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Biogen Inc.

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Eric Schmidt Analyst

Okay. Good morning, everyone. Time for our next presenting company. We're delighted to have with us Biogen. My name is Eric Schmidt.

I'm one of the analysts at Cantor. And with us representing the company today, Uptal Patel, who's the Senior VP of Development at Biogen's West Coast hub. He is a specialist in everything that is felzartamab. So we're going to be speaking quite a bit about that exciting pipeline program. And we're also delighted to have with us from Biogen's IR team, Steve Amato.

Steve is a specialist in everything Biogen related. So we're delighted to have him with us today, too.

Eric Schmidt Analyst

We're going to start with just a couple of questions on the most recent happenings at the company. I think many of you have seen the updates with regard to LEQEMBI's label change in the last week or so. So let's start with the good news, Steve. You've got an expanded label inclusive of subcu delivery, maintenance subcu delivery at least. What does that mean for the company's commercial efforts?

And how might that help competitively and what's going on out there?

Stephen Amato Executive

Yes. And thank you, Eric, thank you for having us. As a quick reminder, we'll be making forward-looking statements. Actual results may vary, and we have a full list of our disclosures on our website. With that, we're very encouraged by how we see LEQEMBI playing out.

So ultimately, we've seen that market start to grow. We announced that in Q2, we saw a 15% growth in the market, which we think is very encouraging. And it's important to understand the objective here is to make it faster and easier for patients that are diagnosed with Alzheimer's disease to receive treatment. And as Eric noted, we received the approval for the subcutaneous formulation for LEQEMBI maintenance utilizing auto-injector, and we think that's a big positive for patients.

Ultimately, now with this approval, patients have the option after going through a treatment initiation period with the biweekly IV, they can either drop down to monthly IV or they can transition to utilization of a weekly auto-injector. So it's again, building out that optionality. That combined with the data that we -- the most recent data that we presented back in July of this year, showing that with 4 years of continued treatment, we get a continued expansion of clinical benefit. We think maintenance, and now even more so with the subcutaneous formulation, is a more convenient option for patients to stay on therapy longer as they aim to maximize -- to actually maximize that clinical benefit.

Now with that being said, we think there's perhaps an even bigger opportunity to impact the patient journey and the health care infrastructure with a subcutaneous formulation for treatment initiation. So here, this would allow an individual who is treatment naive to immediately start utilizing the auto-injector, allowing for potentially at-home administration. And the good news there is that we have already shown bioequivalence between the proposed 500 mg subcu weekly dose and the approved IV for treatment initiation. And we also initiated the rolling submission to the FDA as well. So we're really encouraged by the potential there with subcutaneous LEQEMBI.

Eric Schmidt Analyst

That's a great overview. Thanks, Steve. Can you remind us on pricing, what the price is of the auto-injector, how it relates to what has been going on in IV in a more maintenance setting fashion and how it also may relate to the induction price?

Stephen Amato Executive

So it's premature to speak about induction price, but for maintenance, the annual cost is estimated to be around $19,000, which is around a 50% premium from IV maintenance.

Eric Schmidt Analyst

And won't that be also your subcu induction dose and strategy? Or could we assume that it could -- do you have flexibility on pricing when you get to the induction regimen?

Stephen Amato Executive

So we'll have to see how, obviously, label negotiations play out and what the ultimate label looks like. It could be a different presentation in terms of milligrams per ml, but we'll have to evaluate that label.

Eric Schmidt Analyst

Okay. And then you also had a label update last week that was maybe less favorable. It encouraged doctors to provide yet another MRI scan to try and reduce risk of ARIA. I think now even -- you've got more of an even playing field versus your competitor out there, both products recommending 4 different MRIs. Is that correct?

Stephen Amato Executive

That's correct. There was a label update. We feel good about the safety and clinical profile of LEQEMBI. I think, obviously, ARIA was an area where there was probably early in the launch was a great focus in trying to -- prescribers trying to make sure they got the right patients on the therapy. We've presented real-world data showing that there's no difference -- or no meaningful difference in the ARIA rate that's observed in clinical trials versus what we're now seeing in the real world.

So we're further encouraged by that.

Eric Schmidt Analyst

No chilling effect, you don't think in terms of asking doctors to do another scan.

Stephen Amato Executive

So we'll have to see how that plays out ultimately. So it would be another step for the patients. But ultimately, as ARIA is still a concern within the market, we think that could help alleviate some of those concerns.

Eric Schmidt Analyst

Okay. Let's zoom out from LEQEMBI. Obviously, that's an important product. But looking at the whole view at Biogen, you've got a variety of new product launches that are seemingly growing at a pretty good clip, LEQEMBI, SKYCLARYS, ZURZUVAE. And to some extent, I'd say those are offsetting a base business that has been bleeding in terms of its top line contributions.

Just give us a sense of when you think these 2 dynamics could cross and what the outlook for growth is over the next few years?

Stephen Amato Executive

Yes. It's a great question. Right now, the company believes that we're executing on the actions that are required for generating that long-term sustainable growth. But to your question around kind of near-term top line, I think it makes sense to take a step back and look at where we are as a company right now. So to your point, we have a legacy MS business, which has shown a high degree of resilience, perhaps even more resilience than we thought earlier in the year.

But outside of VUMERITY, we don't view those as growth products, and they're nearing the end of their life.

On the other hand, we do have recent launches, which are showing continued momentum and growth, and we feel good about that. And to your point, actually, at Q2, we messaged that the year-over-year increase in the launch revenue was more than offsetting the year-over-year decline in MS. So we think that's a really encouraging sign. But as we think about the top line over the next couple of years, it is going to be a function of kind of the pushes and pulls between the rate of decline in the legacy MS business and the rate of growth in our new product launches. But with that being said, I think over the medium term, we have a significant opportunity to grow.

And I think perhaps what investors maybe at this point aren't fully appreciating is the potential value and the opportunity we have with the pipeline to transform the company. We obviously have felzartamab, which has pipeline and a product potential and my colleague, Uptal, will tell you much more about that. But even outside of that, we had an investor event yesterday where

we went over our lupus pipeline, which is anchored by 2 late-stage Phase III assets, one of which has already proven positive Phase III, conducting a confirmatory Phase III right now.

So we feel good about those programs. And then even more recently, we had positive Phase I data for our once yearly salanersen, which is an ASO that's being evaluated in SMA, which can have some real impact on the patients on the standard of care in SMA and where we expect to initiate Phase III studies starting early next year. So we think there's a lot to be excited about across the pipeline.

Eric Schmidt Analyst

So you're investing fairly aggressively in internal R&D. Is there a pressure to do an external deal and further augment the top line growth in the nearer term?

Stephen Amato Executive

So I think between our ongoing launches, in addition to the potential we have in our pipeline, we have the potential to achieve significant growth. Now with that being said, the company is obviously continuing to evaluate opportunities where we can augment that growth, but that's going to be something that we do in a disciplined way, understanding those earlier-stage assets are ones where you can ultimately help guide the development trajectory and knowing that later-stage assets are coming at a premium, and that's all being factored into decision-making.

Eric Schmidt Analyst

Okay. From a valuation standpoint, we've argued pretty vociferously that Biogen shares are undervalued on a cash flow basis. I don't think anyone's disagreed with us yet the stock has not been a good performer to what do you ascribe that to?

Stephen Amato Executive

So I think there, ultimately, again, it goes back to the company taking the actions that we think are required to deliver long-term sustainable growth, which we think is the best way to deliver shareholder value. I would say -- and Eric, I don't need to tell you this, but ultimately, in an industry that's defined by finite products with a finite lifespan, there comes up necessities once in a while to reinvent yourself. And I would say Biogen right now is going through a portfolio transformation. I think ultimately, we're working to deliver the new Biogen through all the pipeline assets that we've discussed through advancing those product launches, and we'll provide updates as we go. But as right now, focusing on delivering long-term sustainable growth.

Eric Schmidt Analyst

Okay. Thank you, Steve. Appreciate the overview. Uptal, maybe you can just orient us to felzartamab and what this molecule is and why it's interesting.

Uptal Patel Executive

Sure. Thank you. Thanks for having us. So my name is Uptal Patel. I'm a nephrologist.

I joined Biogen through HI-Bio, where I was CMO and lead the felzartamab programs. So if you look at immune-related diseases, so many of them are driven by autoantibodies. And autoantibodies typically are produced essentially by various cells that actually express CD38 and CD38-directed therapies that deplete these cells is a really nice targeted way to address autoimmune-driven diseases. And felzartamab was developed specifically for autoimmune

diseases. Unlike other marketed products that were developed for myeloma, we have some differentiation that's really important.

We have low complement-dependent cytotoxicity. We have a chronic and reproductive safety profile in nonclinical species that is more amenable for the kind of dosing we'd see in these populations. And we think that the foundation of felzartamab allows an opportunity to, although we're starting in rare kidney diseases, really expand to other autoimmune-driven diseases.

Eric Schmidt Analyst

So why start in rare kidney diseases?

Uptal Patel Executive

Well, it's a huge unmet need. So if you look across the various autoimmune diseases, some of them have not had very clear endpoints. There's some variability in current standard of care. And in rare kidney, there is really white space where there haven't been transformative therapies that get at the underlying root of the causes for those diseases. And so it provides an opportunity for sort of an initial focus with expansion beyond that.

So as an example, antibody-mediated rejection, one of our lead programs, is a really devastating complication for those who receive kidney transplants. Most people receive a kidney transplant. It's sort of a gift. The wait list is over 100,000 people, fewer than 30,000 people a year receive a kidney transplant. It's the preferred treatment for kidney failure and an incredible benefit for patients who actually get there.

But losing a kidney from antibody rejection, one of the leading causes of graft loss is devastating. But it's driven by these donor-specific antibodies and a variety of other mechanisms that don't require that, that allow us to sort of start there with an effective therapy that has been transformational in our initial data.

Eric Schmidt Analyst

And when you even begin to think about beyond rare kidney disease, where do you also think this role, this antibody may be best suited?

Uptal Patel Executive

So within the kidney disease space, there's antibody-mediated rejection IgA nephropathy, PMN. And outside of that, there's a variety of different places that complement Biogen's portfolio, whether that's rheumatologic diseases like lupus, lupus nephritis, we have a program, in neuro, in endocrine. There's a lot of opportunities to expand.

Eric Schmidt Analyst

Okay. So obviously, felza is a CD38 antibody. It works on the B-cell lineage. What's special about this relative to other B-cell modulators? Why CD38?

Uptal Patel Executive

So among the therapies used for autoimmune diseases, a lot of them are broadly active, and they act on earlier B-cell populations that then have the potential complications with safety, limit vaccine response, lead to excess infections. And so CD38-directed therapy really targets the more mature professional antibody-producing cells without affecting the earlier B-cell populations. And that targeted approach then allows you to maintain the rest of your humoral

immune system, mount antibody responses to vaccines, and we've demonstrated that and selectively deplete them. So other approaches also might modulate B-cell maturation. And this selective depletion then allows this possibility of a more durable therapy.

And in some diseases, we think that might be possible to have larger gaps in therapy.

Eric Schmidt Analyst

We've seen B-cell depletion take on enormous importance in a number of indications. How would we expect B-cell -- CD38-directed B-cell modulation to differ from absolute depletion in terms of clinical phenotype?

Uptal Patel Executive

So if we look at the spectrum of diseases that we're initially active in, we see sort of various immunologic pressure, IgA being potentially the lowest, antibody rejection being potentially the highest. And selective depletion we're seeing is, first of all, leading to pretty profound efficacy across these with PMN in the middle. But we're also seeing that with depleting the cells that cause the disease, you can have durable effects without the need for continuous dosing, like in IgA nephropathy, where we can dose for 5 months but see durable drops in the markers of disease activity out to 2 years. In diseases like PMN, we're seeing that, that might require additional dosing, but might be annually. And in antibody-mediated rejection, higher immunologic pressure with an allograft that is persistent causing the immune system to continue to recognize it, that's an area where we might need continuous dosing.

Eric Schmidt Analyst

I mean I assume you would think about an area like IgN is requiring intermittent dosing, dose drug holidays. So what benefit does that bring to the patient?

Uptal Patel Executive

So it's great that there's an evolution of therapies in IgA nephropathy, particularly some that affect B cells and potentially can be disease modifying. What we have done is looked at -- talked to payers, talked to providers, talked to patients and found that there's a real need for therapies that offer a different option, a differentiated option that might not require continuous dosing. These patients are young. They're in their 20s, 30s, 40s and continuous dosing can be complicated. People can miss doses, where our therapy could allow somebody to have a 5-month course, but then a drug-free holiday for several months out to 2 years, we'll see if it can last longer.

Eric Schmidt Analyst

Let's start with your lead indication of what I think most of us might consider your lead indication, late AMR, late kidney -- antibody-mediated kidney rejection. We saw some data, obviously, from an early study published in New England Journal of Medicine. It was phenomenal. I guess the one criticism of that data set might have been that it was a fairly small sample size. How would you address that?

Uptal Patel Executive

That's true. It was a small proof-of-concept study. The results were indeed striking. So we saw about a 20% placebo response to resolution of MVI by histology, but an 80% response in those

treated with felzartamab. Just for context, other therapies that have been tried in this space generally have about a 20% efficacy.

So our placebo rate matched that.

What was different is that the extent of response was markedly different than any other therapies that have been evaluated, specifically, the hallmark of this disease is microvascular inflammation, which is a sort of score on histology of the amount of glomerular inflammation and peritubular capillary inflammation. We saw 2/3 of the sample have scores of 0, so complete resolution of this inflammation that drives the disease. That was after -- that was with a 24-week period. That's never been seen before.

The other supporting data to show that there's a real benefit here is that there's a biomarker called donor-derived cell-free DNA that's essentially a very specific marker of allograft injury. And we saw that drop very dramatically within 12 weeks, stay down through 24 weeks, which is also a very nice marker of disease effect. And then to put all of that together, small sample and cautious in interpreting the GFR comparisons between the groups, but we saw a pretty striking difference in the glomerular filtration rate estimates across the 2 populations, continued decline, about 4.5 ml per minute per year in the placebo group and stabilization in the felzartamab group.

Eric Schmidt Analyst

Okay. So very consistent data as well. Remind us of the size of this opportunity and then time lines for the Phase III readout.

Uptal Patel Executive

So this is not well characterized, but we think that there's about 23,000 people with antibody-mediated rejection in a prevalent population of about 300,000 people who have a kidney transplant. And we think about 11,000 of those have late AMR. And again, the current standard of care has been completely ineffective. It's actually quite expensive. It involves plasmapheresis and IVIg or other immunosuppressants that haven't demonstrated efficacy.

And so if you just take as a base case, this 11,000-person estimate with a modest cost for therapies that could include what we see in IgA nephropathy in the $150,000 per year range, that's a market opportunity of over $1.5 billion. And so that's not trivial. We think it's quite promising.

Eric Schmidt Analyst

Great. And who's going to pay for this? Is there still capitated reimbursement in this environment? And is it an add-on to that capitation? Is that causing a potential payer issue?

Uptal Patel Executive

So I think this is a place where the cost of graft loss is tremendous. So dialysis costs $200,000, $300,000 a year, a new kidney transplant costs over $400,000 a year -- or for the initial episode. So there's a real need for all insurers to sort of be aligned with preserving kidneys that have been provided. And so this is generally late AMRs past the period of that initial 1-, 2-, 3-, 4-year period where there might be some capitated payment plans. So we think there's a real incentive here to preserve grafts that people have received.

Eric Schmidt Analyst

Okay. Let's move on then to PMN. It's maybe the second of your indications. What is PMN? And what's the data to support felza being active here?

Uptal Patel Executive

Sure. So primary membranous nephropathy is a quintessential autoantibody-driven disease where there are antibodies that are targeting podocyte-specific proteins. And this causes a very high-grade proteinuria, a lot of symptoms related to this for patients that generally in their fifth and sixth decades. And the current standard of -- there's no approved therapies, but the current standard of care is generally chemotherapeutic agents like cyclophosphamide or anti-CD20s or a variety of other therapies that include steroids and others.

The challenge has been that because CD20s aren't -- CD20 isn't expressed on plasma cells, plasma blasts, these professional antibody producing cells, you see that there's about 1/3 of patients who receive CD20s don't actually respond. And then another 1/3 who might will relapse within a period of time. And so there's this opportunity for felzartamab to sort of help fill that goal -- fill that gap with patients who've relapsed, and we've demonstrated efficacy with felzartamab in patients who have relapsed or been refractory to prior therapies.

The other is that in high-risk patients, they also tend to be less responsive to upfront CD20. And so our studies in Phase I, Phase II demonstrated that in patients with high PLA2R, an autoantibody that we can measure present in over 80% of people with PMN, we saw pretty striking responses that were rapid within a few weeks, over 90% reduction in PLA2R. Off therapy, the 5-month course of therapy, they continue to have deepening reductions in PLA2R, which then generally translates to responses and reductions in proteinuria.

Eric Schmidt Analyst

Tell us about that correlation. You have shown good autoantibody reductions. What is the evidence to correlate that to kidney function?

Uptal Patel Executive

Yes. So essentially, for PMN, it's hard to have clinical responses prior to having an immunologic response. So essentially until the autoantibody burden is controlled, it's hard to have reductions in proteinuria. And so the correlation is quite striking. When you look at some of the epidemiology, you see that essentially a 3-month drop in PLA2R is predictive of the 1-year reduction in proteinuria.

That correlation held up in our data. It's held up in other data. And so it's a very strong correlation.

Eric Schmidt Analyst

And what's the landscape here with other B-cell targeted therapies in development?

Uptal Patel Executive

So it's growing. I think there's a few BTK inhibitors at play. There's APRIL/BAFFs entering the space. Obviously, CD20 with a humanized form improved from the current available therapy is in trials. And all of these, I think, have some potential advantages.

What I think is important about felzartamab is it provides a differentiated option, particularly for those who may not respond initially or high risk initially.

Eric Schmidt Analyst

And is felza the lead indication? I know it is the leading drug of B cell modulation in this space. Or are there others in Phase III with you?

Uptal Patel Executive

There are others in Phase III. I think there's expected readouts for a newer anti-CD20 next year.

Eric Schmidt Analyst

Okay. And the size of this opportunity in terms of numbers of patients?

Uptal Patel Executive

So about 36,000 people in the U.S. So sizable opportunity, again, allows room for a variety of therapies and particularly different sequencing. We think felza can be an important frontline therapy in high-risk patients and an important second-line therapy if other therapies prove to be effective.

Eric Schmidt Analyst

Great. Okay. Let's get on to IgAN, which in some ways is the biggest of them all. How many patients are affected with IgA nephropathy?

Uptal Patel Executive

So different people have different estimates. We're using a conservative estimate of about 130,000 people. Again, this space is -- there's an explosion of sort of new therapies here. And so we think that although there are some really promising therapies on the horizon, there's a really an important opportunity for a differentiated asset that could allow noncontinuous dosing.

Eric Schmidt Analyst

Why is there a debate about how many U.S. patients have IgA nephropathy?

Uptal Patel Executive

So IgA nephropathy typically affects younger individuals in their 30s to 40s with some variation around that. It presents initially relatively asymptomatically. So it can be hard to detect until people actually are identified as having something that's abnormal typically in their urine. And then the diagnosis requires a biopsy. And so we think that's one of the leading reasons it's underdiagnosed in the U.S.

In other countries where the prevalence is higher and they have screening programs, it's much more -- it's diagnosed in real time, and there's much less of a lag.

Eric Schmidt Analyst

Okay. So what would be a competitive profile given all the newer potential entrants in the space? What do you need to achieve in terms of clinical profile?

Uptal Patel Executive

So the typical pathway for development in IgA nephropathy is a 2-year study with a 9-month accelerated assessment based on proteinuria. The 2-year endpoint is GFR. And what we have seen to be competitive is at least a 30% reduction in proteinuria at the 9-month time point and

stable GFR and at least separate -- differentiated from the placebo. I think what's interesting is although we had a small proof-of-concept study, we had about 12 people per arm dosed compared -- doses of 2, 5 and 9 doses compared to placebo.

And we saw that in the 9-dose regimen, within a few months, you have stabilization of proteinuria, about 50% decline, which continued to drop after the dosing interval. So 5 months of dosing, but a 2-year study, so 19 months off therapy, we see that there's ongoing reductions in proteinuria. That was striking. And this is also paired with data showing that we have stabilization of GFR relative to placebo.

Eric Schmidt Analyst

So why would this drug work after the dosing interval? I know you showed the graph that you just described that there was some diminution or decline in proteinuria post the stoppage of dosing, but that didn't necessarily make sense to a lot of us. And many of us expect it's just a matter of time before you see a rebound anyway.

Uptal Patel Executive

Yes. So the backdrop of the disease is that these autoantibody-producing cells are generally in the mucosal-associated lymphoid tissues which are a much more accessible compartment than the bone marrow. So our hypothesis is that this is readily accessible with depletion of these antibody-producing cells. We see a durable reduction in the cells that are producing this and therefore, IgA levels broadly, but also GAG deficient IgA, the autoantigen in this disease.

Unlike the drops in IgG and IgM that we see, for IgA, we see about a 20%, 30%, 40% drop that endures through 2 years. So what that suggests is that this compartment that where IgAs produce generally mucosal associated lymphoid tissue seems to result in a deeper depletion that's more durable.

Eric Schmidt Analyst

Okay. So currently, felza is IV, I think, once weekly for 5 weeks in this indication. There are now multiple players coming to the IgAN space that offer the potential for subcu dosing. How do you line up those 2 routes of administration?

Uptal Patel Executive

Yes. So the journey for us has been pretty rapid. As a start-up, we didn't have the resources to embark on a subcu program, but been grateful to join Biogen and have the resources of -- across the company. And that's an area of active effort, and we'll look forward to sharing more about that in the coming year.

Eric Schmidt Analyst

Do you need subcu to be competitive?

Uptal Patel Executive

So again, we think it's a nice option, particularly for patients where there will be subcu options and allow outpatient administration. So for IgA nephropathy, a larger population, we think it will be potentially helpful. For -- our assessment in AMR is that it may not necessarily be all that important.

Eric Schmidt Analyst

Well, there's nothing else.

Uptal Patel Executive

There's nothing else.

Eric Schmidt Analyst

Okay. You mentioned other indications, and I think SLEs seem to be maybe on the tip of your tongue. Obviously, there's an ongoing study there. What and when can we hope to see from that?

Uptal Patel Executive

So we have an open-label signal-seeking study in lupus nephritis. There's been some off-label use case reports of commercially available anti-CD38 in lupus nephritis demonstrating remarkable efficacy, CAR-T like. Those are small single center studies. And so there's some caution we have to use in interpreting those data. But what we're looking to do is see if we can replicate some amount of efficacy in patients who've not responded well to other therapies.

So aggressive lupus nephritis Class III, IV with or without Class V, high-grade proteinuria, active immunologic activity. And so we're in the process of conducting that study. Hopefully, we'll have some results to share next year.

Eric Schmidt Analyst

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Uptal Patel Executive

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Eric Schmidt Analyst

Great. Uptal, really interesting program. Thank you for walking us through it from A to Z. Steve, I appreciate you being here today. Thanks, Biogen team.

Stephen Amato Executive

Thank you.

Uptal Patel Executive