Zevra Therapeutics, Inc. (ZVRA) - 35th Annual Roth Conference
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Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

I'm Jonathan Aschoff, Senior Biotechnology Analyst at ROTH MKM. And with me now is Richard Pascoe, the CEO – the new CEO of Zevra once KemPharm, Inc. Welcome, Richard.

Richard W. Pascoe - *Chief Executive Officer & Board Member*

Thank you, Jonathan.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

And why don't we start off talking about why the name changed?

Richard W. Pascoe - *Chief Executive Officer & Board Member*

Sure. So as Jonathan just noted, we are now Zevra Therapeutics, Z-E-V-R-A. Our ticker symbol also changed to ZVRA. A couple years ago, the Board and management embarked upon a strategic assessment of our strengths, weaknesses, sort of the journey that it brought us to where we were following our approval and subsequent licensing of AZSTARYS®, which as you know is our ADHD medication that we licensed to Corium and is now being marketed successfully in the United States.

And as we assessed our strengths and capabilities, we decided that we needed to move faster. We needed to get not only better at what we do, but to do it faster and to be able to create value for shareholders in a shorter window of time. So that led us to this strategic pivot towards rare diseases and not just rare diseases, but also commercializing those products once approved in the U.S. ourself, because we felt like by doing that, not only could we leverage the company and its strengths, we've had great success with our team in discovering and developing products, getting them approved as you know in some cases, very difficult situations, two drugs to be approved in the U.S. under the KemPharm name as well as helping our partner for AZSTARYS® get one of their products approved as well.

So we have this terrific track record and understanding and capability around that. So taking it to the next level, meaning not only focusing on rare diseases, but focusing on commercializing our products led us down this path of acquiring an asset. We can talk about that here in a moment, but we felt that the name KemPharm, which sort of harkens back to our prodrug roots, did not accurately reflect and embody who we are now as a company.

Zevra is the Greek word for zebra. Zebra is the official symbol of rare diseases. And so we felt like having Zevra Therapeutics see here on the screen was a perfect fit for who we are, who we become, and importantly who we hope to be, which is a rare disease focused company commercializing our own products in the U.S. and building on that great platform and legacy that we have with KemPharm.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

And so certainly the things that you were working on as a prodrug company, I mean that's far from dead you have KP1077 in a clinical trial for idiopathic hypersomnia. And why the pivot to rare diseases?

Richard W. Pascoe - *Chief Executive Officer & Board Member*

Well, as I said Jonathan, two reasons. One is we realized that in order to create real value for our shareholders and for patients that we needed to not only focus on disease states where we could find assets or develop assets in the case of 1077, get those through the regulatory process and get them into the market, but to also be able to commercialize those.

And if you think about the footprint required to commercialize a product in the U.S. especially it's an enormous task. Spec Pharma, Big Pharma, those are areas we want to stay away from. But rare diseases not only affords us the opportunity to help patients in areas of significant unmet need, we'll talk about arimoclomol and Niemann-Pick. You mentioned KP1077 and idiopathic hypersomnia. But those are two areas of significant need where we can field an organization in the U.S., we can generate revenue once those products are approved.

We can keep that revenue for the benefit of our company and shareholders. And moreover, create more asset value, not only with those products, but for the company in general. And so whereas before we were relying solely upon developing products that we would out license, this commercial focus that we now have is a perfect match for the rare disease category that we've selected to focus from a strategic standpoint.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

Okay. And so just to drill like a little more specifically, what are your rare disease priorities and therefore, what milestones there from should we be looking for?

Richard W. Pascoe - *Chief Executive Officer & Board Member*

Well, we are fortunate, blessed, I should say, to have two very strong pipeline candidates. The lead candidate is a drug called arimoclomol. It is an asset that we acquired through the purchase of a – asset purchase of Orphazyme, which is a company that was Denmark headquartered, had taken arimoclomol all the way through an FDA submission, and subsequently received a complete response letter, which we can share more details on that here in a moment.

Arimoclomol is for the treatment of Niemann-Pick Type C, which is an ultra-rare pediatric lysosomal storage disorder condition. It affects roughly 3,000 patients in the U.S. If you look at the incidents, there are about 300 that are identified and being treated in some fashion in the U.S., throughout U.S. and Europe, it's probably more like to 1,500 patients. So again, this is an ultra-rare condition where there are no approved therapies in the United States. So that's priority number one.

With respect to that, we intend to resubmit the NDA for arimoclomol to the FDA in the third quarter of this year. And we feel we have a very strong opportunity to not only get it approved, but as I mentioned before get it launched in 2024. Behind that, we have KP1077, this is a drug that we've developed internally. It's for the treatment of idiopathic hypersomnia. It is a prodrug of methylphenidate that we intend to take into clinical studies, including a Phase 2 study that we're currently running. We're dosing patients in the U.S. currently to determine the right dose and dose regimen, and obviously look at safety and tolerability issues as well, but importantly, to be able to inform decisions with respect to a pivotal trial that we expect to run next year.

So our immediate priorities are arimoclomol resubmission prepare for commercial launch and launch next year upon approval. 1077 complete the Phase 2 study this year. We'll have an interim look third quarter at the data final top line fourth quarter of this year and then look to push that into a Phase 3 pivotal in 2024.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

So not only has your role changed, but other people at the company, their roles have changed. Additionally, you've brought new people aboard. So how is this all collectively going to support the new rare disease thrust?

Richard W. Pascoe - *Chief Executive Officer & Board Member*

Well, I think if you look thematically at the company, it's a terrific blend of where we've come from, meaning the origins of the company and its prodrug platform. And the people that are – were part of the

company then and still part of the company today that got us to where we are, which is an enviable position, coupled with assets like arimoclomol that we brought in through business development activities.

In a similar fashion as we think about building the organization, not just the pipeline, but the organization, we have significant bench strength in areas like clinical development, regulatory affairs, CMC, and other areas that are critical for us to be successful as a commercial company. What we lacked however was commercial expertise, some business development expertise and other functional areas within the organization like medical affairs that we knew we had to create and build out.

So over the course of the last several months, we've been adding new team members in addition to some of the executive changes, we've also brought folks into medical affairs and to commercial. And as we continue to build the organization, we'll do that with the opportunity to leverage what we have and the people that we have to their fullest extent because we've got a great team to do that and add talent. We'll do it in a measured fashion, but add talent, we're needed to cover these other critical areas.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

So with respect to business development and I'm talking about in-licensing products, where do you see holes in your jigsaw puzzle. What kind of if you can be a little specific, maybe you can't, where within a rare disease portfolio do you think you want to fill some spaces?

Richard W. Pascoe - *Chief Executive Officer & Board Member*

So we put quite a bit of thought into the pipeline build, which obviously brought us to where we are today. KP1077 as you noted at the beginning was something that we had internally and we felt very strongly was a terrific product opportunity to take forward. So that was the foundation.

We were very intentional about looking for a rare disease asset to take us to that next level and to truly push the company's pipeline down that pathway, which brought us arimoclomol. Now, of course, arimoclomol is targeting a pediatric ultra rare condition. I would say, that's the bull's-eye, if you will, that we're looking at with respect to additional pipeline opportunities, whether it's lysosomal storage disorders, whether it's pediatric ultra rare diseases or whether it is some form of CNS/neurodegenerative type of condition. Those are all areas where we have expertise and experience. So that would be generally what we're looking at. I think it's fair to say as well that we also want to bring things forward that have clinical proof-of-concept and regulatory pathways that are well established, so that we have a risk profile that's acceptable to us and our shareholders.

Now, having said that, there's an external look that's going on right now, we'll continue to drive that process. But we're also looking internally as well, because we have this prodrug platform, we have this technology platform that has served us so well in the past that we can utilize that as well to identify targets and disease states in this rare disease arena that we could bring forward. So as I look at the company going forward, and what I hope to achieve is to broaden the pipeline through both avenues, both internal discovery and development, as well as external, where appropriate.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

So with regard to the whole prodrug business, which is granted it's the earlier business, do you intend on going beyond KP1077 when it comes to applying that prodrug technology to something else? Or do you – and/or do you have other prodrug chemistry that would work better with different molecules that you would intend to even [indiscernible] explore?

Richard W. Pascoe - *Chief Executive Officer & Board Member*

So if I follow the question, so SDX, 1077, of course is one of the foundational pieces of AZSTARYS® the ADHD drug, which is licensed and being sold successfully by Corium. And just as an aside, we see AZSTARYS® is being a very foundational piece of the story, right? It's revenue, it's growing revenue and

it's long-term revenue for the company. And that's always a good thing. And I think as we think about the company and its future generating revenue from various sources, variety of sources arimoclomol, KP1077, AZSTARYS® maybe other pipeline assets, that's critical. That's going to get us to a much different sort of company from a profile, from an investment thesis and from a risk perspective and we certainly hope from a value perspective as well.

Having said that, there are a myriad of opportunities that the team, Travis Mickle, our President, who's focusing on the science now and the team have that they've already worked on. And I think we'll continue to work on to extract opportunities for us to take into rare disease conditions. So I think leveraging SDX further is not likely, but being able to use what we've already generated from a science standpoint and amplifying that and now that Travis and the team have more of their time to focus on that, my hope and goal is that we can bring something forward from that process.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

And like, is that from a collection of prodrug chemistry or is it taking that same chemistry that sort of oriented chemistry and applying that to different drugs?

Richard W. Pascoe - *Chief Executive Officer & Board Member*

I think it's a little of both, but primarily the latter. And I think if you look at what the team's been able to do and having this flat technology platform, which is a proprietary platform. It certainly allows us to look at a myriad of opportunities, not just from a new chemical entity perspective, which we've been able to do and bring those forward as a pipeline asset. But the other way we want to leverage that is through lifecycle management. So as we think about the other side of the spectrum, albeit both KP1077 and arimoclomol have strong IP, they have orphan drug status. And so they'll be afforded additional exclusivity to be able to layer on top of that additional IP opportunities through that prodrug technology is something we're going to be focusing on as well.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

So to kind of go to AZSTARYS®, what can you tell investors about what to expect revenue-wise? Because that's pretty much the entire AZSTARYS® story right now in terms of not just royalties, but some sort of reasonable timelines on sales-based milestones.

Richard W. Pascoe - *Chief Executive Officer & Board Member*

Yeah, so AZSTARYS® is a – it's a critical part of our story, because not only did it validate the company and our ability to take a product, a novel product like AZSTARYS®, which is for the treatment of ADHD, get it approved and get it licensed to a strong commercial partner in Corium. So it validated us in that way, but more importantly, it represents a ongoing and long-term revenue stream for the company.

Corium has been doing a remarkable job lately. I think, if you look at prescription growth, it's been impressive, as I – we used to say, back where I come from is growing like a weed. And so with that in mind, we continue to see growth not only with quarterly royalties that are coming due to us and will continue to grow over time. But as you noted as well, Jonathan, the way that deal was structured, we pushed a number of the milestones much earlier in the process.

So when you think about a traditional licensing structure, typically milestones don't get paid out until you achieve some significant sales threshold. In the case of this license and I'm not at liberty to talk specifics, but bear in mind that, we pushed many of those milestones earlier in the sales cycle. And what that means to us and what we've guided to this year, we just filed our K last week, is that we expect one, if not two of those milestones to be delivered in 2023.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

So what should we get out of the interim data from KP1077 in IH later this year?

Richard W. Pascoe - *Chief Executive Officer & Board Member*

So as noted, KP1077 is currently in a Phase 2 study for the treatment of idiopathic hypersomnia. We have a large number of sites that are already up and running, a few more to be added. We are enrolling patients and dosing patients. That study is – it's a designed to accomplish a couple of things. One is, it's a safety and tolerability study as most Phase 2s are, gives us the opportunity to look specifically in this IH patient population at the use of the drug. So that's the primary endpoint. The study is set up into two parts. The first part is an open-label titration study, where patients are enrolled into the study. They're then optimized to a dose. There are four doses that we're looking at in this particular study. So each patient will be optimized to a dose over that – over a five-week period.

There's then a two-week withdrawal phase of the study, where patients are randomized into one of two groups. It's a two to one randomization, but they're randomized into one of two groups. They'll either receive drug or placebo, and half of those patients will receive the drug twice a day at bedtime and at night, the other in the morning at awakening. So to answer your question with that design, what we're looking to ascertain is, number one, is it safe and well tolerated in this patient population. We have no reason to believe it won't be, but we need to do that. Number two, what is the optimal dose and dosing regimen? Is it once a day? Is it twice a day? At what level are we seeing the greatest efficacy readout in this Phase 2 study?

And then importantly, we're going to be looking at established endpoints, our secondary endpoint in this study is measurements on the Epworth Sleepiness Scale, which looks at effects on sleepiness during the day, which is what affects these patients. They have excessive daytime sleepiness. They can't wake up in the morning their day, they describe as having brain fog and they don't have full control of their cognitive and executive function. So we'll also be looking at a number of secondary endpoints in an exploratory fashion, including brain fog, including sleep inertia.

So from all of that, we'll be able to check the box on safety and efficacy. We think for at least in a Phase 2 setting, we'll be able to determine the right dose and dosing regimen. And they'll also be able to get insight into what will be the appropriate endpoints, all of which will go into the design of the protocol for the Phase 3. So we're focusing on getting this drug in and out of Phase 2, learning what we need to learn as quickly as possible and getting it into a pivotal trial next year.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

Can you help us understand why developing 1077 in narcolepsy has gone from in parallel with IH to maybe later, maybe not?

Richard W. Pascoe - *Chief Executive Officer & Board Member*

Well, I think it's the development of 1077 in narcolepsy, which is another sleep disorder as our audience might know. It does require a larger effort. But to your point, we intend to file the IND with the FDA in the second quarter in narcolepsy. So we'll have the – we already have an active IND for IH. We'll have a separate IND for narcolepsy. And the goal has always been to complete the IH study first, use that apparatus, I'll call it that we've put in place to conduct the Phase 2 trial, be able to roll patients as quickly as possible into a Phase 3 and IH, and then in parallel initiate a study in narcolepsy, which again, will be a larger undertaking. It's a much larger market opportunity, but it's also a more crowded commercial space as well.

So as we're thinking about 1077 strategically, IH is where the most unmet need is. IH is where we can get in and out of the clinic and get on the market as soon as IH is an area where we can commercialize ourselves and generate meaningful revenue. It's a marketplace that there's only one approved drug now,

no stimulants are approved like 1077, Jazz's XYWAV® commence about $180,000 a year price tag. So again, these are very attractive commercial opportunities in areas of high unmet needs. So let's get into the market where there's the greatest need within the fastest possible way with a novel product. And then let's layer onto the backside of that additional indications in this case, narcolepsy.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

I'm going to ask you to be precise about this next question.

Richard W. Pascoe - *Chief Executive Officer & Board Member*

Okay.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

Exactly what do you need to do to file the arimoclomol NDA? What are any and all the gating factors because this is pretty key to your revenue?

Richard W. Pascoe - *Chief Executive Officer & Board Member*

It certainly is, yeah, and kudos to the team that was back in Florida and Denmark and other places around the world where we have folks that are working on this. So just a little history, when we acquired this asset from Orphazyme, which was the company out of Denmark, they had submitted an NDA, got rejected complete response letter. The FDA raised a couple of issues related to the primary endpoint. One was specific to the – how the endpoint was used to assess efficacy. Secondarily, there was some missing data, which is not uncommon in these ultra rare diseases. Patients don't always come in for every visit. There's pieces of data that you have to account for from a statistical perspective.

And then thirdly, and probably most importantly was a request for any confirmatory evidence because this was a single Phase 2/3 study, which if you look at the data, it generated a statistically significant result. And it demonstrated that the drug does in fact work. And one of the things I'll add is that study has continued to and look at patients over the long term just a couple weeks ago at the WORLDSymposium in Orlando, Florida, we put out an update to that study…

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

Talk about that data.

Richard W. Pascoe - *Chief Executive Officer & Board Member*

…where we saw over a four-year window the continued impact on disease progression in a positive fashion. And that's the – not only did we find and learn and we're comfortable with the idea that over time this drug can be used safely and it can be well tolerated by these young people. But importantly, you're seeing impact on disease progression. So that's a huge finding. And to your earlier question about the resubmission, it becomes a very important part of that resubmission strategy because if you dial back the clock to when Orphazyme submitted, they had the Phase 2/3 study, they didn't have any of the other information that we have at our disposal.

They didn't have access to this four-year study. They didn't have the wealth of data that we continue to collect from our early access program. There are roughly 150 patients around the world that are on this drug including the United States. And so we're able to get that data and assess that data and bring it to Bayer. Orphazyme did not do much of the work that the FDA requested with respect to biomarkers and preclinical data. And so as we think about the submission A, it is important for us and we have and continue to listen to the FDA. We've had interactions with the FDA, both formal and informally. It's been ongoing. The FDA has been very clear with what they want from us in this resubmission. I'll make it clear that they did not ask for another clinical trial that's not on the table.

But what they did want is to address the deficiencies that they found in the original submission, which we are in the process of doing. And as mentioned earlier, we have – our goal is to be able to resubmit in the third quarter of this year. Central to all of that is this confirmatory evidence. And so that has been the focus over the last few months that the team has been diving into. And with the addition of this four-year data that just came out, it further strengthens the submission and we want to make sure that gets into the document.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

So we just have a minute or two for this last question, but you are differentially well funded.

Richard W. Pascoe - *Chief Executive Officer & Board Member*

We are.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

That's nice to be. In addition to paying for whatever you intend to pay for 1077, what can you develop, build, and do for arimoclomol with what you have on it?

Richard W. Pascoe - *Chief Executive Officer & Board Member*

So what we've guided to is we have about a 100 as of the end of last year. We just put our 10-K out last week. We have about $102 million on hand that extends our runway into 2026. That includes the arimoclomol resubmission, it includes the arimoclomol commercial build and launch. And it includes 1077 program through NDA. On top of our G&A and other corporate functions that we have to pay for. So we're funded all the way through not just the immediate milestones of the company, which are submission data for arimoclomol and 1077 respectively. But through approvals, launches, and additional data and NDA submissions for the – for 1077. So it's a – I use the term we're a rare beast. We really are a rare beast. So I'm not just playing up on the Zevra title.

We have two late-stage assets that we think we can get over the finish line that are going to generate significant revenue and help a lot of people. And that's important. Number two, we're well funded. So we can move all of these things through without having to go to the capital markets. And we have revenue, not – and that's not to be overlooked between AZSTARYS® and the early access program revenue. We're in an enviable position as a company and we're very proud of that. And we're going to work very hard to continue to create more value from that.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

Wouldn't rare argue for unicorn, not Zevra.

Richard W. Pascoe - *Chief Executive Officer & Board Member*

Well, maybe, but that was a little too obvious. We thought Zevra was a little more clever and it's certainly ties back to the rare disease symbol, which is the zebra so there we go.

Jonathan Aschoff - *Analyst, ROTH Capital Partners, LLC*

Thank you very much.

Richard W. Pascoe - *Chief Executive Officer & Board Member*

Jonathan, thank you. Appreciate it.