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PRESENTATION

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Hi, this is David Amsellem from the Piper Sandler specialty pharma team, and we will be spending the next 25 minutes or so chatting with Harmony Biosciences. And with us is CEO, John Jacobs. John, thanks very much for joining us.

I wanted to flip it over to you, John, for some quick introductory remarks, and then we can dive right in to what will be a lively discussion given the launch of WAKIX. So John, why don't you take it away?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

David, first of all, thank you for the nice introduction, and we appreciate this opportunity to share a little bit more color and context on our launch of WAKIX with Harmony. Our company, for those of you who are new to our story, we were founded in October of 2017. And since then, we built out a company of over 150 employees, and we've launched our first product in the U.S. Market, WAKIX. And we're starting with WAKIX in narcolepsy, although we do see our product as a portfolio and a product opportunity due to its unique mechanism of action, which we can discuss a little bit more later today.

We're starting in narcolepsy. And our focus is on rare orphan neurological disorders and helping patients who are so often underserved that are living with those diseases, David.

QUESTIONS AND ANSWERS

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Great. So I'll start off with a high-level question on the role of WAKIX and specifically in narcolepsy, I should say. So narcolepsy is very much a polypharmacy type of a treatment landscape. So with that in mind, how do you view WAKIX' role over time relative to other mainstay agents such as oxybate or I should say, Xyrem -- low-sodium Xyrem and other wakefulness-promoting agents. What's your general take on the broader role of WAKIX in this space that is admittedly evolving?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

It is David. Look, very good question and an important one for patients and health care providers as well. Narcolepsy is a complex disease, and it is a polypharmacy market. Importantly, WAKIX offers a meaningfully differentiated product profile. WAKIX is a unique MOA. It's the first product of its kind to work through histamine in the hypothalamus. It's the first nonscheduled medication from FDA. It's not a stimulant. There's no evidence of withdrawal symptoms or tolerance building up in our clinical trials. And we're seeing WAKIX used in combination with other therapies across the spectrum, whether that's wake-promoting agents and stimulants or oxybate products, et cetera. And it can be used as monotherapy as well. We're seeing both, and we see that as a long-term role because there's so much dissatisfaction in the market, David.

And of who are currently treated in the space, about 3/4 of them have residual symptoms that are not only measurable but negatively impact patients' lives. So there's a high level of dissatisfaction and a lot of need for additional products in the market. And WAKIX fits very nicely to match those unmet needs with our product profile.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. So you mentioned previously that there is quite a significant number of patients. I think it's roughly about [40,000] or so narcolepsy patients who are diagnosed but not treated. Why is that the case? And what's your view on your ability to access these diagnosed patients over time as they cycle through legacy agents like stimulants and modafinil.

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

David, we're actually sourcing from that pool of patients. And you asked why, it's a question that I asked as well as we really dug deeply into this market when we founded the company. And again, that data, data demonstrates a high level of unmet need and dissatisfaction. So as I said earlier, about 3/4 of patients have residual symptoms. Over 90% of treated patients have expressed dissatisfaction with current therapies, side effects, tolerability issues, scheduling, difficulty getting access to the medication, et cetera. And so many patients have just decided to go it alone and fight the disease without any therapy at all.

But WAKIX has been introduced. We're starting to source from that bucket, and we can access those patients as long as they go through the step that is required by managed care. And the vast majority of those 1 to 2 generic step edits and they can get access to WAKIX.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. And I wanted to talk about the payer landscape in a bit, but let's go through some metrics on WAKIX specifically, your metrics on average number of patients who are on drug, and this is bearing in mind that the product launched in late 2019. So I wanted to put that out there. Just average number of patients on drug and how that's been trending and then also unique prescribers and particularly repeat prescribers. Maybe you can walk us through that.

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

Absolutely. Look, we're very pleased so far with our launch success, David. And it's never ideal to launch a product in the middle of a global pandemic and have that hit in your first quarter of launch or so. But despite that, we're really pleased with the growth. Our average number of patients in Q3 was about 2,200, which represents over a 20% increase from the prior quarter. And that also considers new starts as well as patient behavior like compliance and persistence. It's all baked into that number. And we're very optimistic we can maintain that continued growth. And as COVID lifts, really see the optimal performance in our launch that we believe we're capable of with this product.

And when it comes to unique prescribers, very interesting, there's about 8,000 or so physicians that diagnose and treat the majority of narcolepsy patients in the U.S. And once again, we've had great reaction from them in the market, especially with the new cataplexy indication. In fact, we've had over 2,000 unique HCPs prescribe our drug, and that's about 1/4 then of the entire prescribing universe, at least one time, and the vast majority of those are starting to become repeat prescribers as more and more patients come into their therapy choices.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. And I wanted to touch on the mix of patients who are getting WAKIX as monotherapy versus patients who are getting it in combination with oxybate. Maybe talk to what you're seeing in terms of the usage and mix. And I guess the -- there's a couple of ways that I wanted to sort of slice and dice this. One is portion of patients who are taking their -- staying on their background, modafinil or stimulants, a portion of patients who are on background and oxybate? And then what are you seeing in terms of WAKIX and some therapy?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

And David, we're not disclosing the specific percentages by category. But what we're actually seeing is combination therapy across the spectrum. So it's with your gold standard, modafinil plus a stimulant or it's with Xyrem or it's with the combination of all of those. And why is WAKIX profile? There's no PK drug interactions. That's right in our label with the drug. So WAKIX can safely be added on to gold standard combinations or long-established therapy combinations. We are seeing some stimulant sparing behavior over time. So once WAKIX is added into combo, we're seeing quite a large number of patients stop taking or reduced their use of stimulants, which is really great because there are public health indications there that we're very pleased with. So to offer the first and only nonscheduled medication that's not a stimulant is obviously having a benefit to some patients there.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. All right. That's helpful. And then what about the mix of patients who have narcolepsy with cataplexy versus patients who have narcolepsy type 2? I know you've alluded to this in the past, but maybe help us understand where that is right now. And maybe this is a way to talk about your cataplexy label expansion.

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

Yes, which we're very excited about, David. Excellent question. And what you see in the market, general diagnosis codes, it's about 50-50, type 1 and type 2 when it comes to diagnosis, even though the prevalence is thought to be about 65, 35 or so, it's 50-50 in diagnosis and reality in the U.S. and that's about what we're seeing with WAKIX. So really, in general, it mirrors the marketplace.

I think it's important to realize for everyone who's maybe some of the folks who are new to this market, that every patient with narcolepsy has excessive daytime sleepiness. And that was our first indication when we were first approved by FDA last year. That's the cardinal symptom, whether they have type 1 or type 2 narcolepsy, that's the core symptom. Type 1, they also have cataplexy, but we were on label for all of those patients since our launch. But we are excited about the new cataplexy indication, David, and we've had a very good response from patients and physicians. And there are some physicians we were seeing that might have been on the fence before, brand-new drug that are starting to get more excited about using WAKIX and trying it for the first time now that we also have the cataplexy indication.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

And I wanted to switch gears and talk about the payer landscape. You alluded to it earlier in the discussion, but I wanted to dive a little more deeply. What portion of commercial lives have access? And can you elaborate on the kind of step edits and prior offs that we're typically seeing? And as part of that question, do you see any step edits through -- in other brand's product? I think that would also be important to cover.

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

Yes. Yes, we do. But the vast majority of lives, it's only 1 or 2 generic step edits to get WAKIX. So the vast minority. Right now, we have over 180 million U.S. lives that have access to WAKIX, and that's across commercial Medicaid and Medicare plans. So we've had some, we think, remarkable managed care penetration and access for the product. And so far, no negative formulary decisions to date, meaning an NDC block, et cetera. So very good access to WAKIX so far. We're very pleased with it, and we're even more excited with the new cataplexy indication that, that may happen both the near term and the long term to be protective for the product and to help even more patients have access to it.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

And so you're not seeing any patients having to step through, say, SUNOSI, which is another new narcolepsy agent, but has a very different price point for reasons we don't need to get into. But you're not seeing anything like that?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

I wouldn't say we're not seeing anything. There are a small portion of plans that are requiring one branded step, David, but the vast majority are not. The vast majority are 1 to 2 generics.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. So I wanted to speak about the COVID impact or the impact of the pandemic on the business is something that you've talked to a number of times. So I guess, as a starting point, can you -- maybe I'll ask the question generally is, can you talk about what aspects of the WAKIX commercialization, the rollout are being hit the hardest as a result of the pandemic?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

Well, I'd say, look, in general, the pandemic has affected our entire industry, right, whether it's physicians, patients and companies and how each of those groups interact with one another. And I think most companies, if not all, in our industry, have seen some impact on their ability to generate top line demand because of less connectivity with their sales teams and physician office staff, et cetera, and also on persistency, compliance rates and all those different factors. So I think it's on the top on demand and also on retention.

And patients' relationship with their medicines and with their doctors has been altered temporarily by the pandemic. The way that they take their meds, the way that they're getting counseled on meds, whether they see the nursing staff or not for reminders, all of those things can have an impact on drug uptake, utilization and compliance rates, et cetera.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Yes. So do you think that the -- speaking of persistence rates, I mean, this is a -- again, a polypharmacy market, there's a lot of switching. And do you think that persistence rates, in general, have been lower than what they would be? If it will be lower the pandemic, and specifically, your physicians rates lower on WAKIX than what you anticipated because of the pandemic?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

I think the way to think about it is that, again, that patient's relationship with their med has changed for most pharma companies. And though we're not disclosing our specific persistence or DC rates, the way to think about it, I think, David, for everyone, is prior to the pandemic, DC rates for drugs -- a publicly available data indicates DC rates for all the drugs in this category were between 30% to 50%. And WAKIX is in that range. And you figure WAKIX with a really good profile, excellent tolerability, nonscheduled, right, might be in the middle to the lower end of that range. But certainly, certainly, COVID has put pressure on any medication, in general, you'd have to think within that range and on that range itself when it comes to compliance and persistency.

A big factor in that, David, is unemployment, right? And we've seen them lose their jobs and then lose their health insurance. Importantly, Harmony is a patient-centric organization, and we have a patient assistance program in place. And as long as patients meet our eligibility criteria, they can get the product at no cost if they've lost their insurance or they're unable to get it from us.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. So now let's talk about that a little more. You've talked about subsidization of cost of drug as a headwind. In other words, impacting your net sales. Can you give us some more color on the nature of that impact? You don't have to quantify it, but talk how impactful that's been, not just in 3Q, but as we think about 4Q and early 2021.

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

It's a great question. And I think it's helpful for any investors thinking about investing in pharma companies, whether they're launching a drug or already selling drugs that, in general, publicly available information would indicate about 3% to 5% in general is a number that's a good planning number for patient assistance programs in general. That's pre pandemic in normal times.

Now we saw unemployment rates, and I checked it again, I check the stats each month on it, but they were as high as 15% or 16% in the United States during the pandemic. And so those patients are losing their insurance, unable to necessarily get medication. So you could see some companies might be experiencing 2x or 3x the normal level of demand in their patient assistance programs. And it's hard to predict how long that will last, David.

But as will lift out of the pandemic, patients will go back on employment, go back on their insurance, and we work with our patients. We sustain them on our path. And then as they get reemployed, we work with them to get covered by their insurance for WAKIX again. We've already seen that start in some cases, though.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

okay. So the idea here is that as the pandemic eases, those patients where you're subsidizing, but in some cases, the full cost of drug, ultimately will flip to being paying customers in 2021. That's your expectation?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

Yes. A portion of those patients would, yes. Some would be eligible for the PAP regardless of the pandemic, right, just because they already meet the criteria. Others may fade away. But those who stay on the medication, it's working well for them. They're tolerating it well, and they become reemployed, yes, they would flip back.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. So what portion of your sales organization right now are doing their sales cost live versus virtually? Can you talk about how that's changed or hasn't changed over these past few months?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

Well, we don't have that broken down specifically, but I would say it depends on the region of the country. They've been where each state is and its process of lockdown. We've seen states open up and physician offices open up and some of our reps are able to go out and make some face-to-face calls. And then we've seen those same states shut back down when they've had a resurgence or an issue. And also, we've had -- there has been hurricanes and wildfires and other things that have affected large pockets of geography down South and out West, et cetera. So it's really dependent on the geography. Importantly, our team made a very good pivot, though, to using a digital platform, use Aviva platform, and we can interact with physicians on these machines that we all have a great habit of using all the time. Our cell phones, our iPads, our laptops. So a physician could be at his or her home walking around their living room and interact with a rep on the phone just like we are here on Zoom. But still, it's not a replacement for face-to-face.

And the last point I'll make there, David, is when the salesperson enters an office face-to-face, they're interacting with that entire staff. From the people at the front desk to the nurses to the folks who handle insurance to the doctor. And that's very helpful in a product launch, and you lose a bit of that office connectivity, even if you're getting some of the staff for the physicians face-to-face digitally. So digital is something we'll continue to weave into our mix regardless of the pandemic. And I think we've learned a lot and done well with it, but it's certainly not a replacement. It's not optimal versus live face-to-face selling.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. I wanted to look further ahead and get your thoughts on the role of WAKIX in the narcolepsy landscape that's going to evolve. You've got Axsome's norepinephrine reuptake inhibitors. You've got reboxetine. You have orexin agonist, Takeda has one that's in earlier stages of development. So as the space becomes more varied, how do you envision the role of WAKIX evolving?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

Look, excellent question, David. First of all, any time a new medication comes in to help patients with a rare disease, it's actually a good thing for all of us. And having additional products and investment in this market, given the low diagnosis rate, I mean, it's thought to be that over 165,000 patients are potentially living with narcolepsy in the United States, yet only 72,000 or so are diagnosed. And even less, they're actually taking treatment. So there's plenty of room to grow the market.

And despite all the available treatments to date, up until the launch of WAKIX, there's so much unmet need in this marketplace and dissatisfaction with long-established therapies that there's a lot of room.

Now that being said, we believe WAKIX is clearly differentiated even from those other products that are coming forward. It's the only product to work through histamine in the brain, inverse agonism and antagonism of the H3 receptor in the CNS. It's quite unique. It's a novel MOA. It's nonscheduled, first and only in the U.S. for that and is only the second product indicated for both EDS and cataplexy. So we're extremely pleased with the product's ability to really be, if you will, a portfolio in a product.

And that's important to understand for -- especially for those folks who are new to the Harmony story, is that we're starting in narcolepsy, but Harmony is not a sleep company. And we're not a narcolepsy company. We're a company that's focused on rare orphan neurological disorders and helping patients who are living with those diseases. We're starting in narcolepsy with WAKIX, but we're already underway, David, as you know, with plans to expand the utility of WAKIX in our label to new patient populations, including Prader-Willi syndrome and myotonic dystrophy, and we're considering additional indications beyond that as well.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. Before we get into that, and I did want to cover that in a few minutes we have left, do you think that a more crowded narcolepsy landscape with more entrants, do you think that makes for a somewhat tougher payer landscape? Or do you think that given that this is a relatively rare disease but a highly underserved treatment landscape, the payer landscape is unlikely to change dramatically in the context of more entrants?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

I'd never say that a payer landscape is easy, especially nowadays in pharmaceuticals. The payers are important partners to us, and they help us to provide access to medications to patients. That's why we believe getting the cataplexy indication, David, was so important for WAKIX. Some of those new entrants you mentioned may have an indication for cataplexy. And if we had only had an EDS indication, that might have made it a bit more challenging in the future with payers, but the fact that we secured that indication early without having to do another clinical trial now with FDA reversing its decision in our favor, that's great news for Harmony. We're very excited about it. And we think that helps to moat the business, if you will, even as new entrants come onto the marketplace.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. So let's move on to your expansion opportunities. And as you alluded to, you're looking -- you're running proof-of-concept studies in EDS associated with Prader-Willi syndrome and EDS associated with myotonic dystrophy. I wanted to start with Prader-Willi syndrome and just get your sense of how many of these -- what portion of these patients are taking stimulants or modafinil for their EDS? I mean -- or is it even addressed in these patients in any significant way these days?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

It's a good question, David. What I will say about PWS is we're confident that WAKIX has a good opportunity to succeed there with an EDS indication in Prader-Willi syndrome patients. And why is that? Those patients uniquely, there's a subset of those patients, a fairly large subset that have orexin depletion. And that's mental to patients with narcolepsy as well. So that same -- that same target, that same reason to believe that WAKIX would work on a narcolepsy patient to help with EDS is there, we believe, in PWS. And I know a lot of companies have tried valiantly but unsuccessfully for hyperphagia and other indications in that population. It's a tough population and a tough disease. Right now there's nothing indicated to help those patients in the U.S. market. But we believe because of that phenotype that's narcolepsy like in that population, that will have success. There are good odds for success. That's our intent with WAKIX there.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. And is there a lot of off-label modafinil used in these patients?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

There may be. I don't have the specific numbers for you to share today on that, but there's high levels of dissatisfaction and nothing is really working well to help patients there as far as we know. And we have a subset of patients that's using the product right now in Prader-Willi syndrome, and we had some remarkable feedback anecdotally from that patient group and patient association. They're very excited about the product and thrilled to be working with us to help bring it forward in the U.S.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Okay. And then in myotonic dystrophy, a similar question, how challenging is the EDS? How big is the subgroup? What kind of usage of legacy agents do you typically see?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

Well, there's over 150,000 patients or so believed to be living with that disease in the United States. And whether it's type 1 or 2, DM1 or DM2, so to speak, they all have a significant unmet need. And we believe, similarly, mechanistically that WAKIX has a good opportunity to succeed there with EDS. And there's a wide spectrum of patients there who could really use the help. That's a pretty big patient population within the orphan space, as you know. And that's an example of Pillar 2 of our growth strategy for the company. And that gets to a little bit, David, on business development.

And I know we have a few minutes left here together. But like I said, Harmony is not just a sleep company or a narcolepsy company. That's where we're starting, and it's a very important place to start. And those patients need us, and we believe we're making a difference in their life.

But we have a 3 pillar of growth strategy to grow our company and our business. And that first pillar is continued success with the launch of WAKIX in the U.S. for narcolepsy. I think we've demonstrated that strongly, and we'll continue to do so. Regardless of pandemic, we've shown

quarter-on-quarter growth in our ability to execute the product launch. And as the pandemic lifts, we're excited about our ability to optimize that launch, especially now with cataplexy indication on board.

And that brings me to pillar 2, which is expanding the utility of WAKIX within narcolepsy, that was adding cataplexy, and we're working on a pediatric narcolepsy indication. And then beyond to PWS, DM1 and potentially other indications, and we're looking at a myriad of indications, including IH. I know idiopathic hypersomnia is something we've been asked about. Nothing formal to disclose here, but we're looking at that among other indications. And when we're ready, we will share that. But we believe that opens up tens of thousands of additional patients in these other diseases that WAKIX could potentially help beyond general narcolepsy space.

And then third, our intention is to acquire another asset, David, or more, a company or asset. We won't be a one-product company forever. Those things are hard to predict. Timing on those, they're complex interactions, but we have an intent and a strong focus on that and a team that's dedicated to that internally here in Harmony. And we're underway with our search. And when we have something exciting to share, we will.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

Well, I guess, one follow-up on that topic is are you probably looking for an early-stage asset? Or are you looking for something that is post proof of concept? How should we think about that?

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

That's a really good question. And we're Harmony strengths. We're not a preclinical company. We're not a very shop. But our strengths are on any stage of clinical. So our clinical team is remarkably strong, and we have a good regulatory team as well. We've shown that. So any clinical-stage asset from 1 through 3 we can take, bring that through the regulatory process and then successfully launch it commercially. We could also take an in-line or commercial-ready asset as well. So anything from Phase I on, we're game. And the key is that rare orphan neuro, it fits within our current level of expertise. We want to be able to leverage the existing commercial footprint, David, the existing recent experience we have in Harmony without an exponential increase in our cost, in our expenses and in our commercial footprint. There's a lot of overlap in neurology, psychiatry with these diseases and our current call footprint that we intend to leverage. Okay.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

All right. Well, it looks like we're just about out of time, but really appreciate you taking the time to speak with us. We covered a lot of ground. So thanks, John, again, for your time, and I'll wrap up the discussion.

John Charles Jacobs - *Harmony Biosciences Holdings, Inc. - President, CEO & Director*

Thanks, David. We appreciate you very much. Thank you, everyone, for your time, and we look forward to speaking again soon. Take care.

David A. Amsellem - *Piper Sandler & Co., Research Division - MD & Senior Research Analyst*

All right. Take care.