Biogen Inc. - Former Senior Director, US Market Access at bluebird bio

Interview conducted on March 22, 2023

Topics

Gene Therapies, Payer Coverage, Reimbursement, Medicaid, Negotiation, Outcomes-Based Contracts, Procedure Codes

Summary

A Tegus Client seeks information on the coverage and reimbursement for high-cost cell and gene therapies in the US. The former Senior Director of US Market Access at bluebird bio provides insight into the challenges faced by patients seeking treatment, including the need to travel out of state for treatment and the logistics involved in the process. The conversation covers payer negotiation processes, outcomes-based contracts, and the role of third-party vendors in tracking patient outcomes. The former Senior Director notes that patient portability is a challenge, and the fewer the treatment centers, the higher the markup starts at for those qualified treatment centers. The conversation also covers the difficulty of annuity-based payments and paying over time in the US commercial insurance market. The former Senior Director notes that any gene therapy with FDA breakthrough designation will have universal coverage, but insurance hurdles may vary from payer to payer. The expert also explains that the burden is on the first manufacturer when they come out with something to establish coding coverage and reimbursement.

Expert Details

Former Senior Director, US Market Access at bluebird bio, leaving in May 2021.

Vice President, US Market Access at ANI Pharmaceuticals.

Prior to ANI Pharmaceuticals, Inc., the expert was Senior Director, US Market Access at bluebird bio, leaving in May 2021.

Prior to ANI Pharmaceuticals, the expert was the Director, National US Market Access and Reimbursement at Alexion Pharmaceuticals, leaving in May 2019. Reporting to the Vice President of Market Access, the expert was responsible for developing and executing specialty market access strategy and tactics with the entire portfolio of Alexion's life transformative therapies. The expert established and maintained executive-level customer relationships with key national and regional payers in the commercial, Medicare, and Medicaid channels. The expert can speak to Soliris and Ultomiris having been involved with the launch of both drugs.

Tegus Client

Hello, thanks for taking the time. I’m generally looking to learn a lot more about the current state of coverage and reimbursement for high-cost cell and gene therapies today in the U.S. on the payer side, so how payers think about these drugs, what are the sort of procedures that they have in place to actually successfully cover these drugs?

How do they work with manufacturers? And then on the manufacturer's side, obviously, seeing what were the sorts of strategies and pros and cons of different approaches that you took to secure market access and coverage for these really high-cost gene therapies. So if you could start with a quick background, that would be great.

Former Senior Director, US Market Access at bluebird bio

Sounds great. Yes I guess that aligns well with my background, not just the bluebird bio, but where I was before at Alexion and on kind of the rare/ultra-rare orphan space where I think our most inexpensive drug

was over $500,000 a year, and patients are on it for life, and then kind of go into the onetime transformative treatment therapies of bluebird bio.

I guess a little bit of background on myself. I've been working in the payer landscape for probably longer than I care to admit, about 25 years now. So really worked my way up. I'm currently Vice President of Market Access at a small, rare disease maker, ANI Pharmaceuticals. So I came over here as employee #2 to kind of start up a new business unit to launch Cortrophin, which is a second-in-class rare disease drug for a variety of indications.

In my experience at bluebird bio, probably launched Zynteglo and Skysona about three different times with payers because during my time there, we were basically six months away from launch and then had a series of delays at the FDA around assays and clinical information and a couple of MDS cases, et cetera. But having said that, I had a lot of great experience working with payers because payers spend a lot of time with us, and I'll give you a good example.

I've never seen UnitedHealthcare take a day. Like all their senior leadership came into bluebird bio headquarters and set up an agenda where they really wanted to understand our gene therapies, and understand the process, and then understand our qualified treatment centers because a lot of it doesn't overlap with their existing business, and then they were getting questions from all of their clients because these are onetime high-cost therapies.

And they needed all the information, they could from us to help justify the cost to their plan sponsors. So it was a fantastic experience working at bluebird bio. It's just disappointing that these therapies got delayed, but now thankfully, they're finally approved and out there for patients.

And then I guess we could talk a little bit about the qualified treatment centers, because that's really a severe rate limiting factor that the patient accesses the number of treatment centers that are out there for a given therapy, depending on the patient or channel mix, be it Medicaid, Medicare or commercial patients.

It takes a long time to get coverage at a qualified treatment center, especially if it's an out-of-state patient. So kind of can talk about some of the hurdles, issues and strategies that we tried to develop in advance at bluebird bio to help payers and also qualified treatment centers because you need to get them talking well in advance of your product FDA approval date.

Because it's a complex payment structure and they need to get their arms around not just paying for the gene therapy, which you can't really announce pricing until you launch but also the procedure of qualified treatment center, travel, lodging, logistics and all that done, kind of in one.

Tegus Client

That sounds perfect. That is exactly what I wanted to get into and specifically for Zynteglo and Skysona, so that should be fantastic. And we'll make sure to jump into the center of excellence qualified coverage side as well.

Maybe just to kick things off, obviously, I’m a little bit familiar with the exorbitant costs and the onetime like dosing frequency and things like that with some of these gene therapies. And I know you alluded to a few of these issues. But outside of just the high cost, what's like an exhaustive list or so of the biggest hurdles that you face when you're trying to do coverage for these drugs like Zynteglo and Skysona?

Former Senior Director, US Market Access at bluebird bio

You know, what's interesting is payer coverage is probably the issue that's not the biggest hurdle. It's the actual patient insurance, if you will, with what is the patient's exact coverage? Is it commercial? Is it a Medicaid? Or is it a Medicare patient. And most of the issues are at least for bluebird bio, the most patients had to travel out of state to get therapy.

So that created a ton of logistics issues. I'll give you a great example in Medicaid. A lot of times you get approval for a drug or a therapy, it's typically good for 30 days. And for the gene therapies, you have to come in for apheresis more than a month in advance of the procedure, so they can manufacture your cells.

So if you have an out-of-state qualified treatment center, you have to travel to that center for about three to four days for apheresis, then go back home for a month. And then you have to have that coverage, too, for the gene therapy after apheresis.

So it was on the patient services side, trying to supplement the benefits of that individual patient, not just for the drug and the procedure in the hospital, but it's also the travel and logistics because it requires that patient to travel multiple times to the treatment center for apheresis and then coming back for a month for the conditioning treatment and then post-treatment 21 to 28-day hospital or close to hospital experience after the therapy.

Tegus Client

Interesting. So how do you get the process set up? Like is this something paid for by bluebird like the manufacturer? Is it paid for by the payer? Is it subsidized by the providers? I know there's only a few of these sorts of sites for each of the main gene therapies that are approved today. There's maybe like a dozen qualified sites or so for each gene therapy. So like how do you manage all of that? Who's in charge?

Former Senior Director, US Market Access at bluebird bio

Well, who's in charge is the qualified treatment center. And I guess I'll give you a little bit more perspective, and I guess I have probably more experience than most, because I kind of gave you my experience. My friend worked at Spark, and they launched Luxturna, and I learned a lot from their experience on Luxturna, and basically, it was the first kind of gene therapy approved in the U.S. for that retinal disorder.

They only had 10 treatment centers as well. So it's funny, I learned a lot of issues from my friend because they actually had an approved drug, and it helped me to talk to both the treatment center and the insurance company, because you have to get them talking well in advance of FDA approval to handle those issues that I just talked about.

It's not just the approval for the apheresis treatment, it's approval for the drug. And then the qualified treatment center has to negotiate the case rate for that particular treatment stay. Which is basically a 21, 28-day stay. So they're basically getting compensated for a transplant procedure in addition to the gene therapy cost. So to answer your question, it all starts with the qualified treatment center.

And they have all sorts of like lodging assistance options. So they kind of take the primary lead on it. Secondarily, the payer, if it's a commercially insured payer, they'll have benefits for travel and lodging on top. And then bluebird bio would come in and offer an additional travel assistance once those two benefits were potentially tapped from both the QTC and the payer or insurer, if you will.

And that's what we were working on prior to my leaving is, what's an appropriate dollar amount over the top because Medicaid patients probably need the most assistance in terms of financial assistance. It's the commercial patients where they typically have not only help from the qualified treatment center hospital, but they also typically have coverage to their employer for lodging assistance and one caregiver to travel with them.

Tegus Client

That makes sense. And then that actual process between the QTC and the payer to get these things approved. Like how is that policy set up for like what's covered and what's not? Is that part of the overall coverage strategy that you guys enacted at bluebird or is this a separate process?

Former Senior Director, US Market Access at bluebird bio

Here's the policy I learned, and I can give a lot of insight from my friend’s experience as well. It's the qualified treatment center and the payers have to talk and negotiate contracts for the procedure for each book of business. And typically, it's a case rate. And here's where the payer will step in and they have stepped in with CAR-T therapies because CAR-T therapies have a lot more different options from a QTC standpoint.

Payers such as UnitedHealthcare and others, and they actually have it listed on their site, who their qualified

treatment centers are for CAR-T, that's the other side, as Blue Cross Blue Shield Association has their accredited cell and gene therapy centers where they do accreditation well above and beyond what the manufacturer does.

But the manufacturer typically aligns the qualified treatment centers around the Phase-III trials and who has the most experience in training and administering and caring for these patients, both pre and post gene therapy administration.

Tegus Client
Very interesting. So back to actually securing the coverage, their coverage rates themselves and negotiating that entire process with payers. What does that process look like?

Former Senior Director, US Market Access at bluebird bio
So here's the biggest issue. The biggest issue is sharing the treatment centers a year in advance of your FDA approval so they can start to have a conversation around the case rate, like what's involved in the transplant procedure and then pre and post hospital stay.

And the challenge for the manufacturer is you don't necessarily have approval from the qualified treatment center yet to share their name with the payer. So that was the biggest hurdle. The payers wanted to know who your qualified treatment centers were, at least a year in advance of launch, so they could start to figure that out.

And I'll give you the back-end part of that because a lot of my bluebird colleagues are over at Kite and other kind of cell therapy manufacturers. The benefit of that is the insurers will have their own centers of excellence for CAR-T and gene therapy, where they may pay for travel and lodging, like a great example, CAR-T is in California for like Kite and Gilead.

Like UnitedHealthcare will fly patients to Arizona for treatment. There's qualified treatment centers in California, but they charge so much. It's cheaper for UnitedHealthcare to fly those patients to Arizona for a month to treat them in an Arizona QTC and then fly them back post treatment, if you will. So that's why it's so critical to kind of share your treatment centers because it's not only negotiating the case rate but the reimbursement for the gene therapy.

And based on that negotiation between the payer and QTC, that's what dictates what the payer will offer the patient to travel further potentially to get the therapy, which may be out of state, even though there's an in-state QTC available, if you will.

Tegus Client
Got it. I've seen some of these payer-published centers of excellence and wanted to know if they're sort of overlapped there, so that's helpful.

Former Senior Director, US Market Access at bluebird bio
Yes. And the other issue on the Luxturna side, which is going to happen to Zynteglo and Skysona right away is the Medicaid population. A lot of the QTCs and that's what we were working through with bluebird bio with the QTCs, getting them accredited to do the accreditation so they can treat an out-of-state Medicaid patient, and it takes six to nine months.

And I know my friend had the issue with Luxturna, so like they didn't have a treatment center in New York. And they had to go to Philadelphia to be treated for their gene therapy. And it took six to nine months to get that Philadelphia treatment center accredited to treat an out-of-state New York Medicaid patient. So that's the other thing that's coming into play right now for Zynteglo and Skysona, and Skysona even more so, because there's only three treatment centers in the U.S., or I think there's two and a third being added.

Most of these treatment centers, beyond their state's borders, they'll have Medicaid treatment privileges or they'll be accredited. Once you start to skip states and the fewer the treatment centers you have, the harder it is for the treatment centers to have basically state Medicaid accreditation to treat an out-of-state Medicaid patient. And that's typically six to nine months for each state.

And the other problem is the hospital typically wants money from the manufacturers to go ahead and do that. Because they've got a dedicated personnel to do that accreditation. So that's where the issues kind of need to be worked through.

And the faster you work on that preapproval, the less issues you have with your patient post-approval because there's a lot of Medicaid patients, especially coming to market with sickle cell, it's mostly Medicaid. So those issues are going to be front and center for many folks, although there'll be more treatment centers available in state for folks.

Tegus Client
Got it. Thanks for the additional detail there. So then outside of the qualified treatment center aspect of coverage, just looking at securing coverage, first and foremost, from payers, what sorts of things do they look for? And what does that negotiation process look like back and forth between the manufacturer and the payer?

Former Senior Director, US Market Access at bluebird bio
It's complex, but I'll give you the 40,000-foot view, and then we could talk about semantics. The manufacturer, we're always looking for coverage consistent with your FDA labeling. And your FDA label in rare disease is always, always broader than your Phase-III criteria. What the payer always looks to do is to limit the population that they'll approve based on the Phase-III criteria for starters. And in many cases, they'll add criteria on top of the Phase-III criteria to make it more restrictive.

So the manufacturer, we want coverage for the FDA indication with as little hurdles as possible around your Phase III criteria and then the payer is going to cherry-pick your Phase-III criteria, both inclusion and exclusion to kind of severely limit the funnel of approved patients. But I think the manufacturer's goal is also, like at bluebird, we would give an outcomes-based contract where we'll pay under a warranty or there was a warranty kind of discussion where you can potentially get up to 80% of your money back, and if the gene therapy didn't work.

Payers had a lot of problems with our pay overtime type concept because basically insurance is on a one-year rider, if you will, in most commercial insurers. So they have to absorb the cost in one year for that employer regardless of a payment over time type model. So the feedback was great from payers, because they basically have to absorb the cost in one year versus trying to spread it out over time. So it doesn't necessarily work in the U.S., it might work more overseas in different markets.

Tegus Client
That's really interesting. I want to dive into those last two points. So let's start with the outcomes-based contracts. So how are these contracts set up over what sort of time period? How do you determine the outcomes themselves? How do you measure the outcomes? The whole sort of gamut.

Former Senior Director, US Market Access at bluebird bio
Well, the outcomes are easier with the bluebird bio therapies because you're either transfusion-free or you're not. It's a pretty easy outcome. It gets a little muddier when you get into other potential areas. So we potentially teed up like in outcomes where we could track the patient over three years. And the discussions with the payers were like, it's probably better to have a third-party monitor these, like third-party administrator, if you will, monitor the patient over three years as kind of the independent validator.

The insurer typically wanted that to be on their shoulders, but the reason it didn't make sense for the insurer is they might not have the patient for three years, because that's the other kind of rate-limiting thing is that warranty or potential outcomes-based contract for that payment to be triggered, one of the things that the insurer did like is if you followed the patient for three years, they might not have that patient in three years.

So it was better to have an independent entity follow that patient rather than an insurer, because they may or may not have that patient in three years. So if there was a failure, the payment would actually go to the original insurer, if you will, back to who made the payment back in the first place. So like those were some of

the discussions that we were getting into on the outcomes-based contract.

But it all goes to the coverage and the coverage had to be to label. And that's where the manufacturer had the most kind of bargaining power because if you're going to pay $2.2 million for the therapy, like you have to cover it to label and you can't add additional restrictions on top of the Phase-III criteria, number of infusions or like for TDT, I think, it was eight transfusions per year or something like that.

I was looking at some of the coverage policies. So the payer couldn't say you had to have 12 or more. They wouldn't take your Phase-III criteria and add criteria on top. They could only use the Phase-III criteria per your labeling.

Tegus Client
Got it. And so a question on the patient portability aspect of this. I know you mentioned that like the patient might not even be with the same plan or with the same payer at all after three years. So does that imply so for both of bluebird's gene therapies here. The payment structure was if the drug is covered and approved to be prescribed, the payer pays the full cost upfront and then gets rebates or like money back, if the outcomes don't succeed. And then regardless of if they have the patient drug anymore, the payer will still get their money back if that patient that they used to have failed on whatever the outcome is. Is that correct?

Former Senior Director, US Market Access at bluebird bio
Yes. And that was all being discussed within what's an appropriate time frame for us to follow the patient and the outcome, and I think that was what was being discussed with payers with what's reasonable because the payer wanted us to follow on a limited number of years. On the manufacturer side, it was very difficult to follow that patient beyond one year, let alone three years, but I think we were kind of settling in that three-year time frame to be able to follow the patient.

And then what was left to negotiate in the outcomes-based contract is who would be the entity following the patient? Because the payer didn't necessarily want bluebird to follow the patient, because you know there's kind of a vested interest there and they kind of wanted an independent entity or for them to follow them, themselves. But again, they might not be at that insurer in two or three years.

Tegus Client
So who are these third-party entities that do this outcomes tracking for these outcomes-based contracts?

Former Senior Director, US Market Access at bluebird bio
So here's the thing. There's every consultant in the world approach bluebird bio to try to get some business in that arena to follow the patient. And if you think about it from a patient hub services, so anybody that offered kind of your traditional hub services would want to follow that patient. So think of your traditional rare/ultra-rare hub services providers.

Then you add on all the consultants that we're willing to do it. And then the one that I was kind of leaning towards at the time was Optum kind of Life Sciences because they probably have the best kind of data tracking independent of like Optum or United, the Life Sciences unit, to be able to follow. So I was working with a lot of the Optum Life Sciences folks because we were going to definitely use them for the United contract and then we are potentially looking to template and partner with them for others.

And here's the thing, everybody was trying to get their name in there as kind of the value-based administrator, if you will, and Optum, probably United had the division that I think had the best capabilities to follow these patients over time.

I think there's COEs and other former payer customers that have their own consulting firms, COEs is another one. And I'm forgetting the guy who worked at that and kind of started COEs. But like we were talking to them as well, as well as a few others. But I think those were the two that kind of stood out to us.

Tegus Client
Got it. And can you tell me a little bit more about what these third-party outcomes tracking, whether it's a

consulting company or a hub service center or some other third party. What is it that they actually do? Like what is their entire role here, what you mean following the patients that are reporting their outcomes?

Former Senior Director, US Market Access at bluebird bio
So as a condition for approval for the gene therapy, they would have to enroll with your third-party administrator and basically, the patient would agree to like basically where that third-party validator, if you will, can reach out to the patient quarterly for surveys to see how are they doing, like they had access to their medical records.

They had to agree to that third-party administrator to fill out those surveys and to track their medical records post gene therapy for a period of up to three years. So that was being discussed as a condition for access in the outcomes-based agreement where that third-party, value-based administrator would follow that patient and have access to their medical records. And they'd also have to fill out these surveys to make sure they're still benefiting from that therapy quarterly, monthly, however, it was designed for them to follow up.

Tegus Client
Got it. And how far does their influence go? Is it just pulling information from their medical records and send the service out? Or can they mandate or request that the patient has to get XYZ diagnostic done or like tell the patients that they have to go get this checkup and like get it set up. Like how far does that scope of power go?

Former Senior Director, US Market Access at bluebird bio
Well, the scope of power is dependent on the outcome that you have to track. Access to medical records was easier for like for Zynteglo and TDT, because the outcome was like as long as they were transfusion free. So you didn't necessarily need them to go out and get a genetic test or diagnostic after the gene therapy, you just had to ensure that the patient didn't have another transfusion.

So for CALD, for Skysona, it's a little bit trickier because like at least for the gene therapy for Skysona, it's a lot trickier because you only halt the progression of the disease. And every day, the patient goes untreated, that disease progressively gets worse, and that's not reversible. So for those types, it's a lot harder to do an outcomes-based contract because it only halts the progression.

It doesn't reverse the damage you already have. So that's a lot trickier to develop an outcomes-based contract on, because sometimes with insurer delays or patient delays, the delays can be really due to anybody getting access to the treatment in the qualified treatment center. But the longer the treatments delayed, the worst outcome for the patient.

Tegus Client
Got it. That makes a lot of sense. Interesting. Is there like an industry-wide standard for who it is? I mean, I know the industry is very small today in terms of raw numbers of approved gene therapies. But is there any sort of standard for who manufacturers go to, to use the third parties? Or is it pretty diverse kind of like the Wild West, still?

Former Senior Director, US Market Access at bluebird bio
Yes. Here's the thing. It's Wild West when I was there. It's getting a little bit better than the Wild West because now you actually have some vendors working for different manufacturers. And that's why everybody wanted to get in with bluebird and the Spark Therapeutics folks and then the Kite folks and then somewhat the Novartis folks, as all of the vendors that supported these therapies would go out and shop their wares to other cell and gene therapy companies with what they're doing for them to grow their business model because they had proof of concept with whatever they did, either as a third-party administrator, as a patient hub services vendor or as a qualified treatment center.

And here's where the qualified treatment centers had a lot of negotiating leverage with payers is, the fewer the qualified treatment centers, and that was the other thing. Each qualifying treatment center wanted to know who was in network, because they wanted to know if any of their peers in the market had access to the therapy because that dictated how much they could ask for, how much they would mark up the therapy.

And great example. KYMRIAH, which my friend launched in the CAR-T space, Northwestern and University of Chicago had access to it and Northwestern refused to treat Medicaid patients. And because it was only them in University of Chicago in the whole like market for in-state patients, they were trying to figure out, like for commercial patients, are they going to mark this up 2x the cost of the therapy or 3x the cost of the therapy, because they were making their money on all commercial patients.

They wouldn't necessarily make any money on a Medicaid or a Medicare patient. So that's the dynamic on the reimbursement side for these QTCs is the moneymaker patients for them are all the commercial, where they mark it up two, three times. And the fewer the treatment centers for that individual gene or cell therapy, the higher the markup starts at for those qualified treatment centers.

Tegus Client
Super interesting. And like depending on if the drug is marked up 2x or 3x by the QTC, if the payers are already on the hook to pay for that, the payers have to pay for that. Is that correct? Or does the payer only cover a certain amount?

Former Senior Director, US Market Access at bluebird bio
Well, that's where you read between the lines. When you go to a KYMRIAH or a Kite CAR-T therapy and then you see the payer treatment centers, that's why California patients have to travel to Arizona to get a CAR-T therapy, because they're charging 2x, 3x where Arizona is probably charging 10%, 20%, 30%.

So that's why it's interesting that both the payers and the QTCs want to know who their competition is on the QTC side, so they have better negotiating leverage with the payer. And then the payer, if they don't have leverage with that individual QTC, they'll fly the patient out of state to where they do have leverage. So that's the give and takes with the cell and gene therapies based on the qualified treatment centers you have.

In CAR-T, there's a little bit less leverage now than there was because there's like 50, 60 treatment centers. So payers have better leverage there. However, with the more limited the number of patients and treatment centers, that's where these treatment centers have a lot of leverage.

So that's the other thing too. When you only have 10 treatment centers, sometimes treatment centers can take or not take patients based on reimbursement. That's the other dynamic in Medicaid, that's really going to impact like sickle cell and other therapies where most of those patients are Medicaid. If they're not making any money on Medicaid, they're not going to be taking these patients.

So that's the other thing I don't think a lot of people realize. You really need to understand the channel mix of the gene therapy you have coming out and channel mix being what's the number of commercial patients you're going to have? And then what's the balance between Medicaid and Medicare?

Tegus Client
So what can manufacturers do to solve this problem? Like whether it's something they're already doing today to combat this or like what's an ideal solution here because especially for drugs like in indications like sickle cell where the Medicaid population is so large, no one is going to be happy if all the QTCs stop taking Medicaid patients, not the manufacturer, not the patients, not the payers. So what's the solution there?

Former Senior Director, US Market Access at bluebird bio
The solution there is you need multiple QTCs probably in every state. And then these QTCs need to negotiate with the state for better reimbursement once these therapies come out to where they're not losing money, because if they're losing money, unless it's a not-for-profit entity, that's where the give and take is going to be as the QTC has to negotiate with that state Medicaid agency.

And I guess to give you that Philadelphia example a little bit more, they refused to take out-of-state Medicaid patients. And remember, that's super important because there's only 10 QTCs in that Luxturna network. There's only 10 QTCs with Zynteglo. Sickle cell, you don't necessarily have that issue if you have a QTC in each state, that can negotiate with that state six months in advance or a year in advance of launch. And

then here's the thing. The more insolvent your Medicaid program is or your state, the harder it is for you to be getting approval, but you can't necessarily solve that.

Tegus Client
That is a tough problem.

Former Senior Director, US Market Access at bluebird bio
Yes. But the best way to handle the sickle cell example is wherever your patients are, and that's where your marketing team needs to do a very good job in working with your patient advocacy, understand where all these patients are at, and they're pretty much in big metropolitan areas and make sure you have multiple treatment centers, because for example, the University of Chicago would take Medicaid patients, even though they were losing money on them.

They were doing the right thing for patients. Northwestern would refuse to take these patients. So that's why you need multiple treatment centers in each state because you don't know who's not going to take Medicaid patients at some point. And it forces potentially negative cash flow situation on some treatment centers that actually agree to take these patients and do the right thing for the patient, which is adverse for their financial situation.

One other thing I wanted to mention to you, which I found super, super fascinating that you may as well is, the other thing with bluebird's gene therapies, and this came up at Northwestern, and we didn't know this until we engaged with them.

They had significant presence, and they actually had facilities in the Middle East and Europe. And part of their thing with like CAR-T and then cell and gene therapies, they would recruit patients from the Middle East where their government would pay millions of dollars for them to come over and get the gene therapy or CAR-T and then go back after they received the administration.

So I think that was the other kind of fascinating thing that Northwestern and Lurie, like we're going to start marketing this to our Middle East and European colleagues because they sourced quite a few patients outside the U.S. for these cell and gene therapies.

Tegus Client
Interesting. And was it like a pretty large proportion of their patients that came ex U.S.?

Former Senior Director, US Market Access at bluebird bio
For the fact that they actually have offices over in the Middle East, leads me to believe that it's a decent amount of patients because they actually have recruitment centers over there. They have offices over there in the Middle East to recruit patients, specifically. And TDT, I think they were already getting interest from folks in the Middle East.

I'm not sure if that was patients or governments on behalf of their patients. So that's the other kind of key piece that we found fascinating because we know there's patients obviously ex U.S., but if the therapies are only available in the U.S., these hospitals and treatment centers have a quite decent amount of business coming ex U.S. for these therapies.

Tegus Client
Interesting. So the other aspect I wanted to talk about when we first started discussing outcomes-based coverage and things like that, is the difficulty of annuity-based payments or like paying over time over a year. Because with these outcomes-based payments, like are you saying that they chose to pay all the money upfront and get rebates back rather than paying like a 25% each year, because they needed everything on the books for one year. Is that the distinction between those two methods?

Former Senior Director, US Market Access at bluebird bio
Yes. So basically, the way to think about it, payment over time does not work in the U.S. You can only do that

when you have a government paying for all of your patients to help them manage their books. In the U.S. commercial insurance market, actuaries, and we had many CFOs from these plans in the realm. They have to account for that patient in that calendar year that they're treated, and they have to collect that payment in the calendar year that it's treated because they're only on the hook for that year for insurance.

So from a premium, and that's the other piece too, is working with actuaries, the premiums are all calendarized for that year only. And based on your spend in that year, that's where your premiums adjust for the next year, like open enrollment for us in commercially insured plans. So if you think about it, payment over time is a great concept.

The only areas you can potentially do that are ex U.S. And then the only thing I think that I would add is some Medicaid programs were interested in payment over time, but it's very difficult to administer for them because they're capital constrained, and it's very difficult to negotiate that agreement with them, because they can always claw back the money later.

So that's kind of a buyer-beware thing. But most of our conversations in the commercial space, it was a great thought to do an annuity-based payment scheme in the U.S., but nobody can operationally effectuate that arrangement.

Tegus Client

Interesting. That's a really good thinking, because I would have guessed that payers would prefer to not take that risk upfront. So do they use that to their advantage like by saying, "Hey, by regulation, we have to pay all this upfront, so we want to pay for a lower price than we would have, if we were to spread it over time." Even with the rebates coming in after the fact. So like net-net, you would expect it to be the same, but they're still taking risk upfront rather than over time.

Former Senior Director, US Market Access at bluebird bio

Here's the thing, the cost of the payment wasn't an issue for the insurer in year one. And basically because there wasn't that many patients out there with TDT or with CALD to where this was going to be a budget buster. Like larger disease states like sickle cell, that absolutely will be an issue where they're going to look for some type of reduced cost upfront. But that didn't come into play for limited patient populations like TDT and CALD.

And then the last thing is to just kind of keep in mind, and just the rare disease space and ultra-rare disease space, the gene therapies, you can argue are so much more bang for the buck than rare disease therapies like where I came from, at Alexion, where STRENSIQ for example, it's an ultra-rare disease, but it's basically $1 million a year of the average patient.

So like the these rare disease treatments, it's almost like a gene therapy payment every year for some of these rare disease treatments. So gene therapies are actually very economical for payers. It's just a sticker price, I think, that the consumer sees, or an outside investment firm might see. Having worked in the rare disease space as long as I have, these are actually more affordable therapies because they're one-time treatments, if you will.

And lastly, on the actuarial side, the one thing that makes rare disease treatment so expensive is you can trigger catastrophic coverage through your employer actuarially for a rare disease treatment in year one, but you can't recoup the cost because like for a hemophilia patient, you trigger catastrophic coverage in year one, but then that patients carved out year two and beyond for the insurer and downstream sponsor to where they're on the hook every year carved out from their insurance.

So that the employer is on the hook after that year-one catastrophic coverage, that the insurance that they're buying from the insurer and then gene therapies are actually much more cost-effective when you look at rare disease, because in rare disease, the moment that patient is identified, catastrophic coverage helps you in year one. But then in year two, all those patients are carved out where you're on the hook dollar one.

And for the life of that patient, too. So again, the cell and gene therapy while they see sticker costs are high, payers actually like almost better, because they're one-time curative treatments and then they could follow

the patient and potentially get their money back later on.

Tegus Client
Very interesting. So is there an analog to these carve-outs with gene therapies? I've seen some of these payer PBM solutions that are like reinsurance products and things like that. Are you familiar with any of those?

Former Senior Director, US Market Access at bluebird bio
Yes. We work with Optum Unite and they're on the reinsurance side and basically, kind of the net-net is United and other national payers wanted gene and cell therapies to be part of your natural coverage because these treatments are so innovative and we have curative intent.

However, on the reinsurance side, they were looking for extra money to have a cell and gene therapy rider, if you will, for maybe folks who couldn't afford them. We're not at the tipping point, though, where there's so many cell and gene therapies for larger disease states, there's only one Zolgensma. There's only $1 billion drug for gene therapy out there right now. Zolgensma.

Until there's more therapies, that's when there's going to be more separation of insurance coverage where you're going to see cell and gene therapy riders separate from their individual coverage for employers. There's really no separate reinsurance. It's hard to do, because there's such limited approved treatments right now. Hemophilia may change that, but I really don't think hemophilia is going to change that, and that's a great analog too, is in hemophilia.

BioMarin is going to have a really, really, really tough time with that gene therapy treatment, because what they're looking to do is basically rather than pay whatever hundreds of thousands for that particular year to pay several million dollars upfront when they might not have that patient in three years. The gene therapies for hemophilia are going to be harder to get covered, I think, than what the current standard of care is, what payers are potentially paying for now.

So that was the other thing, I think, that we learned is BioMarin was had an outcomes-based agreement out there even though they got delayed, they were negotiating at the same time we were, and we got a ton of negative feedback from BioMarin from our customers around BioMarin gene therapy because they were asking for things they never should have asked a payer for. So that was the other kind of thing I can probably give you some insight into.

Tegus Client
So from my eyes, like Hemgenix from BioMarin is somewhat similarly priced as some of these more expensive drugs of maybe $3 million, $4 million, it's just a larger patient population. So does that limit them in some ways and they're looking for others? What is the limitation?

Former Senior Director, US Market Access at bluebird bio
So that's the other thing. The manufacturer has a lot of negotiating leverage with these onetime curative treatments. But the interesting thing with hemophilia in general is, these therapies are very, very costly. They're already carved out from coverage to these patients to where the employers are already picking up the cost. So back to that catastrophic coverage. Most of these hemophilia patients, the insurers are not paying anything.

The employers are paying downstream because they're already through catastrophic coverage. So it's going to be harder to get coverage for a gene therapy there, because they're looking for several million dollars upfront when the employer is already on the hook for it already.

Tegus Client
Interesting. I have a bit of a zoomed-out backup question on Zynteglo and Skysona from your experience. Do both of these drugs end up being covered by the majority of payers for the majority of patients? Or like is there some limitation there? Because like my understanding is it's really hard to get coverage for these sorts of drugs. But at the end of the day, it depends upon medical benefit policy. Are they still getting covered?

Former Senior Director, US Market Access at bluebird bio

Yes. These drugs are universally covered, period, full stop. These drugs will have a coverage policy everywhere, whether the payer has it or not. Reason is, any gene therapy that like at least with Zynteglo and Skysona, anything that has FDA breakthrough designation, you basically have an immunity pricing immunity with, because the payer has to cover it.

So if you think of any FDA breakthrough designation, that's the get-out-of-jail-free card where the payer has to cover it. The coverage criteria may be more difficult, it may be more difficult to access. Great case in point with Zynteglo, some insurers are requiring a genetic test and people know right now if you're getting regular infusions, you might have a genetic test from 10 years ago that shows your beta zero/beta zero, but you might have to get another genetic test to show that.

And genetic tests aren't free, and you got to go out and get additional. So the insurance hurdles may be more or less significant as you get to payer-to-payer. But universally, there's going to be coverage there for any breakthrough therapy designation, and the provider has a lot of leverage, too, with requesting expedited review where they have to get a decision within 24 hours.

Meaning that criteria is another story, and that might take some time to get all the documentation together that you need. But again, that's what you work on pre-FDA approval, knowing what you know about gene therapies and how they're going to cover on this, just to make sure that the QTC has all the patient documentation prior to approval, because they know what that approval is going to look like when they get insurance coverage.

And then the last thing, what's interesting is you'll see Zolgensma, you'll see Luxturna, you'll see many of these cell and gene therapy where the state Medicaid programs will probably never publish a policy, because they likely don't want to invite more Medicaid patients to go in and like basically the patients will switch states if they see coverage for a gene therapy in another state neighboring state, where they're potentially indigent.

Like they'll cover it on a case by case, but I don't think they want to publish a policy because they don't want anybody coming over from Indiana or Wisconsin or other areas. Like they got enough problems within the state, let alone trying to attract patients from out of state coming for these expensive therapies.

Tegus Client

That seems very poorly intentioned. So like I mean, the Medicaid is still generating most favorable nation's pricing from CMS negotiation, correct? Like what makes that influx of patients not end up net positive for them?

Former Senior Director, US Market Access at bluebird bio

Well, the only thing they can count on is they're going to get 23.1%. They're going to get 23.1% Medicaid rebate off the top. But again, remember, these states like Illinois, trillions of dollars in the red for everything that they're on the hook for that, and other states are like that. And they'll approve on an ad-hoc basis a review.

And I remember a friend worked over Christmas on KYMRIAH patient over the holidays, like it was a Medicaid patient, and they were trying to get the Illinois Medicaid, Medical Director to sign up on it, which he ultimately approved that patient. But they basically told them we're never going to publish a policy for KYMRIAH, because we don't want other Medicaid recipients coming from other states to potentially get coverage, if they don't have coverage in their state.

So it's kind of an unwritten rule where you're not going to find state Medicaid, even though they'll cover on a case-by-case basis, nobody is going to publish a policy in general. That's probably the general rule, I would say.

Tegus Client

That is absolutely mind-boggling. I have one more quick question. Just because we've only got a minute left, want to get your high-level thoughts. Like it seems like all of these drugs end up on coverage, if they have

FDA breakthrough designation or if they don't, it seems like there is a way to get coverage. So what is the biggest problem then as it stands today with getting these patients, these drugs?

Former Senior Director, US Market Access at bluebird bio

There are so many problems. I think we talked about it for 60 minutes. The problems all depend on that individual patient characteristics, what state are they in? What's their coverage? Are they Medicaid, commercial or Medicare? And then do they have a qualified treatment center in their state that they can go to. And basically, that patient's insurance is going to dictate how easy it is or how hard it is to get the drug.

And then that QTC may limit the number of patients that they get based on the reimbursement of that patient. If they're Medicaid or not. If they're not a state Medicaid patient, they're going to be further behind in that queue for whatever that QTC is, fortunately or unfortunately, depending on your perspective. Here's the thing, if you're a commercially insured patient you have the most favorable reimbursement for that QTC.

You're going to have the best coverage and that QTC is going to make more money off you than any other patient. So commercially insured patients are going to be at the front of the line for these gene therapies. Medicare will be second and then Medicaid patients will be last. So that's probably the way I would sum it up. It all depends on your individual insurance and whether you have a QTC in that state for whatever gene or cell therapy you have.

Tegus Client

That makes a ton of sense. I have one last really quick clarification question from earlier. So I know you mentioned that QTCs are negotiating with state Medicaid for coverage and reimbursement on their administration and of these drugs and the procedures that surround them. Why is it the QTCs that are the ones doing the negotiation here and not the manufacturer that's negotiating with the state Medicaid to get coverage? Am I misunderstanding something there?

Former Senior Director, US Market Access at bluebird bio

No, you're spot on, and we didn't really talk about this, but this is an important point from the QTC standpoint. Remember, these cell and gene therapies are brand new. They require a lot more hospital support staff work in the preparation. I mean, you look at these package inserts with how these are cryogenically frozen. They have to dedicate staff at how to handle these cryogenically frozen products because they're million-dollar therapies.

And here's the thing, there's no procedure codes for these where they can get reimbursed. And they're much better now where there's CAR-T, there's reimbursement codes like CMS procedure codes, but they have to negotiate that for these Medicaid patients based on procedure codes that are just coming, and every gene therapy requires different prep handling and support staff from that QTC and they're not getting reimbursed from the Medicaid or Medicare for that unless there's a procedure code.

So that's where it's on the manufacturer too, to work with CMS and/or Medicaid to help get codes for these treatments because that's where the QTCs are on the hook for negotiating for reimbursement. Apheresis wasn't paid for by a lot of Medicaid or other programs because there wasn't codes for them. Now they're trying to get codes and what have you.

And like manufacturers like Novartis, like the first CAR-T, they actually paid for apheresis for all patients while they were trying to get codes for it. So the burden is on the first manufacturer when they come out with something to establish coding coverage and reimbursement, and they have to advocate for the QTC, which they might not be able to advocate on themselves or have that individual discussion with Medicaid or Medicare, if you will.

Tegus Client

That makes a lot of sense. Well, thanks for the time and insights. Take care.