

State Health Care Authority Meeting
June 18, 2019

Sue Birch:

Great for those of you on the phone. We're just getting people seated and we do have a plane that just landed late about 10 minutes ago. And so we are expecting a few more to come in. But we thought it was important to get started. I meant... for everybody in the room and those of you that don't know me, I'm Sue Birch. I'm the Director of the Health Care Authority and I'm pleased to be with you all here today in Spokane.

I just really want to thank everyone, and especially Rep Schmidt back there. You want to raise your hand? In a few minutes, we'll be doing some deeper introductions and making sure those that are on the phone and those are in the room share with us a little bit about themselves and the work at hand. But we're going to skip to that in just a few minutes because we're trying to buy a little more time till two more of our folks come in. So thank you.

Really for making this trip to Spokane and really helping our state be better prepared for emerging therapies and issues that continue just to pummel us. It's very exciting and also a little scary. I like to think that you are all fellow futurists and helping us kind of stay ahead of things but also really to plot kind of our course forward. And I think now, more than ever, we all understand better how things just continue to so rapidly cycle and change. One of the things before I turn this over to Dr. Zerzan that I wanted to give folks in our state and just a huge shout out about is last week's announcement from the Commonwealth Fund. The Commonwealth has been responsible for the scorecard of state health system performance. I believe this might be almost their tenth year, not quite. But Washington moved 10 channels to the number four spot in the nation. So there are five domains that are state gets ranked on. There are five dominant scorecards in the country. And this one really is probably the most rigorous academically. There are several other good ones, but this one gives us another slice. Those of you that know me, I know I'm a nurse and I always compare things to the medical field, but this is like an MRI. It gives us another slice of information and data about how we're doing. It helps us rank.

So overall, we're fourth in the country. We changed from the baseline 10 points. It ranks us in five domains: access and affordability, prevention and treatment, avoidable use and cost, healthy lives and

health care disparities. We... we moved two spots from the baseline on avoidable use and cost and health care disparities, but it really was remarkable. They are now studying kind of the big movers in the country. And they are making lots of calls to us and trying to understand what are the components that help states move faster up in the rankings? So again, we don't often stop and celebrate the successes of what we're doing, but this was really amazing. And so we need to stop and celebrate because there's no shortage of work that's coming before us. So kudos to you all and helping shape Washington systems.

I am really pleased now to introduce you to Dr. Judy Zerzan. I figure it was like probably one of the most important things I did. I stole her from Colorado. I recruited her to come back to her homeland. She has spent lots of time in the Pacific Northwest and knew many people, but she and her partner and three kids were wonderful enough to make the jump and come join us in really leading the country in health systems transformation. So Judy is going to speak with you and take you deeper into this emerging work. And I'm already getting one big announcement for those of you that are struggling right now with Wi-Fi stuff. Here is the password and whatnot too as we listen to Judy stall and create more time for those that are joining us. So thank you all for being here and I look forward to spending the afternoon with you. Dr. Zerzan.

Judy Zerzan:

Great. Thank you, Sue and thank you for your leadership. So today we're here to talk about emerging therapies and emerging therapies is a super broad term which really means almost any of this new pipeline of drugs that is coming down the pike at us. That largely have to do with genetic diseases, with cancers, with some rare diseases and all of them are the... the one commonality, I'd say maybe there's two commonalities. One is, they're very expensive. And the second commonality is there are relatively small group of people that they're intended to treat. And so this was a topic and the sessions legislature both myself and Donna Sullivan, who's our chief pharmacy officer gave presentations to the legislature about what is coming down the pike. How does Medicaid look at these therapies? How does the Health Care Authority, in terms of the state side of coverage, and soon the teacher side of coverage, look at these therapies? How do other insurers look at these therapies?

And so we brought together this group under guidance from the legislature to create a plan, essentially, of how does the state of Washington want to approach this? What are the potential benefits

of some of these therapies? Which I think many of which can be life changing. But that being said, many of them also carry some risks. Some of those risks are unknown, because they are brand new drugs and how... how do we pay for some of these things? So, I'd also... while I'm doing introductions I'm going to be leading us on this journey. There are four meetings that you all have agreed to. This is the first one and the goal of this meeting is to sort of set the stage and the landscape. And then we'll have three more meetings over the course of which we hope to create a plan and some direction about what's important for us as a state for these emerging therapies. So Donna Sullivan, I think, who many of you know, is our chief pharmacy officer and a lot of this is under her guidance and then Leta, you want to raise your hand? She is the project manager extraordinary for us that is bringing together all the details. And for those of you that need reimbursement you've probably already connected with her, but she can help you sort that out. And then Mike Benetto I'll ask him to come up in just a minute. But he is in a contract with us. He works for the Center for Evidence Based... he works for the Oregon Health Sciences Evidence Based Practice Center. He is going to facilitate this and we thought it was important to have sort of a neutral convener to kind of get people to talk so that the HCA could be part of this group.

And so with that, I think we'll start going around the room, because I think it's important to learn who all is here. So when you speak, please introduce yourself. Say where you're coming from and if you are representing a particular sector, because there are a number of sectors we specifically sought out to get their expertise and then sort of why... why this topic is interesting and what you hope to discuss. So Cody, I'll start with you.

Cody Gillenwater:

Okay. I'm Cody Gillenwater a medical director at Regents, from the payer sector and have been involved with emerging therapies, gene therapies since really the [inaudible] got approved and helping put together our strategy on how we're approaching those and how we approached the gene therapies that are to come. And you know I think it's... it's an area that is intriguing, both from the clinical care side, but also from the system side and providing therapies to conditions that may not have any other options, but also may have very little evidence. So, how we approach and find a balance of enabling access, but also understanding that we need some strong evidence for these.

- Judy Zerzan: So I'm going to interrupt for a moment. For those of you on the phone. If you could please mute your line. So we don't hear extra noise and then I'll ask you to introduce yourself after we go around the room.
- Carly Rodriguez: I am Carly Rodriguez pharmacy director of Moda Health also representing the payer sector and specifically the commercial sector is why I was asked to participate. I have responsibility for our clinical team that evaluates evidence for merging therapies and really my interest is really understanding and learning from others and participating in the conversation of really, how we move forward in a space where there's therapies that bring clinical promise? They may not bring as strong of evidence and how that meets with affordability issues on both the government and the commercial side.
- Petra Eichelsdoerfer: So my name is Petra Eichelsdoerfer. I am the pharmacist account manager for United Healthcare. I manage the Washington Pharmacy Benefit of the Medicaid line of business and I am here because I have a very, very strong and ongoing interest in how the future looks and the... balancing the benefits and the risks, including the financial as well as the cost of not treating individuals with very human means that are out there.
- Vicki Evans: I'm Vicki Evans from Molina Healthcare and I'm not sure why I'm here as I look at everyone's credentials. I oversee the integration into physical health care. My role really is related to looking at models of care evidence basis and [inaudible]. And so I will take what I can and make sure we have the right people at the next three meetings and I will be judicious note taker for myself.
- Yusuf Rashid: Hi. I'm Yusuf Rashid and vice president of pharmacy and vendor relationship management Community Health Plan of Washington in Seattle. So we are mainly a managed care... managed Medicaid plan, but we also have a Medicare product as well which about half of them is special needs on our Medicare as well. I oversee everything related to the prescription drug benefit, drugs only medical benefit and the vendors that support our medical management programs. I'm excited to be here because I think challenges like emerging therapies that we're going to confront... these are opportunities to be really revolutionary and force the changes and improvements in our system that maybe otherwise we could ignore year after year and these emerging therapies, the threat presents us a tremendous opportunity to revolutionize how we do healthcare.

Dawn Sanders: I'm Dawn Sanders. I'm at Susan G. Komen Puget Sound Breast Cancer Foundation. So always really concerned about the costs of medical therapies for our constituents. So I'm excited to learn more about what this group is doing.

Stephanie Simpson: My name is Stephanie Simpson. I'm the executive director of the Bleeding Disorder Foundation of Washington. We represent bleeding disorders and advocate for rare disease and we worked with Representative Smith on this to make sure it took place because hemophilia, as anyone who is a payer, is very familiar, it's extremely expensive. But it's also on the forefront of emerging therapies and gene therapy. But we think it's really important that we don't create policy, just for hemophilia. That we make sure that all the diseases that are coming behind us and patients on have the same access to care that hemophilia patients are able to access. So thanks for being here.

Melissa Tribelhorn: My name is Melissa Tribelhorn. I'm with Northwest Parkinson's Foundation. Our goal on the policy side is to balance innovation with affordability. So one thing that many people don't know about Parkinson's disease is that people have been using the same meds since the 60s. We haven't had a lot of innovative treatment since then. It's also a designer disease. So most people with Parkinson's it's very unique to them. There have been about 16 biomarkers discovered so far for Parkinson's disease. We believe there are a lot more and that most Parkinson's disease patients are genetic factors, rather than environmental so we're very excited to be part of this emerging therapy work group and get some better treatments and therapies out there.

Jonathan Espenschied: Jonathan Espenschied. I'm not 100% sure... I think one of my colleagues threw me under the bus. My background in my prior life was clinical trials. Pre-clinical, clinical phase one studies have been heavily involved in a lot of the work that we've done in Comprehensive Cancer Center out of the state. So this is one of my interests. This is one of the areas I would teach our residents and fellows, so something that's very much of interest [inaudible].

Armen Khatchatouria: My name is Armen Khatchatouria and I'm Senior Director of industry relations at Formulary Consulting at Optimum Rx also representing the payer side. I work primarily the commercial segment with health plans and employers to go through formulary development while also bringing in physical transparency around all those various decisions that are made and give sideline into not only finances of

manufacturer contracts, but also a bit of contracting opportunities, value based, outcomes opportunities and also contracting opportunities on the medical benefit as well. So as we move forward in trying to find solutions to better creative health care landscape, I'm glad that we're all able to come to the table and have these discussions. Thank you.

Tom May:

My name is Tom May. I'm with the [inaudible] College of Medicine with Washington State University. My background is in bioethics and I've spent the last about seven to eight years working in the area of genomic technologies and its application to genomic medicine and I am interested in looking how we can fill disparities and access to treatment care among those who have rare diseases and also in making sure that we are effectively spending money at a population that will benefit as many people as we can.

Rebecca Owen:

My name is Rebecca Owen. I'm in consulting actuary from Yachats, Oregon and I principally focus on pharmaceutical topics but for all lines of business. And of course as an actuary we're really very focused on affordability and quantifying both the cost, but also the offsets of the cost of emerging therapies, but several years ago, I took a sort of a jog in my career and suddenly became bitten with the public health bug. So now I answer to both disciplines, the affordability and the access but as an actuary always.

Jean-Baptiste Rouillet:

My name is Jean-Baptiste Rouillet. I go by JB, for those who cannot pronounce French correctly. I'm a Clinical Professor at Washington State University College of Pharmacy, the department of pharmacotherapy. I have training in pharmacy clinical chemistry and research and have done rare disease research for about 15 years now in very specific areas, mostly diseases that impact kids with genetic disorders. I know what it costs. What it takes in terms of dollars to do research on rare diseases and developing new accounts and testing new compounds. I have no experience in how this translates into the cost of drugs for patients. I know it's very expensive. But besides that... so why I'm here? I don't know exactly. But I will learn and if I can be of use I'm willing to be part of the team.

Donna Sullivan:

I'm Donna Sullivan. I'm the chief pharmacy officer with the Washington State Health Care Authority. The reason why you are all here is that there was... Representative Schmidt can introduce some legislation over this past session trying to create this work group and we asked him, and he graciously agreed to take it offline and there were... we wanted to bring together a bunch of different

stakeholders. So people that were experts in rare disease, patient advocates, the payers, everybody in the room so that we can talk about this together because we're all in it together and then a medical emphasis as well. So that's kind of why we're all here. I'm here because what the Health Care Authority... we manage the Medicaid population, the public employees and soon the school employee, so we're managing the health care for about a third of the residents in the state of Washington, the pharmacy budget alone is over \$2 billion. That's a B and \$10 billion in overall health care costs. So we need to figure out, how are we going to treat the patients that have these debilitating diseases with these new therapies and also be able to afford to continue to provide basic services to the rest of the population? So that's why we're here.

Sue Birch: Again, I'm Sue Birch. I am a nurse with an MBA and really to have teams and interested parties that come together was part of the attraction of coming to Washington and continuing this very transformative work so to have a process where we can really focus on value meaning cost, quality and member experience and really engage in this arena and be very futuristic about being prepared for what's coming out of this is very, very exciting. So again, thank you all for being in on this.

Robin Williams: I'm Robin Williams. I'm the budget assistant to the governor for Medicaid and affordable health care at the office of financial management and I think my interest, not to be too on brand, is to, you know, provide the most care for people that access service, as well as stretching the dollar as much as possible, so I'm looking forward to that. I think the third one.

Judy Zerzan: Excellent. So can we go into the back?

Amelia Davidson: I'm Amelia Davidson, coordinating care and our senior pharmacy director, Kerrie Fowler, is on the phone, but we have our HCA team auditor today. So she's popping in and out. So I'm just make sure she has notes about what happens today if she doesn't make it.

Terri Levien: I'm Terri Levien. I'm a clinical professor here at the WSU College of Pharmacy and Pharmaceutical Sciences. I work in our drug information center on reviewing new drugs as they come along for formulary helping P&T communities evaluate drugs for formulary. So I've been asked to give just a broad overview of some of the emerging therapies.

Sandy Stith: I'm Sandy Stith. I'm with the Senate Ways and Means Committee. I do low-income health, benefit exchange and the Office of the Insurance Commissioner are all friends of my portfolio. Robin is my counterpart at the Office of Financial Management. I have similar interests to Robin for why I'm here. I am very interested in the serious components. I don't want to sound also like I have no feelings about how this impacts of the lives of people I absolutely do in working... how we do rate setting for this. Sometimes we get very focused on just what's the cost of a thing is a drug, a service, it's very helpful to listen to other perspectives and other areas where this actually impacts people's lives, how this works in different ways, because there are other things, fiscal as well, again, not to sound cold or too on brand here either, but it's useful for us to be able to talk to our members like our representatives and they can say, you know, it isn't just a cost in this one area or a savings in this one area. There is something over here too. So I think this will give me a more rounded experience to be able to present a more clear, fuller picture to my members as well.

Joe Schmidt: I'm Representative Joe Schmidt. I represent the 9th legislative district.

Leta Evaskus: I'm Leta Evaskus with the Health Care Authority. I'm going to be setting up these meetings. I'm going to be your contact for meetings and for those of you who I'm reimbursing for travel if you didn't pre fill out the form, I printed one and put it next to you. So if you could please fill that out and sign it and if you need help with that I can make sure you fill it out correctly before you leave today. Thanks.

Mike Bonetto: Hi. I'm Mike Bonetto, OHSU.

Judy Zerzan: Great. Now we will move to the phones. I can see your names so I'll call you out one at a time. I think David, do you want to start?

Woman: It's client 10.

Judy Zerzan: Oh it's client 10. Who is client 10? It looked like David from here.

Dan Kent: That's probably Dr. Dan Kent from United Healthcare.

Judy Zerzan: Thank you. Dan, do you want to say a couple words about why you're interested in this topic?

Dan Kent: Vitally interested in new therapies, some of which have been really dramatic recently, and we all are very, very conscious of financial stewardship as well.

Judy Zerzan: Great. Kerrie?

Kerrie Fowler: Hi. This is Kerrie Fowler, senior pharmacy director with Coordinated Care. Thanks for the opportunity to participate in this. Sorry, I couldn't be there in person today. I am very much interested in this and I really echo what Donna had mentioned. We're really taking care of a vulnerable population and at the same time I feel the need to be physically responsible. So I'm just very happy to participate and I think we have a great group of people on the phone and in person.

Judy Zerzan: Shawn?

Shawn Akavan: I'm Shawn Akavan. I'm the chief medical director for AmeriGroup Washington. We're a Medicaid managed care plan and I am sitting here to learn more about emerging technologies of the personal interest and also as part of our fiduciary responsibility as a managed care organization to our members and the state. Our [inaudible] pharmacist is not here with me, but I work closely with her and definitely would like to put our input around this topic.

Judy Zerzan: Thanks. Mellit?

Mellit Winston: Yeah, you pronounced it correctly. Mellit Winston, I join Petra and Dan Kent from United Healthcare as a medical director here and it is such an important discussion. I think all of us are aware of how important each of the sectors that we represent need to weigh in, because it's a balance—a balance of what we all represent between the access to medication and the cost of those medications, and the awareness that there is limited funding and that we need to advocate for our vulnerable population, the Medicaid population that's not able to have [inaudible] for itself.

Judy Zerzan: Thanks so much. So Mike if you want to come up and go over our charter and our work plan and we'll start moving along the agenda. Thank you.

Mike Bonetto: Everybody in your packet you should have the agenda and then you turn that page and the next thing is the charter. We put this charter together just so there is a bit of a road map. You get a big group like

this together we want to make sure kind of everybody knows where we are starting and then kind of where we are finishing. Welcome!

Judy Zerzan: Perfecting time. We are just finishing introductions. Introduce yourselves and why you're here. Not to give you a breather at all.

Monica Thakar: My name is Monica Thakar. I'm a pediatric bone marrow transplant physician. I work at Seattle Children's and Fred Hutchinson Cancer Research Center. My area of research is k-cell adoptive amino therapies. So I work with overlap in [inaudible] immune therapies, gene therapies and other types of even rare diseases that we would treat with bone marrow transplantation.

Sean Sullivan: Good afternoon, everyone. I'm Sean Sullivan. I am professor and Dean of the School of Pharmacy at the University of Washington and I'm trying to help the cause and my specialty area for the last 30 years has been on pharmaceuticals and pharmaceutical pricing.

Mike Bonetto: Excellent. Sean and Monica, we were just getting going. You've got a pack in front of you. It should have an agenda and then we just turned the page to where you've got a Work Group Charter. So if you open up your charter we put a few things down just so everybody kind of has some context. Background, again, I think sort of kudos to Senator Schmitt in terms of putting this group together. I think this is... I've seen this happen many times when you have a difficulty policy issue and, you know, the best way is to get a group of professionals and subject matter around the table to really start to dive into this and get some details on the table. But at the same time we kind of also want to understand where we are going. If you look at the scope I think this is something I think HCA has put some time and energy into and all of those bullet points, that really is the course of the next year of spending some time kind of dedicating within each of these meetings so we can understand each of those a bit more.

If you look at the end of that scope I mean this is really where we understand kind of how we put the best charge to this group of really starting to synthesize anywhere from three to five, you know, kind of key recommendations and next steps that you're going to be... have all the information and then really looking to HCA and "now what"? What are we going to do next? And then how do we actually think about the implementation plan or what are the recommendations? So from an advisory standpoint, you know, what is this group going to come up with? We may get into that just a little bit today. This is very much a level setting day. The next three meetings we'll get into much

more detail where I think you guys are going to be grappling with, you know, what recommendations would you be making to the state?

Judy mentioned this earlier. If you look at the schedule we've got four meetings outlined. Today we really were highlighting this as a level setting meeting. We've got a lot of folks around the table. We haven't necessarily met each other before. We are introducing this top, getting some background on it, both from a private sector and also from the public sector. We will hopefully have a little bit of time just to have some group discussion to get some initial thinking based on the presentations today.

Then if you go to the next page we've outlined October as the next meeting date where we really will be dedicating some time to some case examples. I think theoretically it's always interesting to talk about kind of what is in the pipeline and what is coming down, but then when we talk about real life examples it starts to hit home a little bit more around what does that actually mean from a policy perspective? And then you'll see February and April talking about funding options and then quality oversight. I would say that could be subject to change based on kind of the conversation of this group, but this is kind of the initial outline of how we are looking at over the next year.

I think Leta and I are going to be point on a lot of this so if there are questions, comments that come up, even in between meetings, you know, certainly we'd like to be able to facilitate that and get those questions answered. Did you guys look at this? Questions? Comments around the Charter and kind of your task at hand? Okay, we're ready to go. Next up is Donna.

Donna Sullivan:

Again, I'm Donna Sullivan. I'm the chief pharmacy officer with Washington Health Care Authority. I'm going to go over just kind of a brief overview of the U.S. health care system and kind of how we got to where we are at and then kind of set the stage for the conversation for the rest of the day.

So the evolution of the health care system really started in World War II due to the war that was going on, the lack of workers, there was wage freezes, as well as price controls. And so employers got creative and they started to offer health benefits as a way to attract employees to their companies and to maintain those employees. So it really started back at World War II. It then it involved into an employment-based private health insurance system. Again, based on

WWII and then as unions evolved the health benefits became really something that the unions were going to negotiate on as a benefit for the employees of those companies. And then in 1947 we had the Taft-Hartley Act that was passed and then health benefits were declared a condition where they could actually negotiate with their employers on the health care benefits that are provided, as well as the cost sharing that goes along with the health care benefits, the deductibles, the co-pays, and anything else. If you're interested we can make these slides available to you going forward.

So what really happened? Health insurance we can buy it from the private insurance companies, we can... there's options for purchasing private health insurance. Many of it is employer offered. We have the exchange. There's self-insured employers that offer health insurance and we also have national and regional health plans. So the national and regional health plans are like United, Anthem that are here in the room. We have regional health plans such as Premera, Regence, and then the Kaiser Northwest that is also here. They may be small in size, large in size, but they are here in the state.

The employment model does not cover the elderly or the poor. So we have public options as well. So we have Medicaid which is a federal/state partnership that is state managed, but federally funded in partnership with the state. We also have dual eligibility with Medicare—so the elderly health plans. And then in 1990 the covered outpatient drug program was passed, the pharmacists in the room will know that this is over 90, which really established the Medicaid Federal Rebate Program. It level sets at a floor for pricing for pharmaceuticals across the country and it really put us into the situation now with the pharmaceutical companies and their willingness to negotiate on prices. We have Medicare, which is a federal-funded coverage for the elderly and the disabled. The Part A program is basically the hospital insurance. It is free. It was established in 1965. Part B was the physician services. They realized that patients, you know, waiting until they were in the hospital was kind of taking too long. So in 1965 the Part B program became the physician services. It is limited coverage and it is basically funded through federal tax and the beneficiary premiums. So patients have to pay a premium into Medicare Part B. Then in 1997 managed care started to become strong and Medicare evolved into Medicare advantage plans where you have a managed care plan that is actually providing the care. Medicare members can sign up for their individual care plans. United is a big provider of Medicare advantage plans. And then in 2003, finally, the prescription drug company piece came. So

up until 2006 when Part B actually became effective none of the elderly programs, the Medicare programs provided prescription drug coverage. You might have seen some of it in the advantage plans, but it wasn't a required benefit for the Medicare program.

We also have the Department of Defense, as well as the Veterans Affairs and the Veterans, you know, they provide... the VA provides health care, limited health coverage for some veterans, full health coverage for others to the tune of about 23 million American veterans in the country.

So I'm going to focus now mostly on the pharmaceutical side of health coverage. It's complicated. That is the... you just look at this particular diagram and you realize that trying to follow the dollar of how our pharmaceuticals are created and bought and sold in the United States... we have the manufacturer who creates the drugs and sets the price who then sells them to a distributor who then sells them to the retailer or the pharmacy who then sells those drugs to the patient. So we have multiple areas where dollars are passing hands, each one of those entities taking, you know, they have to have their piece of the profit for the drugs that they are selling. And then on the right hand side we have the pharmacy benefit manager who is negotiating rebates with the manufacturer. They are also setting the reimbursement rates that the health plans are going to be paying to the retailer or the pharmacy. They might actually even own pharmacies. So they might own their specialty pharmacy, their mail order pharmacy. They might have chains of pharmacies that they prefer in their networks. And then they also contract with the health insurance companies. So some of the carriers that are here in the room they might directly contract with an employer. So at the State of Washington we have a separate pharmacy benefit management contract than our health insurance contract. They also provide the pharmacy benefits for Medicare programs, for the Medicaid programs. The pharmacy benefit managers also have a big role. And again there is also dollars that are passing hands on the right hand side. And then of course you have the patient that is buying their prescription drugs and then paying their premiums to whatever their health plan is. Like I said, the best way to explain it is, it is complicated.

So now we're going to switch to kind of what is driving the health care trend on the pharmacy side? And it's really the specialty drugs and they are a major driver of the increasing costs in the United States. In 2008 about 10 years ago they accounted for about 24.7% of total

pharmacy spending and in 2017 it's up to about 46.5%. What's alarming though is it is only about 2% of all of the prescriptions that are actually dispensed. I do want to point out this is data taken from just drugs that are covered through the point of sale, the pharmacy side. This doesn't include drugs that are IV drugs or infused in a doctor's office.

The pipeline shows no sign of slowing. So the number of specialty drugs in 2008 was 249 and in 2020 we're expecting there to be over 700 specialty drugs in the pipeline. It's a three times increase over the course of 12 years. Terri is going to be going over some of those drugs that are actually in the pipeline.

We have the 20th Century Cures Act of 2016 which really spurred innovation. It facilitated the development and the approval of genetically-targeted treatment for rare diseases. It modified the FDA approval process that allowed and expedited approval with new drugs and devices. It allowed submission of real world evidence such as registries, observational studies, insurance claims data and anecdotal data. So it lessened the burden of the manufacturer to create evidence supporting the use of their drugs. It also allowed single-arm studies to be used in the approval process. So instead of having to have a placebo-controlled trial there might be a single-arm trial that's not randomized, it's not blinded and there's a historical control that the therapy is being compared to usual care based on a registry that might exist. But we've had some really breakthrough specialty drugs. We've had certain types of cancer, the [inaudible] therapies, the gene therapy for blindness, hemophilia has a gene therapy in the pipeline, Alzheimer's disease there's a lot of research around that, and then also neurologic disease. And we know that we just had the other gene... the second gene therapy approved for spinal muscular atrophy.

So specialty drugs bring exciting innovation. They also bring enormous price tags. So this was a newspaper articles about a year ago where they were talking about the hemophilia treatment might be about \$4 million. So the gene therapy for hemophilia. I think we got a bargain with the SMA gene therapy. It was only \$2.1 million. This is why we're here. We're going to start talking about these medications or therapies that are really just set at a price that are not affordable and it might actually increase the... or decrease the access to care.

So they continue to grow and influence the clinical and the financial landscape. Of those drugs in Phase III trials 60% are specialty drugs, 33% are orphan drugs, and an orphan drug is a drug that treats a disease that impacts less than 200,000 people in the United States. 13% are considered breakthrough therapies. Breakthrough therapies get an accelerated process through the FDA. So they can come to market faster with potentially less evidence that is provided. They might get approved after the Phase II trial instead of a Phase III trial and I think Terri might talk a little bit about that. In all of the applications 25% of them have been given priority review. In addition to the accelerated or breakthrough therapy designation there's a priority review designation. And priority review might be it's a novel treatment for something where there's really no treatment today.

Sean Sullivan: Donna, before you go on could you go back one slide?

Donna Sullivan: Sure.

Sean Sullivan: You've got some dollar signs and numbers underneath each drug. What does that represent?

Donna Sullivan: So those represent the estimated annual cost and I think that is in millions.

Sean Sullivan: Cost or revenue?

Donna Sullivan: I would have to check. I would have to go back and check. I'm not sure. I apologize.

Sean Sullivan: So it must be some market forecast about...

Donna Sullivan: Yeah, it's Magellan's Rx pipeline forecast and I can't remember if that's projected revenue for the drug company or Magellan being a health plan benefit manager if it is their expected annual expenditure nationally.

So drug coverage decision making. Before any decision is made for Medicaid the drug has to go through the FDA approval process. Until it is approved by the FDA it cannot be legally sold in the United States unless a patient can get a compassionate use approval from the FDA to get access to a drug that might currently be in clinical trials in the United States or it might be available in another country. But we don't have it approved in our country. For example [inaudible] which was for [inaudible] disease and it was for horrible seizures. We used

to import it from France for many, many years for patients, little kids that have these horrible, debilitating seizures, but now it has been approved. It has been approved in the United States. The FDA when they approve a drug they approve it for a certain indication. But once it is approved the doctor can prescribe it for really whatever they want to prescribe it for. We've also seen that the studies that are being conducted for some of these drugs are being very focused on a targeted patient population and then the FDA approves it for a very global indication. An example would be the gene therapy for SMA was mostly studied in infants with SMN, the spinal muscle atrophy type I, but when the FDA approved it, they approved it for all types of SMA, spinal muscle atrophy, some of it that they hadn't even studied the drug in yet. They also approved it for kids under the age of 2 as opposed to the infant population that it had been studied in. And then regardless of the approved indications, like I said, doctors can prescribe it for whatever they want to. And then the health plan can decide to cover it or not cover it, sometimes Medicaid we have to cover it if the manufacturer has a federal rebate with the Department of Health and Human Services at the federal level. Health plans might decide not to cover it, but often times they will get sued and they might be compelled to cover it. But we can control when we do cover it under what circumstances we cover.

So the decisions we have to make they are complex and they are affected by everybody's different perspective. There's a societal perspective that us as taxpayers what do we want to put our hard-earned taxpayer's dollars towards in health care? There's the patient and care giver perspective. There's the parents that know that their kid has a disease that's going to kill them before they are most likely two years old. From the payer perspective we have to not only pay for these new emerging therapies, but we have our entire population that we need to give health care to. So Medicaid for example dental care for adults is optional. So if we run out of money in our budget then often times that coverage, that care, gets cut and so, you know, we have to talk about what's equitable across all of our patients. And can we provide basic care? And then we have the supplier industry. So the manufacturer's, the life science, the researchers, they are all out there. So the decision-makers we value and how they value is complex. We try and take all of this into consideration when we are making these decisions. Washington State that we want to put forward? And can we come up with ways to try to tackle the affordability and tsunami really of these therapies that are coming. Any other questions?

So next Terri Levien is going to come up and talk to you about the pipeline. So this is really just to set the stage of we're here. We're talking about it now because this is eminent. These medications are being approved. We have two gene therapies approved. So Terri is just going to walk us through some of those. Go ahead.

J-B Roullet: Can I ask a question?

Donna Sullivan: Please.

J-B Roullet: So you have these specialty drugs that are the major driver of prospective drug costs in the United States. You have a [inaudible] of that and you talk about specialty drug of that common drugs and the total pharmacy standing. You didn't show those...

Donna Sullivan: I apologize. I don't have that slide. I don't know what the figure is in the United States. I know in our Medicaid population, public employees and school employees the total spend on drugs is about \$2 billion, \$2.1 billion. And we're at the same point, about 50% of that is in the specialty drug arena, the drug space.

J-B Roullet: I just wanted to make sure that if one was increasing the common drug cost might be decreasing.

Donna Sullivan: They are both overall going up and Rebecca can probably answer the question being the... yeah.

Rebecca Owen: It's like this. As a percent... when you do a percent then the percent is increasing.

Donna Sullivan: The specialty piece of the pie is getting bigger, but the entire pie is getting bigger also if that makes any sense. Go ahead.

Man: Two real quick questions, I think. It's just about skills of our worker to get into my head. So I understand we're looking at emerging therapies, new drugs that are in the pipeline, but are we also concerned with... I'll give you an example real quick. There's a group at the University of Alabama that's looking, Matthew Mike is leading it. They are looking at how to repurpose existing drugs for more diseases that match up through genetic markers and literature. Is that also the sort of thing that we will be looking at in scope? Or are we only looking at new drugs?

Donna Sullivan: I think it is something to be very concerned about because once you have that word orphan tack six zeros behind the cost of that drug. So I can see them getting new indications. They will patent it under a new brand and they will attach a much, much larger price tag. So yes, that is something to be concerned about.

Man: And the second is, are we looking at something that might be not directed at an orphan disease, but at a smaller subset of the population? The example I would give here I've done a lot of work with adoptees. Right? So some things like genetic screening aren't justified in full population levels, but might be for a small group that lacks for example family health history for certain targeted diseases like [inaudible] syndrome or BRCA 1 and 2. It might be cost effective for those groups in a way that's not for the population at large that has access to other means to access that information. Would that be included in these sorts of...?

Donna Sullivan: I think so.

Judy Zerzan: Not the testing. More the therapy part.

Man: Very helpful.

Donna Sullivan: And we're not just focusing on gene therapy. So there's other things that are in the pipeline that are very expensive—there's cancer treatments, there are... Terri will talk about treatment for NASH, there's lots of drugs with high prices that are in the pipeline. There's also things like, you know, hepatitis C which was the perfect storm. The prices now, or discounts. I won't say prices. Prices haven't changed. Discounts are getting better. Those... so our costs have come down, but that was a very, very expensive drug in a very large population of people. So we're looking at those types of situations, as well as an individual drug that might be really expensive with targeting a small number of patients or something that is moderately expensive.

Man: So more pharmaceuticals than other types of technologies that are emerging like testing? Which is fine. I'm just trying to get clear.

Teri Levien: We're going to give several examples in several different areas of emerging therapies. So one of those specialty drugs, a good portion of the specialty drugs are orphan drugs. So just to update people kind of on that. She mentioned it is diseases that affect fewer than 200,000. In 1983 the Orphan Drug Act was passed. At that time there

were only 10 drugs approved for rare diseases so this was passed in order to encourage manufacturers and provide financial incentives to develop drugs for rare diseases. We can say they were very effective in doing that, especially in recent years. Some other things though to note are 80% of rare diseases are genetically based. So along with the gene therapies we're also seeing other therapies that are definitely targeted at these orphan drugs. And currently only 5% of rare diseases have treatments. So we are still seeing a big push to find more treatments for these. The mean drug cost for one year for the top 100 orphan drugs is about \$150,000. It is substantially higher than the top 100 non-orphan drugs.

So this is just a slide showing the increase in orphan drug designation. Designation doesn't necessarily mean approval. The FDA hands out designations to drugs while they are still investigational to encourage companies to continue to developing them. But in 2010 there were 350 approved. That was from the 10 back in 1983. We are continuing to see an up-tick, but we are seeing more and more approved. This last year 58% of the drugs approved were actually qualified as orphan drugs. That's up from a little less than 50% in 2015 and we've seen it consistently increasing. So we've past the point where more than half the drugs approved are for orphan diseases.

So then the sales are also going up substantially. In 2010 10% of sales were for orphan drugs. It's projected it will be about 20% of sales. \$242 billion worldwide for orphan drugs by the year 2024.

Not all high impact drugs though, like Donna eluded to, are orphan drugs. So we're kind of going to give some examples from a lot of areas on this particular chart, but this is just showing in terms of prevalence. So as we move across those are more prevalent. And the larger circle too and then the cost per treatment. So we see some of these gene therapies, new SMA gene over here. Not very many people affected by it, but it's a huge cost. Moving across peanut allergy is about 1.5% of the population is actually affected by peanut allergy. And that's growing. So that is becoming an increasingly prevalent condition and then NASH, which I'll talk a little bit at the end has the potential to be kind of our next hepatitis C in terms of impact on the budget.

Man: Can I ask you a question on your slide there? You are using PMPY.

Terri Levien: I corrected it on here. I borrowed these slides from someone who gave another presentation and I modified some of them. I missed

that one. So yes, it should be per person. Not per member. So these are the costs. Like \$2 million per patient. We estimate the NASH therapies will cost about \$12,000 to \$80,000 per patient per year, not per member. The per member cost would be substantially lower. So, yes. Thank you.

A couple of the orphan diseases that were on that list then were cystic fibrosis and Duchenne muscular dystrophy. Cystic fibrosis has a new treatment coming down the pipeline, anticipated to be approved next year. This is a three-drug combination. So we have some two-drug combinations right now. This is a genetic condition. The CFTR gene isn't working correctly. So we have some drugs that can kind of boost the effects rather than providing gene therapy at this time. The current therapies target individuals with two mutations. This one can actually target people with just one mutation. So it's expanding the potential patient population substantially. Duchenne muscular dystrophy there's a drug already. This is also working at a genetic condition, but it's working not as a gene therapy, but just forcing it to skip an exon and create the protein on its own then with skipping. The drug is already out there. It can be used in about 13% of the population. This is going to add another 8% of patients, potentially. So not a huge group, but it is a potential impact for sure.

Cystic fibrosis affects around 30,000 Americans right now. About 60% are eligible for the current two-drug regimens. It's estimated about 90% will be eligible for the three-drug combination. So between patients who are decided to switch to this new therapy, which is estimated to be about \$350,000 per year, and those who are now newly eligible, there's definitely a potential for a big budget impact there. There's also an impact on the clinical side. That's where the information is a little bit lagging because this is a condition that slowly progresses. So we can see with these drugs that they improve lung function, measuring FEV1, what kind of impact they are going to have in terms of overall survival, progression of the disease, decreased need for transplants, follow-up infections, longer lives. It is still evolving because these are relatively new therapies.

So for some of these conditions we'll have more clinical evidence as these drugs come out. For others we don't. We have a lot of surrogate markers instead of clinical actual endpoints to see if people are doing better. Duchenne muscular dystrophy about 10,000 American males have this condition. About 400 to 600 newborn males are born with it every year in the United States. This drug would be about \$25,000 per patient per year and again this is

designed to increase a protein that is deficient because their gene doesn't correctly code to create this protein. And so they've developed these drugs to try to increase this protein. Mostly effective in increasing the protein. We don't know the long-term impact yet with these therapies, you know, the one that is already available or this one, the Golodirsen. That's investigational. So that's an area that's still because it's not as rapidly progressing as a disease it will take time to really truly evaluate how effective these therapies are.

So both of these are obviously candidates though for gene therapy because we just talked about how they are caused by a gene mutation. So spinal muscular atrophy is the condition that was just talked about. So the onasemnogene abeparvovec was approved in May. This targets SMN, which is a specific gene and so they use a vector to bring and code for this particular missing trans gene. So this is a one-time dose. As Donna said, \$2.125 million for the one dose. I think if I understood right they are going to price it out over five years. If it works you pay for it, if it doesn't you don't, are some of the things I've heard.

Right now there are 35,000 Americans with SMA of some form. There's a type 0 which is almost universally fatal shortly after birth. The symptoms start before birth so that one treatment has not been studied. Type 1 is the most common then. About 50 to 60% of patients with SMA actually have the type 1. Its symptoms first appear usually before the age of 1, often before six months. And so that's who they have targeted in the studies was infants... first signs they tried to give the gene therapy. And so what they say in terms of clinical outcomes was an increase in motor function improvement using a specific measure. It's called the CHOP-INTEND. Where they measure motor function in infants with this condition. So they do have data, a small number of patients, a very small numbers of patients, but they did see survival. These kids did survive to two years of age some of them. Some of them haven't reached it yet because the studies are that recent. So we don't have years and years of data with this product, but we do have comparator historical controls kids surviving, kids being able to hold up their head, sit unassisted. The oldest in this study are walking so long-term is it a durable response? Those are still the unknowns. So those are some of the questions with all of the gene therapies because they are all just so new.

So gene therapy targets. Just as an overview they are basically being directed at diseases obviously caused by genetic mutation and right now we're focusing on the monogenic diseases. So if there's one

defective gene that is responsible those are going to be the easy ones to target. And then beyond that if there is another therapy out there and working great that's probably not a great target. So we are targeting disease states that don't have effective treatments, or the other treatments are extremely difficult, expensive, just not preferred or they are not working. So that's kind of how that is decided.

There's a lot of other conditions then and hemophilia obviously is an obvious target then. It has a single genetic mutation, requires a lot of different treatments of which they are not universally effective and it's expensive. So it became a quick target for gene therapy.

So this is the pipeline right now. There's a couple on this that aren't gene therapies. You see the majority of these in late stage are therapies right now in the hemophilia pipeline. The ones on this table are all in phase 3 so they are farther along. With gene therapies, like Donna mentioned, the approval process has been changed a little bit. Historically a phase 1 study would be in healthy individuals. We don't do that with gene therapy. So they combined phase 1 and phase 2 and give it to individuals with the disease in small numbers. If they see results generally in terms of increasing the levels of whatever the protein is that they are coding for them they can move on usually into a phase 2/phase 3 study. So that's where these are. These have moved into phase 3. We probably will see the first submission this year or late this year/early next year. It's kind of the hints that are out there. And there could be others. There's 13 companies that have studies in clinical trials right now, in human studies. So there could be other products that are further along that just haven't been necessarily publicizing where they are at in the pipeline. But these are the known ones that could be submitting within the next couple of years.

Like I mentioned hemophilia is an obvious target then. 20,000 Americans with hemophilia. Hemophilia A is more common, about four times more prevalent than hemophilia B. About 60% of those patients overall have a severe form of the disease where they don't make enough clotting factor and have severe bleeding episodes and require extensive treatment. Estimated cost for the gene therapy is right now at \$2 million. That's the number that is being consistently thrown out probably because that's in line with the other gene therapies so far. But that could change for sure. Clinical outcomes we're actually seeing a little bit better data so far with the hemophilia and I think it's just because you can see an impact quicker. So we're definitely seeing factor levels increasing. So whether they are coding

for factor 8 or factor 9 they can measure that within weeks and see if the person is actually creating the factor. So within weeks they know if the drug is going to have an impact or not. And then they can look at, down the road, do they require as much replacement factor? Do they have serious bleeding episodes that require treatment? Patient reported outcomes, obviously how are they doing? Quality of life and then safety concerns. That's going to be one that is still a little bit of the unknown. Do we have a downstream organ toxicities that we haven't seen yet?

So this is very few published studies on any of these gene therapies at this time. So this is just an example of one of the products that is probably farthest along in the pipeline and this is from its phase 1/2 dose escalation study. Basically first time you give the drug to somebody you're going to give them a low dose, then they are going to go up to a medium dose or intermediate, usually they will have dose escalation. So this was their dose escalation. They gave a low dose to one individual. These were all males with hemophilia A who had severe disease. The low dose really didn't increase the factor 8 levels very much so they moved straight to the intermediate dose, gave it to one individual, also didn't see much of an impact, went to the high dose and saw a pretty significant increase in factor 8 production within weeks. In those seven patients then that got the high dose the levels did stay within a clinically normal level in six of the seven through the full year that they followed them up. All seven had a substantial reduction in bleeding and a substantial reduction in the use of factor 8 concentrate. Bleeding rates went from 16 in the year before annual bleeding rate to 1 per patient in the year after they received it. Use of concentrate in the seven patients who got the highest dose, including the one who didn't even have a full response, after week 22 they had no more need for factor 8 concentrate administration during the rest of that follow-up year. So preliminary results look really favorable. Moved in to phase 3 and those results haven't been published yet, but are anticipated to be the basis for the submission for approval.

Beyond hemophilia then the gene therapy pipeline is kind of busting at the seams. These are examples of products that are the farthest along. So these are products that are in phase 3. They made it past phase 1, phase 2, look like they at least increased the coding for whatever it was that they are. Each one is a different thing. So Duchenne muscular dystrophy they are coding for micro dystrophin in these studies. They did see increases and they saw some

improvement in ambulation. Whether that will be sustained, whether that is going to be significant still we haven't had full results.

The Lenti-D is an example of an autologous gene therapy. So this is a little bit more like [inaudible] in a sense where you... or stem cell therapy where you take stem cells and you attach the gene therapy and you reinsert those stem cells into the patient. So that's a disease state. Obviously all are genetic. Not all of these are tested for and some of these do require recognition of the disease before the symptoms necessarily are extreme. SMA as an example, the one that is already approved. So you want to... if you're going to use gene therapy you would want to use it as soon as possible. They did a study in kids who were already ventilator-dependent and they wanted to give it early on. I think the state of Washington was just addressing whether they were going to test for that in the newborn screens routinely last week. I didn't hear the outcome on that. But that's another cost associated with these therapies; not necessarily part of the pharmacy benefit, but the recognition that we might need to do the screening to detect these conditions early. So the same with the cerebral adrenoleukodystrophy. There are some forms of that condition that are tested for currently, but that one is not in the routine newborn screen. Muscular dystrophy isn't routinely screened for although some states are experimenting with that right now. I think New York is one of them. There's some other forms of the mucopolysaccharidosis that are routinely screened for, not the type 3, which is the one that might be the first one to have the gene therapy approved. And then there's some other gene therapies here for some retinal conditions. So we already have one approved. These are the... the top one here, the one from GenSight and the bottom one by Nightstar those are forms of vision impairment that occur a little bit later in life. So they typically first present maybe in the teens or early 20s where the leber congenital amaurosis actually is one of the leading genetic causes of childhood blindness. So that one would be another that would need to be detected early in order to administer therapies.

Some others... the top one there, Zynteglo was just approved in Europe about a week ago. The price tag there in U.S. dollars is \$1.7 million for that one. Right now it's indicated for beta-thalassemia, transfusion-dependent, which is a smaller population. It's a very rare condition in the United States. But the same gene is a hemoglobin encoding gene and so the thought is it might also work in sickle cell disease and so that has studies ongoing but not as far along. So addressing the question of, "If something gets approved for one thing

are we going to allow its use for something else?” This is a condition with a much broader population that may have a particular interest in this particular gene therapy should it be approved here and those studies aren’t nearly as far along to know if it will have an improvement in sickle cell disease. And then these others are some relatively rare conditions, but are farther along in development than some others.

These were all very targeted. This are orphan drugs, smaller populations. All of these were less than 200,000 patients in the United States. The other big impact we can look at as far as emerging therapies is the potential for a therapy for non-alcoholic steatohepatitis. So this condition affects 3 to 12% of adults in the United States. This is a fatty build up in the liver. It’s associated with inflammation and fibrosis. In about 10 to 15% of patients it progresses to cirrhosis. In about 7% it progresses to liver cancer. They think is probably going to be the most common reason for a liver transplant by the year 2020 to 2025. So it has a huge potential patient population. Estimated cost of the therapies are guestimated to be about \$12,000 to \$80,000 per year. This would be a chronic therapy. And the FDA, because there are so many drugs coming down the pipeline for this condition, did establish guidelines for the approval for this condition. They want to see two particular clinical outcomes. So the drug to be approved must show resolution of the steatohepatitis and also much show improvement in fibrosis.

So this is a partial pipeline. I saw a story today where they called this the multi-billion-dollar race. Every company out there is trying to get a drug in this race right now. So far the anticipated approval dates on there are kind of like the dream date. So far nobody has progressed far enough along that they are going to be able to submit immediately except maybe the obeticholic acid, which is already available for another indication here in the United States. We’re seeing more and more combinations. We might see two- and three-drug combinations for this condition. That’s kind of the direction that it is moving in because they are not finding any one drug that is effective so far. So three of the drugs that were farthest along and have clinical data did not meet the FDAs requirements. So they did not produce NASH resolution and improvement in fibrosis and actually they all had safety concerns as well that could derail their marketing. But this is just something to be aware of. This is a huge population with a pretty expensive drug that is being actively pursued.

And then the cancer. The cancer pipeline is still by far the largest drug pipeline out there. It eclipses all other disease states by at least ten fold. So some of the trends right now that we're seeing are definitely the gene-based therapies though. So either specific conditions, you know, as non-small cell cancer with a specific genetic market or as these drugs are a genetic market that's not specific to a particular tumor type and that seems to be the trend we're looking at now. So pembrolizumab was a check point inhibitor and when it first was approved it was for melanoma. One specific indication. It's now approved for 12 different indications including a pretty broad indication that could apply to any tumor type. Last year we saw larotrectinib approved and it was for a specific gene fusion mutation. It didn't specify the tumor type. It's a pretty rare gene fusion mutation, but it is still kind of a hint of things to come and this is the direction they are definitely moving in. Yesterday a product was approved like that, entrectinib, in Japan. It actually was indicated for 10 different tumor types that all express that particular gene. And then this other product up here is being investigated in 35 different tumor types that are thought to express that similar gene type. We're seeing more directing at a particular genetic mutation or a particular gene type rather than specifically renal cell carcinoma or something like that.

And then another area of continued growth is the biosimilars. This has a potential kind of two-fold. It has a potential to reduce costs once these products actually start to become available. Think of these as somewhat generics of biologics. So once they start to hit the market we will see a reduction in cost. So far the first ones out are only reducing costs about 10%. Once more get out there we could see a potential for a lower but. On the flip side we could see increased use because as the cost comes down people might start to prefer them as a first-line therapy or at least in earlier use. Unfortunately, of these nine where we do have biosimilars to these biologics, only four of them have actually marketed at this time. So we only have the infliximab, the [inaudible] filgrastim and pegfilgrastim that have come to market. So while [inaudible] has five biosimilars approved none of them have actually been marketed yet because of patent discussions with the proprietary manufacturer. Similar for Humira, adalimumab we probably will not see those products until 2023. They will continue to approve them, but we probably won't see some of these for years. The trastuzumab they are estimating by the end of this year or early next year those ones could actually be available. And here are some others. None of these are unique biosimilars. We already have biosimilars to the particular proprietary products for

each of these, but this is continuing to be an area where we're seeing more and more... so potential to decrease costs or expand use or both. I think that's pretty much it. These are the things you're going to be discussing. I'm not going to focus on these because this is kind of your focus and your scope, but some of the things to consider are the uncertainty around the long-term benefits. That is certainly something that needs to be at least considered and discussed. And genetic markers, not necessarily strictly in oncology, but genetic markers in some of these other rare diseases, in newborn screenings, there is definitely a lot of need to identify particular genetic markers. Any questions on anything? I know that was a broad overview of a lot of things.

Dan Kent: Just a couple questions. This is Dr. Kent on the phone. Is there anything brief and summative you could say about the impact of making current best therapy unnecessary as a way of getting some cost mitigation?

Terri Levien: Making current best therapy unnecessary? Yes, that is certainly an area, especially with the gene therapies. That is the hope with those that especially when we have high cost or very invasive therapies currently that maybe some of those would be. So spinal muscular atrophy for instance.

Dan Kent: Right. Okay.

Donna Sullivan: In spinal muscular atrophy the ongoing studies there's one of the studies that's in the older kids that is I think estimated to 40% of those patients are now also getting Spinraza after getting the gene therapy. So the durability I don't think is quite there yet in that particular...

Terri Levien: Yeah, right. So right now they are using them. They are studying them in combination. They are also studying when identified administering it before symptoms to see if we don't need to provide another therapy so that's... it's an unknown, but it definitely isn't... and then hemophilia definitely there the hope is with the gene therapy that wouldn't need to be using the other products either as prophylaxis or acute treatment if it works.

Dan Kent: So it creates... instead of a single step threshold that we step over from experimental and investigational into sort of proven across the board we step over many small thresholds as we expand our field of knowledge in each of these situations, which is a much more fuzzy boundary and therefore difficult to manage regulatory wise. That will

factor into all of our thinking over the next four meetings. Second question is, has anybody built a research cost model for particularly gene therapies? It seems that we are getting good enough at the molecular technologies to have a fairly standard idea of what that costs in the lab. I certainly don't know it, but I can imagine somebody has pretty good estimates. And then the number of patients needed to prove out efficacy and so on. It's getting much more predictable. So somebody can start modeling the anticipated production costs of new therapy X. The one thing that is probably really unpredictable is the hunting around among the molecules to get started on what the next X might be. Maybe we can understand better the long-term production costs of the producing industry and therefore come up with different ways to maybe co-invest and have the sticker shock at FDA release be mitigated.

Terri Levien: I'm not an expert in this area but I have seen some articles that have talked how the cost is relatively inexpensive with the current technology to create some of these, but with some of these first products coming out right now we also have about 20 years of development trying to identify the correct vector to use. We had some problems probably 20 to 30 years ago with the initial gene therapies, the vector wasn't the correct one. We had some deaths. Went back to the lab, took many, many years to identify a safer vector that could be used to transmit these genes and so I think at least right now we're probably seeing some of that catch-up on that time that was invested trying to figure out how this is going to work. We're also seeing a lot of these little companies that were developing being bought up by larger companies. So we'll probably see fewer companies developing gene therapies in the relatively near future.

Dan Kent: You mentioned patents as another form of reducing competition.

Woman: It also seems that the cost of gene therapy is going up. I was pretty shocked at some of these prices. The Novartis product costs about half a million dollars, you know, and this is \$2 million. So, you know, some of the lessons learned with developing some of these genetic products I understand it is different diseases, but, you know, the price seems to be going up, which is interesting.

Terri Levien: And I think part of the difference in the price is the difference between the Car-T type therapy where you're extracting the cells, you're... it's a personalized therapy. You're sending it back. It is for that individual patient. They are hospitalized. There are a lot of other things that have to take place as opposed to some of these more off

the shelf these can be used in any patient with the condition. It's not unique to that particular patient. I think that is part of the difference in the pricing. We're seeing a different price between those two different kinds of therapies.

Donna Sullivan: I think the manufacturers are also looking at cost offset. So like with hemophilia if you're spending \$200,000 a year over the course of the patient's lifetime that's more than \$2 million so they are trying to figure out how does the care that they are going to replace and they are setting their prices based on that.

Man: I agree with that, but I think they are also looking at their lifetime of pricing power where the first to market has that \$2.1 million price tag, but they are looking at projections for when new market entries come and want to make as much money as they can before they lose that pricing power. If they are looking at research costs and then avoidance costs, but also how much time do they have before stiff competition.

Man: Yeah, that's the answer if you tax the manufacturers directly. The answer I've gotten directly from two of them is, yeah, but we have shareholders.

Man: To which one might say the shareholders knew the risk too.

J-B Rouillet: It would be a historical point. So emerging therapies, therapies have emerged for decades. So is it more costly now to develop emerging therapies than it was when statins were developed? Or is that a new problem? Has it always existed?

Terri Levien: It always existed to some extent. It's just things like statins and the small molecules are more like the hepatitis C and the NASH where the cost isn't in the millions for a single patient as opposed to these more biologic and gene-based therapies.

J-B Rouillet: How is the cost of those historical emerging therapies, how is it absorbed by society and the state over time?

Donna Sullivan: Twenty years ago we were talking about statins breaking the bank. I mean that's where we were. It's just the price tag, you know, we've added three or four more zeros I think to the cost and that's why I created... the legislature passed a bill creating the Pharmacy and Therapeutics Committee for the state of Washington. So we have a Washington State preferred drug list. Atypical antipsychotics were

coming out onto the market and, you know, we were worried about the cost of them. We were worried about drugs that cost \$200, \$300, \$400 a month and then when they started being \$500 a month we were worried about that. Now we're talking about therapies that are, you know, several thousand dollars a month and then some of these infusions like the Spinraza is \$325,000 a year and we have adults now that are being prescribed it. It's just some of these... it's just unsustainable and I think that there is such an incentive now at the FDA regulatory level to get into the orphan disease category. They basically get a "go" pass let's say and they are bartering these go passes amongst the different companies when they get one. So they come up with a novel disease or therapy and they get special access to the FDA that they can actually, you know, sell their past to another company for profit. So there's a lot of perverse incentives, I think.

Dan Kent: I'm just echoing with that and thought I think it was the seventh statin that was released that finally started a bit of a price drop. There were six of them out on the market and no drop in price.

Sean Sullivan: But Dan, remember it's the second hepatitis C drug that caused the major competitive price drop. So it's not always the case that it is the seventh product.

Dan Kent: Right, right.

Donna Sullivan: It was the fourth or fifth. It was the second genotype.

Sean Sullivan: It was the second one that caused a 50% drop in the price of Sovaldi.

Dan Kent: The other thing that is striking, among the gene therapies, at least so far it is striking how low the side effect profiles are. Ordinary drugs have apparently much narrower therapeutic [inaudible], but it is still early days on the gene therapies. Somebody is going to scrape something off the genome for the good gene that we want and pick up the nearest neighbor gene that's, you know, delayed but lethally toxic.

Sean Sullivan: I mean what you showed here... this is a good presentation. The dichotomy in emerging gene therapies is that some are claiming cures and we have no data on, as you said, durability and the lasting effect of let's say, you know, lifesaving. So they are making lots of forecasts over that and setting prices on some hypothesis about cure and then a number of the emerging therapies are not curative. They arrest the progression of the disease at a particular state and leave the patient

in that same health for a long period of time. So they are not truly cures and how will they go about pricing their therapies? So it's interesting to see that the conversations amongst the companies about what is a cure and what isn't. That's the debate in these next couple of years is, "So what is a cure?" And if I arrest the disease progression at today's sort of health state level is that a cure? The manufacturer that makes the SMA 1 product has a new therapy in development for Rhatt syndrome that will not cure and they've had some experts weigh on what is a cure because they want to argue that a cure means just resting the progression. It's going to be a very fascinating conversation about cure and whether you, you know, what's the value of a cure?

Terri Levien: Last comment, Sandy, and then we'll take a little break.

Sandy Stith: To add to that Dr. Sullivan, what kind of capacity do we have on price negotiation for these types of drugs in negotiating payment for outcome? Do we have any ability to do that when you have no studies that show it is curative or that it arrests the development of some typical disease that you're trying work on here? Do we have the ability to do that in negotiations or for payment on this? Great, here's your therapy; however, you have no proven outcome for this. You mentioned that with one of the drugs, Donna, I don't remember which one it was.

Judy Zerzan: So maybe some of it depends on whether the manufacturer is interested in negotiating or not and not all of them are, but we did get actually, last week, I was going to say this week, but last week, a template approved by CMS that would allow us to do contracting based on outcomes and value-based reimbursement, but, again, that requires both sides to negotiate on what's going to [inaudible].

Sandy Stith: So that would allow some flexibility with the FDA approval, I'm assuming.

Donna Sullivan: An example is with the zolgensma, the company has actually offered to... once the outcome-based contract based on survival, but these kids are surviving. I think they've only had one or two deaths. But they are surviving, but still disabled and they are not willing to do an outcome based on motor function and achieving motor skills.

Judy Zerzan: They are still needing a wheelchair, needing ventilators, like surviving but still pretty sick. And if the goal is to stop the disease in its tracks, you know, where is sort of that r? I think that is a "stay tuned for

meeting three". That is some of what we want to talk about and how we decide to pay for these and how to approach them.

Sean Sullivan: One of the big barriers to doing that of course is, you know, best practice. And so that is perhaps something to really dig into because if best price is a barrier to actually getting the manufacturer to a reasonable outcomes-based contract then, you know, a potential policy solution could be to find a way to rid yourself of... or to get an exemption or best price.

Carly Rodriguez: I think to add onto that too were, you know, when we think about outcomes-based contracts we're not talking about manufacturers saying, "Oh, if this kid with SMA dies you get 100% of the money back that you paid for it." I mean we're talking single digits. So, you know, potentially... or low double digits. Let's say optimistically 10% of \$2.125 million is not, you know, it's better than \$2.125 million, but it's still not great.

Sandy Stith: There are certainly a lot of different ways to do the math on these, but I'm with you, Dr. Sullivan.

Cody Gillenwater: One of the challenges on those that we may get into in a later meeting is the administration behind them is extraordinarily complex and challenging. It's a whole other animal. The manufacturers certainly, I don't think, are willing to take all that on. What do you do? How do you track whatever outcome is aligned?

Judy Zerzan: So we talked at you for a while. Stand up, take a break. There are restrooms I think around the corner. There's some upstairs and a drinking fountain. I don't know if the café is still open upstairs. Please be back in 10 minutes because now we are going to get into the good stuff and talk a little more. Thanks.

I'd like to first invite Foxy to introduce herself even though you haven't been here. Say who you are and what you do to...

Foxy Davidson: My name is Foxy. The thing is I was here you guys, but I went to Pullman. I thought the meeting was in Pullman. It was a great drive and not a problem, but I was listening in on the Zoom call. I represent families with sickle cell disease across Washington State.

Judy Zerzan: Great. Thanks for being here. So we're going to hear from a couple of payers next and this is going to start to be a little more interactive and so if you comment, please tell us your name before you start

commenting so that we can get to know each other and know who you are. Who's next?

Carly Rodriguez:

I'm Carly Rodriguez. I'm pharmacy director of Moda Health. So presenting on the payer perspective of how we look... the commercial payer perspective on how we look at emerging therapies.

So I first wanted to start by talking about, you know, how we establish what is or is not covered and there's a variety of steps that we take to determine how drugs are covered. I think the foundation of how we determine what's covered is when we look at a certificate of coverage, member handbook, different plans call them different things, but essentially this is a benefit booklet that clearly articulates what is or is not covered by a member's health plan. You might ask yourself "who defines this"? And the answer is, it depends. I'll try to talk a little bit about how this gets defined. So when we think about different types of groups who contract for health insurance we can think about fully insured groups. These are employer groups that contract with a health plan. A variety of us are in the room here and they say, you know, we're going to pay premiums to the plan and you're going to take on the risk. The health insurer, the health plan is going to take on the risk for paying those premiums. And then similarly with, in the individual market, the plan takes on the risk for paying claims. And so in those cases the sponsoring health plan defines the certificate of coverage or what is or isn't covered. Employer groups, depending on the health plan has say in what is or isn't covered. So they can help define that member handbook language. But in general the sponsoring health plan defines what is or isn't covered for those groups.

Then we think about self-insured or ASO administrative services only groups. So these are employer groups who take on the risk themselves for paying claims. So they contract with the health plan to provide administrative services, process claims, prior authorizations, contract pharmacy networks, contract provider networks, those kinds of things. But they pay the claims. So in those cases those groups are defining what is or isn't covered and they may draw on language that is suggested by a health plan but ultimately the group at risk or the self-insured group defines what is or isn't covered. State laws and insurance divisions may also help define or provide guidance or influence as to what is or is not covered. Insurance divisions review and approve certificates or handbooks every year and state laws may dictate what is or isn't covered. In the state of Oregon we're now seeing where laws are being introduced

but dictate coverage for very specific disease states and I'm not aware that any of them have passed yet, but there have been some bills that mandate coverage... proposed bills that would mandate coverage of like IVIG for certain immunotherapy conditions. So state law comes into play certainly, as well. And when we think about the pertinent language in these certificates of coverage medical necessity or what is medically necessary drives what is covered and that's typically a variety of factors play into the definition of medical necessity. Those are things like the intervention is for the prevention, evaluation, diagnosis or treatment of a medical condition. It's generally accepted by the practicing medical community as standard of care. It's proven to be effective in producing the intended impact on a health outcome. It's not primarily for the convenience of a member, benefits outweigh risks, those kinds of things. Experimental and investigational language also comes into play. So something could be defined as... yeah?

Man: I just have a question about the medically-necessary. Will every carrier have a different interpretation of medically-necessary?

Carly Rodriguez: Yeah. So a carrier can define what is medically-necessary. Yes. I think in the experience of multiple plans that I've worked for the language is very similar. It might not be identical, but, yes, an individual carrier can define what is medically necessary. Again, depending on if a group insures themselves they could have input on that language, as well.

Woman: In a follow-up question if a drug is FDA approved for an indication are you mandated to...?

Carly Rodriguez: Not in the commercial space, no.

Sue Birch: Medically-necessary is also defined differently by providers too. They can interpret things different so we have to be careful because...

Man: That was the follow-up question that I...

Sue Birch: Thank you. I want to be careful that it's not just plans and carriers because medically-necessary...

Man: So the interpretation of that is very broad, that term.

Dan Kent: This is Dr. Kent. The debate has gone on for many cycles, many years with many stakeholders and it really has converged to a fairly stable

set of principles and then both CMS and Medicaid are really quite standardized on a fairly mainstream set of what medical necessity is. So there are lots and lots of variations, but it really has converged on a fairly well understood core set of elements. It's just enough variation to keep armies of regulators and lawyers working on it for another round, but it is fairly centralized and fairly standard.

Man: Thank you. Do you see a difference then between the MCOs and the private commercial carriers in that?

Carly Rodriguez: I think to Dr. Kent's point, I think, so, you know, CMS drives medical necessity language. I think a lot of the commercial payers are adopting at least a large portion of how CMS defines medically-necessary.

So I briefly was touching on experimental investigational. This could be drugs that are not yet approved by the FDA. It could be for off-label uses. So drugs that are approved, but don't have supporting evidence. For example, to support their use or effectiveness or a regimen might not have been studied. So we could be getting requests for combinations of drugs we use together that haven't been studied together. We don't know what the safety or long-term outcomes are with combination use. So those are just some examples of how plans might define experimental investigational and then others also exclusions called out in contracts. I think, you know, some exclusions are pretty straight forward—things that aren't FDA approved. Drug that are acquired in a foreign country aren't eligible for reimbursement. Those are excluded, but then, you know, some groups or plans will also exclude things like weight loss medications or cosmetic... medications used solely for cosmetic purposes, those kinds of things.

So I just wanted to level set, I guess, in the commercial space that really... what is or is not covered is dictated by a Certificate of Coverage and then beyond what's covered or not covered by your Certificate of Coverage we start to look at clinical evaluation. That's what I'll get into next, but I just want to make sure there's no other questions here.

So, um, you know, we certainly look at clinical evidence as well within a health plan. This is often done by clinical pharmacists or nurses or physicians, as well, you know, potentially other clinical specialists, as well, evaluating evidence for emerging therapies. So they look at evidence from a variety of different sources. I listed some of them

here. This isn't an exhaustive list. I think we're looking at ex U.S. data sometimes if a drug has been available in Europe or Japan for decades we'll look at what the experience has been there. As we evaluate the clinical evidence for a drug and not only are we looking at what evidence exists, but we're looking at the quality of the evidence that exists. And there's a variety of different methods, well established methods that can be used to evaluate quality. We tend to look at the Delfini Group's methodology for how you evaluate the characteristics of an individual clinical study to say if it's a high-quality study or low-quality study. But then we also have to look at, you know, what does the entire body of evidence say? You could have five clinical trials, for example, or you could have a systematic review, which is a summary of a bunch of clinical trials. And so how does all of that clinical data come together to provide an overall assessment of... does the drug work or not? Does it provide a clinically meaningful benefit? The Institute for Clinical and Economic Review or ICER I think is kind of emerging as a leader in this area in terms of how you synthesize evidence and how you quantify or qualify a meaningful benefit or clinical benefit of different drugs. So we tend to look at ICER methodology for how you describe what a body of evidence means in terms of clinical benefit.

Again, this tends to be done by this evaluation process... tends to be done by clinicians that work for health plans and then I'll talk about how that plans out in terms of formulary decisions or coverage criteria. Yeah?

J-B Rouillet: Just a quick question. So clinical benefits is there a financial benefit evaluation, as well?

Carly Rodriguez: Yeah, I'll talk about that later. Yeah, because we want to make sure that first and foremost we're looking at the clinical picture of a drug and we don't want that to be influenced by the cost of a drug. So we don't put in front of our, you know, our clinicians that are evaluating the clinical evidence. We don't say, you know, "Here's a clinical trial for the drug and by the way this costs \$2 million." So think about that while you're looking at the study. We have those as two separate processes. So I will talk about the financial analysis later.

Monica Thakar: And one other thing that I think is kind of important, I'm sorry to keep reference to Car-T, you know, that's the one I know the best. So the drug may cost a certain amount, but the supportive care and the hospitalization and all of the other issues that surround it have a whopping huge cost. So when you're approving the drug costs you're

actually approving a whole package and I have no idea what that total cost is, you know, per patient, which can be extraordinarily high.

Carly Rodriguez:

Yeah, in the Car-T world I think we see the drug cost accounting for half or maybe even less than half of the total cost of care. The Car-T aspect of it. But I will talk a little bit about that too as we think about the whole financial or reimbursement picture.

As we talk about the concept of emerging therapies and orphan and, you know, rare diseases, it's also important to think about, you know, ideally we like to see these, you know, huge clinical trials that study, you know, outcomes as death potentially, you know, being the ultimate outcome that you want to avoid and like cardiovascular conditions for example, but as you think about emerging therapies and gene therapies and rare and orphan diseases we realize that you're not going to get that. Right? So we still have to make decisions based on the evidence that we have in front of us. So it's still important that we have evidence that describes that there is a clinical benefit associated with a drug. But there's also factors that come in to the clinical evaluation picture besides just a clinical trial. So to talk a little bit about those different aspects. So we look at the disease characteristics, the severity of the disease. Is it life-threatening? What's the prevalence of the disease? Is it ultra-rare or do we have another SGLT2 to treat diabetes or something like that? What's the disease burden? Is the disease well understood versus poorly defined? Is there a clear need for treatment in the condition? Are there defined treatment goals and outcomes? I think that's, you know, a challenge that we saw with the really expensive therapy for Duchenne muscular dystrophy that has been approved already is that there weren't necessarily defined treatment outcomes or the outcomes that were shown in clinical trials there was not necessarily a correlation to something meaningful. We can see lab values changing, but we did know does that lab value correlate to improved function? Longer life? Those kinds of things. So we considered that aspects as it relates to disease characteristics. Looking at treatment alternatives the availability of them, are there tons of products available to treat this condition or are there none? That can play an important role in the overall evaluation of a product, as well. Looking at evidence of those treatment alternatives is there evidence? Is there direct evidence against a comparative treatment or not? Did the alternative treatments look at outcomes versus lab values or something like that? We think about when new drugs come to market once of my old bosses said, you kind of think about it like you think about buying a house. You obviously care about what the house

looks like that you're buying, but you also care about the neighborhood that the house is in. So that's how we think about, you know, new drugs that are approved. You care about the new drug and the quality of evidence, but you also care about the treatment landscape surrounding a new therapy.

And then we also think about crowded markets. When I mentioned yet another SGLT2 or something like that, another branded statin comes to market or something like that it really... the burden is on the emerging therapy or the new drug to market to establish that it has a benefit relative to what's already out there. I think that's, you know, obviously more common in some of these... in some of the larger chronic conditions than some of these rare diseases where we're talking about zero therapies being approved and emerging gene therapy or maybe their supportive treatment or non-disease modifying treatment that's available, but not a gene therapy. So that's considered a little bit differently. We also look at administration considerations. What's the route of administration? Can it be self-administered? Does it have to be infused by a provider? What's the frequency of administration? What's the formulation? And also the burden. So looking at infusion times, or pill burden, does a new therapy offer an advantage in one of those spaces relative to existing treatments? And then expert opinion we are also reaching out to key opinion leaders or treating providers in our network about emerging therapies, especially as we look at rare disease treatments or orphan diseases or gene therapies, you know, we don't have, unfortunately, you know, the childhood neuromuscular specialist on our P&T Committee, for example, so we're reaching out to key opinion leaders in those spaces to get feedback from treating providers about their perception of new therapies as we're evaluating them.

When we think about clinical criteria... so let's assume that, you know, a therapy meets the definition of a covered service for, you know, a particular member then we start to look at what's the clinical criteria for ensuring clinical appropriate use of that medication. So developing clinical criteria for us is really an evidence-based process. I think it largely is for most payers. We are looking at study protocols to see how was a drug dosed? What's the frequency? The duration? Is it used in combination with other therapies in the study? What indications are studied or disease states are studied? What's the age of the population it studied? Or in some cases the gender of the population studied. There's, you know, been cases where drugs are only studied in females or only studied in males or we've seen drugs

that have different outcomes depending on gender, as well. Or looking at comorbidities so studies very clearly identify who's, you know, included, what comorbid conditions did they have or did they exclude? And then also looking at prior therapies and alternatives that could have step therapy implications from a clinical criteria perspective. So if a drug comes to market that's only been studied after treatment with standard of care that exists then we maybe don't know what it looks like in absence of that treatment.

Other influences so the FDA approved label, of course, but has to be supported by evidence. I think several speakers have pointed out already that, you know, what we're seeing from the FDA is changing. The level of kind of robustness of clinical trial data that the FDA requires is decreasing. So it was also mentioned that FDA labels often are expansive and beyond the populations that were studied. So the FDA is saying something is approved to treat a condition where... or a subtype of a condition where it maybe has never even been studied. And then also looking at treatment guidelines. Again, you know, if they are supported by evidence and factoring that into clinical criteria development.

So then what happens after we've evaluated the, you know, clinical picture or clinical data that supports emerging therapy? We've evaluated the quality of the evidence. We've drafted clinical criteria. This goes to our P&T Committee, our Pharmacy and Therapeutics Committee, and I'll just briefly give an overview of what that is in case someone is not aware of what a P&T Committee is. This is an independent group of practicing providers that are not employed by the health plan. I guess I'll say specifically to our plan that's the case. So we have an independent group of practicing providers that are not employed by MODA. So this includes physicians, pharmacists, nurses, health economists, could include lay members that represent unions or just a member of the health plan. It could be on a P&T committee and they represent a variety of practice settings and specialties. So what's the role of the P&T Committee? It's to develop and maintain formularies. So establishing what tier or what level of coverage if it's covered or if it's on formulary and covered or not on formulary. If it's on a preferred tier or a non-preferred tier which influences copays. They establish clinical criteria. So they review clinical criteria that's been drafted by health plan pharmacists as an example and then establish what the criteria are and they also direct drug utilization review activities. So those are things like they drive quality improvement measures that we look at. We go to see if we have issues with poor adherence to maintenance therapies for asthma, if

we have combination use of dangerous drugs like opioid and benzodiazepines. So they direct these types of initiatives that we go out and implement quality improvement programs. Ultimately our plan... health plan pharmacists present evidence and they propose recommendations around formulary coverage or clinical criteria but only P&T members have decision-making authority. Yeah?

Yusuf Rashid: Just to clarify a question. I appreciate how this is a clinical consideration and we're looking at the cost, but if you bring in ICER as evidence and economists where is... how is that different than looking at costs later on? Is that not bringing a factor and can you help me verify?

Carly Rodriguez: Yeah. So we all... I guess I can clarify how we use like the ICER reports as we think about the... like disease background, the treatment landscape and that kind of thing. Our pharmacists are not considering the quality... or cost per quality of life year or those kinds of things that ICER puts out in their report. We're just, you know, not at a place where we are sophisticated enough to utilize that information at this point nor is it relevant to our P&T Committee. So we're not using that aspect of ICER reports. And then our committee... these are just examples of what I've seen on other committees. Our particular committee does not have a health economist, but I know other plans that do have health economists and it may not be looking at the specific cost of a drug, but really more kind of the economic burden of the disease as a whole. Does that help?

Yusuf Rashid: Yes. Thank you.

Sean Sullivan: I have a question for you too. I would pause it that you absolutely are smart enough to use ICER's economic evaluation data. I know personally that you are. But here you say that the committee members have decision-making authority. That's interesting. They actually make decisions or they make recommendations? And then your office makes the final decision?

Carly Rodriguez: So that's a great transition into, I guess, how I was going to close this slide and move on. So different P&T Committees are structured differently in terms of the decisions that they make. So our P&T makes decisions that one of the decisions could be that they are giving us flexibility to make the decision. So they could say "The clinical evidence says that this must be on your formulary. It must be in a preferred position or it should not be. You should not cover this drug. It should be off formulary. It should be non-preferred." Or they

can say, “There’s not a clinically compelling reason why this should or should not be on your formulary and therefore we’re giving you the flexibility to make that decision based on finances.” Different P&T Committees are structured differently, but I’ve seen a variety of plans that have this similar concept. Thank you for pointing that out. One of the decisions can be flexibility to the plan.

After our clinical evaluation then we go through the P&T process and then we do a financial evaluation and that is in the context of the P&T decision. But the goal with the financial analysis as we think about financial factors and decisions related to finances, to drive to the lowest net cost option when it is clinically appropriate. So I’ll give you an example. I think there’s a lot of misconception that plans would put a period at the end of option and delete the rest of that. Just say, “Drive to the lowest net cost option.” But I think there are plenty of examples where that’s not the case. I think hepatitis C is a really good example of that where we had emerging, you know, potentially curative therapies. There were still cheaper alternatives available, but I don’t think many or any plans said “Let’s continue to push towards interferons instead of these curative therapies.” I think HIV is another space where branded products tend to be the products that work the best. There are generics available, but you don’t see a lot of plans driving to generic HIV medications. So those are just a couple of examples. But, again, it is evaluated in the context of the P&T decision. So they had to give us flexibility to make a decision based on finances or we see if there is a financial benefit given the decision that they make. So they said, you know, you have to put this on your formulary, you have to put this in a preferred position. We can say, “Okay, well, we know that we have to do that. That might help us actually negotiate a lower price with the manufacturer by saying your drug is going to be covered in a preferred position. Is there a discount or rebate available for that drug?”

Armen Khatchatourian: I have a question. This is Armen Khatchatourian. When you say it is important to make this distinction because in the last few months of governmental regulations, you say drive to lowest net cost. To whom?

Carly Rodriguez: Right. So I’ll talk about that a little bit. So as we think about what are the different factors and getting to lowest net cost there are several factors. There’s the list price, there’s manufacturer rebates. There’s channel or benefit management and there’s the member cost share. We think about both the cost to the member as well as the cost to the plan in terms of driving to lowest net cost. We have tended not

to be a plan that embraces like brand over generic type of strategies because it's disadvantageous to members and can be confusing for members. I think there is also cases where plans put branded drugs on generic tiers so that, you know, there is an aligned incentive. It's the lowest net cost for the plan, but also at the lowest cost share tier for members. We look at, you know, the list price or the price that you see printed in news articles and the \$2.125 million. You think about manufacturer rebates, you know, are rebates available that lower the cost of the therapy that make them the lowest net cost within a group of treatments but that fits within our clinical context from P&T? And then when we think about channel or benefit management this could mean is there an opportunity to drive towards specialty distribution where we have, you know, specialty pharmacists that are doing outreach related to adherence and overcoming barriers to adherence. Is that, you know, does that potentially help the financial picture by doing that or not? Does it make sense to keep it out of the specialty channel? When we look at benefit management does it make sense to drive a particular therapy to coverage under the medical benefit versus the pharmacy benefit or vice versa? That has, you know, can have major impact to member out-of-pocket costs where a drug is covered. Members tend to have better coverage under pharmacy in terms of, you know, they might have a cap on their specialty copay versus, you know, 20% of whatever is billed under medical. So those are aspects that are looked at and this is a place where we are looking at kind of the total cost of care and reimbursement, as well. So when we think about Car-T as an example we know that drug cost is only a portion of that. So when we look at channel or benefit management is there an opportunity to contract with the Center of Excellence that we know, you know, has more favorable reimbursement rates or higher quality of care that kind of thing as it relates to a specialty therapy like Car-T as an example.

Then again member cost share. We think about exchange plans look different than group plans in terms of cost sharing or copays. And so we think about, you know, how does that impact members and does that make... does that influence our decision to put a brand on a generic tier, you know, insulins would be an example of that where many plans cover a branded insulin product at a generic tier, because they tend, you know, generic tiers a lot of times have fixed copays. So it is less costly to members.

And then we put it all together to come up with our final coverage policy or, you know, prior auth criteria. So this is, I think, a simplified

way of representing what I just talked about. But we have our clinical evaluation. We have the context of what the P&T did or didn't give us flexibility to do and we have the financial evaluation and that ultimately leads to our final coverage policy.

And then lastly talking about how we evaluate coverage decisions that we make. So we might put prior authorization on a drug or put it in a preferred or non-preferred tier, but we want to evaluate that... pan out how we thought it was going to, if that makes sense. So we look at, you know, cost and utilization trends for drugs. We look at prior authorization data in terms of approval and denial rates and feedback that we get from the provider community in the prior auth space. We look at claims analysis on the medical side to see, you know, are we seeing these adverse outcomes potentially for members that are using certain therapies. Are they getting admitted? Those kinds of claims data we're looking at. We're also looking at clinical data. Are there new trials? Have safety data emerged that influence coverage of a particular drug? And then we also look at outcomes based agreement results. So if we have an outcomes based agreement with different manufacturers we want to see what the outcomes have actually been. Does the drug do what the manufacturer said it was going to do? You know, unfortunately for our plan in the Car-T space and this is a place where, you know, Novartis came out and established an outcomes-based contract with CMS and, you know, unfortunately our Car-T members have not fared well. We've had a handful of them and none of them have survived beyond a couple of months, which is really unfortunate, but it's also important that we track that and see how drugs are actually playing out in terms of effectiveness in the real world. That's my last slide. Happy to answer any other questions or turn it over to Donna.

Donna Sullivan:

I'm going to ask that we hold questions until after. That way we will have time for a longer discussion.

So my presentation I'm going to make it a lot shorter than it was. I'm just going to say ditto to most of what Carly just said and I'm going to just kind of talk mostly about the differences between a commercial payer and what a state government or public employer... what our policy is. For our Medicaid program I mentioned earlier we have that covered out-patient drug program and it requires that the Medicaid programs across the country cover any drug that the manufacturer has entered into a federal rebate agreement. So our drugs coverage policy first it must be FDA approved. Second, it has to have a federal rebate, and third, it has to be medically necessary. And to answer

Monica's question earlier, just because a drug has an FDA indication doesn't mean it's medically necessary in the person that it is being prescribed from our perspective. So those are two rules that our Medicaid program has to meet. It has to be FDA approved, but it has to be medically necessary for that particular individual Medicaid client. So that's something maybe slightly different from the commercial payers. It's also different from our public employee plan, as well. And full disclosure Moda Health manages our pharmacy benefit for our self-funded, public employee's program and Regence manages the medical benefit for that same plan. And then on the employee and retiree benefit side for public employees and school employees, again, must be FDA approved, they must be medically necessary. This last bullet where new drugs emerging needed to go be reviewed, do a budget impact analysis, and sent to our public employees board, was something that the legislature put into the budget several years ago. It has now sunsetted, but the cost of drugs that were impacting premiums was really alarming to the legislature and they wanted to make sure that we were looking at these new therapies and making a conscious decision on whether or not they should be covered. We had a \$50 per month increase in our Medicare retirees' premiums, I believe in 2018 and so... because Medicare is primary we cover the pharmacy side of their benefits as the drug costs really impacted the Medicare retirees' rates. We also performed an evaluation of the evidence for members of the Drug Effectiveness Review Project that is administered by Oregon Health Sciences University and the Medicaid evidence-based decision-making group. We also look at ICER and then we have clinical staff like Moda does.

We determine the strength of the evidence. I'm just kind of plow through these.

But what we have to do is we have to do a budget impact analysis. So when these drugs come out onto the market we, as a Medicaid program, have a set budget that we have to spend for all the prescription drugs that... for all of our members. So when we see really expensive drugs come out we are going to try to estimate how many patients that we have in our population, what is the cost, and then I have to go have a conversation with Robin and Sandy and say, "Holy cow, we are going to over spend our budget. What are we going to do?" So based on all that evidence review we have to let our authorizing environment know ahead of time that, you know, winter is coming and we're going to have all of these expensive drugs that we're going to have to pay for. And that is a really fun conversation because they're going to say, "Well, don't pay for it." And I'll be like,

“Well, we have to pay for it.” It becomes a struggle for us. On the public employee side it’s a little bit easier but those drugs then are going to impact our member premiums. So we know that our employees are going to pay more money out of their pocket for their health insurance as we start paying for these drugs and we also know that state employees typically are paid less money than their private counterpart. So we’re not... we have stagnant wages at the state level at times where we don’t get raises, but our health care costs are continuing to go up. And so that’s a challenge also. Then we also want to attract and retain good employees for the state.

Sue Birch:

Can I just also say that we brought in a new tier here when HCA has the ability to negotiate better scenarios we still have to go back through that process. That should not happen. We need to take that away when HCA can work a better deal. We want to be able to move more quickly and we shouldn’t have to go back through and advise that all those layers that hey, we’re going to save a boat load of money. We are going to move quickly when we have downward savings and only need to go [inaudible] when we have upward impact. Because of the changing environment and the value-based formulary and the way we negotiate things can go a lot quicker if we didn’t have to go through that laborious process. To me it’s a real critical flaw in Washington. We did not have this problem in Colorado. If we were saving money we typically checked it to say, hey we’re going to save a boat load more. And they would say, “Move faster and don’t come through this process.” Just want to point that out because I think it is a significant modern [inaudible] that we need to deal with in these processes.

Donna Sullivan:

And then we develop our clinical criteria. Very similar to what Carly mentioned we have a... for Medicaid a Drug Utilization Review Board. The same members also serve as the Washington State Pharmacy & Therapeutics Committee. To Sean’s point they make recommendations to the state agency on drug coverage policies, the DUR Board does, our P&T Committee makes recommendations typically on how many drugs should be preferred, if they are equally safe and effective, also if they can be subject to therapeutic interchange, which is something that Washington State passed about 15 years ago now where if a drug is non-preferred and a doctor writes “dispensed as written” they’ll get the non-preferred drug. But if they write “may substitute” the pharmacist can substitute that drug at the point of sale and then just send a note to the doctor stating what they dispensed and they don’t have to call the doctor to get a new

prescription. I don't think that's done very much, but it is a law that is permissive for pharmacists.

And then I already mentioned who our partners are for the Uniform Medical Plan. We are developing a high-cost policy because as these high-cost drugs come out we set our rates for our managed care plans on a capitated rate based on two years of historical data. So when we get a new drug that comes out that costs \$2 million we know that the current capitated rate that we're paying to that plan does not include that \$2 million drug. We also know that some of these drugs... these conditions are disproportionately spread across the plans. We might have one plan that has 200 patients with hemophilia and all of the other plans don't have any. So we need to take that into an account. So we're developing a new policy on how to react to these drugs in our managed care arena so that we make sure that we're covering those drugs, we're getting to the patients that they need to cover or that need the drugs, and that the managed care plans are still solvent.

And now we're back to discussion. Mike, I'm going to call you up.

Mike Bonetto: I think we've got about 15 minutes. I heard that there is a flight that is leaving at 5:30 that I think many of you folks are on. So I want to make sure that we get through in 15 and kind of capture some key thoughts. I would like to see if there are any kind of questions, comments, just based on Carly and Donna's presentations?

Man: How many lives are we talking about, Donna, when you breakdown Medicaid and PEBB lives?

Donna Sullivan: So Medicaid is just under 1.8 million.

Woman: Just over 1.8, yeah. And on our PEBB product we're about 350 and we anticipate that SEBB, school employees are going to be an additional 300ish. Could be a little higher, we don't know yet.

Donna Sullivan: Two and a halfish. Yeah.

Mike Bonetto: Other thoughts or comments?
Carly, I have a question for you. As you were listening to Terri's overview of kind of just the pipeline, do you see anything changing within your current process?

Carly Rodriguez: Yeah. I think we, you know, we're a smaller plan that has tended to review things like as they come to market and I think just the way that

the treatment landscape is changing there will be a more forward-looking or I guess, yeah, a more proactive or perspective look at drugs. So we're not going to know price at the point that we have our clinical decision made and that's fine because we don't consider finances anyway. I think it will change the timing and how we review things and I think our actuary team is also, you know, significantly more involved earlier on in saying, you know, "When are you reviewing this? Who do we anticipate is actually going to be treated?"

Mike Bonetto:

Got it. Thanks.

Any other questions? Okay. So what I'd like to do is make sure I can capture some of the main themes in some of your thinking today. I had some notes just from earlier on from some of the conversations. Dr. Kent, I know you had some points that you were making just about kind of the impact of current therapies, proving out efficacy long-term production costs. Sean, you brought up best price implications early on. We don't want to lose sight of that. Cody left, but he had some issues just on the administration of outcome-based contracts. I'd love just to get a sense from other what kind of resonated today? What were some of your bigger takeaways? Some of the things that you'll be looking at kind of in the course of the next two or three meetings? What kind of stood out today?

Monica Thakar:

For me there's an unmodifiable issue, which is someone out there is creating what the cost is and then we have to kind of deal with that cost. I don't know if there is any way or... I have no idea where that cost really comes from and how modifiable that is, because we're just kind of given a number that's been developed by pharma, probably. Or a combination of groups that are profiting from this therapy. So how do we deal with that?

Armen Khatchatourian:

And we talked about this at break in a smaller group, but what are the actual levers we have to play with here? Right? We can either fight them on pricing and challenge them to lower their price, or we can buckle down on what is going to be covered. Are we willing to take that stance as a state entity on what we cover and tighten up the, you know, what's available and what are the ramifications of that decision? I think it's harder to fight a pharma manufacturer what they are allowed to charge. We can fight that battle, but everybody would have to be on the same page. Versus what... this group can decide on what they want to allow for coverage. I don't know any other levers that we can really pull here.

Monica Thakar: The hard this is as a pediatrician who looks at these families and tells them that this a therapy. It's very difficult because there's a lot of marketing out there. Whether that's the correct marketing, but it's incredibly hard when these families are Googling everything. They come to you incredibly sophisticated with a lot of knowledge and they are requesting certain therapies because they got on a blog sphere, they get on Facebook. They are getting their information from lots of ways. And so I think there is a lot of push and pull from a physician's standpoint. It would be really hard... I mean I've had to, you know, definitely for certain therapies that I felt were really important I had to speak with medical directors at different insurance... I'm not saying at a state level, but on the private sector it's really you go to tell them to support what I think is the best for my patient. It's a lot of effort. It's hard because I'm coming at it from a more personal standpoint. Back in the day when insurance companies weren't covering many things and you heard about these families, you know, these patients dying, I think, you know, they have made movies about this, you know, all of that stuff it's just like it has affected our culture right now.

Armen Khatchatourian: I think we all want to be compassionate, but at the same time educating the patients on... it's hard to go through clinical data at an evidence-based level that... I think we all try and go through it on the P&T level and for the patient to say, "Well, 10% or 15% potential positive outcome rate may not be..."

Donna Sullivan: And that's where we've been talking about, you know, developing... requiring a patient or even hiring... so the Washington State we now certify patient decision aids. And so hiring a consultant to do patient decision aids on some of these treatments so that, you know, people can weigh all of the benefits based on their values.

Thomas May: I think that's going to be difficult though because for them it's not a population decision. Right?

Donna Sullivan: But as a parent, you know, and my values if I'm going to get a gene therapy that, you know, my child gets diagnosed with spinal muscular atrophy, they have type 1, you know, it's a death sentence before the age of two, but we have this evidence that shows, "Okay, now they are going to live, but they are going to live and they are going to require a wheelchair and they are going to require all this much more care," do you want to move forward with that? It's horrible a child would die, but somebody might decide I can't live with this disability... with this patient disability. My values are different and they might be able to make that decision because they are being sold a cure on

television and they don't know what they are really signing up for and, you know, if they get it would they... if they had known would they have made the same decision? And I think that's where it is really important. And also an outcomes registry, should we be requiring that there be some sort of registry so we can start tracking the outcomes in our state about these particular therapies in order to go back and really do our own analysis on whether or not they work or don't work.

Cody Gillenwater: There's a big medical ethics question built in there. The ethics going into that question of who... what are the decision points around when we don't have the evidence or adequate evidence to say what the actual outcome of a certain treatment could be? Especially when you're dealing with the pediatric population. But more broadly with these rare conditions there is a significant element of hope being built in and ultimately does that hope pan out or...? We don't have the evidence and we're not going to for quite some time.

Monica Thakar: That's very true. I have to say when I meet with a family I will sense in about 10 to 12 hours of my time over the course of multiple visits talking with them... so these are not like, "Oh, there's a cure? I want it. Yes, you can have it." There's a lot of, to your point, Donna, there's a lot of discussion that happens that I as a physician don't get reimbursed for. That kind of stuff never gets reimbursed, but you know, we look at the whole package and especially on the pediatric side this idea of, you know, quality of life and some families it almost... they are so focused on life and you cannot make that... speaking from an ethical standpoint you can't define life for that family. You know, some families, you know, you have someone like a Jehovah Witness for example you get one blood transfusion and that has made the decision for them that they cannot get over. And other families they will want the chest compressions. They will want to go all the way, all the way, all the way for that .1% chance of life.

Man: Because for them that's better than a 0% chance.

Monica Thakar: Yes.

Man: And if it delays the inevitable the hope then is that in the meantime there will be another emerging technology. So it becomes a complicated decision at a personal level. That's different than what I think we're faced with.

Rebecca Owen:

There's another player in these sphere that we haven't talked about. I'm sorry I'm going from human to actuarial now. About half of the people in commercial insurance that are... work for businesses between fewer than 1,000 lives are covered under self-insurance and they reinsure and the reinsurers are starting to build this idea that they have a national negotiation on orphan drugs because this is their world. They think about different pricing or different outcomes and I'm wondering if it is possible to join up with them as they build a sort of national consensus with more data, more access to information and more leverage to think about different ways of paying for this.

Sean Sullivan:

That's a very good point. They will also find the same roadblocks that the health care system and other players have found over the years. That is that the manufacturers will negotiate with them to a point. So you asked the question, what are the big takeaways? I have two of them that I will offer. One is that we are in a health care system where we are price takers. That is we have very little power to negotiate with what are essentially monopolists in the pharmaceutical industry. No matter what regulations we have over 90 or sort of third party evidence synthesizers like ICER manufacturers have baked in all of that to their pricing. We will in this country always be a price taker because of the way our economy and our political system treats the pharmaceutical industry. I'm not saying that with any amount of sort disparaging comment. It's just the reality of what it is. And so when we think about solutions we have to recognize that we'll always be in that situation unless federal congress acts. That's the first thing.

Second is both Carly and Donna showed their sort of process. Those processes are built on strategies we've had for 30 years or more. They are not going to work. Those strategies aren't going to work in the future where we have drugs like larotrectinib that was approved on a single-arm trial with 55 patients with 26 tumor types. No comparisons. So there is no ability to do all the work on comparative effectiveness. Right? For which the FDA allows a label that says, "If you have this track [inaudible] mutation you can have the drug." So we have to start thinking about new ways of doing what Carly and Donna just showed in a world where those therapies are going to be approved by an FDA that is constantly changing and allowing a whole lot of different kind of, you called it, Carly, less rigorous information. I would say it's even potentially more difficult than that, than less rigorous. You know, non-small-cell lung cancer in larotrectinib approval database was one patient. A single patient treated and now it's approved for use in non-small-cell lung cancer, which by the way is not an orphan condition.

Donna Sullivan: I wanted to tag onto Sean's comment.

Sean Sullivan: Can you do that while I'm walking?

Donna Sullivan: Yeah, about the price takers and little negotiation. Not only that, but we're all complicit with the manufacturers because we're all investing in their companies. Like our retirements are invested in their companies and so there's not just this... we can't just slash prices by 30%, you know, we're going to crash the economy. Our entire economy is all, you know, tied into the fact that they have shareholders and that's how they are making their profit. So it's complicated.

Yusef Rashid: I just want to add to this as well that pharma is also floating Medicaid in a lot of ways and there's an [inaudible] the whole system to rebates and what Sean was saying about how these are processes that we use and hearing the words of us, them, fight, and don't use the word unsustainable. For a takeaway for me it was that slide that you had, Donna, with the circles of society—payer, supplier and patient care giver and if we are to put our futurist hats on, if we could find a way to align the incentives all of the players towards one common goal and have everyone's skin in the game on that then this us versus them unsustainable and pulling out these tools to try and fight when you don't have the power to [inaudible]... I agree, it's not sustainable. So we need to rethink the whole, if we can, it's not going to be easy, but I think it is aligned to those [inaudible].

Sue Birch: Totally agree. I think one thing we also need to add to this is provider education. Because if you're in an integrated system and you have access to a lot bigger integrated established pharmacists or have a lot more know-how than other systems I just think we have enormous unevenness around providers making those involved in the conversation. So I think provider education... I mean when you look at the advanced practice providers and... that's another huge challenge in all of this and by disease and volume and also [inaudible] incentives.

Man: The fact that manufacturer's aren't at the table. We're just not ready for that shows...

Donna Sullivan: What I've been talking in my smaller circles one is there's no gene therapy to modify trees to make money grow on them. I want to see that one in the pipeline. But we keep talking about the state has to

pay for this, the federal government has to pay for this, the purchasers have to pay for this. We're paying for this. You as a taxpayer, you as a resident in the state are going to be paying for this out of your hard-earned dollars as you're income taxes that you pay to the federal government. They fund Medicaid, they fund Medicare, so you as an individual is paying for that and I think we need... I don't see the public outcry. Where is the public outcry on how expensive drugs are getting? We're starting to hear a little bit about it, but until we really wake up the residence in our country and in our state and they understand that they are the ones that are paying for these therapies that sometimes they are demanding, I'm not sure we're going to make a lot of progress. But that's where I really want to change the conversation away from, you know, "Donna is the state and Judy and Sue and I have to come up and figure out how we are going to afford these drugs and Medicaid." It's a taxpayer... we're all in the same boat.

Woman: I just want to add onto that. I think something that is probably for one of our future meetings to talk about is really the societal impact and then even thinking about within a population within a given, you know, employer group or health plan is that when a \$2 million gene therapy is funded for a single individual that raises the premium the following for every other individual in that pool.

Woman: Or bankrupts an employer group.

Man: I agree with you absolutely whole heartedly, but I just, as a warning, we've never been able to get the public to realize that. The public just does not want bedside rationing or anything that looks like...

Woman: [inaudible] this committee and making them start using table tents or, I don't know, do you want to call on people. I'm really excited we have actual people that represent patients and have perspectives and so I think before we end if any of you have thoughts on the presentation today or sort of where we're going I'd be interested in hearing that.

Man: Love your feedback on that.

Stephanie Simpson: I think that... first of all I'd like to say thank you for a robust presentation because I have gotten to spend a lot of time listening to these kinds of things and you guys would all be excellent at many of the national conferences I go to. But I think that's really exciting, right? Some of you may not know like this is really the only public

conversation in the country at the state level happening. So this is super exciting. But also thank you because I felt like the patient voice was represented. It wasn't, you know, yes, the word member, but not in an unlawful manner. Right? But I think that to, you know, from the perspective of the pediatrician since so many of us, you know, think of children and it doesn't matter if it's your kid or it's your adult parent um is understanding that everyone pitches in, everyone is paying. You know? And though there is a public plan here talking, but to remember that we are speaking of Medicaid patients. That's a very different population when you represent patient groups than somebody who has a parent who works for Microsoft, which I think is actually really exciting to know that we are representing our most vulnerable patients plus, you know, the plans that you are playing to. If you are going to apply this to those other groups, but to know that we are making sure that those most vulnerable, you know, members of that population are being represented here I think is really valuable. But, yes, the [inaudible] I always say that hemophilia. When a patient says, "Oh my gosh I have to pay my premium," and I say, "I pay for your premium." And that's my representing them because it's important for the patient to remember that everyone pays for them. Right? Expensive patients are hemophilia's expertise because the state has a \$20 million patient. So we struggle with those ethical questions. But an emerging treatment took care of that and that patient is no longer that. So that is kind of my thoughts.

Mike Bonetto:

I know we are running out of time. One thing that was talked about just briefly at the beginning was the middle bullet here—care versus maintenance. I don't know... I'd be kind of interested in everybody's thoughts on that. I mean when we talk about these orphan drugs and the impact and what from a societal cost perspective are we paying for the ongoing and I think Donna had the example of, you know, somebody who would continue to be wheelchair bound and how do we quantify that quality of life if there's not an actual cure? I mean do you guys have thoughts on that from a particular...

Monica Thakar:

I think the patients define cure. I was just telling Representative Schmidt at the break, very differently, right? So there's a new hemophilia treatment. They no longer have to infuse in their veins. Some patients view that as a cure, because it feels like such an improvement in quality of life. For those of you who are agreeing to pay for this, thank you so much. Um, and so they... the kids see it as a cure. The patients who have inhibitors see it as a cure. So I think that part, you know, I look forward to hearing what you have to say

about that because I think that definition is really broad for a patient versus, you know, a data and a doctor who sees zero, you know.

Donna Sullivan: And I think it will be different. I think the impact on hemophilia is going to be huge. The impact on some of these other diseases that might not have had treatments before may not be so huge. So I'm not sure the cure versus maintenance. It's going to be a disease-by-disease patient-by-patient conversation.

Monica Thakar: But I'd still like to say the improvement in quality of life for a family may feel like a cure. Right? Their child has been dying at two and being in a wheelchair at four may feel like a cure.

Woman: I think it's so personal.

Woman: Yeah.

Donna Sullivan: And it may not feel like a cure.

Woman: Totally. Yeah.

Man: Like cystic fibrosis not a cure, but that probably feels like a cure compared to just trying to get congestion out of the chest. It's actually more of a cure.

Donna Sullivan: I think we're in this position where we have diseases that have treatments now that are burdensome and they reduce quality of life. We have new treatments for diseases that haven't been treated before that are actually going to, you know, they might make the patient's... the family's quality of life worse because of the treatment. Because before the patient died you had to go through the grieving and I'm the one who gets to pull the plug in my family. I'm just saying from a medical ethics point of view, and I'm not one, but that one person... their child would die. They go and they grieve that, but they get back to some quality of life where now they have a disabled child and I... I don't have disabled children so I can't speak to what that might be, but I just know that when I... I pray that... I'm excited that I don't have disabled children. And because it's so difficult for me as a person that would have been very, very difficult for me.

Man: vul[inaudible] wide spread [inaudible]. I'll give you an example where I think there's a clear... if we were justified as a society to step in at a policy level and make this quality-of-life decision on any day it would be PVS, persisted vegetative state versus [inaudible] and yet we have

a wide variety of perspectives going. And so we don't actually have policy set for whether or not we will treat... we allow that decision to be made and then [inaudible] level which I think is partly what you're saying. Many people decide. Now that's not a colleague or my mom or my whoever. And so, you know, at the policy level that's really where the challenges will be at the societal impact thing. We can say, "Is this worth it?" To them it is. At a personal level even though... if we could get them to look at it as a policy level and realize that this isn't just paid for by MODA or Kaiser or Regents, it's paid for by you through your premiums, but that's a hard road.

Mike Bonetto: Guys, sorry, I know we have just a few minutes left. I want to be sensitive to folks on the phone. Comments? Thoughts?

Dan Kent: Just a quick one. Dr. Kent, I'm just amazed at the expansion of this discussion that's coming at us. The expansion of pipeline, the expansion of possibilities, the driving costs of all of it, the business consolidation which accelerates all of that. So, overwhelming.

Petra Eichelsdoerfer: I find myself wondering about informed consent and that is informed consent of the family. Do they really understand what decision they are making or are they clinging to... understandably, are they clinging to that little hope for that 1/10th of 1% chance of either prolonged survival or cure and not even realizing that the 99% either don't survive or survive with extraordinarily poor quality.

Man: I think they do. It's the problem...

Petra Eichelsdoerfer: It's a challenge. Yeah, but the challenges, do they really...

Man: [inaudible]

Petra Eichelsdoerfer: They say that they do, but do they really? And I don't know the answer to that. This is a problem with any of the quality-of-life decisions people are making. You used the example of someone in a vegetative state. There's lots of these where people may not, you know, geriatric surgery is the one that comes to mind for me. They may not really fully understand the decision they are making because they are so focused on that hope.

Monica Thakar: I work in pediatric hematology and cancer and transplant. It's a very niche field. Take that with a grain of salt because my perspective is, at least at Seattle Children's, the way that we... they are so focused on quality-of-life of that child that even though we think that there is

potentially a treatment... one of my mentors a long time ago told me, "As a well-informed physician who is working very closely with a family who has developed relationships it is horrible to give a family a decision and say you with limited medical knowledge you are supposed to make that." It kind of takes me off the hook and that is not providing really good care. So I think that education level I think we had talked about for the physician and having that relationship it provides that support. It still doesn't mean that you have people who escape through that, but for the most part, at least on the pediatric side, where maybe it's a little more boutique and you have a little bit more time with the families you are having some really deep conversations that take potentially weeks, you know, to make a decision and get retracted. So just comments here, but it's not easy to your point to establish that informed consent.

The other point I was going to ask is... so if you don't work for Microsoft but you work for a family who has insurance, but you reach a cap, those kids they end up going on Medicaid. Right? So there's another...

Woman: [inaudible]

Woman: No, they don't?

Rebecca Owen: A fully insured plan cannot have any caps. There's no lifetime...

Monica Thakar: Okay. I just recently moved here from another state. That's why I...

Rebecca Owen: That's the ACA.

Monica Thakar: So that's not a state law?

Man: Yeah, caps went away, but caps are a real thing not very long ago.

Woman: Is that every state?

Man: Yeah, \$2 million set off to Medicaid.

Sue Birch: I do think another area of the discussion should be in lieu of, because I think we're seeing more and more people saying, "I don't want this [inaudible]. I want medical marijuana and I want it paid for." I think there are all sorts of other kind of alternative things that are going to play out in this conversation. I see heads shaking. That is certainly in that pediatric seizure arena. We were seeing that in Colorado big

time and so this alternative treatments in lieu of and... and/or that sense of what's my... I don't want to spend \$2.1 million, but give me \$20,000 in support.

Rebecca Owen: Back to your cure versus maintenance question, so you have sort of two things happening here. So NASH is kind of a different thing. It's maintenance and it's not... there's not heart tugging children's heart strings on that. Maybe we might want to approach these two things differently and think about them separately.

Man: That's a good point.

Cody Gillenwater: I think one of the related things when we're talking about pricing and economics is, again, the development of additional knowledge in a space so when we have things that are approved and [inaudible] every early evidence it's almost that the price of that research is pushed onto the market. So how do we factor [inaudible] development, as well?

Mike Bonetto: Closing comments from HCA?

Sue Birch: I just want to say having witnessed in process I'm really... I applaud the efforts here and the staff that put this together and I just know that this will be a fabulous journey of exploration and I know that we will serve Rep Schmidt up some great recommendations and this will be ever-evolving work and I just thank everybody for your participation in getting here safely and please travel home safely, as well.

Mike Bonetto: Thank you guys very much.