of what we're looking at is how do we really [inaudible] populations [inaudible]. [inaudible] medication regimen, but one of these other therapies [inaudible].

Male: [inaudible] more affordable and [inaudible]. [inaudible] to focus on credibility and [inaudible] apparently [inaudible]. [inaudible]

Male: Can we try this? We've got some slides [inaudible].

Emily Transue: [inaudible] I wanted to completely redo what I was talking about. So, [inaudible]. So, I'm going to pull us back a little bit, back into the [inaudible]. I want to talk a little bit about trips we've been making. So, this is a formal [inaudible], which [inaudible] work together [inaudible] outcomes [inaudible] patient [inaudible] and values. [inaudible] tapped into that and we'll sort of compact that a little bit. So, [inaudible] normal practice, and [inaudible] confident that Monica and Marco are [inaudible]. But I think that many of them [inaudible] clinical practice kind of think of themselves as doing this, but aren't necessarily as [inaudible]. [inaudible] that we'll talk about. [inaudible] interviewing an old communication that are really important that are taught in medical schools, but thinking about how is actual shared decision making a little bit different. Really shared decision making is a structured way of doing exactly Monica has been talking about, dealing with families, and what Stephanie brought up earlier, thinking about really, do people understand what they're getting? Do they know all of the grounds on which they should be [inaudible]? How do they think about decisions in relation to their values? So, a couple of things that have come up in this

[inaudible]. That question of a patient who is deciding I may have [inaudible] therapy, and if I end up spending the rest of my life with a chronic disease, there's no right or wrong answer there. That's about somebody's values. [inaudible] with them being able to make [inaudible]. Am I OK with infertility? So, these are different from the situations we go to and say, this is [inaudible]. You think about [inaudible] beginning to know, before you say [inaudible] and needing to know what's right and wrong, motivational [inaudible] kind of that I really want you to . . . I want to change your behavior and get you to a point where you're seeing the way I am. You're [inaudible] and figure out what the barrier to that. Shared decision making really looks at that space where there are multiple appropriate options and [inaudible] in the way they [inaudible] somebody's values.

These are kind of their clinical [inaudible]. You could do it. You could not do it. They're pretty much the same. Or [inaudible] about [inaudible]. So, we really don't know how long this is going to last. We really don't know the . . . what happens down the road. These are situations where we're really helping the patient deeply [inaudible] the options and make a value congruent choice, which is particularly credible to them.

One of the components of this, and in many ways the things that [inaudible] the thing that we've been talking about around the table and making it into something that is really structurally addressed in [inaudible], and list some tools that we'll talk about. So, ensuring somebody understands the conditions, make sure that they understand all of the reasonable options, the risks, benefits, the pros and cons of each of those, really eliciting those patient values and exploring how this decision will play out relative to their values. Then, sharing that decision, which we're not always [inaudible]. It's a little different [inaudible] the right thing for you may be different than the things that I would [inaudible].

A couple of examples [inaudible] so things like joint replacement and spine surgery. You don't necessarily have to do that, but different components of what you're going through could push you in one direction or another. It can also give [inaudible] to really sort of lower level decisions. Do you want to take a statin for your cholesterol?

[inaudible] These are the potential effects and your risk of heart [inaudible]. Prostate cancer screening was one that was really [inaudible] where this was developed. Again, it's sort of a [inaudible] prostate cancer, but it also may increase your risk of interventions [inaudible] problems with [inaudible] and [inaudible]. Where are your values falling in that? Then, end of life care, of course, another very kind of well-developed area in this regard.

` Here are some very interesting data around really systematic use of shared decision making, not just finding that it can improve patient experience and health outcomes, there's really interesting work around impact on health disparities. So, looking at populations that typically underuse certain healthcare [inaudible]. For example, [inaudible] very, very low risk of surgery, probably inappropriately low relative to the quality of outcome. Use of the structured shared decision making process and patient decision aid tool really brings the level [inaudible] down in some other populations that are [inaudible], but this is, I think, one of the tools of the [inaudible] help even out some of those societies not addressing kind of the fundamental social [inaudible] but at least according to the evidence, really helping to even out the amount of [inaudible] population.

So, most of these studies have shown involve the use of an aid. Patient decision aids come in lots of forms, but especially their tool that lay out all the [inaudible] and help them make sure that the doctor [inaudible] with the patient through [inaudible]. It can be as little as a one page [inaudible] doctor during the visit. It can be a two-hour long interactive video where you watch people describing their [inaudible] different choices and [inaudible]. There are lots of different forms to these, but each of them achieve that goal of making sure that the patient has accurate information about the options, that they explore their values, and that the conversation with the doctor is really structured.

Where do we fall into this? So, there's some history that I won't spend too much time on today, but there have been foundational legislations to support this in Washington State about ten years ago. It was in response to really looking at the degree and variation of [inaudible] trying to reduce duration and approve appropriateness of utilization. Some

components of that were creating a role for the Health Care Authority certified decision aid. They are [inaudible], and they are a very valued [inaudible]. There was one on [inaudible] where you could enter in information about your problem, your values and everything there, and it [inaudible]. [inaudible] to have a process that helps to really use specific criteria to identify. It is also part of our [inaudible] support this and our role in [inaudible]. There has been a lot of implementation work around the state for a number of years, Group Health, now Kaiser, has done a lot of work on this. I know [inaudible]. There is a free implementation workgroup, which has been going on this year. I am thinking that [inaudible] that are out for [inaudible].

So, healthcare where we are, we certify aids that we really think about how to promote this work into Washington. We have an opportunity here around emerging therapies, and this is sort of self-evident, but really if we have a high degree of uncertainty, a lot of this is [inaudible] around this room, who is going to benefit? How big is the impact? How long-lasting will it be? Short-term, longterm, are many of the questions that many of our earlier presenters [inaudible] around therapies. These are critical things to think about and talk about, even if we don't [inaudible] people make a decision about going forward with these. Again, core patient values, very much in play. Risks can be high, benefits could be high. The financial toxicity, as we've talked about, could be high. Really understanding somebody's individual values in relation to the [inaudible] is very, very important.

We have heard concern from the advocates around people being pressured emerging therapies. [inaudible] Am I going to have to, because I don't want to, or I don't get to choose [inaudible]? I think this is a place where we're seeing a lot of concern in both directions. I [inaudible] these are [inaudible] want people to be making a decision that's wrong for them. They should have access when it's right for them to have access, [inaudible] know that it's right for them before they make a decision. All of these conditions kind of scream shared decision making to me in what we're talking about. We've talked to other states about whether they were involved in shared decision making [inaudible] therapies that essentially will be approved for everybody is [inaudible].

There are some challenges. Again, the use of patient decision making has really been shown to be important in making sure that this process happens in a really high fidelity way, and I think, [inaudible] a structure that will be [inaudible] necessarily easier in a high fidelity way, and aids are important to that. There are really [inaudible] to make. You have to gather all the evidence. You have to put it together. You have to [inaudible] overwhelm people. So, normally, most of these things are proprietary and are usually developed for higher volume conditions. [inaudible]. It's good to have a [inaudible] ratio [inaudible] level here. I think that if this group decided that this was a [inaudible] thing for us to do, I kind of gave us direction to work on that. We [inaudible] sort of exploring different ways that we might help to support [inaudible] the material [inaudible] commercial development.

This kind of replicates the other side. So, that's kind of my introduce. We do a lot of work around this [inaudible].

Female: So, is this [inaudible]?

Emily Transue: Absolutely, involving [inaudible]. We've got that the other team members who are very important to them. Often, there is a good [inaudible] to the [inaudible], but [inaudible].

Female: So, using this [inaudible] method [inaudible] first slide. When it [inaudible]. I thought this was all around the world, but [inaudible].

Emily Transue: I do have [inaudible] there for 30 years or so, this definition is the one that [inaudible].

Female: And I, I, uh, it's great, but I [inaudible]. [inaudible] decision making, then explain to me why am I [inaudible]. Nobody had told me that [inaudible]. [inaudible] and they say [inaudible] or you don't need antibiotic. Now it's just [inaudible].

Emily Transue: I think you're [inaudible].

Male: [inaudible]

Female: [inaudible]

Male: [inaudible] University of Washington and [inaudible] individualized consult [inaudible].

Mike Bonetto: [inaudible] those certified tools that [inaudible] medical associates [inaudible].

Female: [inaudible] criteria [inaudible].

Emily Transue: There are a few things that are triggered by certain [inaudible]. There is heightened legal protection in the State of Washington. When a certified aid is used. So, normally if [inaudible] the burden is on the provider to prove that [inaudible]. With the certified aid was used actually, the burden goes to the patient to prove [inaudible]. In fact, a lot of people come to our site and look at our aids when they're looking for high quality aids. So, it's not a push, but there is a lot of pull of people are out there looking for high quality and we are apparently the only organization that is doing that. The hope from the beginning was the National Quality Foundation or someone else would take over this in a high volume [inaudible] not able to [inaudible] replicate.

Male: [inaudible]

Emily Transue: [inaudible] proprietary, they would have to [inaudible], and there's a [inaudible] other [inaudible].

Female: [inaudible] pharmacists? Or is this just [inaudible]?

Emily Transue: That is a great question. At this point, the [inaudible] are all in [inaudible], but I don't see, I mean, as pharmacists, the [inaudible].

Male: [inaudible]

Emily Transue: [inaudible] Absolutely. [inaudible]

Female: [inaudible] earlier [inaudible].

Male: [inaudible] It is what it is. [inaudible]

Male: [inaudible] a sense of prioritization. [inaudible] So, you've got this [inaudible] upcoming [inaudible].

Emily Transue: We go through a number of things. I think there's the question of what's getting developed, and there's the question of what we're certifying, and I don't think the certification needs to be actually part of the publication. We would figure that out. We [inaudible] based on state priorities based heavily on the BREE collaborative areas of focus. It included [inaudible] [inaudible] number of areas that they find if you have focus on, I think we will be sort of working through those. So, a number of factors go into it, but it's essentially, priorities defined one way or another.

Male: [inaudible]

Emily Transue: [inaudible] develop them based on the [inaudible]. Then, we want to have a conversation with the doctors about [inaudible]. We do have an influence on that discussion.

Male: [inaudible] into [inaudible]. [inaudible] conversation earlier [inaudible].

Emily Transue: I think that's just [inaudible] to the financial [inaudible]. There's no way to force people to develop anything [inaudible] in it for themselves unless we come up with a different model. So, I think thinking about [inaudible] is important, but figuring out what's within our sphere of control with how to [inaudible].

Female: I think it's a [inaudible].

Female: [inaudible] Even at multiple [inaudible]. [inaudible] condition [inaudible]. [inaudible] perhaps [inaudible] social working, [inaudible] somebody who is not the prescriber [inaudible]. The advocacy groups would [inaudible]. [inaudible] because we know that I can't drink anymore might be a huge deal breaker. Or I have to give up carbonated sodas or, you know [inaudible]. [inaudible] covered by insurance because I have special [inaudible] special lifestyle needs that might impact the [inaudible] disorder or [inaudible]. So, these are [inaudible] take into consideration.

A lot of people are so focused on the positives [inaudible]. They're so focused on that, that they may not even be [inaudible]. So, it's a . . . it really does need to be a multistep process.

Male: [inaudible] collaborating on [inaudible] therapies [inaudible] trials are [inaudible].

Female: And I would add to that, too. Progression-free survival is the [inaudible], because sometimes [inaudible] with progression-free survival is you're [inaudible]. [inaudible] below 20% you're progression-free, and if you're [inaudible] 30%, it's [inaudible], it's considered progression-free. So, with that, [inaudible].

Male: [inaudible]

Marco Mielcarek: [inaudible] prolongation [inaudible].

Male: [inaudible]

Mike Bonetto: [inaudible] first meeting and [inaudible]. [inaudible] You guys are OK, because an hour [inaudible] an hour [inaudible] see if you want to stand up and maybe do a little stretch, but we'd love to start with this [inaudible].

Wylie Burke: [inaudible] and I want to start by saying [inaudible] complicated problems that you're already digging into, but when I think ethics [inaudible] have to offer is perhaps some ways to clarify what the issues are and to create some parameters for developing what [inaudible] or stakeholders might consider [inaudible] to approach this [inaudible]. Let me start by saying that it's very important in thinking that the questions raised by emerging therapies, the ethical considerations, it's very important to remember there are two kinds of decisions, policy decisions and personal decisions. Policy decisions really are a lot of [inaudible] should this therapy be approved for use? Should this therapy be coverage [inaudible] healthcare plan? Some of those policies [inaudible], the FDA, for example [inaudible] determining when [inaudible], but very much talking about how healthcare is. When I think about [inaudible] policy

[inaudible] policy position. Then, there's a personal decision. Personal decisions are those of the patient, should I accept this therapy or not?

So, I wanted to start first with the policy level decisions and talk about healthcare coverage policy. It's very important, and I think the obvious, but it's important ethically to stress the healthcare coverage [inaudible]. So, we do have a [inaudible], which is Health Care Authority and [inaudible]. One is the Medicaid pool. Another is the [inaudible], and the importance for [inaudible] from which healthcare funding comes is the payer resources that provide Health Care Authority [inaudible]. So, they are shared resources. [inaudible] of that, they're shared resources. We need to take into account [inaudible]. They also [inaudible] as to [inaudible] and healthcare [inaudible].

All [inaudible] offer here two definitions [inaudible]. One of them is Medicare's way of defining [inaudible]. [inaudible] treatment of your medical condition and meet accepted standards of medical practice. [inaudible] Cigna says [inaudible] exercising prudent clinical judgment, would provide to a patient. What these speak to is not [inaudible] to say [inaudible]. There's nothing personal here about patient condition. They [inaudible], I think it's important to note it's [inaudible] social component to our society [inaudible]. In other words, it really is a necessity to [inaudible] better. Besides this language [inaudible]. And the question then is, who comes together, and what process is used to decide what is [inaudible]?

And [inaudible] I think [inaudible] that those decisions are more difficult [inaudible] with their [inaudible] virtually by definition. Certain [inaudible], and all the moreso because [inaudible].

Let's talk about standards for the process of healthcare decision making. Here I am [inaudible] were done by [inaudible] and colleagues. I believe she just got [inaudible]. Actually, we [inaudible] and, uh, she has proposed that when we do healthcare decisions, healthcare [inaudible] decision about what the [inaudible]. There are four standards that she would use. One is the [inaudible] medically [inaudible]. So, [inaudible] should understand exactly why they were [inaudible], how they would [inaudible], and how [inaudible]. [inaudible] early [inaudible]. In the

terms of [inaudible], I think [inaudible] things that [inaudible] was [inaudible]. [inaudible] and why, not just [inaudible], but here's [inaudible]. This is how we [inaudible]. People are going to disagree with that [inaudible]. However, the decision making process shouldn't [inaudible] by promising [inaudible], and that argument [inaudible]. [inaudible] are full medical [inaudible]. Decisions [inaudible]. So, [inaudible], and they [inaudible] explain that any healthcare convention has [inaudible]. We understand what those are. That means [inaudible] used to define what methods we're using. Shouldn't we identify methods [inaudible] and explain what [inaudible] or examples? Or are we just [inaudible]? [inaudible] what the tradeoffs are. [inaudible] I would say, that is hard to do, um, and then how are those different types of [inaudible]. When we [inaudible], whose got [inaudible]. [inaudible] getting to [inaudible]. [inaudible] justification for a coverage decision. [inaudible]. [inaudible] all the necessary complication [inaudible]. That means [inaudible]. They have a valid [inaudible]. [inaudible] make decisions without the information and [inaudible]. It adheres, again, [inaudible] all the people covered. What that means is, there may be a lot of different ways to [inaudible] decision, but those [inaudible] needs to address the [inaudible]. [inaudible] our decision [inaudible].

[inaudible] particular moment [inaudible] that is [inaudible] decision [inaudible]. That is [inaudible] for healthcare health systems. Here's a therapy we will cover, and here are the patients we [inaudible]. [inaudible] therapy works [inaudible]. [inaudible] justifiable [inaudible]. [inaudible] just [inaudible], because this is where [inaudible] is [inaudible] therapy [inaudible]. [inaudible], but because it's very [inaudible], we're going to be much more cautious about patients with whom [inaudible]. If we [inaudible] that what we're talking about is an ethically sound approach to medical decision making, we have to acknowledge stress to [inaudible], the analysis of the evidence, which these decisions are made could be open to question. [inaudible] very open way. We can [inaudible] evidence, [inaudible], and [inaudible] whether [inaudible]. There could be misrepresentation of the problem. I think when you [inaudible] pharmaceutical companies will [inaudible] evidence [inaudible] rest [inaudible], but also [inaudible] that we might [inaudible]. Or are we hearing from the people who [inaudible] truly understand the [inaudible]. [inaudible] and that [inaudible]. I just want to say, one of the

main reasons why [inaudible], and so apparently, not just [inaudible] is that emerging therapies [inaudible]. On the other hand, [inaudible] because [inaudible] in which [inaudible] a situation which we would [inaudible] and [inaudible].

This is a [inaudible]. [inaudible] public and private and [inaudible]. These are [inaudible] statistics [inaudible]. We are seeing [inaudible]. So, [inaudible] described here [inaudible]. [inaudible] emerging therapies [inaudible]. Again, just to sort of [inaudible] the only alternate solution is payer content aimed at payer allocation of a [inaudible] doing our best to maximize the solution and [inaudible]. It certainly seems that some individuals can [inaudible]. [inaudible] attention to the kinds of [inaudible].

[inaudible] Now, let me return to personal decision making. So, it's very important to note that [inaudible] in context. In that context we first of all show [inaudible]. [inaudible], and they will also potentially [inaudible]. [inaudible] the social context of a personal decision, whether or not to [inaudible] emerging therapy. Having said that, in my last slide, [inaudible] too, and the [inaudible]. That is [inaudible]. Anything [inaudible] focused on [inaudible]. It had to be [inaudible]. [inaudible] autonomy is absolute. That [inaudible].

Male: [inaudible]

Wylie Burke: Yeah. I think I'm articulating something that's very important. I think I was trying to get at that [inaudible].

Male: Absolutely.

Wylie Burke: Yeah.

Male: [inaudible] very clear, very accessible. I'm going to try to get your ideas also. I think [inaudible] about this. [inaudible], if you think about things like fair procedures and stability, we run the risk of having [inaudible] exacerbated [inaudible]. Something's bothering me, like [inaudible] social resources that are available [inaudible] potential [inaudible] of a transplant [inaudible] has a way to [inaudible] disadvantaged people that

we're trying to keep out of the [inaudible]. So, you know, these things kind of run not just [inaudible]. [inaudible] as [inaudible] to take a 30 minute walk in the evening [inaudible], very plausible for me, but given the certainty [inaudible], stay home [inaudible]. So, I wonder if you had some ideas. I'm not expecting to solve this problem, just [inaudible] trying to protect against [inaudible] criteria, which are necessary for the reasons you've laid out. [inaudible] exacerbated to [inaudible] discrimination, bias, and [inaudible].

Wylie Burke: Yes. No. I'm really glad that you're making that point. I think it's very important. If I might say that the first ever to address that problem in [inaudible] to make sure that the point of view is correct. In other words, [inaudible] population, and if we take [inaudible] coverage decision ought to [inaudible], then I think one of the things we have to [inaudible] reality, if we're [inaudible] then every [inaudible], every [inaudible], and our goal should be [inaudible] condition in our system should have that therapy. To do that, [inaudible].

Male: [inaudible]