



The Our American States podcast—produced by the National Conference of State Legislatures—is where you hear compelling conversations that tell the story of America’s state legislatures, the people in them, and the policies, process and politics that shape them.

You can subscribe through iTunes, Google Play, Sticher or Spotify.

The Fiscal Challenge of Emerging Gene Therapies | Jan. 11, 2021 | OAS Episode 118

Ed: Hello and welcome to “Our American States,” a podcast from the National Conference of State Legislatures. This podcast is all about legislatures: the people in them, the policies, process and politics that shape them. I’m your host, Ed Smith.

“Even before COVID when these new generation, orphan drugs that treat novel and rare diseases emerged over the last few years, some of these drugs can cost into the millions of dollars. And so, being able to predictably budget for these drugs can be very difficult.”

That was Anne Winter, a Medicaid strategist with Health Management Associates, a national research and consulting firm. Winter has particular expertise in pharmacy benefit management and is a guest on the podcast. She’s here to discuss how states are dealing with the challenges of new gene therapy treatments.

There are gene therapies approved by the U.S. Food and Drug Administration that can treat certain cancers and other rare disorders, offering life-changing treatment. But they also carry extremely high costs that often fall on the Medicaid system.

Also, here to discuss the issue is Colleen Becker, a policy expert with NCSL, who tracks the approaches states take to deal with the high cost of these treatments. Let’s start with Colleen. Welcome to the podcast.

CB: Glad to be here, Ed.

Time Marker (TM): 01:35

Ed: Now, I expect many of our listeners are not very familiar with gene therapies. So, I wonder if you could set the stage and tell us what kinds of therapies we’re talking about and why state legislators should be interested.

CB: There have been some promising curative treatment options emerging for some pretty life-altering diseases including genetic disorders and certain cancers, and these are cell and gene therapies that are coming out. They are extremely high-tech processes that either add, delete or correct a gene that's causing a particular condition.

So, for example, a condition called spinal muscular atrophy, or SMA, is typically diagnosed early in childhood and causes muscle weakness, difficulty breathing and eating, and sadly often means a short lifespan. Now there's a gene therapy approved by the Federal Food and Drug Administration that's a one-time treatment. It's given to children under two and it stops the disease's progression.

There are at least five treatments that are approved with several hundred more in the pipeline, so you can imagine how encouraging this news is, especially for people directly affected by these treatments.

One concern for legislators is that they're extremely costly. Some are approaching several million dollars and those costs are felt across the healthcare system, particularly for Medicaid and state employee plans.

There's a federal rule that says if the manufacturer participates in the Medicaid program, then the state must cover all FDA approved treatments by that manufacturer. The tradeoff is that Medicaid receives the best or lowest price.

TM: 03:12

Ed: Can you give us some examples of approaches states have taken in the past or are taking now to address these extraordinary costs?

CB: Again, these therapies are just coming to market, so health plans and policymakers both are just starting to find ways to pay for them. For Medicaid, a state might have an option if it's administered through a managed care organization versus a fee for service. If they have a managed care organization administering their benefit, then it can be carved in or out of the contract.

States can also choose to carve out certain populations or classes of drugs and, while these strategies do help mitigate financial risk, they may restrict some access to certain folks.

Some manufacturers already offer a mortgage-based or pay-over-time arrangement by offering a five-year payment plan, and others are pursuing outcomes-based reimbursement models where a manufacturer offers rebates should certain patient health outcomes not be met.

And lastly, something I'd point out, states are also implementing value- or outcomes-based contracts. So, for instance, about two or three years ago, after gaining approval from the Center for Medicaid and Medicare Services, Oklahoma pursued outcomes-based purchasing contracts where certain metrics such as decreased hospitalizations were tied to how much the state paid.

More recently, Oklahoma and Massachusetts both reported striking an outcomes-based agreement for one of these novel therapies used to treat SMA.

TM: 04:49

Ed: The outcomes-based approach certainly sounds like a promising way to go. Now, I understand the details of many of these contracts are confidential, but what sorts of outcomes are states reporting since the implementation of these new reimbursement models?

CB: Well, since gene therapy is so new and these reimbursement models are just being explored, there isn't much to relay to your listeners yet. But Louisiana is an example a lot of people are familiar with. It was an early adopter of the outcomes-based reimbursement framework.

The state applied for a state plan amendment to implement a subscription-based model to pay for a curative treatment for those living with Hepatitis C, not only in their Medicaid populations, but also in their state prisons and employee health plans as well.

There are some other key components. One is that the amount the state will pay is consistent, so there's a guarantee in how much they will pay on the five-year contract. And this is also important should there be an increase in price.

Again, the details of the contract are proprietary, so it's uncertain how much, if any, short-term savings have actually been made. But one of the long-term goals of these policies is, again, it's a curative, so we're looking for savings overall to the healthcare system.

TM: 06:06

Ed: Well, thank-you Colleen. Before we wrap up, is there anything else you would like to share with listeners, or any resources you can direct them to for more information?

CB: NCSL is always tracking these types of initiatives on both our Medicaid webpage and also our prescription drug policy resource page. So, both of those are good resources to access to find more information.

Ed: Colleen, thanks so much for filling us in on the background of this issue. Stay safe. I'll be right back with Anne Winter.

MUSIC Gene VO:

The 2020 edition of Mason's Manual of Legislative Procedure is now available in both print and digital versions. Designed specifically for state legislatures, Mason's Manual is used by 79 of the 99 legislative chambers in the United States. Prepare for the next legislative session and order yours today at nctl.org/masonsmanual.

Ed: I'm back with Anne Winter. Welcome to the podcast.

AW: Hi. I'm happy to be here.

TM: 07:11

Ed: Well, Anne, first let me thank you for taking the time to be on the podcast. Now, earlier in this pod, I spoke with Colleen Becker and she gave us a big-picture sense of the types of gene therapy treatments that are involved and some approaches that states have taken.

Now, I understand you've done a recent survey of state Medicaid pharmacy directors that offers some insight into why these therapies are presenting a challenge to them. Could you talk about that?

AW: Well, there are a couple of things happening with state Medicaid agencies. First is that one thing, we have COVID and state budgets are really stretched. But even before COVID when these new generation orphan drugs that treat novel and rare diseases emerged over the last few years, some of these drugs can cost into the millions of dollars. So, being able to predictably budget for these drugs can be very difficult.

In the survey that we conducted in conjunction with the Kaiser Family Foundation, we asked what their biggest challenges were and, sure enough, it was really: how does a Medicaid agency predict that there is going to be utilization of these medications, how many people will utilize them, and then what their expenditures are going to be.

And the other thing that compounds the budgeting issue is the fact that, as many of your listeners know, most states do budgets annually or biannually, which means if one of these curative drugs has cost savings in the future, you're not able to capture those cost savings during the budget building period. So, it can look like you have this big, one, initial outlay, but you don't really factor in what savings you may have later.

Part of the problem with that is not only how the budgeting cycle works, but also, when we talk about Medicaid, patients churn on and off of Medicaid, so they may get that curative medication once and then the next year they're no longer on Medicaid and are ineligible for those and the state doesn't accrue to those savings.

So, that's a really big issue. With the pipeline coming up, there's a lot of activity and a lot of thinking. And even in the survey, we asked them what their top concerns were going to be for 2021 in the next few years. Really understanding how to reimburse and cover in an appropriate way high-cost drugs was at the top of the list.

TM: 09:29

Ed: Now, have you seen state efforts that were successful in mitigating the cost of other high-dollar therapies, other high-expense therapies?

AM: Well, again, so much of this is in the nascent phase of understanding the utilization of these drugs and how they're going to be coming into the markets. There are some preliminary strategies that states have used. I think we're going to see more increasingly. And I'll talk a little bit about those later.

One of the challenges, and I don't know if Colleen mentioned this, but there is the federal drug rebate program. What that means is that if a drug manufacturer commits to offering states a drug rebate, then the drug has to be covered. States don't have this ability to create narrow

formularies like commercial payers do. So, they have to come up with strategies in order to do that. One of them is prior authorization.

So, that's the first thing that we've seen states do with respect to these high-cost medications and orphan drugs is to develop very specific clinical criteria around who is really eligible. That's really not that hard because these drugs, because they're gene therapies, work with very specific diseases and even certain individuals within a disease state that have a particular genetic profile.

So, first of all, even the drug manufacturers will have what these are indicated for and their own recommended prior authorization, because they don't want this drug to be given to somebody it won't work on. So, the first thing they do is they create these prior authorization strategies. What that does is it really makes sure that somebody isn't prescribed one of these therapies and it gets administered and it was to the wrong person.

The other thing that has recently come on to the horizon in the last couple of years is value-based arrangements, so this is really around paying for a drug only if it works or has outcomes-based arrangements. About eight states now have state plan amendments with CMS that allow them to negotiate these types of alternative reimbursement strategies with drug manufacturers.

There are some states, even though they have the authority, that are pursuing these more actively I would say than some other states based on the experience I have. There is a variety of reasons for that. Of course, COVID-19 has really kind of slowed some of this activity down. But the states that I would say are leading the way on this right now are Massachusetts, Oklahoma, Alabama, Arizona and Texas.

I know of some drug manufacturers that have active engagements with them on developing these value-based arrangements. So, this is really promising because I think with the flexibility on reimbursement it's not just a formula, but it can be more creative, and I think we're going to see things emerge from that.

Another thing that I see, and I'm not so sure it's necessarily controlling costs, but it might be on the managed care side... So, a lot of states, a majority of states have moved Medicaid individuals into managed care organizations. Sometimes you might have three, four organizations and you might have a small managed care plan, and they get one member that will receive one of these medications and it can be very financially difficult for them.

So, what some states have done, Arizona and Kentucky notably, is create a reinsurance pool, so it helps offset some portion of the cost of the medication. If a smaller plan gets three hemophiliacs, for example, and they all need a very costly medication, this reinsurance kind of follows the member. So, the plan actually submits information on the member and the cost, and they get reimbursed some portion of it.

These are called risk mitigation strategies and it's really a way to make sure that health plans remain financially solvent when they get adversely selected and have no control over it. So, that's kind of a managed care strategy that I'm seeing out there.

Some states will actually carve out of the managed care plans responsibility for high-cost drugs. A couple of states are doing that: Texas and Michigan. That strategy has been around for a long time, ever since the hemophilia factor and HIV/Aids drugs came out, and some mental health drugs. But we're starting to see it with these new gene therapies as well.

A couple of states have these drug review processes, high-cost drug review processes, and notably these are New York and Massachusetts. In New York, for example, they have an overall global cap on their Medicaid spending and within that global cap, they also have a pharmacy cap. And if that pharmacy cap is pierced, they'll look at the utilization and try to identify which medications caused the piercing of this cap. And if they identify that drug manufacturer, they bring them in and they work with them to negotiate some additional supplement rebates.

If they can't come to terms on those supplemental rebates, what New York will do is put that drug on a prior authorization, so it defaults back to that original prior authorization. This is really the mean mechanism states have, but it's also a way, even though the process is pretty detailed, and Massachusetts just implemented theirs, which builds on top of the New York process. There have been some discussions between manufacturers in the state that I think have been very helpful and, again, everybody wants to make this work. Everybody wants these drugs to be available. And so, I think even if a process can appear to be onerous, it can also lead to maybe some better discussions.

Those are some prominent areas that states are taking right now.

TM: 15:19

Ed: Well, these are certainly some life-changing drugs from what I've read about it. Colleen and I did talk about some of these approaches and one thing that seems clear is that data collection is critical. Can you tell us about the different ways state Medicaid programs are collecting data and who they are relying on for their data collection?

AW: That is a great question, Ed. In fact, in our survey, states identified data collection as the number one barrier to implementing value-based arrangements. We're just setting the budget aside, but the fact that getting the data and collecting it on a person level basis and following that person throughout parts of their life in the future is almost impossible.

So, if you think about a Medicaid agency with half a million, a million, four million, 13 million like California Lives and their program, these programs and data analysis are really built for scale. So, when you're thinking about the reporting that they do and have to do for CMS and then you say now we need you to track one individual that received this medication, they don't have the resources to do that.

When I met actually with one state to interview them on this survey, they cited not even having staff to do it because budgets are lean, and they don't just staff positions to track one person. The data collection is really hard. The data comes from claims data, it comes from reviewing provider medical records, it can come from the manufacturers themselves, it can come from pharmacies, it can come from a number of places. But being able to collect it on a person-specific level and then tracking it through the system is almost impossible.

Some states have all-payer databases and even though that can help solve some of it as a person moves... if their originating payer is Medicaid, moves into a commercial plan, becomes uninsured... hopefully these all-payer databases can account for some of that, but they're not regional.

We're very mobile, so if a person moves out of state, then all of that outcomes-based information is lost. So, this is a barrier where I think there's an opportunity for someone to come and solve it.

TM: 17:36

Ed: It does sound like the data end of this is extremely challenging. Let me ask you, is there an ideal model? You're an expert on Medicaid. Do you think there's an ideal model for Medicaid reimbursement of these gene cell therapies?

AW: Well, I think I'd win a Nobel Prize if I could answer that question, seriously, and I think a lot of people would be very, very happy if we could crack that nut. I think because this is still new, as I mentioned earlier, I think we're figuring that out and we're trying to come up with innovative strategies around the value-based payment. CMS has recently encouraged states with new regulations to develop new payment models, and I think we're going to see something emerge from these efforts.

Medicaid programs have always been the incubator of new and innovative ideas, and so I think with this new flexibility that CMS has offered, I think we're going to really be able to start seeing something emerge.

I'm aware of one manufacturer, biotech company that is trying to get creative with their value-based arrangements and announced that they were going to be offering a prevalence-based adjustment on their medication costs. In other words, because these medications treat rare diseases, you might be a state like Montana and you get one of these, or you may not get any of them, because the prevalence is so low.

But there may be a situation where Montana gets five of them for some reason because there is some genetic connection in a family, and this drug manufacturer negotiated a value-based arrangement. But what they're saying is: Hey, we think you're only going to get one. But if you get five, we'll adjust our cost for you.

And so, it's another way that we can get creative around prevalence, particularly around rare diseases. So, that's something I just learned about that I think is really innovative.

I like the reinsurance model personally, and I think if we were to take that reinsurance model that some states have for their Medicaid health plans and thought about it as a state reinsurance pool... not to pick on Montana, but I was born there, so I like to use it as an example.

So, if Montana and North and South Dakota and Wyoming and Idaho, maybe Washington, if they all look at something like one of these novel medications and said well, we've met with the manufacturer and I'm going to get one and you're going to get two and you may have one in

two years. But it's all unpredictable. How do they form through a budgeting process? They come together to form their own interstate reinsurance pool. That might help each other offset those costs.

So, I think there are some things that states are doing, again, and financing that maybe goes up to a regional level for some of these medications. It's a thought that could be explored.

TM: 20:31

Ed: Well, great things for state legislators to be thinking about. We've talked a lot about the states and certainly working with Medicaid, but are there other things going on at the federal level to address the cost of or the access to these therapies?

AW: Yes. I think there have been some most-favored-nation proposals out there; they're in the courts right now. It was done through I think an executive order in the current administration. But one of the things CMS did, and I alluded to it just a little bit earlier, is they issued a regulation around value-based arrangements and one of the things that has kind of been a barrier for manufacturers to go into some creative arrangements is this notion of best price. And what that means is as part of that federal drug rebate program, the state Medicaid program gets the best price that a manufacturer offers anybody. Medicare is actually exempt from best price, but commercial payers for example.

So, what this regulation did is try to disentangle value-based arrangements and the payment out of that broad, best price regulation. So, by disentangling it a little bit, it may encourage manufacturers to be able to expand these value-based payment arrangements, get a little more creative, and not have to worry about this best price rule.

I think it's promising and it's the right way of thinking. As I said, all of this is so new in the last couple of years, what kind of innovation will be coming out of that regulation.

TM: 22:06

Ed: Well, this certainly is, as both you and Collen pointed out, such a new area that there's still quite a ways to go before we probably have enough experience to know for sure what the best approaches are. Let me ask you, is there anything else you'd like to share before we wrap up?

AW: Just a couple of things as I think about wrapping up. There's an opportunity if you think about as a legislator and a policymaker and developing a budget, is funding an analyst position within a pharmacy department could potentially save you millions of dollars. There really is an opportunity where an analyst who is dedicated to being responsible for following outcomes could really be a very cost-effective opportunity.

Better data sharing across states and payers, these all-payer databases, can be used to track some, but what if you created a patient registry within one of them where doctors entered into it specific to these value-based arrangements so we could know what the outcomes are, so sort of value-based arrangement, outcomes-based patient registries.

There are patient registries in many places, but maybe something very specific to these arrangements to help the Medicaid agency with the tracking of them.

Again, the reinsurance pool is something that I think could be an interesting idea. Louisiana did this “Netflix” model where they negotiated a price with Gilead for Hep C drugs for not only the Medicaid population, but also for their prison population. And so maybe these states could come together to negotiate a similar kind of Netflix model for themselves as well. I think we just watch how states implement the new flexibility.

I think finally, one of the things we forget sometimes is: What percentage of overall spending is drugs? And if you look at the information from the office of the actuary, over the last several years and into the future, their projections, drugs are going to stay around 5 to 6% of total healthcare expenditures. So, even though we see these and they’re very prominent, there is not an expectation because they’re so rare that they’re going to really change those financial projections.

But, on the other hand, I think it’s really important that lawmakers keep up on the pipeline and are aware of what’s coming down, not even this year, but next year and future years, just to see what impact it could potentially have with the states.

Ed: Well, Anne, thank you so much for your time and expertise, and it sounds like something we should check back with you on in a year or so when the states have had some more experience with these novels. Again, thanks so much and stay safe.

MUSIC

Ed: And that concludes this edition of our podcast. We encourage you to review and rate our episodes on iTunes, Google Play or Spotify. You may also go to Google Play, iTunes or Spotify to have these episodes downloaded directly to your mobile device when a new episode is ready. For the National Conference of State Legislatures, this is Ed Smith. Thanks for listening and being part of “Our American States.”