

Emerging Therapies Workgroup
December 18, 2019 transcription

Leta Evaskus: Hi, everyone, it's 3:00. So, I think Emerging Therapy Workgroup should get going. Judy, do you want to go to the podium or do you want to stay there?

Judy Zerzan: I think I'm going to stay here.

Leta Evaskus: Okay. Make sure that your mic is on.

Judy Zerzan: We're on now. So, hello everyone. Good afternoon. Welcome to our public meeting on Emerging Therapies. I thought I might start by seeing if we have some people on the phone, perhaps? Or maybe not.

Mike Benetto: Hello, Judy. Mike Benetto here.

Stephanie Simpson: This is Stephanie Simpson.

Judy Zerzan: Great. Okay. Well, I'm Judy Zerzan. I'm the chief medical officer at Health Care Authority. We wanted to have this meeting to get some public feedback about the work that we are doing and sort of help level set on what we are doing for the Emerging Therapies Workgroup, which had been introduced as legislation, and it did not pass, but we agreed to do. So, I am going to step through sort of a number of slides about what the workgroup's purpose is, and where we are. Then, we'll have time for open discussion after that.

The Emerging Therapies Workgroup is charged with helping Health Care Authority identify perspectives, concerns, ethics, proposing potential solutions around Emerging Therapies. Emerging Therapies is sort of a loose definition, but it is roughly focused on the fact that there are some very high-cost drugs that have come out recently. Some of them are potential cures for new diseases. Others change the course of an illness fairly remarkably, and sort of figuring out how does the State pay for these things, and what might coverage look like. So, some of the issues related, and I guess some of them are on here, are related to Emerging Therapies are how do we pay for these longterm, particularly for the

State programs of Medicaid, the public employees, and the school employees. Second, how do we think about quality, oversight, and outcome tracking? Some of these drugs have been approved under the FDA Fast Track mechanism. So, there will be sort of in the future updates about their effectiveness and potential side effects. So, how might we locally sort of keep tabs on that and figure out what's happening locally in Washington. Third is management of patients eligible for these therapies and potential improvements to their health outcomes and their quality of life. Potential longterm either savings, expenditures, or maybe both, or it depends, depending on the drug that might happen to the State. Then, helping sort of link to the quality oversight. What are the metrics that could be used to sort of help measure these fiscal and health impacts, recognizing that that might be different from condition to condition.

So, to give sort of an overview of where we are, we had our first meeting June 18th. This was in Spokane. It was really sort of an overview, a kind of table setting of what are new therapies coming to market? What is the current financing of those? What's the private sector perspective? What is Health Care Authority's perspective, and sort of getting everyone on the same page about what we meant? The October 18th meeting was really focused on the patient and family experience with this. Part of that is, we have three or four patients or patient representatives on the workgroup to give their perspective. So, we heard from them. We heard about shared decision making and decision aids, and how that might be in there. We heard some about patient experience and ethics around Emerging Therapies. We are now at the December 18th meeting that we are going to give a summary of what we've done so far and answer questions. The February 19th meeting is also a public meeting. It's really going to be focused around the financing. So, what are longterm funding options for Emerging Therapies? What might some of those be? How might we explore that? The April 15th meeting is really more based on the measurement and the quality piece. So, how might we measure some of the potential benefits and harms and what should we be looking at? What should we be looking at in terms of savings and expenditures? Then, the final meeting will also be an open public meeting on June 9th and be a summary of the workgroup's progress and questions and feedback. The goal of this group and why some meetings are open and why some aren't is really to get at ideas. We aren't making any decisions

about what to do with these Emerging Therapies, but we're really listening to people's experiences and getting feedback from a variety of perspectives that we can use to then create some potential solutions and then have some public meetings to figure out, what's the approach that Washington State should take.

Moving onto the June 18th meeting, we're just going to go over a couple slides from some of these different meetings. I should note that all of these slides and the transcripts are on a website that we've made around Emerging Therapies. You can feel free to go back and look at those.

So, the first slide of today is something that I am sure all of you know is that specialty drugs, however you define them, have really changed a lot. In 2008, they accounted for about a quarter of total pharmacy spending. Now, they're at 46-47% of total pharmacy spending, even though they represent only about 2% of prescriptions. There is a big pipeline, some of which, since this slide, have come out. There is a number of really breakthrough specialty drugs. They treat a variety of conditions, some of which are fairly rare, and some of which are much more common. It's really a super spectrum. There are a variety of therapies, some of which have come out, some of which are in the pipeline, around treating different types of cancer, cystic fibrosis, Duchenne Muscular Dystrophy, spinal muscular atrophy have gotten a fair bit of press. There are a couple of things in the works on nonalcoholic steatohepatitis, blindness, hemophilia, Alzheimer's disease, and then certain other neurologic disease. So, all of these are important drugs. Many of them are quite severe illnesses that changes in current therapy, I think, are much needed.

Of the drugs now that are in Phase III trials, about two-thirds are specialty drugs. About a third are orphan drugs. However, only 13% are really considered breakthrough therapies, so substantially different than what's out there now, and 8% are biosimilars. Of the applications that have been submitted to the FDA, about a quarter of them have priority review, because they fall in this category of potentially providing a new therapy for something that is out there.

We also talked a fair bit in this first meeting about current approaches to determining coverage. As is, I'm sure not surprising to any of you in the room, there are similarities between commercial insurers and the Health Care Authority of how coverage is determined, as first by looking at the evidence and evaluating that, by looking at a financial impact analysis, developing clinical criteria, which is largely based on the evidence, and then tracking outcomes to sort of see what happens to people that are treated.

We talked some about, and this was really sort of the bulk of the discussion, about these decisions are very complicated. They are certainly effected by your perspective. There are the perspectives of society, of the payers, of the suppliers or manufacturers, and of patient caregivers and their families. Some of those things overlap and some don't. How decision makers value these new drugs and how they think about it really depends on the perspective. Certain things may be weighted or valued differently depending on your perspective. So, the challenge that we have, as the State, is to take all of those perspectives and figure out how do we find the right path forward.

So, I think this is the last slide on that June meeting. We talked some about... some of this would be things that we talked about, and some of it was things that the workgroup would like to learn more about. Under the broad categories of sort of cost, quality, and access. So, what levers do we have? Could there be reinsurance? Medicaid has a best price provision that is part of federal law and how might that interact? What's the societal impact? Could there be outcomes based contracts? HCA has recently gotten the template approved from CMS that would allow us to do some of these similar to Oklahoma, Colorado, and Michigan. So, what might that look like in paying different prices for different outcomes? Are there longterm side effects of new drugs, and how might those be measured? In both this meeting and the next meeting, we talked a little bit about cure versus maintenance and what that looks like, and how the definition of a cure or what a patient and family are looking for might vary by disease to disease. There was some desire to learn more about patient decision aids. So, we talked about that in the second one. Outcomes registries, provider education, informed consent of patients,

and that was actually a thread that we picked up in the second meeting quite a bit. Then, alternative treatments and how those might fit in.

So, that was a whirlwind, and here is the next whirlwind of our last meeting on October 18th. That meeting, we actually had some audio difficulties. So, the transcript has some holes. There were some people on the phone in the workgroup that couldn't hear. So, it was a little bit challenging, but I think, I don't know. This overview might help give some of that... and yes. So, we had the October 18th meeting, some patient advocate presentations around Parkinson's disease, hemophilia, and sickle cell, which are three of the patients, or the patient advocates that are on the workgroup either have those diseases or have family members that have those diseases. So, they each talked a little bit about what drugs are in the pipeline that their organization is tracking, how they evaluate new drugs and sort of keep after it, and how a lot of these organizations, part of their mission is really educating patients and families about treatment options and what that looks like. So, they talked some about how they did that and shared their websites.

Next, we had a panel of providers, or sort of a series of providers talking about their perspectives on how they helped patients and families on treatment decisions. The first two providers were both oncologists, the first one a pediatric cancer specialist. She is on the workgroup and had talked some at the first meeting. Her story was very compelling about how she approaches some of these new therapies. She gives CAR-T therapies in her practice and things like that. So, how does she talk with families about sort of what are the potential benefits? What are the potential risks? Her sort of challenges and best practices on particularly approaching this as families making decisions for children. Then, we had an adult oncologist that specializes in BMT treatment talk about sort of similar perspective, except his patients are making the decision for themselves and what that looks like and some of the uncertainties and sort of how he follows the evidence and how he sometimes has to make difficult decisions about this might be the only thing out there. It might help, and it might not. How do you help patients through sometimes very difficult therapy? Then, we had Dr. Emily Transue, who is an internist, talk a little bit about shared decision making, which is a topic that the HCA has done a fair bit in. We have certified shared decision

making aids in different areas. So, talking a little bit about shared decision making as an idea and how might we insure that sort of good shared decision making happens for these Emerging Therapies so that patients and their families really understand the potential benefits and the potential risks and can make an informed decision about that.

Then, finally, we had Wylie Burke, who has recently retired from the University of Washington come and speak about ethical considerations. She talked around the ethics about health payer policy decisions versus personal patient decisions, about care for yourself, about medical necessity and sort of the process of healthcare decision making, and how to sort of weigh and think about the benefits and harms of treatments. I think it provided a nice framework.

So, the takeaways from that conversation is that one thing that came up, and this is part of where is here is the workgroup really wanted pharmaceutical manufacturers to come to the next meeting and be a part of that conversation about financing, and thinking about how do we approach this. We had some discussion about, in the next meeting, FDA approval and looking at that process and learning about that process more. We talked a fair bit about sort of cost containment or funding options and what that might look like. In particular, many people in the workgroup were interested in how manufacturers determine cost and what that looks like. Then, how does we sort of keep all stakeholders in this sort of nice process of a variety of perspectives continuing to be part of the conversation. There was some talk about reinsurance or risk corridors particularly for our Medicaid managed care organizations, sort of who gets the cost and who gets the savings and what does that look like, because sometimes savings are made not necessarily in the medical system, but they are made because there is less money needed to be spent in the educational system, or people are able to be part of the workforce. So, there are savings in sort of other places that aren't completely captured. Then, there was a fair bit of enthusiasm for this sort of idea of how do we help both patients, families, and clinicians be educated about these issues, and how might we promote shared decision making. So, that is my whirlwind presentation about what the workgroup has been up to. Now, we would like to sort of open it up for questions, comments, sort of more discussion. I think we've got a couple of people

that are going to have microphones, so we can get a better capturing of this conversation than we did the last time.

Leta Evaskus: I have one announcement to make before that. We are recording the meeting for transcription. So, before you ask your question, please state your name and who you represent before you speak. If you want to just raise your hand if you have questions or comments, and we have two roaming mics that we'll need you to speak into so that it gets recorded.

Mark Cummings: This is Mark Cummings with Life Science Washington. So, we represent the Life Science companies and research institutes in this State that have actually literally spent decades in some cases and billions of dollars developing these Emerging Therapies that this workgroup is charged with reviewing. You may or may not know that Washington, Seattle in particular, has really become sort of not just a national hub, and international hub for cell and gene therapy for the development of those cures, literally the most cutting edge work in the world is happening right here, and I've got to say, I mean, it's sort of stunning to me that the State would put together a group like this to have the conversation around Emerging Therapies without having anyone at the table that's actually responsible for inventing and bringing those therapies to patients. After all the issues that you just went through are super important, but they only matter if there is actually a treatment available to the patient in the first place. We worked really hard last year to be proactive in the legislation hearings to set up this taskforce, because we realize that these therapies are totally different than the traditional drugs and how we treat those. We need to get ahead of that curve. To bring an effective Emerging Therapy to market, you've got to have a much broader conversation. You've touched on some of those topics, but the structural differences between delivering a personalized cure, I think, at some point some people pointed out in one of the sessions. Some of these are actually done on a patient by patient basis. It's that personalized. The discussion on value and at risk payment models, the implications of payment models for curative therapies versus longterm maintenance therapies, related administrative issues associated with that, and the efficacy of targeted personal therapies versus traditional drugs. So, as we participate on those hearings, not because we have all the answers, but because the science has advanced so quickly over the past couple years,

that now these are on the cusp of becoming reality. We want to have a thoughtful conversation about how this system needs to respond and adapt to handle them before they arrive, rather than sort of chaotically responding to them as they get approved. Unfortunately, as I went back and read the minutes from the previous meetings, it seems that the group sort of interchangeably uses Emerging Therapies, Specialty Drugs, and High Cost drugs. When we think about those things, those are each really important issues. They're three very different issues. To put them all in a bucket together and treat them as sort of one policy process really sort of misses the point of sort of what's coming with this new round of therapies. Secondly, I was also concerned that some of the remarks about pharmaceutical companies demonstrate a limited understanding of the companies who are actually developing many of these Emerging Therapies. So, just a reference point, over the past decade, most of the large pharmaceutical companies have pushed out much of the risk for developing these types of Emerging Therapies on smaller, more innovative biotech companies. So, many of these companies have no revenue. Let me repeat that. Companies have no revenue, yet they're on a path that often takes over a decade, hundreds of millions to billions of dollars to develop these therapies. As you guys know, these aren't sort of the traditional small molecules that get delivered to your door. So, the risk that these companies have to undergo to pursue this kind of research is really challenging. They have to work in an environment to raise those billions of dollars to continue to do the research to bring those to market. Without a clear path to market, without a clear understanding of the payment and reimbursement models, and without a clear understanding of the administrative requirements, it's hard for those companies to continue to go back and raise money year after year to pay for the research to bring these to market. Again, I think from the comments, the concept here was that there's a thought that these are sort of companies with a deep bank of the abilities to fund this work. These are folks, in many cases, a founder of the company probably had a family member that, you know, was impacted by one of these diseases and then either built a company or committed their life sort of doing that work. That is tough to do if you have no revenue coming in, because the one product you're trying to bring forward is the one that we're talking about here. So, again, the original legislation and charter to this group sought to include experts from all corners, including the biotech industry

on the workgroup. This region is literally one of the strongest places in this country, in terms of expertise in these issues and as a hub for this kind of work and research in cell and gene therapy in the country. We've asked repeatedly to have our experts included in the conversation. We can't understand why this group would not want to include those experts in the conversation, as the State seeks to charter its way through sort of uncharted waters on these issues. Having reviewed the transcripts, it's clear that not having that perspective reflected is having a real impact, and issues are getting missed, as this group does its work. So, we hope you will reconsider your position to work in isolation and engage these experts that get up every day, doing the research, to bring these kind of therapies to market, and include them in the conversation. I think what you may not realize is that these companies are as anxious as this group is to figure out answers to these solutions, because they all understand that we're chartering new territory here, in terms of what it takes to get these to market. They want to keep investing, but they need to have a partner to work with to think through how to do this and do it in a way that is most sustainable and makes sense for the system. With that, I'll turn the mic over.

Brian Warren:

My name is Brian Warren, and I'm with a biotechnology innovation organization, BIO. To follow up on some of Mark's comments, we know, and I know you guys know, that this workgroup is being watched very closely, as this is a very new area of medicine. We only have right now just a couple of therapies on the market. There are a number in the pipeline, but it's being watched closely for a couple of important reasons. Right now, determining coverage or how you will determine coverage for these types of therapies not only impacts which patients will receive the ones that are on the market, or will soon be on the market, but it will have a significant impact in terms of, as Mark was saying, that future investment into the remaining therapies that haven't yet gotten that far, because if therapies are approved by FDA, they are being recommended by clinicians but coverage is restricted for some reason. That will severely hinder the ability of many of the small and emerging companies to be able to attract the investment revenue that they need to bring those products all the way to the market. We continue to believe that manufacturers should be part of the discussions of this workgroup. We think that the perspective, expertise, and information that we have fits

perfectly within the workgroup charter. I think a number of the things that are supposed to be discussed, in particular at the next meeting, and I think it may be a little bit developing, because I heard Dr. Zerzan's comments about perhaps manufacturers being more involved at the February meeting. We think that that would be very important, because as you know, that meeting is scheduled to have discussion about alternative payment models. It is our hope that the manufacturers who are the ones developing and hopefully working with payers, such as yourself, to implement those alternative payment models, that we be able to have a more robust conversation and not just a two-minute public comment at the end of a meeting. We strongly support the exploration and adoption of alternative payment models, because we know that paying... we are very aware that many of these transformative therapies, while truly transformative to patients in terms of the clinical outcomes, they are high cost. We know that these medicines are different based. They need to be treated as such. We are working, as much as we can, to try to ensure that they are part of a sustainable system so that patients can get those therapies, and that they are also still affordable to payers. So, we know that the State of Washington has already received State plan amendment approval for outcomes based reimbursement for hep C for example. Those are the types of things that we want, that our bio and our members, want to talk more with the State about, in terms of a way to ensure that these therapies are able to be available to patients. Part of a sustainable innovative system and also affordable to the State. So, we just, again, really urge the Health Care Authority to include manufacturers in some of these upcoming meetings, and in particular, the February meeting in a significant way and not just as a public comment at the end of the meeting. Thank you.

Judy Zerzan: Is there anyone on the phone that has any comments, questions? Anything else?

Rick Dabner: Hello. I'm Rick Dabner with Alnylam Pharmaceuticals. Speaking of the next meeting, that being open and getting feedback from the industry, have you determined what that process might look like, and how one would go about making sure that we can participate in that process?

Judy Zerzan: We haven't yet figured that out. I thank the first two folks for their comments, but we are thinking it might look like a panel of some sort. So, it would be sort of a presentation or part of a meeting, but we haven't completely figured that out yet. We were thinking that this meeting might help give us some ideas or sort of gage what interest there was in doing that sort of process.

Mark Cummings: Well, again, we would certainly offer to work with you to figure out what that panel looks like and make sure the right perspectives are represented and make sure we sit down in advance and think through kind of the issues that should get covered to make sure that's a comprehensive conversation. So, I look forward to working with you on that.

Judy Zerzan: Okay. Well, if you have ideas in the interim, there is our email, and there is the website. If you haven't been there yet, please feel free to give us your sort of ideas and thoughts and questions. Yes. We will continue to figure out what the February meeting looks like. Then, our hope is that this workgroup develops some ideas that then we can talk about in different ways and try and figure out what our State's approach will be to these new therapies. So, thank you for coming. Have a good afternoon.