Vertex Pharmaceuticals Incorporated

# Vertex Pharmaceuticals Incorporated presents at Goldman Sachs 45th Annual Global Healthcare Conference

Tuesday, June 11, 2024 11:20 AM

## Event Participants

**Analysts** 1
Salveen Richter

**Executives** 2
Charles Wagner, David Altshuler

### Salveen Richter Analyst

Great. Good morning, everyone. Thank you so much for joining us. Really pleased to have the Vertex team here with us. Today, we do have David Altshuler, Chief Scientific Officer; as well as Charlie Wagner, CFO.

### Salveen Richter Analyst

Maybe to start here, could you give us a snapshot of the business today and what we should anticipate in terms of strategy and commercial execution and pipeline in the second half of the year?

### Charles Wagner Executive

Yes. Listen, Salveen, thank you for hosting. We're happy to be here, and good morning, everybody. I would just say this is an incredibly exciting time at Vertex, and the company has never been in a stronger position. If you listen to our recent Q1 earnings call, we continue to grow in CF, TRIKAFTA continues to grow.

We're expanding geographically. We are increasing our penetration into younger age groups and the business just continues to execute very consistently in Well. Not only are we growing in CF, we continue to innovate. There's a lot of interest, of course, in our collaboration with Moderna for our mRNA program for CF. And we'll have more to say on that program late this year, early next year.

I would say significantly 2024 also represents an important milestone in terms of commercial diversification for the company with the launch of CASGEVY, our therapy for sickle cell

disease and beta thalassemia. The early launch is going incredibly well. And keep in mind that we've received a number of approvals in different geographies around the world in a very short period of time. We've commented that a number of patients have already had cell collection and have begun the patient journey and the interest from the patient and physician community is outstanding. And so we're really excited about that launch as well.

We recently closed on the acquisition of Alpine Immune Sciences, which brings with it an asset called povetacicept or pove, it's Phase III ready in IgA nephropathy, and it's in basket trials, Phase I/II basket trials in a number of renal indications and cytopenias, represents -- truly represents a pipeline and a product and is something that we're very excited about. And so overall, the business continues to grow. The pipeline has never been broader. We will have updates on some of our other pipeline programs this year, notably type 1 diabetes, mRNA, as I described, and others late this year, early next year. And so in terms of both growth and diversification and the pipeline, the company is really in a fantastic place.

And maybe that's a great place to start for this conversation.

### Salveen Richter Analyst

Perfect. Let's maybe start with the commercial aspect here. So on your pain programs, you do have an NDA submission for your drug for acute pain. Help us understand the go-to-market strategy here, and how you're negotiating in terms of contracting, thinking about pricing?

### Charles Wagner Executive

Yes. So with acute pain, we have a rolling submission, and we'll have more updates on that soon, represents an incredible opportunity. As we look at the treatment of pain with VX-548, we see the ability to transform the treatment of pain. There is a lot of unmet need. There's a lot of undertreated pain because, quite candidly, the standard of care right now, particularly with opioids in acute is not very attractive for many patients.

And so we see a huge opportunity here. Commercially, we think it's a multibillion dollar opportunity and one that we're poised to go after.

As we think about the commercial opportunity, though, we want to take a very focused approach that's consistent with the Vertex specialty commercial model. And so, for example, if you look at acute pain, there are over 80 million people a year who are treated for acute pain in the U.S., represents north of 1 billion treatment days in a given year, roughly 50% of those -- of the volume is either prescribed in an institution or at the time of discharge. An institution might be a hospital, might be a surgical center. But the institutional setting is really significant in terms of prescribing. And within that, we see a high concentration.

There are roughly 2,000 hospitals that roll up into 200 IDNs that drives the majority of prescribing around acute pain. And so we see a real opportunity to focus there, particularly working with key opinion leaders and institutions like that whether they be orthopedic surgeons, plastic surgeons, ER docs, pain specialists, anesthesiologists, all of those really carry a significant share of voice in those institutions. So we're going to target our commercial efforts early. We think we can do that with a specialty sales force that's targeted

at the physician and hospital level with key account managers that are targeted at the IDN level. And then with MSLs, or medical science liaisons, really helping to drive a high science approach to this.

We also are looking at opportunities for inside sales and digital enablement to make sure that we're amplifying the voice of the sales force. So all of that is in great shape. We have -- we are essentially going to be fully staffed in pain for the commercial organization in the current quarter. And so we feel very, very good about that.

In terms of next steps, we'll be -- and the institution will be focused on -- right now, we're focused on compliant information exchanges in this pre-approval setting, but we know that we're going to work through P&T committees. We know that we need to get on formulary. We know we're going to be talking to PBMs. We know we're going to be talking to payers. Those conversations are already happening at this point.

And there is just a ton of interest in an alternative to opioids. So those conversations are going very well. There's a lot of work to do to make all of those things happen, obviously, but we've built a great team and feel really confident about our ability to launch upon approval.

### Salveen Richter Analyst

Great. David, I think we spoke earlier about just the health economics around it, the hospitalizations around patients who just have to deal with the pain aspect. Could you just speak to that and how the hospitals itself start to incorporate that into their thinking?

### David Altshuler Executive

Sure. Thanks again for having us. What we were talking about at breakfast is the fact that the implications of taking an opioid go beyond just the opioid itself, both because opioids have side effects that can affect patients ability to get up and about and leave the hospital and also how well they do outside the hospital. There's pain relief, which opioids do cause pain relief. So it's not that, that's really the challenge.

You need more pain relief. It's actually the tolerability, the safety and the addictive potential. And so I think that certainly for society, whether it's government payers, hospitals, doctors, et cetera, thinking about the overall impact of being able to avoid opioids, I think, is a very important thing.

### Salveen Richter Analyst

Great. And could you just comment on the legislation that comes into play starting next year like the NOPAIN Act?

### Charles Wagner Executive

Yes. I think -- listen, I think that is a really exciting element and really tells you how much government and societal interest there is in an alternative to opioids. So the NOPAIN Act was passed a couple of years ago. It goes into effect in January 2025, essentially creating the opportunity for an add-on payment for an opioid alternative so that there is not any sort of economic disincentive for physicians to continue prescribing opioids simply because they're

cheap or for patients to choose opioids simply because they're cheap. It's not -- it doesn't cover the whole market, but I think it is very indicative of a tailwind and a strong interest there.

Additionally, there is something in Congress now called The Alternatives to PAIN Act bipartisan support. There's very little that has bipartisan support these days, but I think both sides can agree that addressing the opioid epidemic and creating an alternative to opioids is something that people can agree on. That in -- legislation has not been enacted. But again, it's -- it would look to level the playing field between opioids and non-opioids, ensuring, for example, that there is not -- no utilization management, so prior auth or step-through requirements. We would look to limit those.

We'd look to ensure that there's co-pay equalization wherever possible.

And so I think when you see State and Federal Governments being willing to put legislation in place to either reduce barriers or at least level the playing field, I think it's very significant. It then dovetails very well with the conversations that we're having with hospital systems, with payers. They know that there is strong demand for a product like VX-548. We're going to have to work through all the details of contracting and everything else. We understand that.

But we feel like we're starting with a tailwind on this one.

### Salveen Richter Analyst

You've also noted that Vertex has identified an initial set of specific acute pain conditions and procedure types that are a high clinical fit. Can you speak exactly maybe about this initial set? And then give us your viewpoint on what a successful launch will look like.

### David Altshuler Executive

Maybe I'll start and Charlie may want to comment. I think the most important thing to say is that the NaV1.8 mechanism and suzetrigine specifically, we believe has broad utility across all different types of acute pain as well as neuropathic pain. As you know, we've done 9 studies with this mechanism, 6 with suzetrigine, they've all been positive. And in the case of the Phase III data for acute pain, in addition to the bunionectomy and abdominoplasty study, we also had an open-label study with many different surgical and traumatic pain types, and the medicine works well across all of them. So the question, I think, is more about where is the concentration of patients, where would there will be the greatest ability to get going?

And with regard to that, that's about finding again the right combination of -- for our specialty market approach of concentrated patients, the right health care systems that are interested, so we can make progress. I don't know if there's anything else...

### Charles Wagner Executive

Yes. No, that's really -- it's really -- as we mentioned, some of those procedures and specialties, it's really about where is the greatest and most immediate opportunity to displace opioids and certainly gain traction and the commercial [indiscernible], that's really what the strategy is about.

### David Altshuler Executive

But everything we've seen to date across [Audio Gap] peripheral nerve blocker. Mechanism works for all the different types of acute pain we've studied to date for neuropathic [Audio Gap] with osteoarthritic pain as well. So it's a very broadly [Audio Gap]. The peripheral nerves are sending a signal from the injured or painful place to the brain or in the case of neuropathic pain firing unnecessarily. And if you can suppress that, you can reduce the pain they experience without any of the other side effects that come from a medicine that acts in the central nervous system, which VX-548 does not.

### Salveen Richter Analyst

David, can you touch upon the sodium channel work that's being done here to then bring NaV1.7 into it and maybe even this triplet opportunity on the [ Forward ] and how you're evolving this group? Because it seems to parallel CF in many ways.

### David Altshuler Executive

Yes. So our -- as we did in CF, where we started with 1 medicine, we brought forward additional mechanism and also serially innovated to bring greater and greater benefit. That's our general approach, and that's what will be our approach in pain. suzetrigine itself, as you know, we have the Phase III data. We're filing acute pain [indiscernible].

We had our end of Phase II meeting for neuropathic pain and diabetic peripheral neuropathy, we're pursuing that, and also a study ongoing in lumbosacral radiculopathy, which I could talk more about. In addition, we always try and find even better medicines, if that's possible. And so in the case of NaV1.8, we have what we call the 9 series, which is another set of medicines and VX-993 has completed its Phase I orally, and we're planning to start Phase II studies, and it also has an intravenous Phase I study ongoing. And the goal of additional NaV1.8 inhibitor is simply to see can we drive even greater efficacy if we can go higher and higher up the curve and also obviously make sure we have the ideal drug-like properties, PKs, et cetera. And then there's also NaV1.7.

NaV1.7 is another genetic -- the one other genetically validated target for pain and the mechanism of NaV1.7 and 1.8 is they work together as a pair. NaV1.7 initiates and NaV1.8 propagates the pain signal. So right now, we're suppressing or blocking NaV1.8. Everything we know from the human genetics, for example, people inherit knockouts of NaV1.7 have what's called congenital insensitivity to pain. Everything we know from that and preclinical data says that a highly selective NaV1.7 inhibitor will have effect on its own and potentially in combination.

And that's been 1 of the things that people in the field have worked on for decades. And it's not that hard to find a nonselective inhibitor. Lidocaine is a nonselective sodium channel inhibitor [Audio Gap] so you don't run into any of the other challenges of [Audio Gap] inhibitor.

So about 2 years ago, we were able to -- in the labs -- in the laboratories, sort of crack the biology of being able to bind a highly selective NaV1.7 inhibitor. We're now in the late stages of preclinical work, where we're finding the optimum medicine, and we hope to get that into the clinic as soon as possible with the ultimate goal of having, as Charlie said, transforming the treatment of pain. The one [indiscernible] is we don't see a need for a triple therapy. We think [indiscernible] we don't know that we'll need combination, but if anything, it would

probably be NaV1.7, 1.8. And so that's sort of on the horizon.

But first, we've got to get suzetrigine approved, the additional neuropathic pain, we got the 9 Series and 1.7. It just seems like a tremendous opportunity in the coming years to really transform the treatment of pain.

### Salveen Richter Analyst

On CF, with the vanzacaftor triple, which is on its way towards a potential launch here, could you speak to how that launch will roll out? And anything we need to understand about payer dynamics or physician view over the existing portfolio?

### Charles Wagner Executive

David, you want to talk maybe just a little bit about the profile of the medicine, and then I'll take it from there?

### David Altshuler Executive

Absolutely. So as you know, our goal for a long time has been and remains to bring as many patients as possible, ideally all patients to carrier levels of CFTR function as early in life as possible. The reason for carrier levels is that people who inherit 1 copy of the CF mutation, the other is normal, they have no disease or symptoms or disorder whatsoever. And the reason early in life is because if you've had CF for years, you can get organ damage, some of which may not be reversible if it's scar or what have you. So with TRIKAFTA, it's a really remarkable medicine.

As you know, its clinical data. It's been in the market 5 years. We have long-term follow-up. And it brings a fraction of people to carrier levels and is now proved down to H2. But our goal is, as I said, to get as many people, ideally all patients to carrier levels.

So the vanza triple is more potent and effective, has higher efficacy, first in vitro in our translational model and then just as predicted in the Phase III study, where it had superior sweat chloride reduction. Sweat chloride is a direct readout of chloride transport from CFTR. And maybe the most important numbers to recognize are, if the -- there's 2 numbers for sweat chloride, one, which is a sweat chloride level 60. It's a diagnostic threshold. If you have a sweat chloride over 60, you're diagnosed with CF.

And if it's below, you might or most likely do not, depending on the symptoms, et cetera.

And then below 30, that's carrier levels. They're like someone who is a carrier. So with vanzacaftor, the chance that the person is below the odds ratio, that the person is below the diagnostic threshold is twice as high. The odds ratio that they get below carrier levels is 3x as high as with TRIKAFTA. And maybe the most amazing result is in the 6 to 11 arm study, which normally we'd start 12 and above, we were able to do 6 to 11 study as well and submit all that together.

In the 6 to 11 study, 95% of the children had a sweat chloride level below the diagnostic threshold.

So if you imagine that CF patients today have a life expectancy that is predicted in the recent

publication, if you start TRIKAFTA under the age of 12, it's predicted to be in your 80s. When I started medical school, it was in the 20s, that's just a remarkable thing. But if you're a 5-year-old, parent of a 5- or a 10-year-old and they're predicted to live 70 years, you want to -- going to want to get them as normal a CFTR function as possible, but you don't want any residual disorder that might, over the time, lead to some health consequence. So that's -- and 70% of doctors, I believe, in our market research have said that they're highly interested in this medicine, both because they understand the sweat chloride, the importance of CFTR function and also the once-a-day convenience, while not critical, is certainly helpful to patients.

### Charles Wagner Executive

And so maybe building off of that point, what we know from our previous launches is that CF patients and physicians are incredibly well educated and pay really close attention to what we're doing and innovation and the profile of our products. So we see strong enthusiasm for vanza. From a launch standpoint, given that we have reached so many patients already, I expect there will be strong interest in the drug. There are likely to be a number of patients who want to switch from TRIKAFTA or previous medicine to vanza, though we're not making any real effort to predict what the rate of switching is. I think that's really a choice that patients and their physicians will make.

Again, given the profile that David mentioned, we think switching will be significant, but we're not looking to drive that.

Importantly, even though our [indiscernible] have a fantastic profile, there are some patients who have discontinued use of CFTR modulators. So there's roughly about 6,000 people who were on medicine previously who are not on the medicine [indiscernible]. What we have seen is that when we launch a new medicine, a large percentage of those people who have discontinued want to come back and try the new medicine, see if it works for them or if it works for them at this point in their life. So there's the opportunity to bring some patients who were previously treated, currently untreated back on medicine. I think that's a really exciting opportunity.

I would expect that uptake and interest to be very significant and very quick.

So we're fully prepared in terms of doing our part of compliantly educating on the profile of the medicine. We are -- obviously, the commercial organization exists, our supply chain will be ready to go, and we're really excited for this launch upon approval.

### Salveen Richter Analyst

How is the CASGEVY launch progressing?

### Charles Wagner Executive

CASGEVY launch is great. We said this was a foundational year for CASGEVY. Think about the fact the number of approvals we've gotten around the world in the last, call it, 6 to 9 months, really remarkable. We commented on the Q1 earnings call that at that time, as of late April, there have been 5 patients who had gone through cell collection, representing both sickle

cell and beta thalassemia in all of our major geographies, so U.S., Europe and the Middle East.

So I think that's significant. What you see is broad-based interest. Patients and physicians have expressed deep interest. Payers, there are no [Audio Gap] supply chain obstacles. Honestly, this is ready to go.

And it's just a matter of patients and physicians kind of having a conversation, patients going through the screening process, making the decision to start the journey on CASGEVY. So we feel great. We'll have more to say in the next quarterly call, but off to a really great start so far.

### Salveen Richter Analyst

And David, where do you stand on next-generation efforts, be it in vivo editing, which seems quite complicated, or just a more benign preconditioning regimen?

### David Altshuler Executive

Yes. So obviously, the foundation of our approach is that the gene editing with CASGEVY has demonstrated both high rates of efficacy in the current approach for both sickle cell, beta thal as well as a safety and tolerability profile consistent with the bone marrow transplant. And so the goal is now to be able to get improved patient experience and get to more patients by either reducing the intensity of conditioning, but still doing bone marrow transplant, I'll come back to that, through in vivo editing and also through a small molecule approach we're working on to boost hemoglobin up. And the nearest term 1 is the improved conditioning where we have a substantial effort ongoing preclinically, looking to use essentially antibody-like approaches to target specific cells needed to be removed to enable the bone marrow transplant with some sort of payload to do that. And that is in contrast to busulfan, which, as you know, is nonspecifically affects cells all throughout the body.

And I believe that, that's very doable. I think that it's now a point of -- it's clear that you can find antibodies that are specific for these cells. They're obviously payloads, and we're working hard to do the preclinical work to find that. We also have more exploratory work on the possibility of in vivo editing. And for all of this, the experience with the CASGEVY gene editing itself, the guide, the CRISPR and all the data will be foundational to say that if we can now improve the conditioning or the delivery that you can bring that to even more patients with an even more favorable profile.

### Salveen Richter Analyst

The American Diabetes Association meeting is coming up, and you'll present updated data on your stem cell-derived type 1 diabetes program. Perhaps just to level set everyone, where do you stand in terms of the update that you're going to provide in terms of number of patients and the duration of follow-up? And at this point, is it fair to say you've kind of gotten to that functional curative status, and what is it that you need to show on the [ Forward ] to make this commercial?

### David Altshuler Executive

Sure. So let me just, as a foundation, say that there's over 3 million patients in the U.S. and Europe with type 1 diabetes. And actually, despite all the improvements in care, only a

fraction of them, about 20%, reach the glucose control of the hemoglobin A1C less than 7 that is the ADA guideline. And doing that requires literally like hour-to-hour, minute-to-minute attention to your diabetes.

So there's a great unmet need. The foundation of our approach is the ES cell, embryonic stem cell-derived fully differentiated Islet cells that we first showed preclinically and now we've shown clinically can engraft, make insulin, bring down blood sugar to that goal range and actually, in a number of individuals, no longer have the need for exogenous insulin and also their hypoglycemia in this trial, everybody has hypoglycemia, which means in the attempt to lower their blood sugar, unfortunately, the regulation is such that they go too low, which is very dangerous because if you have too low blood or you can have a seizure or lose consciousness, et cetera, but the patients also have benefits in terms of the hypoglycemia. So that's sort of the basis. And then there are 3 approaches to how we're trying to deal with the immune system. VX-880, which is the 1 we're going to present at ADA, is using standard immunosuppression.

The second is VX-264, which is a device -- custom device, new material, new design, et cetera, invented at Vertex, [indiscernible] before that. And that's just the beginning of its clinical data, and we also have some gene editing approaches. At the ADA, we'll be presenting updated data on more patients with greater duration of follow-up. The study, as you may remember, was initially 17 people, and we have completed enrollment and will very soon have completed dosing them all. And we also proposed and got approval to expand that study to 20 more individuals.

And the reason for that is, as with CASGEVY or any of these cell and gene therapy programs, once you have the kind of promising data we've seen, the key thing to do is to continue into more patients so you can build the case, and we look forward to working -- to collecting that data and working with regulators to figure out exactly what would be needed to get to the market. But we do think the data we've seen thus far as we -- if we can continue to see it and expand it is exceedingly promising for patients.

### Salveen Richter Analyst

Do you think it can be a commercial product in the current version?

### David Altshuler Executive

I do. I do. I think the key thing to understand is that, again, there's over 3 million people with -- I think 3.8 million is the current estimate in the U.S. and Europe. But a subset of those have severe hypoglycemia.

They don't have control. They failed every possible way of doing it. And those patients, in which we estimate there's about 100,000 or so, clearly are potential candidates for a medicine that would restore that even despite the immunosuppression. There's also a set of patients who have transplant already on immunosuppression. So we definitely believe that even though that's a small fraction of the 3.8 million, it will be a very meaningful product for them because of their great unmet need and also could be a good starting point for commercialization.

And then obviously, we'll be working on -- in addition to moving that forward, other ways that I described to modulate the immune system because that really will be the -- I think the barrier to further expansion isn't really so much the profile we've yet seen in cells, admitting it's a small number of people, so we need a lot more data to support that, it will be how we manage the immune system, assuming that data continues to make it available to more people.

### Salveen Richter Analyst

I believe, by year-end, early next year, we'll see data from the Moderna partnered CF program. And it would seem like you've unlocked pulmonary with mRNA delivery in that context. But maybe help us understand what would be meaningful in this population.

### David Altshuler Executive

Sure. So in CF, as you know, we've estimated there's about 92,000 people in the markets we serve with CF and about 5,000 of them are predicted not to be candidates for our CFTR modulator medicines because that 5,000 set of people don't make any protein. And the medicines we have, the oral medicines require the protein there to act on. So for those 5,000 people, some sort of genetic therapy seems necessary. And we worked with our colleagues at Moderna for over 8 years now to identify a novel LNP to deliver, not only to deliver -- to be able to deliver to the human primary bronchial epithelial cells that are the target and that are the model that we have in our labs with our conditions that is predicted quantitatively clinical outcomes for our small molecules.

When we started the program, we actually tried all the other LNPs we could find in patents and published and none of them could get into the HBE cells. The reason being HBE cells exist as the lining of your lung to keep out particles, to keep out bacteria, to keep out viruses. That's why we don't get sick from those things that we inhale. And so it took like 7 years to get that LNP that both could get into those cells and also had a favorable safety, biodistribution, et cetera, in NHPs in animal studies. That's what's in the clinic right now.

And you asked what would be the meaningful. Obviously, there's a sense, obviously from -- a good sense from the CFTR modulators of what type of change in lung function would be meaningful to patients. Obviously, we're hoping to have as high as possible, and we think that's certainly a potential. But I think even something that wasn't as great as like the kind of TRIKAFTA like levels to be very meaningful to people who have no other way to improve their lung function right now. So a little early to say, but I think a meaningful improvement in lung function would be very important to these people.

And obviously, as always, our Vertex goal for CF patients is to get as much benefit as we possibly can.

### Salveen Richter Analyst

Great. Last, maybe a 2-part question here. You acquired Alpine recently, which is, I believe, 1 of the biggest transactions if not the biggest transaction that's happened. Speak to the importance of [ BD ] on the [ Forward ] here for the company, but also what really drew you to Alpine and what we should be excited to see from this portfolio over the next year?

### Charles Wagner Executive

Yes. Maybe I'll start, and you can add. The -- we're really happy to have completed the Alpine acquisition. I was just out there a couple of weeks ago, working on the first days of integration. There's just a ton of enthusiasm with our team and their team about what is possible.

I will say the acquisition, it really is a fantastic example of our corporate strategy and our innovation strategy in action. We have looked at IgA nephropathy, lupus nephritis, membranous nephropathy for years. We have this concept called the sandbox, David can talk a little bit more about it. But we look at all the diseases, we run them through our filter, are they serious diseases, unmet need with biomarkers, clear regulatory pathway, specialty sales force. Once you do all that, the universe of opportunities is relatively focused.

The disease areas that Alpine focuses on have been in our sandbox for some time. And so we have studied every company, every asset, every program in that area for some time.

Recently, we felt that it was the right time to approach Alpine and look to see if there was an opportunity there, there was. Because we've studied the area for so long, we were able to move very quickly. And I think the capital outlay, again, is -- represents, for us, we think, a fantastic deal. The opportunity, the unmet need in IgA nephropathy alone is significant. And with pove, we've got a Phase III-ready asset in the IgA nephropathy.

Additionally, some of the other indications that I've already mentioned represent significant opportunities as well, and they've got a basket study in cytopenias as well.

So it's a great asset with near-term potential. It's also a pipeline and a product. It's a great fit with our strategy. And from a size standpoint, while it was the largest transaction Vertex has ever done, it's well within our means and doesn't reduce our flexibility in any way. So all around, we feel great about the transaction.

### David Altshuler Executive

Yes. No, just -- I think I'd say, our strategy, IgAN fits in perfectly as do some of these other diseases, serious disease, great unmet need, no highly effective therapy, specialty market, underlying biology well understood, both genetics and the lab, but also now pharmacology, and we've been following this and pove looks to be -- have the best chance to be a best-in-class program. That's based on its affinity and its potency in animal and preclinical studies, but also its clinical data. And because we've been following it for a long time, it's not like we saw a few people's clinical data and based it on that. It was based on having followed the field, and we also, having studied the field, do believe that these basket studies that I should say, IgAN is probably ready to go into Phase III, so that's exciting.

But also these basket studies, 1 that includes lupus nephritis and membranous nephropathy and also the other, the immune cytopenias are not only promising in terms of additional opportunities, but also our 2 areas that we happen to already be commercializing in. So in the case of -- we don't have therapeutic areas. We're not going to do something simply because we're already in it, but there is a -- we've obviously, with APOL1-mediated kidney disease, and we also have now a new program in autosomal dominant polycystic kidney disease, IgA

nephropathy will sort of be able to leverage that both clinically and commercially and similarly, with CASGEVY, we're now in the benign hematology space and so the cytopenia. So there's just a lot of ways scientifically, strategically that this is a good fit for Vertex.

**Salveen Richter** Analyst

Great. With that, Charlie and David, thank you so much.

**Charles Wagner** Executive

Yes. Thank you, Salveen.

**David Altshuler** Executive

Thank you, Salveen.