

**Washington State Pharmacy and Therapeutics Committee
Drug Utilization Review Board
Emerging Therapies Workgroup
February 19, 2020**

- Mike Bonetto: Well, guys, why don't we kick this off. I think we're just waiting for Sean. I think he should be in momentarily. So, thank you for your patience. I know we've got a number of folks on the phone today. Okay. So, for any speakers, let's make sure we know that we've got to lean into this mic today. A couple things before we get going. I know we've had a lot of people fly in from other areas. We've got a lot of folks on the phone. So, we certainly thank you for taking time this afternoon to be with us. I think I've met many of you over the last many months, but I am Mike Bonetto with OHSU Center for Evidence Based Policy. I've been working with Health Care Authority in helping kind of facilitate these workgroup meetings. We have today. Then, we have one more in April. Then, obviously, Health Care Authority is going to be kind of compiling all of your thoughts and recommendations around how they could proceed forward. I'm going to go through some housekeeping in just a bit and then kind of get into agenda and kind of our discussions for today. Before we do that, if we could maybe just go around the table. Then, we'll go on the phone of who is here with us today. So, we can just go through... I'll start with Leta.
- Leta Evaskus: This is Leta Evaskus with the Health Care Authority.
- Donna Sullivan: Donna Sullivan, and I'm the Chief Pharmacy Officer with the Health Care Authority.
- Robyn Williams: Robyn Williams from the Office of Financial Management.
- Carly Rodriguez: Carly Rodriguez from Moda Health.
- Danielle Walters: Danielle Walters with Bluebird Bio and Meghan Gallagher will be joining us later by phone, and Debbie Echiveria [sounds like] also from Bluebird Bio.

Yusuf Rashid: Yusuf Rashid with Community Health Plan of Washington.

Petra Eichelsdoerfer: Petra Eichelsdoerfer with United Healthcare.

Armen Khatchatourian: Armen Khatchatourian, Optima Rx.

Leta Evaskus: I think you had your mic off, because the red light didn't turn on. Rebecca's, yeah. Just hit the talking person.

Rebecca Owen: Okay. I have the talking person. Rebecca Owen with Health Care Authority Solutions.

Stephanie Simpson: Stephanie Simpson, Bleeding Disorder Foundation of Washington.

Ryan Pistorosi: Ryan Pistorosi, Health Care Authority.

Mike Bonetto: Thanks guys. Folks on the phone?

Jonathan Espenschied: Jonathan Espenschied, [inaudible] College of Medicine, Washington State University.

Rachel Currans-Henry: Rachel Currans-Henry with the Center for Evidence Based Policy. I'm a colleague of Mike's.

Leta Evaskus: I see David Robertson.

David Robertson: Yes. Hi. I'm David Robertson with Regence, Vice President of Clinical Pharmacy Services.

Leta Evaskus: Foxy Williams? I see her on, but she is raising her hand.

Mike Bonetto: Foxy, if you're there, let us know or send us a text. Anybody else on the phone?

Leta Evaskus: That's it.

Mike Bonetto: Okay. Sean, not to put you on the spot, but we were waiting for you to come back in so we can put you on the spot.

Sean Sullivan: Okay.

Mike Bonetto: Just do a quick introduce, name.

Sean Sullivan: Great. Hi. Sean Sullivan, university of Washington.

Mike Bonetto: Okay. That's our crew for today. So, thank you, guys, very much. A couple things, just in terms of logistics today. We are going to have a break about halfway through. I think for those of you who got here early enough, bathrooms are down the hall on the right, but we'll hopefully try to spread this out so we can get a bit of a stretch. When we did this last time, make sure... Leta is recording this. So, every time that you're speaking, if you could please make sure that you announce yourself, just so we can have that for the record. It's really important, as we go through this.

Last time, at our meeting in October, we had some technical difficulties, just with the audio, for the folks on the phone. So, folks on the phone, if you have anything that's going on today, please just, again, send us a text or shoot Leta something so we can get that corrected, as soon as possible.

Okay. So, before I jump into agenda planning for today, I wanted to touch on, in your packet, Leta is going to pull this up for folks on the phone. I think it's helpful, just to make sure that we know where we've been and where we're going before we kind of launch into this topic today on the funding options. So, again, this group is this body of subject matter experts to really help Health Care Authority think through what are some next best moves when it comes to these emerging therapies. So, HCA is kind of taking all this in and then really trying to, you know, kind of formulate their own path moving forward. When we met in June, you guys kind of... it was a level setting of really understanding kind of what is in the pipeline. What are we currently seeing? How are things with emerging therapies being managed, both in the commercial setting and then even within the public sector, as well. We transitioned in October and heard kind of specific examples from the patient experience, patient navigation

aids, and then even more, really, with that provider/patient experience, specifically dealing with these emerging therapies. Today, we are really focusing on these funding options. What are other states doing? What are other innovative ideas that, again, HCA could or should be looking at. I think we kind of have this mantra today to say, certainly when it comes to this financing piece, if you were in HCA's shoes, what would you do. That's going to be kind of a centerpiece, even for later this afternoon's discussion, as you guys are thinking through some of these options.

The next piece that we've got, only one more meeting left in April, and that is really going to focus on the outcomes. How do we track these outcomes? What are the metrics? What's the overall value of some of these emerging therapy drugs, and how should HCA really be thinking about this. So, that's really going to tie this up. We'll kind of leave that with a final package for HCA to think about.

So, I think, again, before we jump in today, I just want to make sure everybody was kind of clear on the level setting of kind of where we've been and where we're going. Let's jump back to today's agenda. So, we've got a lot to go through. I think it's going to be helpful, as we have Robyn Williams from the State to give us a bigger overview of just how she is even looking at it from a budget perspective with the impact of these high-cost drugs. They had a little bit of that back in June, but this really starts to get into, if we're going to have kind of this pipeline starting to hit, what are some of the State's options, and the impact, and what are the tradeoffs that the State is going to have to look at, which kind of gets into these policy options around how we can fund these things differently.

We're going to transition over to Donna, where Donna is going to give us kind of an update on where I think Washington was one of the leading states in the nation of putting together a very innovative package on hep C. So, we're going to hear a little bit more about that, and how that has occurred, as well as what other states are looking at. We're going to turn these over to Sean and Carly to look at some of the stuff that they've proposed, in terms of a summary of funding options. So, this is going to be a lot of information, but I think

what we really want to make sure that we do, we've got a whiteboard over here. I want to make sure that we capture as many kind of outstanding questions, other issues that you guys want to address in our later discussion. So, we might not get everything discussed during some of these presentations, but we want to make sure that we capture all of your thoughts and ideas there.

We're going to turn things over to Bluebird, where we have Danielle and Meghan. Meghan is going to be on the phone. We'll hear their thoughts on the sustainable payment models. Then, we're going to open this up for some discussion with the manufacturers, and again, everything really framed around they know what's coming in their pipeline. How would they kind of recommend HCA be looking at funding options? Then, from there, we've got some open time. I think this is really where this workgroup is going to be able to dive in and really talk about pros, cons, and again, your thoughts and recommendations around HCA when you think about some of these funding models. So, with that, any questions, concerns about agenda today? Okay. So, with that, I will turn things over to Robyn.

Leta Evaskus:

Can you hang on just a second. I seem to have not put that on my computer.

Robyn Williams:

I'm Robyn Williams. I'm the senior budget assistant to the Governor for Health and Human Services. I lead the team that does the Health and Human Services budget for the Governor and advises him on budget-related impacts for all of Health and Human Services, which includes Medicaid, the Social and Health Services, Corrections, the whole spectrum of human services that we provide in this state. I'm talking today at a really high level about the Medicaid budget and kind of the size of it and the process to show you the challenge that we face with new therapies coming onto the market, if they come off a little challenging.

So, jumping right in. Medicaid is within the HCA. The total HCA budget in the state, we care about general fund state, but most everyone else would care about total funds. So, 5.8 billion, it's a huge, huge budget. Of that, most of it is Medicaid, 4.6 billion general

fund state, 17.7, all of these are biannual budget, so two-year. The managed care... I highlight here for you managed care rates and drugs, because those are, as you can see, about 3.5 billion out of the 4.6. So, the big drivers in Medicaid are managed care rates and drugs. Then, just to give you an overview of what the other things that the HCA does, there's community behavioral health. That's all outpatient behavioral health therapies for the most part. Then the drugs that are related to behavioral health, those are lumped in with this drugs amount here. There's the Public Employee Benefit and the School Employee, which is the new book of business. Those are not part of the Medicaid program. They are separate, but the Health Care Authority oversees that. Then, the Health Benefit Exchange is not part of the Health Care Authority, except for that they receive state funding that has passed through HCA. So, that's just there to show you the full book of business that HCA has here.

I wanted to go over how we do the Medicaid budget. It's a pretty collaborative process. There are people... most of the population is in managed care, as you saw, 2.5 billion out of the 4.6. So, that's most of the 1.8 million people are covered by managed care. So, the managed care organizations are right now out there spending money on people that they are getting a capitated payment for. When they present their actuals to the actuaries who do the managed care rates, they take that, update them annually for the calendar year. Then also, all of the other expenditures that are not covered by managed care, the fee for service population and things that are pulled out of managed care that are fee for service, that's all submitted into the forecast. The forecast is a collaborative process with the Office of Financial Management on both the budget side. We have a forecasting group. There is the Health Care Authority is obviously involved. There are representatives from the fiscal committees of the legislature. So, it's a collaborative process where we use actuals to project the cost going forward. The forecast, which is updated twice a year, is used first for the Governor's budget in the fall. Then, for the legislative budget, which they are putting together right now.

What I really wanted to highlight for you is, all this is going on, it's all based on expenditures, and it's all basically based on expenditures

that have happened. So, managed care rates for the year that we're in, the data is from calendar year 2018. So, obviously anything that came on the market in 2019 is not captured in that. The actuaries use information and trends that they know and apply that to the data, but if something comes on after the actuary, the actuary is more anticipating, that's just not part of it. Similarly, the forecast, the February forecast that they've just completed right now, that is using data through July. So, that's also not very recent. It uses past expenditures. If things we know are happening, like there's a health insurance tax, as part of the Affordable Care Act, we know that that's going to start to kick in. So, we can plug and say, we're going to have to pay that in these coming years, but if we don't know that something is happening, then there's no plug in the forecast. The budgets are set on a biannual basis and updated, but basically, it's really challenging. If something comes on the market that's not anticipated in the managed care rates, or not anticipated in the forecast, and it's expensive but only applies to a few people, then it might be a tenable situation, but if it's an expensive thing that applies to something that effects a lot of people, it might be very difficult for the managed care organizations whose rates are based on information that didn't include that. Or the fee for service population, or if it's something for a high cost drug, we've been pulling those out of the managed care rates, because it isn't necessarily part of the capitated estimate. So, it has the potential to break a budget. If something came on after the legislature was in session, but before the budget is updated, it could be really, really challenging for the Health Care Authority. So, that's what I wanted to show you with this slide.

I'm sorry. I'm going very quickly, because I have a very short time here. What I wanted to show you here is to remind you that this is all part of the State budget. The total pie, if something here in the Health Care Authority gets bigger, it doesn't necessarily mean that the pie gets bigger. If there's new revenue, then the legislature or the Governor are making choices of how to spend the new money on what their priorities are. Medicaid is an entitlement program. So, odds are, that it would be part of the consideration, but other things are priorities. There are emergencies, as we all have seen in the past

few years. There has been an emergency in the schools. There have been... it's just, you know, other things going on all the time right now. I'm sure there's probably coronavirus considerations, things like that, that the State has to respond to that are up against the Health Care Authority and the Medicaid budget. Just because the costs for Medicaid go up doesn't mean that necessarily the pie goes up. So, it can mean that the slivers for other areas have to get smaller, which is the challenge of the State budget. So, that's really what I wanted to talk about. I'm here for questions.

Mike Bonetto: Robyn, can you maybe talk a little bit about the history around hep C and how that impacted?

Robyn Williams: Sure. Hep C is a good example. I can tell you that when I started on the Medicaid program four or five years ago, the new drugs had come on the market, and they were part of the forecast, but at that point, the Health Care Authority was limiting who could get them. The Health Care Authority was being sued about it. So, I'm not a doctor. So, I think it was for fibrosis scores 3 and 4 were qualified, or were people who could get the drugs. Then, 1 and 2 maybe as special cases, but mostly 3 and 4. So, the forecast took the cost of the drug, put a step in. We knew how expensive the drug was and included that. Then, the lawsuit expanded it so that everyone who had hep C within some clinical guidelines at that point, could get access to the drugs. So, then the forecast adjusted. The forecast, each time another manufacturer came on the market, or there was another option, the price of the drugs would change. The assumptions around what percent of the people who were coming on month by month would take which type of drug, and it was based on, I believe, Donna correct me if I'm wrong, like, clinical assumptions about what percent of the population would be right for each type of drug. That was all included in the forecast so that HCA would be funded to cover the population. Then, now that we're in a new world, it's still being included in the forecast. The new world is a little bit more complicated to put in the forecast, but it's still in there so that HCA continues to have the funding they need. Does that help?

Mike Bonetto: It does, very much. Other thoughts, questions? Let me throw one more out there. As you've been a part of this group now for these last many months. I think you were at the first meeting when we had that overview of all of the pipeline drugs. You see this from a budgeting perspective. What keeps you up at night, in terms of how we balance this in the future?

Robyn Williams: So, the sliver of time, let me go backwards, on the last slide, the sliver of time that's after the legislature has enacted a budget that is before the end of the fiscal year is what really scares me, because something could come onto the... so, to give you the specific dates. The legislature is scheduled to end March 12th. The fiscal year ends June 30th. So, there's that three month period of time where something big could come on that came out of nowhere. Probably, if it was big and going to affect a lot of people, we would know about it now, but maybe not. That really makes me nervous. The other thing that makes me nervous is the pipeline of expensive things coming on the market. Odds are, at some point, we're going to have a recession again. This whole pie is going to have to shrink, but this wedge is going to need to grow, because the dollars are going to grow, but also the pie is probably going to have to shrink. That's really hard. We're still recovering... when I started on this workgroup, I just had the Medicaid Affordable Health Care perspective. Then, I've changed jobs. Now, I'm looking at the whole human services spectrum. There's not a lot of room to give anywhere. There are a lot of institutions that need funding. We're still recovering from the last recession across the human services spectrum. It just makes me nervous that we're going to have expanding costs and really competing and compelling stories. There's just... human services, as you can see, a good portion, at least a quarter of this is human services right here. So, we're an obvious target. Well, not target, but a place to go if the general state fund amount becomes less.

Mike Bonetto: Thoughts, comments for Robyn? Okay. Thank you, Leta. Just a comment to folks in the back, stakeholders who are sitting in today, we do have a couple mics. So, if you do have a question, we have some roaming mics we can get to you if you do have a question that you'd like to pose to some of our speakers. Thanks. Robyn, thank

you. I think that's a helpful backdrop before we get into all of this, in terms of just understanding a bit of the mechanics and how this works. So, thanks.

Robyn Williams: Yeah. Sure, thanks.

Donna Sullivan: I'm the chief pharmacy officer with the Washington Health Care Authority. I'm going to... my presentation is going to be shorter than 20 minutes. I'm really going to try to focus on some of the high level state strategies that not only Medicaid programs but public employees programs are considering, as we're trying to figure out how we're going to pay for these expensive drugs, or these emerging therapies, as they come out.

So, I'm going to talk to you about pharmacy benefit management strategies, multiagency purchasing strategies, briefly go over some alternative payment models, and then some affordability approaches that some of the states are looking at.

With pharmacy benefit management in the Medicaid space, some Medicaid states are considering carving out their pharmacy benefit from their managed care plan. So, about 70% of all Medicaid programs across the country are in managed care right now. There's probably a half a dozen or more states that manage the pharmacy benefit within their fee for service program, even for those enrollees that are in a managed care plan. Other states that currently have their pharmacy benefit carved out, some of them are actually considering pushing it back to the managed care plans. Then, overall, a lot of the states are considering implementing a single preferred drug list. So, if you'll remember in Washington state, we started to implement our single preferred drug list in January of 2018, but on this slide, if you can't read it from the audience the states with the stars are the ones that are currently considering a carve out. The grey states are fee for service only states. So, they don't have any managed care plans. The light blue are those states that allow their managed care plans and Medicaid to have their own formulary or preferred drug list. Then, the kind of medium blue/dark blue, those are states that have some sort of single preferred drug list, either a

comprehensive list. Or they might have part of the drugs that they cover on a common PDL across the different programs. Then the light green is managed care where the pharmacy is actually carved out of the managed care plan.

So, multiagency purchasing approaches, you'll see many states are looking at how they can either work together or partner within the agencies within the State. So, like, Department of Corrections, their public employee programs, their Medicaid program, Department of Health, whoever is purchasing healthcare within the state, they're trying to figure out, how can they all work together to try to leverage their ability to purchase or negotiate prices within their states. So, in that attempt, they're looking to gain market share. You might have heard about the Northwest Prescription Drug Consortium, where Washington State works with the state of Oregon to contract with a pharmacy benefits manager for our public employees' plans and other group plans in the State. It's a transparent contract. We have a lot of pass through pricing at the pharmacy. So, we don't allow spread pricing. Then, we also contract with a group purchasing organization through the consortium. So, our Department of Corrections and our state hospitals can get access to bigger discounts. Then, there are other states that are looking at doing some sort of combined interagency within the state purchasing. Those are noted here. California recently passed legislation that actually requires their state agencies to work together and to form a commission and work towards a bulk purchasing arrangement and also recently in California, there was legislation passed that would require California to start manufacturing generic drugs. So, that's one of the things that California is doing that's pretty exciting.

Alternative payment models, so these are also approaches where when we talk about alternative payment models, we're typically referring to an outcomes based contracting or a financial arrangement, financial based contracting, which is what Washington did with their hepatitis C model. So, with our elimination strategy, our purchasing strategy was to negotiate with the manufacturers for our Medicaid program so that we could cap our costs and really be able to predict what our costs were going to be. So, the model that

we have implemented, you'll hear it called, like, the Netflix model, or a subscription model. It's not a Netflix model. It's not a subscription model. It's really a financial based model. So, we've negotiated kind of a threshold, a treatment threshold where we have a guaranteed net price, unit price for hepatitis C medication up until we spent a certain amount of money, treated a certain number of patients, and then the guarantee net unit price drops substantially to the point where it's about a penny a pill, or really no additional cost to the State. It would be negligible. This would allow us to treat as many people as we possibly could without spending more than the significantly higher predetermined threshold that we've already decided. Another state, Louisiana, has a similar arrangement for their hepatitis C program. I believe it's up and running now. I'm not quite sure what Louisiana is. Ours in Washington, we started July 1st.

So, outcomes based arrangements, and I know the other speakers are going to go into more detail. These are mostly based on a performance metric. So, either a clinical outcome or cost savings, some sort of event driven event that drives the rebate. Then, Colorado, Michigan, Oklahoma currently have outcome based contracts with their Medicaid programs. Washington does, as well, but ours is more what we call a value based contract, but it's on the finance based side.

Then there are some affordability strategies. So, there's a lot of legislation being introduced in states looking at controlling costs. I don't know how many states are looking at drug price transparency legislation. I know in Washington. There was a bill passed in California, as well. I would say about half the states are looking at either they've passed drug price transparency legislation, or they're currently considering it through their legislative process. Other approaches are, like, New York set a spending tab where their budget office determines how much they're going to spend on drugs every year. Then, they'll look to see which drugs are going to pierce that threshold. Once they've identified those drugs, they've contracted with a third party to try to estimate what would be a cost-effective price to pay for those particular medications. Then, they will negotiate with the manufacturers in order to try to get down to that

price. If the manufacturers don't come to the table and negotiate, then there is a process in place to bring them to the Drug Utilization Review Board and open public meetings and different types of activities. Massachusetts also has a similar model. I'm not going to go into detail with that. Then, the other kind of piece that we're seeing are these affordability boards or drug price advisory boards. There is legislation currently active in Washington State. There is also legislation that was passed in Maryland, and I believe my reference to Maine is incorrect. I think it's Massachusetts. That's like a Board convening together and looking at just drugs that either cost a certain amount or have a price increase of a certain percentage. Then, the Board would determine what would be a threshold. What would be the cost for that drug? The legislation in Washington actually has the Board set an upper payment limit. Then, all state purchase healthcare programs like Medicaid, corrections, the public employees, school employees. They can't pay anything above that upper payment limit. There's all kinds of challenges with that, but that's just a legislation that's being set in Washington.

Other strategies that we're seeing, importation. Some states are passing importation rules to reimport drugs from Canada. There are states that are considering manufacturing. So, California has decided it is going to be manufact-, or contract with a manufacturer to create its own generic label. Then, again, the price transparency legislation that I spoke to earlier. Any questions?

Mike Bonetto: When you think about what you just outlined there, how do you view Washington's current position? I mean, as you think about moving forward.

Donna Sullivan: I think that we, as Robyn was talking about, is it's really a struggle. We have a finite amount of money, and all of these options sound great with the outcomes based agreements with the financial based agreements. We can make those types of contracts, but the medications are still unaffordable. So, it makes it really challenging in order to figure out where that money is going to come from. The money doesn't grow on trees. There's no gene therapy for that yet. So, you either have to increase the revenue, which means taxes.

That's our taxes out of our pockets. Or, those programs that Robyn was referring to are going to, you know, education is going to get cut. Transportation, I don't know if you could tell, it was already at 0%. Look at your roads. We're not going to get new roads. All those other programs are going to be cut in order to pay for some of these therapies. So, it just becomes really challenging when you have to weigh those different competing priorities, because it's all important. So, that's kinda where we're at and why we're here.

Carly Rodriguez: I wondered what your thoughts are on the legislation that's being passed. Oregon is one state that has passed kind of the price transparency legislation. I just wondered your thoughts on the effectiveness of that legislation and what they're trying to accomplish.

Donna Sullivan: That's a good question, because it was passed in Washington, too. So, I'm not sure how effective it will be. I don't necessarily know that it will cause price increases, but I think it will let people see what drugs are really costing. So, a lot of people that we cover in our plans, they're paying their coinsurance or their copay, and many of them really don't know what the true cost of the drug is. So, possibly that will bring more attention to just overall drug expenditure, in general.

Robyn Williams: Donna, I should point out that those pie charts where you saw transportation being 0%, that was the operating budget. Transportation has its own budget. So, there is more spending there.

Donna Sullivan: Maybe it was less than 1%.

Robyn Williams: I think that's the little bit that pays for State Patrol people to be out and things like that.

Rebecca Owen: The hep C is kind of a specialized situation where the drug had a hump. It was kind of a cure, but do you see that when you were thinking about the next drug that you're going to put into a finance based arrangement, do you have an idea of whether it should be an ongoing maintenance drug that you'd be looking at? Or would you be

looking at this short-term treatment type? Or what's the best... maybe that's the question for today. I've asked it too soon.

Donna Sullivan: My response to that... I've been asked that question before. When are these different alternative payment strategies most appropriate? So, the finance based approach is really when you're looking at something that is moderately expensive. It is used to treat a whole bunch of people. So, like, insulin would be a great example to get into some sort of arrangement with that so that you could get everybody treating... treat their diabetes using insulin without having your expenditure for insulin keep increasing. Other models, the outcomes based models, might be more for drugs that are really, really expensive but treat a very small population.

Mike Bonetto: Armen, we'll get you in just a second. One other thing I would add, Donna, when you think about the hep C work with Washington, and Rebecca this is what we've seen with other states. When you're looking at even aligning multiple agencies, having a drug class that has competition can be a big thing. Right? So, what's one thing for this group to think about when you think about some of these orphan drugs? There's no competition, again how could or should the State handle that. Armen?

Armen Khatchatourian: So, refresh my memory, though. What's the breakdown percentage wise for different types and lines of business you guys have, like, Medicaid population, state employees that are probably under the commercial plan. Do you guys manage all of them in house?

Donna Sullivan: Yes. So, let me caveat that. We have about 1.8 million lives in Medicaid. I would say 85 to 89% are in managed care. So, we manage the managed care plans, but our fee for service Medicaid program is about 250,000-ish. We manage those in house, literally in house. We're at the PBM doing the claims processing, the prior authorizations. For Uniform Medical Plan, our school employees program, and our public employees program, we have our self-funded program, which is Uniform Medical Plan. Ryan, that plan has about 280,000-ish for the public employees side. How many for the school employees?

Ryan Pistorosi: That's right. In UMP about 106,000.

Donna Sullivan: So, we have almost 300,000-ish in our self-funded program that we don't manage... we're not doing that work in house. We manage those contracts in house. So, Ryan is very involved with the Northwest Consortium Vendors or Moda Health. So, he is very involved with them on a day to day basis to manage those programs, but there is also probably another 300,000 covered lives that are managed through a fully insured public employee or school employee plan. So, like Premera, Kaiser Northwest, and Kaiser Washington. Is there another one?

Ryan Pistorosi: No. That's correct.

Donna Sullivan: Okay. Thank you.

Armen Khatchatourian: Do retirees fall into that, as well?

Donna Sullivan: Yeah. So, retirees right now, we cover our public employees and school employee retirees in the associated plans. We are in the process of trying to contract with a Medicare advantage plan with a Part D component, but that procurement is ongoing.

Mike Bonetto: Other thoughts, questions from the audience? Donna, I've got another one for you. If you wouldn't mind backing up to the slide. You had a really great state map. I'm gonna maybe put Rebecca on the spot a little bit, because there has been a lot of discussions of to carve in, to carve out. What's the right balance? Right? I'm interested even in Donna, in your perspective, and Rebecca's, sometimes we talk about a carve out being the full pharmacy benefit being carved out. Sometimes, we talk about a partial carve out. We talk about a single drug class being carved out. I think that is something for this group to be thinking about. When you think about the newer emerging drugs, how should HCA be thinking about that even from a carve out perspective.

Donna Sullivan: I can tell you what we're doing right now. As the new drugs are coming to the market, we have worked with the managed care plans. It really started with the hepatitis C that there was the cost and the number of people that needed to be treated. It was obvious that the plans capitated rates were not sufficient to treat everyone. So, in 2015, we carved the hepatitis C medications out of the managed care responsibility, and we cover them through fee for service. It was after that point where we then started seeing the other drugs that were coming out that were just really expensive, one after another. Over \$100,000. So, we have now set up a process where we're looking at the pipeline, we're identifying drugs that are going to cost \$100,000 a year or more. We just automatically are carving them out of the managed care rates. So, eventually, or soon, we'll probably have 5% of the drugs carved out, but 90% of the drug expenditure carved out.

Mike Bonetto: Rebecca, thoughts?

Rebecca Owen: It isn't just your Medicaid plans that will benefit. The State can consider all of the residents within the State self-funded employers that are too small who could be put out of business by having a whole family with \$700,000 in bills. This is a new way of thinking about what has been a really tough problem. One of the things that you want to think about, as you go forward is, how do you keep the medical side under control, as you carve out the pharmacy, in search of managing costs? Because it's a... 18% of your costs, although they're huge, are in pharmacy. You want to make sure that you keep those two worlds talking to each other very well. That's sort of two big parts of this. Getting everybody under the umbrella and making sure that all kind of care stays integrated. Incidentally, I think that Kentucky is bluer than you have. As of 2:00 this afternoon.

Donna Sullivan: I just borrowed this slide. I didn't make it. So, I won't... thank you, though.

Mike Bonetto: Hey, we've got folks on the phone. Questions from anybody on the phone? Comments? Okay. We'll keep moving. Donna, thank you. So, we'll turn things over to Sean and Carly.

Carly Rodriguez:

My name is Carly Rodriguez with Moda Health. Sean and I were asked to talk about funding options, as it relates to emerging therapies. So, we'll focus primarily on some of the ultra-expensive drugs, like, gene therapy and cellular therapies. I'll start with just a brief background on what gene and cell therapies are, just to set the stage and make sure everybody is on the same page. Then we'll talk in more depth about some of the funding options before turning it over to Sean. A couple of you in the audience have seen this presentation before. I don't know if that's good or bad. Alright. So, just to get started, I wanted to level set on what cellular and gene therapies are. They are overlapping fields of research that have similar therapeutic goals. It's kind of difficult to distinguish between them sometimes. You hear them used interchangeably, but cellular therapies, what we're really looking at, is transfer of whole live cells to produce an immune or other biological response in someone's body. So, some examples are listed here, cancer vaccines, while they don't exist today, are some of the examples, I guess, are stem cell transplants. Gene therapy really using genetic material to modify or manipulate the expression of a gene or alter how a gene is behaving in the body. When you look at different kinds of gene base treatments, I'll reference... in fact, this website actually does a really good job of kind of in plain language highlighting what some of the different types of gene base therapies are. So, there's gene replacement therapy. This gives cells new working copies of genes using a vector. Those are the products that we see available on the market today. Gene editing can insert or remove, change or replace specific genes. CRISP-R is an example. Many people have heard of this technology. We don't see it widely used yet. Then, CAR-T is another example where a person's immune cells are modified and then delivered back to the body to fight cancer currently. That's used with a gene based therapy to modify the CAR gene.

This is just a visual depiction kind of what I just said. You can deliver in the in vivo setting. You can just deliver via a vector gene therapies to the body, or on the ex vivo side you can extract cells, like stem cells, modify them, and deliver them back to the original host.

I just wanted to really quickly talk about the current landscape and emerging therapies just to give a sense for kind of the magnitude of what we're looking at from the payer perspective. So, first starting with cellular therapy, I just wanted to give an example. I'm not going to go into a lot of detail in the interest of time about what some of these are, but just to give an example of what kinds of products are available on the market. So, stem cells, CAR-T, which many of us have heard a lot about and know a lot about. Immunotherapies, like Imlygic and Provenge that are available to treat cancers. Then, there's others that are used for various things like wound treatment and even cosmetic uses.

We see a pipeline that's more robust than the one I've put here, but just a couple of notable therapies that are in the pipeline and the cellular therapy space, a couple of CAR-T therapies, as well as another immunotherapy for prostate cancer.

Moving onto gene therapies. We have two that are currently approved. Lexturna is used for rare form of inherited retinal dystrophy. This is a one-time treatment. It has a list price of \$425,000 per eye, or \$850,000 for both eyes. There is also the cost associated with ongoing treatment and monitoring. Zolgensma is the other. This is the most recently approved therapy for certain patients with spinal muscular atrophy. I think most people are aware of the price. We've seen it all over the news, 2.125 million dollars is the list price for one-time therapy, and then it also has associated administration costs, of course. There is also some misconceptions about Zolgensma, in that it replaces Spinraza, which is another therapy approved for SMA that's a non-gene therapy. It's interesting that I hear that in the pharmacy community a lot, that it offsets the cost of Spinraza, or replaces the need for Spinraza, but we know, based on some of what we've seen from clinical trials that some patients actually went on to be treated with Spinraza, which, in itself, can have a \$750,000 a year price tag. Also, Spinraza and Zolgensma may not have the same eligible treatment populations.

Really big pipeline for gene therapy. I just made a whole slide for hemophilia by itself. A couple that we might see this year, one for

hemophilia A, one for hemophilia B. I think this is a really interesting area that payers are watching really closely, because this isn't an ultra-rare disease state. Many plans have, for example, we have 40 hemophilia members that we know of right now. So, the thought of these coming out to market with a 1 million or 2 million dollar price tag and thinking that we have 40 hemophilia A members. Obviously, they're not all going to be eligible, but if a good half of them are, thinking about being faced with a 20 or 40 million price tag paying for gene therapy once is pretty daunting. I think this is also kind of... it's a really exciting category to see innovation. We know that these patients have significant burden associated with their disease and using huge amounts of factor in some cases that really impacts their quality of life. I think one of the challenges here, which is similar to some of the gene therapies that we see on the market is that we don't really know what the durability of response looks like. There have been some conversations around potentially an eight year durability of effect. So, are we going to see treatment again after eight years? I think the other piece is that if the thought is that gene therapy replaces the need for other therapy potentially, we also haven't seen that completely. Some clinical trials where patients have continued to use factor, albeit less factor, there is still going to be a significant cost associated with factor treatment.

This is also again just a subset of some of the other gene therapies that are in the pipeline. A couple that might be approved this year or next year for some very rare but potentially life threatening conditions. I think we'll hear more about them in a later presentation. So, I won't talk about them in a lot of detail, but again, a really exciting innovation here for disease states that have not had treatment before. We'll look to clinical trials and other evidence to see what the efficacy and durability of response looks like for these patients.

So, I'll move on to some of the alternative payment strategies, payment models, or things that payers are looking at to really help manage market entry access and the cost of these therapies. Given that many are going to come with 1 million dollar plus price tag, and as I mentioned with hemophilia, a disease state where we're not

looking at, as a plan, potentially paying for one gene therapy patient a year. We're looking at potentially ten or more. I also wanted to just pause for a minute to think about what a payer is. I think a lot of people think about payers as insurance companies and PBMs as an example, but when we think about employers and public entities that are self-funded, I mean, thinking about public entities, in particular, that ultimately the payer is the taxpayer. So, I just wanted to make sure we're all thinking about who payers are in the same way. I think with gene therapies, we'll continue to see payers using fundamental approaches, like prior authorization, to really continue to ensure that it's the right population based on how gene therapy has been studied or shown to be safe and effective and ensure that those are the candidates that would be eligible for coverage of gene therapy. Interestingly, this coverage and looking at who the right patients are may differ from the FDA label. We're seeing that with Zolgensma now with some payers coverage Zolgensma based on how it was studied, not based how it was labeled, which are different. Other use, I mentioned earlier, that is kind of being monitored closely is pre and post therapy with other treatment alternatives. I gave Spinraza and Zolgensma as the example. We're seeing some payers who are managing closely whether or not people can use Spinraza before or after Zolgensma. Right now, for the therapies that are available, looking at a one treatment per lifetime, but as I mentioned with hemophilia and maybe some of the other gene therapies that we'll see, whether that's appropriate moving forward, we'll see. I think again, we've kind of heard potentially the eight-year timeline as what we might see with some of the hemophilia gene therapy products. A lot of plans may outsource prior authorization review to their PBM, as an example, or a medical management company, but I think with gene therapy, you're going to see a lot of in-house management in terms of prior authorization review that it's going to be up to the plan, itself, to make that coverage determination, rather than a PBM for some plans, at least not in the market. I think, in addition to prior authorization, I think kind of billing and coding management is really important across departments within healthcare organizations. The same people that are establishing coverage criteria are not the same people that are reviewing claims. There can be a lot of confusion with billing units. I gave the billing units for Luxturna as an example.

No claim system is going to be able to take in 1 billion... that many digits in their claim system, but that's also just not the correct billing. So, in this case, we would see probably a lot of times providers billing for way more units than they should be. So, we'll see a lot of manual review. It's just important to have that kind of communication across departments within a payer.

So, I wanted to shift focus to maybe some less fundamental, but things that I think have become really commonplace in discussions and payer organizations, and stop loss and reinsurance is one of them. For the first five or so years of my career, I had never heard of the reinsurance concept. Now, I talk about it every week. To level set, when you look at stop-loss or reinsurance programs, specific stop-loss, you can have a reinsurance program that's for a specific member to tie cost. Or you can have aggregate stop-loss programs that set really kind of a dollar threshold for a plan's spend across the board and then provides protection for claims that are incurred above those thresholds. So, I think in the simplest terms, what I tried to represent on this slide is, you can think about it as insurance for insurers, for self-funded plans. So, really, you reach an agreement on the defined coverage terms and limitations, plans pay you know premiums or deductibles associated with those coverage terms. When a plan incurs a large claim, either for a specific member or just across their book of business that is submitted to a reinsurer. Then, the reinsurer can reimburse above that based on the coverage terms, I should say. These kind of arrangements provide a safety net for payers. I think Robyn talked a lot about the unpredictability. You're making guesses in these arrangements based on the claims experience you've had maybe for part of the prior year based on what you know about the pipeline, but you can never be certain. I think the other challenge here is that reinsurers can exclude certain services, or they can laser certain patients where they are either not going to reinsure for that specific member, or that specific treatment type, or they're going to be associated with a much higher deductible for that particular member. So, there are definitely challenges here, but it does provide somewhat of a safety net.

Another model that I'll talk about is kind of the pay over time model for Zolgensma. I think it might have a specific name, but I'll just call it the pay over time model. The thought here, I mentioned the list price earlier, but the thought, I think, from the manufacturer is that it's easier to budget or think about paying smaller installments over a period of time than paying a lump sum of 2 plus million dollars all at one time. These kinds of arrangements could be coupled with outcomes based arrangements also. I'll talk more about that a little bit later. Some of the challenges with this model, I think this model can really work for some smaller plans potentially. I think the vast majority of plans that I've talked to, this isn't a model that they're interested in. That's because of stop-loss and reinsurance, like I talked about, that are in play for many self-funded groups and commercial payers. It's better to pay the lump sum at one time and have it hit reinsurance and not be liable for the whole cost of the therapy, because if you pay \$425,000 one year for a patient, it's going to hit the reinsurer's radar. That patient is potentially going to get lasered. They're either going to be not covered, or they're going to be associated with a higher deductible or premium. So, that will kind of impact the safety net that you have over time. Significant financial risk still remains on the plan. Whether you're paying it all at once or over five years, the financial burden really falls on the payer. I think the other piece is that from an actuary perspective, and maybe Rebecca you might have thoughts on this, but the feedback that we hear from our actuaries is that it's really more difficult for actuaries within payer organizations to accommodate or account for the longer term payment models, especially when you have turnover. So, your member leaves a plan, but you're still paying for a therapy for them for four years. It can be challenging from the feedback that we've gotten.

I'll switch and talk about what's called the Embarc benefit protection. So, this is a program that's available from CIGNA, ESI, and eviCore. This, I think, is more of the kind of subscription model that people think about where it's a prepaid program. You pay into this program to have access to the network that dispenses gene therapy products like Zolgensma and Luxturna, and you're protected if you're making these monthly payments, if you have a member that is eligible for

coverage of Luxturna or Zolgensma. So, the way that it's being promoted is that it really eliminates out of pocket payments for employers or unions, but I think what they really mean is that it eliminates surprise out of pocket payments, because you're still paying into the program. There really are, at this point, few details known about this program. I think more and more details are emerging kind of every month, but it's available to help plans, other payers, employer groups. It really provides kind of a network for gene therapy providers. So, there's pharmacies in the network. You pay a PMPM rate to eviCore to have access to that network. Then, if you have a member that's eligible for treatment with Luxturna or Zolgensma, the cost of that therapy is covered by the monthly payments that you're making into this program. The price is rumored to be about \$1 per member per month, regardless of the number of covered lives that you have.

I think that's turning out to be more than a rumor at this point, that it's \$1 per member per month to participate in this program, but there are, you know, definitely some challenges with this program. I think, again, this is a program that could work for some smaller payers. It looks like you can't necessarily, on the fully insured side, you can't necessarily identify a specific subgroup that you want included in this program. It's really more like your entirely fully insured book is part of this program. So, it may not make financial sense depending on the size of the plan. So, as an example, if you're a 300,000 member plan, and you're paying \$1 per member per month, at the end of the year, you're going to have spent 3.6 million dollars on these payments. So, unless you have a Zolgensma member and maybe a couple of Luxturna members, you're not getting your money's worth. Buy and bill could be advantageous, I think, in the gene therapy space for some products that have agreements with specialty treatment centers where there is an agreement to not mark up the list price of a drug. So, there are some cases where it could be advantageous to keep these drugs on the medical benefit. It's also a little bit unclear, getting some more clarity, around what utilization management requirements might be. It looks like you probably will have to follow express scripts prior authorization guidelines. It's unknown if per express scripts criteria a member wasn't eligible for

coverage, but then an independent review organization overturns that and says that they are eligible, is that member still covered under the program. Of course, plans are still liable for the expenses associated with administering these drugs, and we don't know what contract term limits would look like. There is also some talk that there could be a refunding portion of this agreement. The 300,000 member plan example that I gave, that you could get to the end of the year, and if you haven't used it, that there is some portion of the fees that you've paid in that are refunded, but there's not a lot known about that at this point.

Outcomes based agreements are another thing that many plans are looking at. Donna mentioned them briefly, but basically manufacturers are backing the clinical performance of their therapies, defining outcomes where there is a rebate or incremental rebate available if and when that defined outcome occurs, usually a negative outcome, meaning it didn't work. This partially addresses the unknown in efficacy or durability in response. I think the challenge is that at least in a lot of the agreements that have been talked about, so far, for the products that are available to date is that there is still substantial financial risk that remains on the plan. If a portion of that is rebated back, it's probably not a huge portion at this point, because of issues with Medicaid best price potentially. There are other challenges from an operational perspective with member turnover if the outcomes are over several years. It's difficult to follow members over several years, if they're not in your plan anymore. Then, some of these outcomes based agreements we've seen have terms that include things that providers are responsible for. So, as a plan, it's not an integrated delivery network or system. That's difficult to control what a provider is doing, as we all know.

Lastly, I will just touch on preferred treatment sites. With the gene therapies we are seeing that there is really just a subset of select treatment centers that manufacturers might be contracting with to deliver these gene therapies, and payers might contract even a smaller subset of those preferred treatment centers. That can have a lot of advantages, in terms of negotiating reimbursement if all patients are going to one place, as a center of excellence for example,

but also in terms of consistent quality service and care. A lot of these treatment centers were involved in clinical trials. So, they have had the vast majority of experience in treating these types of patients using these drugs. Some of the challenges can be that support for patient and family travel. We've seen with at least one of the gene therapies available today that the manufacturer does provide support for that travel for the patient and a family member that includes transportation and housing for the period of time that they need to have treatment. Another challenge could be that this causes network disruption. If you have a strong relationship with a provider in your geography, but you're steering members elsewhere can create some challenges.

So, I'll turn it over to Sean and just say that I think gene therapies are really bringing exciting innovation, and payers are really excited about that. I think payers are also really anxious about the price tag that comes with them, especially as we start to think about disease states like hemophilia where you could have again 10, 20, 40 members a year that are eligible for a multimillion dollar treatment. How you afford that is a question that is unanswered, I think, at this point. Thank you.

Mike Bonetto: Carly, thanks. Hang on at the mic. Is it better for you... you guys have tag-teamed before? Is it better to do questions all at the end, or do you want to do questions for you now? Is there a preference?

Carly Rodriguez: It doesn't matter. I'm happy to take questions now and later.

Mike Bonetto: Any outstanding questions? Does anybody wanna get out on the table now? Rebecca, any followup on the stop-loss reinsurance?

Rebecca Owen: Maybe this year we're okay, and next year they'll make sure that they're not losing. So, it's a good thing. It's not something you can rely on, or you're going to have to pay a lot for it, if that makes sense. You either pay a lot, or you've got lean coverage, especially two years down the road, for instance.

Carly Rodriguez: That's a really good point, Rebecca, that even if you're covered this year when you pay the 2 million dollars for a gene therapy, you're going to be making it up in premiums the next year.

Rebecca Owen: Right. And if you shop, am I supposed to be on here? If you shop, it will be something that you will have to reveal to your next stop-loss carrier. So, it's not like you can come all naïve and look for the best first year deal again, which is another reason to think about this from something other than a commercial stop-loss carrier. If you're trying to take care of many, many people like this.

Mike Bonetto: Carly, one thing, I was taking notes, and we don't need to get into detail now, but it certainly plays into the next meeting in April. You had the great phrase, the durability of response. I like that, and I'm just kind of curious, in your thinking, all of these new drugs have this kind of difference in durability of response. How is Moda looking at this in terms of how you guys categorize that?

Carly Rodriguez: I think the way that we're kind of taking it as they come, right, with the new approvals, but I think the existing gene therapies, they were studied and promoted as one-time treatments. I think one of them is actually maybe, they are potentially looking at retreatment. So, we take the approach that they are once per lifetime per member treatment. Again, with hemophilia, that may be a different story, depending on what the evidence looks like, but I think that one of the big challenges is, I think for Zolgensma, as an example, I think we have, like, five or six years' worth of data now. That's approximate. It could be wrong. So, we know that there is at least some durability of response, but the studies aren't going to be 20 years long, and rightfully so. Patients that are eligible should have an opportunity for access earlier, but I think the reality is, we just don't know. So, that's the approach that we're taking right now. I think that hemophilia end up kind of changing how we think about retreatment.

Mike Bonetto: Thank you. Anybody on the phone with questions, comments? Okay. Thank you, Carly.

Sean Sullivan:

So, Mike, in the interest of time, I'm just going to focus on three slides, if that's okay and talk to those. So, let's start with this slide here. So, we're talking about these Emerging Therapies as if they sort of all have common characteristics. It turns out, they actually don't, but there are some characteristics about them that make them different. I just want to talk about these for a second so we can put those on the table, as we're thinking about potential strategies. The first is that these Emerging Therapies that are high cost are largely therapies that are directed at what are nontraditional pharmaceutical industry targets, disease targets. They represent small populations, rare and ultra-rare diseases, some genetic targets, and patients who are incurable or resistant to treatment, particularly on the cancer side. It's interesting, I was at a conference last week in London. One of the evaluators from NICE said the more we know about genetics, the more we're going to turn every disease into a rare disease, which leads to the possibility of very targeted precision medicines that can come with high cost, even for diseases like asthma and COPD, etc., that's something we can talk about at some other point if you'd like. So, that's a first, nontraditional targets. The second is that the FDA, our regulator, along with the EMA, which is the regulator for Europe, have been very open to a whole host of different approaches to studying whether the treatments work or not, which challenges those of us who are involved in host approval evaluation, as to whether or not they were, and as to whether or not they are safe. That's because the clinical research programs that are designed to support regulatory approval don't follow the traditional models that we're used to, two large well controlled randomized trials. Rather, what we're looking at now are these oftentimes single arm studies, sometimes with a basket of populations, not a single population, not a single homogeneous population, and we're very focused on biomarkers as the outcome, leaving us asking questions about whether the treatments actually work on the outcomes we're interested in. Of course, the regulators have been pushed by patient groups, by the industry, and by their own sort of scientists to try to encourage early market introduction, get these therapies out faster. That, of course, means intensely faster access to patients for patients, but it also means a whole ton of uncertainty on the part of those of us who have to make decisions about how we cover price, etc. We just

don't know much about them. These therapies also, many of them, come with the potential for cure, but rarely with the clinical programs, back to bullet number two, do you know whether they cure or not. Secondly, as you raised, we don't know the durability of that response. Very significant questions, and I'll talk about that on the third slide when I point to some potential opportunities for how to address those issues. All of this raises the question for these kinds of therapies is what is the true value. I mean, that's really the question we all skirt. What's the value of the cure? We have some benchmarks. For patients with hepatitis C, we're saying we're willing to pay about, I mean, if you do take your contract and sort of do the math on it, it's somewhere between 45 and \$50,000 a cure, roughly. That's to cure someone of a disease that would potentially lead to significant complications, lots of extra costs, maybe even death or early mortality. On the other side, we've got therapies like Zolgensma, which are 2.1 million, [inaudible] for the \$350 to \$450,000. Again, for a potential cure. So, we've got 50,000 on one side for a cure, all the way up to over 2 million dollars on the other side for a cure. So, what are we willing to pay, as an organization, as society, as a state funded agency for a cure? So, why this matters is because these treatments are coming at a very fast pace. There are over 800 therapies that are in the pipelines right now, both in North America and Europe that have the potential to target these very rare small population conditions, potentially cure, and are very likely to come at a price tag that is significant. I use the word here astronomical, alright? Astronomical because the idea that we're going to pay 2 million dollars for an hour infusion for a therapy just is mind boggling, maybe not to those of us sitting around the room, because we think about these things daily, but you talk to the general public about this, they are just blown away by this possibility. So, 2.1 million dollars for a single dose gene therapy for SMA type 1 is one thing, right? But that price also signals others. Donna, you made a statement when you were up here, and you said this is not sustainable, and it's not affordable. It's unaffordable. Those were your words. So, for me, a Lamborghini is unaffordable, right? I don't own one. I can't buy one. Therefore, I didn't buy it. That's unaffordable. These therapies that we've been talking about, you guys are actually paying for it. So, it's only unaffordable the moment

in which you say no. You're not saying no yet, right? So, that's a signal to the marketplace that says, we haven't reached, really, the non-affordability threshold in healthcare. So, I want to make that point, as well, that if we really want to make a dent and want to say something about affordability, what it means then is we have to say no. I mean, significantly full stop no. There are, as you know, other countries around the world who do that, who say no. They pay, on average, a whole lot less for therapies than we do.

I'm going to skip all the rest of this stuff. Those of you who were at the AMCP meeting in the fall when Carly and I were there, I talked about these. I want to just focus on the last slide here, which is really what you asked us to talk about, which is what are the emerging payment models and Carly has mentioned a few of these. I'll make a few points and then talk about some things that aren't on here. So, the annuity model, which is payment spread out, made over a defined period of time from months to years is being experimented with right now. Folks are trying it. They're trying it both by asking the payer to accept some annuity payments over time, but also using a third party, like Embarc as an example. Have Embarc actually take the annuity risks, pay the health plan. That's a sense of what Embarc is doing. In the case of Bluebird Bio's product that's coming, will be here soon, is the five-year annuity payment, \$356,000 x 5 years, but linked with the promise that the payments would only continue, as long as the treatment was effective, right? So, linking an outcomes based contract to an annuity payment, and the question is whether this is affordable or not, this doesn't address affordability. What this addresses is, how do you get the therapy paid for, right?

I want to talk about the sort of bottom orange there, discounts and revenue caps where the therapy prices is reduced by a percentage of the total revenue and achieves a certain target. So, we have some experimentation in the country with revenue caps, but we have a country in Europe, France, who has been doing this for quite some time. They found this to be very effective. The way they do it is, they'll say to the industry, we are going to, because we can't by law negotiate on price, we're going to negotiate on volume. That volume is going to be based on the total number of patients that we see in

our system who could potentially be exposed to your drug, and with accounting for an assessment that France does with respect to the significance of the treatment, arrive at a price, multiply that by the number of patients that France believes should receive the drug, and multiply those two, and cap the expenditure on the therapy for that year. Any expenditure above that, any sale of the drug above that, falls at the feet of the manufacturer. They've been doing this for decades and have been very successful at it. Of course, if we were to try something that like in the U.S., we would need a law for that and need special authority and permission to do that, but it works. It works for France. They have innovative therapies in France, just like we do. They have control over their budget, although they are worried about the same future that I described a little bit earlier.

I want to talk about what's not on this slide, because some of these things Carly has mentioned, but there are three things I want to talk about that are not on this slide. The first is the patent. In this country, the patent is at 20 years, by law, but for biologics or for treatments that come with multiple patents, so the product is not just patented, but the delivery system is patented, etc., that effective patent life can go on way beyond 20 years. What if, let's take hepatitis C, what if the State of Washington declared hepatitis C to be a public health emergency, right? Through that declaration, then indicated that they were going to use eminent domain to snatch the patent from whichever manufacturer, turn that product manufacturing, because they now sort of wiped out the patent, turned that product over to a manufacturer, a generic manufacturer, or maybe one of their own, in California that's going to emerge here, and said manufacture that for us. We'll pay you a reasonable price for manufacturing, and then we're going to distribute that, now no longer a patent protected product within the State of Washington, because hepatitis C is a public health emergency. People have talked about that. It's not farfetched to think that could happen for things like coronavirus, for example, or other types of public health emergencies. So, that's one thing. Second, international price referencing. So, this has been made public to us from a Trump Administration proposal for Medicare Part B, which is essentially to identify 12 to 14 countries, outside the U.S. where there are products

just like the ones we're interested in reimbursing, and finding out what those countries pay, bringing all that data in, and then making some calculation on the prices from those 12 to 14 countries. Then, telling manufacturers if you want that product available in the State of Washington in the Medicaid program, we need the average of those 12 prices. Or we need the median of those 12 country prices. International price referencing happens in about 50% of all countries in the world. We are very slow to this game. The Secretary of the Department of Health and Human Services for the U.S., who was a former executive at Eli Lilly said that A) We should be doing this, as long as we find the right countries to reference. B) Doing so would only have a 1% or less impact on the industry's ability to innovate, which has been the industry's pushback on any kind of regulation, which is it'll take resources away from our ability to innovate; 1% is what he said, as a former executive of a large pharmaceutical company. So, that's the second thing. The third thing is, how do we address issues... by the way, third and final, how do we address issues like durability of benefit, because what we're doing with a price... making a payment on a drug like Zolgensma is, we're taking a risk, right? We're buying the notion that this is potentially curative, although there's no evidence. There is no evidence that treatment cures. If you look at the clinical data, the word cure isn't in there. It says it has the potential, right? Now, I'd love to... if I had a kid with SMA type 1, I would love to take that gamble, because I know the alternative, but we don't know. So, why not move to a program that Medicare put forward 20 years ago, which is, let's cover with evidence development. Let's say, we'll pay for Zolgensma. We'll negotiate the best price, but what we're gonna do is, every patient in the State of Washington who gets put on that therapy is going to enter a registry, and at the end of five years or six years or whatever, we're going to assess whether, in fact, the claim of cure happened. Now, we know, from the clinical trial data, that there is already 17% of the patients in the clinical study that are receiving the alternative therapy, Luxturna. That tells you it's not curative in everybody. So, move to a potential platform where we cover, and we ask the scientific question to prove things like cure, durability, etc., the things that we don't have answers to, because the FDA and the EMA are allowing manufacturers to run innovative clinical programs, but they

leave major clinical questions unanswered. So, I'm going to stop there, Mike. I'm happy, along with Carly, to take any questions that people have.

Mike Bonetto: Thanks, Sean. Thoughts? Questions? Nobody? Folks on the phone? So, Sean, you just outlined all of those areas from annuity all the way down to durability. Are you looking at kind of pushing on all? Where would you, if you had kind of a laser focus, where do you think there's the best energy spent right now?

Sean Sullivan: I guess to point out that none of these programs here focus on price setting, right? The U.S. is a free pricing market, one of the last free pricing markets in the world for industry. So, when AveXis and Arvator set the price for Zolgensma, and they said 2.1 million dollars, there was no entity in our country that could push back on that and say, hey, wait a minute. So, we moved, instead, to pushing back on price to focusing on, well, okay. So, how are we gonna get that paid. It was sort of a tacit acceptance that we're going to accept that price. That's how we all behave. All state Medicaid programs behave this way. All private sector health plans pay... it's almost like we've kind of given up on the notion that we have to push back on price. We're waiting for some federal attempt to do this. So, given that we don't have a federal attempt to do this at the moment, other than potentially international price referencing, which may in fact crack that nut for Medicare Part B, I think you have to think about affordability and focusing on paying for the outcome that you want, because there's so much uncertainty in the clinical programs for these therapies, that linking payment to the assurance that the therapies are actually doing what they say they're gonna do, is key, from my perspective. Otherwise, we're potentially paying for vaporware. Not just \$50 but millions of dollars.

Mike Bonetto: Other than Medicare, what you outlined, any other examples of where you highlighted...

Sean Sullivan: International price referencing?

Mike Bonetto: No, more of this cover with evidence development.

Sean Sullivan:

Yeah. So, Medicare has been the one who has done this most. They've done it for nondrug technologies. The most recent they did was for lung volume reduction surgery. They had a very advanced program, because they were paying for lung volume reduction surgery, not knowing whether it worked. So, they went through a situation where they actually, themselves, Medicare, paid for the trial, studied it, found out that guess what, it doesn't work. Then, they decided not to cover it. You saw coverage rates and reimbursement rates go from thousands pre-research to almost zero after research, because that research answered the question. Medicare has been the one who has done that in this country. In other countries, Sweden does this, as a matter of course. Whenever there are uncertainties around outcomes of medical technologies, they will insist on bringing patients into a registry post-approval, and then studying whether that therapy actually produces what the manufacturer or the innovator says it will produce. Australia actually has a very big program and coverage with evidence development. You used to be able to see it on their website. They had everything, but a few years ago, they actually made that less transparent. So, you can't really see all the details there, but there are investigators who will publish the results of those kinds of studies. So, there are places other than Medicare, just not in the U.S. We haven't done that routinely.

Mike Bonetto:

Thanks.

Jonathan Espenschied: Quick question. I understand the transitions that we've seen in clinical trials, phase 1 being the benchmark, phase 0 at a brief stint. You're now seeing gene therapy, and then you're seeing CAR-T cell therapy and all these others that either look at biomarkers or it might look at tumor markers. Is there any approach that you've seen with this cost control that could be done at a state level that we could maybe use as a pilot or a proof of concept in the State of Washington, with your experience? Is there anything that could be looked at on a smaller scale?

Mike Bonetto: John, you're talking about a coverage with evidence development strategy?

Jonathan Espenschied: I think that's probably... And you would know better than I, but I think that's probably the best approach to approve something, but I'm open to any kind of discussion.

Mike Bonetto: Yeah. It's a great question. If you're okay with this, let me think about that a little bit, because I want to just review in my own mind the criteria that make coverage with evidence development work. Sean Tunis, who used to be the head of the coverage division at CMS for Medicare, he wrote a paper in JAMA a number of years ago when he was describing coverage with evidence development and what characteristics they looked for in terms of the therapy or the surgical procedure that would actually trigger them to think about coverage with evidence development. I think that would be the . . .

Jonathan Espenschied: I remember that.

Mike Bonetto: ...yeah. That would be a good reference to sort of unearth and revisit.

Jonathan Espenschied: Yeah. It would be helpful, I think, to see, especially with this group, if there could be any small measure that we could even suggest at a statewide level that might be helpful, but thanks. Very good points.

Armen Khatchaturian: Okay. Sean, do you foresee any... I get what you're saying about kind of reference pricing and even capping, but do you see any of this kind of squeezing the balloon, cost being shifted over to commercial lines of business.

Sean Sullivan: Great question, Armen. So, you know, it's clear that whatever we do with policy, when we're still in a free pricing environment in the U.S., anytime you squeeze part of a massive balloon, it's gonna pop up in other places, whether it's the commercial sector or whatever, whether we restrict patient access programs or 340B gets adjusted in some way, yes. So, because the industry operates on a target average price for their product across all their lines of business both

in the United States, but globally. If they see things being squeezed in one area, there are discussions and adjustments that are made in other areas. That's not just the pharmaceutical industry, by the way. That's any manufacturer of any product of any type. They do this in the computer sector. It's done in the transportation sector. If you lose a little bit from a pricing perspective in one market, then there are attempts to make it up in some other segment, for sure.

Armen Khatchatourian: Yeah. I guess, obviously, as you're well aware, many probably in the room are aware, our industry partners do a very good and thorough job of producing beautiful health economic models to justify pricing. From a payer perspective, we can pick those apart to a degree and negotiate pricing, as best we can on our end; however, we still are stuck with that position of liability from restriction of coverage. I don't know if you have any thoughts or suggestions around how we can play our side on that front.

Mike Bonetto: So, this is what I said to Donna. I was just challenging Donna tongue in cheek. She knows that what we really need is some sort of federal intervention to be able to allow protection if you say no. Right? We don't have that right now. So, you can't. We have state law. We have federal law that restricts that, right? So, that's the biggest hammer is to say, nope. You can do that when you've got competitive products. The hep C space now is loaded with competitive products. You can say no to one of them, or two of them, but when you've got a gene therapy for 10,000 individuals, there likely won't be another one. So, what's your ability to say no? Limited. How could you tell a parent of a 2-year-old, I'm not gonna cover that for 2 million dollars. I'm sorry, I'm not gonna do it. I'm thinking for myself. I would say really? So, we have to have a legal framework that encourages and shifts us to some more equitable stance the balance of power between industry and payers. We don't have that in this country, and we do in others.

Carly Rodriguez: I think that in the state of Oregon, we got a glimpse into what this might look like, because Oregon has a waiver from the federal Medicaid program. They have to prioritize lists of covered services. They went down the road for drugs of saying, we're going to pair a

drug with a condition and say it's not a covered line. There was a therapy approved a couple of years ago for Duchenne Muscular Dystrophy, as an example. The state of Oregon went down the road of saying this is not a covered line of service. It resulted in a threat of a lawsuit, because of the federal drug rebate program. So, I think that's a place where in the Medicaid space there is probably going to have to be some reform around the requirements to the federal drug rebate program to be able to kind of make those determinations in a Medicaid space. We see it in commercial, obviously, where plans take a stance and say the evidence isn't there. This isn't a covered service, but it's much harder in the more highly regulated areas, from my perspective anyway.

Rebecca Owen: Carly, you might remind everybody what the Medicaid rebate rule says about coverage.

Carly Rodriguez: Yeah, just that it... this is a summary, but I don't have all the technical terms maybe, but basically that if a manufacturer is participating in the federal drug rebate program, there has to be a pass to coverage for that drug.

Danielle Walters: I have a question for Sean. Your registry concept, how do you see that distinct different or not from particularly gene and cell therapy the, what we're expecting 13-year post-treatment registries that each of the manufacturers would be required to do as part of their approval. Are you envisioning something different? I just would love your feedback on that.

Sean Sullivan: So, the FDA has asked you to have a 13-year post-approval registry.

Danielle Walters: They haven't yet, but that's...

Sean Sullivan: You're anticipating that.

Danielle Walters: ...that's what the others have been required to do.

Sean Sullivan: For what specific purpose? Safety?

Danielle Walters: Durability of response, ongoing obviously vary by product I would assume.

Sean Sullivan: So, if they were doing that, I could see that that could potentially be useful for... you wouldn't have to replicate that for the evidence development process.

Danielle Walters: I just didn't know if you were talking about something different.

Sean Sullivan: No. No. No. Could very well be the same. Yeah. In fact, could be incredibly helpful, if you're doing that from an FDA post-approval requirement perspective, that could very well be linked to a coverage agreement, both current and in the future. Yeah.

Donna Sullivan: I would potentially replicate it so that we have the data, access to the data. A lot of these therapies are conditionally approved with the label saying that additional when clinical benefit has not been shown and additional research or studies may be required for continued approval. We're not seeing these studies. So, that's the concern is that a lot of drugs, a lot of cancer therapies are being covered and approved that way saying conditional approval based on post-marketing research, which isn't happening, or it's not being published.

Carly Rodriguez: I was gonna say basically the same thing that you just said. I think there was actually an article published recently about some of the follow on therapies required for a muscular dystrophy drug that basically didn't happen. So, I think that's the concern is that something being mandated or required by the FDA doesn't always lead to, at least from our vantage point, something being produced, but I think that's great if there is an effort being made for registries with these therapies. That's great.

Danielle Walters: We're anticipating it.

Mike Bonetto: Okay. Anybody else on the phone? Well, guys, we are a few minutes behind. So, thanks for your patience. So, if I could ask we take a five-minute stretch, maybe get some water, go to the bathroom, and we'll

be back here at 2:55. So, folks on the phone, maybe back in five minutes. Then, we'll get into the second section. Thanks.

Meghan Gallagher: Can everybody hear me?

Mike Bonetto: Thanks for everybody being patient. Meghan, this is Mike Bonetto. Danielle has a clicker ready to go. I think it's appropriate we... I think Bluebird has been mentioned several times today, as we kind of talk about some of these new funding models. So, we're kind of interested to hear what you guys have to share today. So, thanks.

Danielle Walters: Great. Thank you. I'm gonna kick it off and then hand it over to Meghan and we're gonna tag team our remarks. Obviously, yes. We've been brought up a lot already. I want to thank you for inviting us and thank you for all of the obviously very provocative comments, because this is not easy. Gene therapy is not easy. We, as Bluebird, obviously, are not afraid of the hard and afraid of the difficult. We know that we are embarking on an amazing era, in terms of medical care that equally needs amazing thought and innovation on the system side to prepare for it and to prepare for it sustainably.

So, a thumbnail on who Bluebird Bio is. We are a gene therapy company, also cell therapy. We have a partnership with cell gene, multiple myeloma that was referenced earlier. That is our sole focus as a company. We received our first approval in Europe last summer, and we just filed our first rolling BLA submission for LentiGlobin for TDT here in the United States. One of the things about Bluebird, in particular, is knowing where we are in this space and being very cognizant of trying to be partners very early in the process. Obviously, there's a lot of discussion about budget and the ability to forecast and prepare. And so, we have been engaging on these topics in states in Washington for several years now, in advance of our first approval.

The other piece I wanted to mention was our footprint here in Washington State. Our headquarters, our home nest as Bluebirds, we have nests, is in Cambridge, Massachusetts, which is Meghan

Gallagher, who will be joining us shortly. We also have a very real footprint here in Washington. We just recently expanded a research facility here that is working on the next generation, focusing on gene editing, in particular. We have multiple partnerships in Washington State research partnerships and such. So, we definitely, again, thank you for inviting us. We're very privileged to be here to share with you.

What we want to do today is go through our value model, which has also been touched on a couple times today. Meghan is a director of value demonstrations. She's one of our health economists that actually works on our model. She'll kick it back to me, and I'm going to discuss basically the reimbursement structure and the policies that we've proposed. We don't have all the answers, but we have formulated our approach based on a lot of learning and evolution over the last several years, and I'm very happy to share that with you. So, with that, I'm going to turn it over to Meghan.

Meghan Gallagher: Thank you, Danielle. I think it's a build slide. It's a build slide. So, the second half of the slide, the right hand side of the slide is a reflection. That's okay.

Danielle Walters: It's up on the screen.

Meghan Gallagher: Oh, it is. Okay. Great. Yep. I see it now. Okay. Great. Good.

So, hi everyone. Sorry, I can't be there in person, but it's really nice to join in on the discussion. Hopefully, I can contribute a little bit, as well. So, as Danielle mentioned, I'm a health economist. I'm working at Bluebird specifically supporting our sickle cell disease gene therapy program, which is in development. I'm going to be speaking today to an example that we have spoken to publicly in regards to our LentiGlobin for thalassemia, which is under review, as Danielle mentioned, in the U.S. and has recently received approval in [inaudible]. What I'd like to bring to the discussion is really to talk about how we're innovating not only in science, but as well as with action. Danielle is going to get into a little bit more of the details around the access schemes that we are proposing, but I want to

speak a little bit about how we're innovating in a world of health economics. This is really fundamental to the fact that we recognize that the gene therapies that we are developing, hopefully, will offer transforming potential to patients, communities, and the broader healthcare system, but these therapies are only [inaudible] as long as they are accessible. So, that's really the reality that I know that we all share. We're all making sure to make sure that these medicines don't live in the storybook, but live in the lives of real patients. So, with that, so what are we doing to innovate in the world of health economic evaluations? How effectively a product's value for money or cost-effectiveness is determined. So, typically, a cost-effectiveness, or value for money therapy, is determined based on three components. How long a therapy might extend life, to what degree that new therapy improves patient quality of life, and thirdly, the value expressed in dollars or local currencies of the full amount of cost offsets that a new therapy enables? This means by introducing a new gene therapy, the case of our question today, what costs are no longer relevant for treating these patients, as we disrupt the system with these innovations. Typically, historically, drug manufacturers will account for cost offsets when setting price. That is part of the incremental or cost-effectiveness ratio. Bluebird has chosen to take a different approach. We are proposing a different approach. So, we recognize that certain payers and HCA bodies may still want to look at those costs, and we will make them fully transparent, but we're not going to account for them in terms of the value that we lay claim to. That's because we believe that the cost offsets that our technologies enable should be shared with society. That's why the title is really about the proposed sort of Bluebird shared value approach. We don't want to lay claim to all of the value that we're going to be bringing to society with these new technologies, but lay claim to what we believe is most central to patients. That's the intrinsic value, and I'm going to get into that in a few minutes.

With regard to cost offset, this is something that we believe should be shared. We hope that given the innovation of these technologies and their transforming benefit to patients, that in fact, this societal value, these cost offsets, will hopefully be tremendous. That's not to say that we're not going to be held accountable to the same measures

and metrics that are stipulated by payer governing and advisory bodies, including the U.S. ICER that we're all increasingly familiar with. Institutions like ICER will measure a therapy's cost-effectiveness according to its incremental cost-effectiveness ratio, which I've just mentioned a moment ago. This is typically expressed as a cost per QALY [inaudible]. QALY, I appreciate, is probably a nebulous term for many, but it is, in fact, a very standard metric, in which to express value for money. What a QALY is, one QALY is the value of one additional year of life lived in perfect health. QALYs are effectively composite metrics. So, they take into account how much a therapy extends life, and how much a therapy improves life at that extent with the goal being not only to extend life, but to make sure that the life extension is coming with a certain improvement in quality of life. So, institutions, such as ICER and NICE and others, PBAC in Australia, as mentioned as well, that has the foremost technology assessment body around the world, these institutions all set a willingness to pay threshold. In the case of ICER, it was \$150,000 per QALY gained. Now, it's upwards of \$175,000 per QALY gained. So, when we went to calculate the value of our treatment, in which to determine what is the total value that we're bringing to society, we looked at three components. We looked at the quality of life. We looked at the life extension. That composite is the QALY gained. So, if you consider that patients with beta thalassemia, or patients with sickle cell disease are living a severely stunted life expectancy, dying upward 30, 40, 45, 50 years of age, and our treatment could potentially deliver a near normal life expectancy for these patients if treated early, the calculation that we have done is basically taken a look at the additional quality of life gain, or life extension gain multiplied by the willingness to pay threshold set by in this particular example, in NICE and highly specialized technology, and that's given us the calculator for the intrinsic value, which is displayed on the right, which is the 2.1.

That is the intrinsic value meaning that is what we believe we are bringing to the market in the case of LentiGlobin that is of central importance to patients, their families, and the community. Now, healthcare systems will evaluate cost and value beyond that. They will look at cost offset. In some cases, they may even look at societal

value. These additional components in value are what we are saying should be shared and should be returned to society. That's society reward for investing in the innovation. We have made a commitment that our price, and I can't speak to price today specifically, but that our price will never exceed the intrinsic value that we've assigned to these therapies in the case of LentiGlobin for transfusion dependent beta thalassemia it's around 2.1 million. Now, that's not the price. That's just what we're saying is the intrinsic value. As we progress and hopefully achieve approval in the U.S. and then progress the pricing of the therapy for the U.S., we've made a commitment globally, as a company that our therapies will never exceed the intrinsic value that has been calculated.

So, that is sort of the math that we're doing and how we're a little bit taking some innovation in how value for money is assigned and saying we're going to give part of this part back to society. We're going to still be rewarded for innovation, but make sure that we share part of that reward for innovation with patients and the broader healthcare systems, as well. That's really what I wanted to speak about today. The other thing to mention is these numbers are not, and these systems are not chosen by Bluebird. We are not responsible for studying the metrics that are the components that go into these value calculations. It's a very well established rigor and practice. The example that I'm showing you today is the example from the National Institute for Clinical Effectiveness in the U.K., which is an incredibly rigorous HTA body, that I'm sure many of you in the room are familiar with. ICER also becoming an incredibly rigorous body in the U.S. These agencies look at very distinct attributes for us to be able to calculate. So, quality of life improvement, life extension, societal benefit, cost of cure reduction, these are not defined by Bluebird. These are defined by these agencies. So, we are working within the bounds of the expectations, and the full transparency of these bodies that set the guidelines for which value for money is determined. The inputs that we apply to these different components of value are, again, not picked out of the sky. They are clinical trial data, they are heavy, heavy investments in outcomes research and real world evidence, and pursue into an economic model and strategy that is transparent and robust and rigorous, and it's fully vetted through the

HTA body that we are preparing a submission for. So, that's just simply to convey that what this slide reveals here is robust quantification of value to deliver an intrinsic value for patients. The notion that let's be a little innovative with how we approach value for money and not lay claim to all that value, which we believe we will deliver to society. In the case of LentiGlobin for transfusion dependent beta thalassemia, that was actually an excess of 4 million, if you consider the cost offset for patients who are no longer needed to be on a lifeline of regular blood transfusion, then for whom will hopefully no longer live a shortened life expectancy, but can go on to live a fuller life. So, if you factor in all of the cost offsets that are actually resultant or driven by this innovation, the all-inclusive calculation is actually much broader than the intrinsic value. Again, Bluebird's approach, trying to be a bit innovative, not only in the world of medical advancement, but in the world of access advancement, and always keeping patients first and foremost at the center of everything that we do. This is the commitment of the company. This is our global commitment. This is our global model. So, hopefully, we achieve more global approvals. We will have the opportunity to put this principal into practice and really help to realize access for patients and their families around the world. So, that's what I wanted to speak to today. I'll pause, if there are any questions specifically. Danielle, I don't know if you want to just continue on, and then we take questions at the end.

Danielle Walters: Let's take questions if anybody has any questions of Meghan.

Sean Sullivan: Meghan, this is Sean Sullivan. I have a couple questions for you. So, the first is, this slide that you're showing here, this is your own internal calculations, right? This is not NICE's evaluation. Is that correct?

Meghan Gallagher: Exactly. That is correct.

Sean Sullivan: And NICE, presumably, is going to weigh in with their own estimate and calculus, and that'll be done in the next few months or something? Is that?

Meghan Gallagher: Yeah. It's actually ongoing now. It's under review now with NICE.

Sean Sullivan: Okay. Great. Thank you. I also want to ask you about the... you focused this presentation on the all-inclusive calculation, right? So, if I were looking at this with my own economics hat on, what I see is cost offsets that are roughly 1 million dollars, a price that is 2.1 million dollars. So, it's cost increasing to the healthcare system, but for that, there is the possibility of additional life extension and quality of life along the extended life, right? So, I guess I'm just a little curious about how you've monetized life extension and quality of life and sort of indicated that you're returning those to the system when the system actually doesn't really see a monetary gain from that. In fact, what the healthcare system is very likely to see are additional costs, because just in a straight up crude calculation perspective, those folks are going to live longer, develop other diseases, and cost more. So, tell me sort of how you think about the all-inclusive calculation with that in mind.

Meghan Gallagher: Yeah, a very good question. So, to be clear, the cost offsets are what we are arguing are the returns to society, or the part of that shared value approach. The quality of life and life extension is a factor of estimation of mortality gains. So, patients with beta thalassemia are living between 30, 40 years of anticipated life. So, treating a particular age of intervention, we can bring them to a near full life trajectory with a tremendous improvement actually even more pronounced improvement in the quality of life for that gain, given that these patients are no longer on regular blood transfusions and have a pretty significant utility deficit loss associated with taking blood transfusions out of the system. I think you raise a very good point, in that gene therapies are not cost saving to society, right? We expect that gene therapies will require incremental investment, but that the benefits delivered to patients exceed that, right? We're not arguing that by introducing LentiGlobin into the system, we can be cost saving to the system, but we are arguing that we can disrupt a whole lot of cost that is being driven to these patients on a chronic basis, remove that cost, improve their quality of life, and enable a new life trajectory for these patients. I mean, I know you're trained as an economist, and that's certainly... I am as well, and it is very...

the grim picture of the economist who would say that these patients will go on to develop secondary health problems that will then cost the health system more money down the road. I hear you. I don't think we can look at it like that, because I think we have to look at the opportunity to significantly improve the quality of life for these patients that are suffering pretty desperate situations and circumstances right now. So, I don't know if I fully answered your question, but just to clarify, I agree with you. We're not expecting to be cost saving, but we are expecting to be cost-effectiveness, certainly by the improvement in the quality of life and the life extension that we're going to bring for the incremental value that we bring versus a lifetime of blood transfusion.

Sean Sullivan: Great. Thank you.

Meghan Gallagher: In the case of thalassemia.

Mike Bonetto: Any other questions? Folks on the phone? Okay.

Danielle Walters: Now, we'll jump to... which was also referenced earlier, is basically our global approach to pricing and reimbursement. Again, we don't claim to have all the answers. This is our best effort in looking at what type of a model could be appropriate specifically for gene therapy. Again, not necessarily a model that's appropriate for all types of emerging therapies, but again, with our lens of gene and cell therapy, looking at that specifically. Number one there, obviously Meghan already went through kind of the cornerstone of this model, how we determine value. Meghan did an excellent job of kind of overviewing our approach. The other piece that was previously referenced is, we believe, in a shared risk model, we call it value based payment over time model, that would allow for Bluebird to be up to 80% at risk, and that that risk is based upon the durability and success of that particular therapy. The other piece is that the realization of that risk would be over a five-year period, and that we have committed to even before an approval in the U.S., no price increases. Again, this is our global policy. No price increases beyond CPI, as a matter of policy. So, that is basically our approach. I think we welcome a discussion in terms of how could an approach like this

be operationalized, say in a state like Washington. We are having these discussions across the country, and across the globe. In fact, several sick funds in Germany, just recently have adopted this model with us, just literally in the last month. Again, we are having these discussions across the country, both on a Medicaid state payer side, as well as on a commercial side. Obviously, there are challenges on the commercial side with regards to best price and the regulatory barriers, but again, we are not afraid of the hard. Even operationalizing something like this on a state basis, on a Medicaid front, has its own challenges, which a number of people have already articulated in regards to portability and such, but we do believe, at least in the case of Washington with their innovation on value based arrangement that they've utilized for hep C, that there is a framework in place that could be built upon to operationalize this type of an approach. We would be absolutely happy to work with the State on that and work through those hurdles that have already been identified. I'm sure we would uncover others, because again, this is not easy. So, with that, I open it up to any questions you might have of myself. Meghan, of course, is still on the line, as well.

Yusuf Rashid:

Number one, I appreciate that you're bringing to the table a proposed pricing solution versus just conversations about funding solutions or a pathway to coverage. This presents the idea of potential for a negotiation of a solution. Having said that, I'm curious, from country to country, how do you... is there variability in life extension or quality of life in the different countries that this might be in?

Meghan Gallagher:

There is, because standard of care varies. So, a little bit more in the case of sickle cell disease. So, LentiGlobin is being developed for both beta thalassemia and sickle cell disease. In the case of beta thalassemia, the treatment approach is a little bit more homogenized. So, patients are on blood transfusions. There are differences in outcome in both from an efficacy standpoint and from a quality of life and cost standpoint based on the actual transfusion approach that is given for each patient. So, I can get into the specifics if you would like, but there are two or three different types of approaches to blood transfusions that have [inaudible] different cost, quality of life to the patient, and efficacy of outcome. In the case of sickle cell disease, we

see even more disparate outcomes globally. When I say globally right now, I'm really speaking to the Western markets, because of course we know that the prevalence of these disease extends into lower and middle income countries quite significantly. That is something that is on our horizon that we are very aware of, but right now, for Bluebird, we are focused on the Western markets in North America and Europe. In the case of these two regions, we do even already see disparities, as far as outcome to the patient, most notably in the case of sickle cell disease, less so in thalassemia. This is due to differences in standard of care approaches that are given for patients. In the case of the U.S. with two new recently approved therapies for sickle cell disease, that may even become more pronounced in coming years, as new treatments have recently been made available to patients that may even have better outcome potential, as relative to their European counterpart. Let me know if that addresses your question.

Yusuf Rashid:

Thank you. That helped. I was just wondering how quality of life or life extension would vary from country to country, but that helped explain it. Thank you.

Meghan Gallagher:

I think what will vary most significantly in the context of a health economic evaluation for these therapies from country to country is not the quality of life in the mortality, but the resource use cost. So, we have much higher cost for a transplant for transfusion for medications that are used to chronically manage these patients. The costs are significantly higher in the U.S. than they are in Europe. So, when we look at that cost offset perspective and what we are removing from the system from the standard of care perspective, it will be potentially more significant in the U.S. than in Europe. I think we'll see less differential, though there still will be some meaningful differential with regard to quality of life and mortality between the two regions, the U.S. and Europe. Even more pronounced in two other regions of lower and middle income countries.

Leta Evaskus:

I have a question about pay only if the treatment works. How do you define that if each patient has a different outcome after treatment?

Danielle Walters: Obviously, this is a general model. So, we could speak to TDT. We're looking at very binary outcomes, obviously, that have been clinically proven. In the case of transfusion dependent beta thalassemia, our endpoints are transfusion independence. So, something that, in terms of something that could be measured on a claims basis. So, those are the types of things we're thinking through, but that's what we would like these outcomes to be binary and measurable, obviously, and the simpler the better. Clearly, to pilot something like this in order to really measure it and measure it successfully, we believe the simpler the better in terms of binary outcomes that are tied back to our clinical setting.

Mike Bonetto: I want to keep us moving. Anybody else on the phone, questions, comments? Meghan and Danielle, thank you very much. That was really helpful to get that backdrop. Next up on the agenda, we have kind of a placeholder here to hear from manufacturers. We have one speaker who requested to speak, Brian Warren. If you don't mind, if you could come up to the podium. If anybody else is interested in the audience, we certainly have some time for that. I think, as you see on the agenda, I think everything that really is, as I mentioned earlier today, really tried to tailor the comments around if you were in HCA's shoes, what would you be doing.

Brian Warren: Thank you, Brian Warren with Biotechnology Innovation Organization. We are a national trade association for biotechnology companies. We have our affiliate here in Washington State, Life Sciences Washington. I'm kind of speaking on behalf of both of us, because my counterpart with Life Sciences Washington wasn't able to make it.

I think first we wanted to level set a little bit, since this is the first of the meetings where we've been following along the transcript when they post it online. The first one that we've been able to really present at. I think some of the things that we wanted to talk about first to level set were that despite some of the figures that have been talked about before, about the growing dollar expenses on prescription drugs, the percentage of overall healthcare spending that prescription drugs account for has remained steady at about 14% over the past ten years and looking forward into the next ten years

when you account for rebates. So, the post-rebate dollars have remained steady. Medicine spending on a per member per year basis, a per capita basis, which is usually how we would measure spending or increases in expenditures has grown nationally only \$44 since 2009. So, yes. It's true, as also has been discussed at some of these previous meetings, I think especially in the first meeting of this workgroup where there were a lot of facts and figures. Spending has grown exponentially on specialty medications and on these Emerging Therapies, but that has been largely offset by a lot of patent expirations and as other traditional medicines have gone off patent, generics have come to take their place. That's the way the system is supposed to work. So, we just want to point out some of those issues. Then, as it relates to the core issue of today, we were invited to come talk. The agenda for today was supposed to be focusing on how manufacturer payment methodologies might be a solution to some of this or how that takes into account how the Health Care Authority can look into its affordability. We use a slightly different term. We use transformative therapies. I don't know if it's... ours is not based on a dollar amount but based on the nature of the therapy, itself, and how they treat patients. So, transformative therapies are aimed at serious often rare diseases where patients frequently have limited treatment options. Cell and gene therapies, as I think what's presented. I'm going to try to breeze over some stuff that's repeated. I hadn't seen the previous presentations before getting here. So, some of my remarks are a little bit repetitive. I'll try to reduce that as much as possible. So, cell and gene therapies are a good example of transformative therapies. These are therapies that are giving patients treatment options that they haven't had before. There is certainly the case, I think as Sean mentioned, where looking into the future, you could have a cell or gene therapy for a lot of other conditions that there are already are treatment options for patients, but at least right now, a lot of things we're looking at, since severely improved treatment options or the first treatment options for many patients. Some of those examples would be spinal muscular atrophy, Duchenne muscular dystrophy, hemophilia, and sickle cell. According to one study, sickle cell disease and B-cell lymphomas and leukemias are supposed to make up about half of the pipeline anticipated over the next five to eight years in terms of cell and gene therapies.

So, they have a potential to transform the treatment of some of these serious and rare diseases, but we also recognize the challenges around access and affordability for these medicines. These can be further exacerbated by structural challenges with the system, as Robyn discussed earlier. There is Medicaid budgeting. You have a single budget that you have to meet for within a given year. You have an allocated amount that you are allowed to spend. If you're looking to go over that, it has to come from somewhere else. Also, states have to have a balanced budget. I think in Washington, it may be a constitutional requirement that you have to balance the budget every year. We also acknowledge the limited longterm clinical data on many of these transformative therapies. I think also, as mentioned, I think Sean mentioned it in some of his slides that these are unique in a number of ways, many of these therapies. Because of that, we don't believe they should be looked at as simply a high cost drug, because they are very unique in terms of how they treat patients. They usually are very small patient populations. Frequently, you have a single episode of care, instead of chronic therapy that the patient would be taking one of these medicines for the rest of their lives. They are often also potentially curative. I think there is one publication that described these, I think, that made a lot of sense was while a boon for patients, this could prove a financial pain for payers. Patients usually take and payers reimburse the medications over time, which matches years of benefits with years of payments. Gene therapy condense all of those payments into a single, much larger, upfront payment at the time of treatment. We fully acknowledge the challenge that that presents, especially to an organization like Health Care Authority. So, in terms of how to manage this, I think first and foremost, negotiations about price still exist, even if you're using some of these alternative payment methodologies, and I'll talk about them a little bit, negotiations about the price paid still exists. So, even if you're doing a pay over time, or a value based payment, there are still negotiations between manufacturer and payer, or manufacturer and PBM about what those prices will be. We do strongly support states working with CMS in the case of, like, Health Care Authority, as well as other payers working on what we call innovative payment models. I'll go through them really quickly, since

I think just about all of these have already been mentioned, but there is value based or outcomes based arrangements, pay over time arrangement or payment in installments. I think the one that is making a lot of sense and has been getting more traction recently has been outcomes based with payment in installments where it's a hybrid of paying over time, as well as it being evaluation or outcomes based. There are also subscription based arrangements, such as was I think was referred to earlier and is often referred to as the Netflix model. One other option that I had here on my list, and I didn't want to bring it up necessarily, but I'm glad that it was earlier mentioned by Carly, is the reinsurance, because that's less of something that a manufacturer enters into, and that's not looked at as a solution to us, but something that I know that payers, such as HCA, are looking at in terms of how you manage this.

I think that these alternative payment models are an acknowledgement about the uncertainty of the durability for some of these treatments. The risk in paying now for benefits that the patient makes well onto the future, this is part of what happened with some of the hep C. You have a patient where you are paying to cure them, but you may have never, and although there may be cost offsets in terms of not needing a liver transplant, that patient may not be covered by whatever payer is currently responsible for that patient.

Each of these different innovative payment models has different benefits and challenges to both patient and payers, which is why we do encourage that if HCA were to look into some of these that they remain flexible and not necessarily say that we have a given one that we are going to go with for everything, because something for a curative treatment may not work for something that's a little more chronic. We are working with CMS to resolve potential conflicts with federal anti-kickback statutes and policies, such as Medicaid best price. We've seen Washington has its [inaudible] amendment, and a number of states have already been doing those, as well. We've been encouraging those to progress. I think also, as discussed earlier about the hepatitis C and the big flip that happened in 2014 when all those medicines came to be available to patients, that one therapeutic area alone increased spending by 21%. However, within two years,

spending on a lot of those specialty drugs was already back on trend. We believe that if you had innovative payment models that we are looking at now that are starting to be in place, at that time, that trend would have [inaudible] a lot better. We also believe that HCA should avoid a one size fits all approach to coverage decisions. I think, to that extent, rare disease advisory councils can help. A number of states have adopted rare disease advisory councils to help aid their DUR boards in terms of how they're looking at coverage decisions, because these are a unique set of treatments. I also had a couple of additional resources. I don't have a slide.

Mike Bonetto: We can send that out. Sure.

Brian Warren: But MIT has a new drug development paradigms program where they are looking at a lot of value based outcomes and other innovative payment models, as well as the Duke Margolis Center for Health Policy. So, I can give you guys that information. There are a number of groups that are really carefully studying innovative payment models and how they can help balance [inaudible] affordability for both patients, as well as payers.

Mike Bonetto: Great. Alright. Thank you. I want to be sensitive to time. So, are there any other speakers that have not signed up that want to come up? Okay. Brian, can you hang on a second. Any questions for Brian, either on the phone, people around the table?

Stephanie Simpson: Stephanie with Bleeding Disorder Foundation of Washington. This is for both drug companies. It's more of a comment maybe or a thought than a question. I represent hemophilia, so I've been working on high cost drugs for a really long time. From the patient perspective, I think that there is something that you guys are forgetting. When you say that the patient is at the center of all of your decisions, and I try not to really get upset, which is really kind of funny, because I don't normally get upset, is that we're forgetting about the fact that if a parent all of a sudden has a child who has sickle cell, and that child is going to be no less than 2.1 million dollars, that parent is likely going to be fired. Discrimination is illegal, but it happens all the time. It wasn't that long ago where patients... if you

had a child with hemophilia, or you had hemophilia, you did not tell your employer. That's why we didn't have people to raise money for us, because they didn't tell anyone they had hemophilia, because they were terrified of being fired. So, if you're putting the patient at the center of this, what's gonna happen is, they're going to lose their job, or they could lose their job. Then, there's going to be even more people that the HCA is going to have to pay for. We don't know if HCA is going to stick around forever. Granted, there are some protections in Washington. So, if you say the patient is at the center, how are you going to ensure that these parents, or this individual who has been working their whole life and now wants to be able to cure their hemophilia or their sickle cell that they're not going to lose their job, because this is 2.1 million dollars? And I just don't think that that has been added into this conversation or into your chart. So, I just ask that that be considered.

Mike Bonetto: Thanks. Okay. Other thoughts, questions? Rebecca.

Rebecca Owen: So, many of these pricing models don't seem to account very well for member turn, which is a continual problem in Medicaid. The only place that I can think of that it's not a big problem is Medicare supplement where patients are more sticky. How do you visualize a program with a five-year payout working in places where populations where we have 1 in 3 turnover?

Danielle Walters: Very good question, probably the first question we always get in terms of affordability. We call it loss to followup, in terms of a patient. So, I think we're thinking of a number of different approaches. It's obviously something that is subject to come to negotiation now that would be handled in the contractual situation. I think one question is if that patient just essentially disappears from the system and you're no longer able to get outcomes data from that patient. I think there is some opportunity, whether it's from a registry or other sources to gather that data. I think we will have that ongoing requirement. We expect to. So, I think that's one issue, but it is obviously a tough question, as far as how that should be handled. I think it has to be handled payer by payer contract by contract in terms of what the parties feel comfortable with, but the other issue,

too, is I think we're doing some research in terms of patient... again, we're dealing with patients that have been highly tethered to the healthcare system. So, should we look at those patients differently than obviously a general turn number? So, I think that's one factor that can be looked at, in terms of how these particular patients in these very narrow disease states, what their turn is like versus a general population. So, again, all stuff that definitely needs to be addressed and discussed as part of any sort of an arrangement.

Rebecca Owen: They don't tend to turn by payer.

Danielle Walters: No. That's what I'm... yeah. That's what I'm speaking to.

Rebecca Owen: So, this is the Donna argument here.

Mike Bonetto: Anybody on the phone, questions, comments. I want to keep moving, as we've got about 15 minutes left. So, we have some time for some group discussion, maybe not as long as we had hoped for, but we also have a whole separate meeting in April, which this will tie into, kind of the values and the outcomes, and the quality discussion, as well.

Leta Evaskus: I just have one person on the phone. Foxy Williams asked if Stephanie's question will be addressed.

Mike Bonetto: Go, Stephanie, please.

Stephanie Simpson: I guess that's up to the drug companies.

Danielle Walters: I would just say that obviously we look at all of the societal issues that impact our patients. I think we definitely hear what you're saying and are very sensitive to that. I think the question is, how to address that. I think working with patient and individual patient groups, which we do, are very close to the patient organizations of the disease areas we're involved in. We would be happy to continue to dialogue on that, because if it's a real issue and clearly impacting access to these treatments, it's definitely something that should be discussed.

Donna Sullivan: I'm not sure, the discrimination about firing somebody... it would be really hard for us. There are laws in place. We understand it happens. I think the other thing also is that not just you might lose your job, but I think a more likely situation would be is, your employer would no longer offer health insurance to you, and you might have to go purchase it on the exchange. Are you going to be able to afford a plan that has these medications and therapies? I think that's just going to be a bigger challenge for more people is that the small employers are going to be hit the hardest, and they're going to pull out of the market.

Rebecca Owen: Donna, I want to emphasize that it wouldn't just be the person whose child had the disease if a small employer decided to no longer offer coverage.

Donna Sullivan: Absolutely.

Meghan Gallagher: I think we have to acknowledge that in the case of sickle cell disease, this is already a concern irrespective of these new treatments. We understand very well the burden that this disease presents to patients and family members, and their caregivers. I think we have to look at how can novel therapies, such as these potentially one time treatments enable patients and their families, including their caregivers, to get back to work in a capacity that they have not been able to perform. That's something that we're also looking at. So, I fully hear you and empathize with the point that you make. I think we acknowledge that this may already be an issue. So, how can these new treatments kind of disrupt and enable patients and their families and caregivers to get back to work, too, is an opportunity of these treatments, as well.

Mike Bonetto: Stephanie, followup?

Stephanie Simpson: I think it's important to bring up the point, especially as we talk about... we, at the Bleeding Disorder Foundation, we actually work super close with sickle cell. Our goal is to make sure that patients try to grow out of Medicaid. So, that they go and live full lives. That's part of our... every practice in all of our programming, and if we want

to keep people from leaving their jobs to go onto Medicaid so they can get these treatments, and all these different patients come up with a lot of strategies and techniques. There's a difference between on the ground and a national entity. I think that it was an opportunity to bring it up. I hope that the companies bring it up at the forefront, because yeah.

Mike Bonetto: Thanks.

Meghan Gallagher: I'm really pleased to hear you say that. We're actually working on a modeling project at Bluebird addressing that exact issue right now, which, so hopefully more to come in the public domain on that work, as well, in the near future.

Mike Bonetto: Okay. So, guys, Meghan, thanks. Ten minutes left. This is what I want to do. We've got some folks around the table still and some folks on the phone. I threw up just again some pieces that kind of came out of today's discussion. I had those up on the whiteboard, but I thought it would be easier if we just had them up on the screen. There was a lot more, obviously. What I want to do is go around the table and kind of start to tease out from each of you what resonated? Where do you think, again, HCA should be looking into? What do you want to even see come back at the April meeting that starts to tie in with what Sean was really talking about, the value and how do we start to talk about this from an almost higher level. So, that's just a starting point, but I really want to kind of get inside your head, in terms of what really were your takeaways from today. What do we do with it?

Sean Sullivan: I have a suggestion. So, each of these potential options comes with opportunity to potentially reduce some cost expenses. It also comes with some unintended consequences. I think it would be interesting, and I don't know, Donna, if you and Judy have access to some support help. I'm not looking at Ryan, but to sort of go through a little exercise between now and our next meeting about, for each of these, or for the top whatever 60% of them or something, what are the opportunities? What are the challenges to implement any of these within Medicaid? What might be some unintended consequences?

Then, when we come together again, we can talk about these with the benefit of your deep dive into some of these things. You may just straight up rule out, hey, we can't do this, because we don't have a legal framework for allowing this. That legal framework would be just too impossible to overcome as a barrier, but maybe these other couple of things are possible. Here are the opportunities. Here's what we think might be a benefit computation. Here's some potential risks. That, to me, I think would make for a really good April meeting. There may even be some things that aren't on here, Donna, that you guys have been thinking about that you want to add to that. That could be the framework for the April meeting.

Mike Bonetto: Donna, what are your thoughts on that?

Sean Sullivan: Ryan, did we just give you some homework?

Ryan Pistorosi: Yeah.

Sean Sullivan: Yeah? Sorry.

Donna Sullivan: Are you sending us a student soon?

Sean Sullivan: I sent you Ryan a few years ago. We can.

Mike Bonetto: I think that's a great framework for a couple months. I am interested in others, just in terms of takeaway.

Yusuf Rashid: Just thinking about how we might approach this and just a caution about using too much of a rear view mirror reactionary approach. As an example with the carve out, and referring back to that map of the different states. So, carve out PDL, the different solutions, a lot of them are reactionary based on past history and don't, I think, adequately contemplate future state. So, for instance, with carve out PDL, I'm not an economist, but some of the variables, I would think, are Medicaid best price being better than managed care's ability to negotiate a best price in the market. That's contingent not just on the initial launch price discount, but also the CPI cap that Medicaid experiences for several years. So, it makes sense in an environment

where there has been significant inflation over time. I think we're moving to a future where it's going to be pretty easy to guarantee inflation at the CPI end. I would predict even some deflation, as competition enters the market for the high cost drugs. So, I think we're moving away from over time where that solution makes sense anymore. Right? So, when we think about these, I just want to call out thinking about future trends that will occur, as well as the rear view mirror that led us to these solutions, and whether they'd be relevant going forward.

Mike Bonetto:

Great point. Others? Stephanie? What, Stephanie, other than that, no. Stephanie, no. I'm gonna put you... what... your takeaway though from today?

Stephanie Simpson:

I think my takeaway is that people want to figure out how to provide these drugs. So, I think it's always important, because as patients, we often hear that payers, whoever they may be, don't want to pay. So, I think that I always appreciate the thoughtfulness of everyone. So, continuing that part of it. I think to figure out what I really want at the end of the day is that whoever the patient is, whether... let's use sickle, because they don't have any treatments in the pipeline that are great today, and hemophilia has a lot, that they kind of know the framework. Patients know the framework. As a treatment comes down the option, or down the pipeline in Washington, will I or will I not potentially be able to get this treatment if I'm in the State of Washington, because part of why I always thought this was important is that it's terrible when you sit at a presentation and a doctor goes over this amazing new opportunity. Every single time the presentation ends the same way, but we don't know who will pay for it. So, I want the patients to start being able to have hope, can I start preparing. There is a lot of lifestyle questions and thoughts and changes. Like, sickle cell, if it's a four-month treatment, I need to start strategizing. Do I need to stock up on my sick leave? Do I need to see if grandma is available? Like, people want to start planning new lives, but do I know that... that's what I want out of the framework at the end of the day for the patient, is that they can start creating some hope.

Mike Bonetto: That's good. Thank you. Rebecca?

Rebecca Owen: It's going to be hard to build predictable and stable budgets with highly sophisticated models. I think that's something we're all going to have to remember is that, at the end of the day, there is going to have to be a number put on these. That's going to be very difficult.

Mike Bonetto: So, bigger assumptions. Right. Thanks. Armen?

Armen Khatchatourian: I'm philosophically more of a proponent of outcomes based strategies. However, obviously, there are a lot of operational hurdles. If there is a way we can figure out whether it be through legislation or whatever, that a more streamlined kind of template strategy can be implemented so that we can enforce the clinical outcomes and trial data and make them perform in real life settings, and in a manner that isn't so operationally cumbersome. I'm a big proponent of it over any of these financial kind of Netflix modeling, annuity model, whatever you want to call it, just because when 800 of these are on the market, those small payments are going to add up again to where we're in the same boat with 800 of them. So, eventually, the cost is going to end up in the same position. So, I would frankly prefer, just from a philosophical standpoint, pushing outcomes and enforcing outcomes and tighter contracting strategies. Obviously, we talked about some strategies around not covering drugs or to leverage and negotiate with manufacturers and international strategies. That's probably not going to be viable in this country. Obviously, our advocacy group feels very strongly against it. So, the only alternative I can see, other than straight price reduction is outcomes based models.

Mike Bonetto: Great. Thanks. Petra?

Petra Eichelsdoerfer: The one thing that comes to mind to me is how all of this fits into the context of a very rapidly changing healthcare system, for lack of a better description. I'm not even sure what the right word is, but the overall world of healthcare itself is changing so rapidly right now in the United States viewpoints, especially if you look at the political realm, and I wonder how all... this is obviously going to be part of it,

and it's playing a role in why things are changing, but I wonder how it plays a role moving forward, as a progressive improvement, as opposed to looking forward, not the rearview mirror piece.

Mike Bonetto: Got it. Thank you. Sean, we got you. Any other comments? Donna, can I wait until we get folks on the phone? Anybody on the phone, observations, comments from today?

Jonathan Espenschied: This has been a great discussion. Would love to see everybody who needs this actually be provided cutting edge therapy. We all know realistically that's not necessarily going to happen in a short amount of time with our current environment. I think we really need to look at what can be possible here in the State, whether it is a Medicaid approach. We looked at France. Europe has great models and great ways of bringing a drug to the people, but I would love to see us maybe... and I think we initially discussed it just at the beginning of this discussion is, what can we do here, as far as the State of Washington goes. There might be winners. There might be losers, per se, but how do we get the ball rolling? What's going to be feasible in this current environment?

Mike Bonetto: That's helpful. Thanks Jonathan. Others on the phone? Okay. Donna, I would like to close with you, as you were kind of thinking about all of this, and even thinking about how you'd like to tie this into the next meeting, as well.

Donna Sullivan: Well, I like Sean's idea about trying to do a deeper dive on some of these different alternative models. I think we have some of that work already done through other projects, but the ones that really resonate to me that seem more doable, one is the public health emergency is something that exactly would be interesting to do if we had the political, I guess, courage to go down that road. I would love to see something like that happen. I think with the coverage, based on evidence, is really another great idea. Not only... to have these patient registries, so that we can really look at the quality, and not just the effectiveness of the drug, but the quality of the care from the provider to make sure that the provider of these therapies, is there one that's better than the other. Is there one that is causing harms,

not because of the therapy itself, but because of the administration, the technique, whatever. International reference pricing, I think, is also very interesting. We all talk about negotiations, but I want to remind everybody that from a payer, it's not a negotiation if both parties can't equally walk away from the table. So, we end up being price takers instead of negotiators. So, it gets to be really hard. So, every one of these, it is very difficult for the state or even for a payer, because at the end of the day, if we don't cover it, have some pathway to coverage, we will get sued. There will be lawsuits. So, we really don't have... we can't just say no. I wish we could. Many of them I would I would say no, but many of them transform people's lives, and I think that it is important to get those medications to those patients. So, I'm glad that everybody was here. I would have loved to have heard more from a manufacturer's perspective. So, I'm looking forward to any comments that you want to submit onto our website. We do have an Emerging Therapies mailbox. Email list, you can sign up if you want information. And you can submit questions or comments to their website, as well. That's it.

Mike Bonetto:

Thank you. We are a few minutes over. Thank you for your time. Thank you to all the speakers. Meghan, I think you're still on the phone. Carly and Robyn left, but Sean, it was really great info today. Danielle, thanks. So, we will go back and start building out that April agenda. I think, Sean, you've provided some great framework, and getting meeting minutes out in the upcoming weeks. I look forward to seeing everybody in mid-April. Thanks, guys.