enGene

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Etzer Darout: Great. Thanks, everyone. My name is Etzer Darout, Senior Biotech Analyst at Barclays. It's my pleasure to have enGene with us this morning, CEO Ron Cooper. Ron, maybe if you can just give us an introduction of enGene for those that are less familiar with the story, and then we'd take it from there?

Ron Cooper: enGene is a unique company with unique technology at actually a really unique time. Our technology is called the DDX Platform. It's a proprietary sugar platform that we developed ourselves over many years. The lead asset from our platform is a product called Detalimogene, which we're developing for non-muscle invasive bladder cancer. And we are right in the middle of an open-label pivotal study, and so it's an exciting time.

Detalimogene itself is a non-viral gene therapy, so that confers a lot of benefits. Very unique. And this year is a pretty exciting year for us in that first off, first up in Q2 this year, in the spring of this year, we will be providing -- we plan a data update at an upcoming scientific conference. In the second half of this year, we plan to share the long-term data, 12-month data, for our pivotal study which is fully enrolled. We'll have the final results of our pivotal study. We have a planned filing of the BLA, and we expect an approval in 2027.

You wrap that up with a company that has cash that's over $300 million, we have cash in the second part of 2028, that's why I said it's a unique company, unique technology at a very unique time.

Etzer Darout: Awesome. Great. Thank you for that. Based on our conversation with KOLs, non-muscle invasive bladder cancer feels potentially larger I think than I think the market assumes. Certainly, higher unmet need. Maybe if you can, comment on the disconnect if you will between how the market views this opportunity and how key opinion leaders are talking about it.

Ron Cooper: Yeah, this is a market that's undergoing a lot of transformation. The first misnomer is that people are very focused in on breast and lung as big cancers. But first of all, bladder cancers is number 6. It's a top 10 cancer, so it's pretty large. And then NMIBC within that, the prevalence is around 740,000 in the U.S., so that's quite large as well.

What people are not quite understanding where the transformation that is occurring is, when you think about NMIBC, traditionally, if you were diagnosed with that, the only

real treatments that you had were BCG and removal of the bladder. And the urologist said, oh, you have cancer. If you fail BCG, we're going to take your bladder out.

Increasingly though, it's understood that NMIBC is a slow progressing disease. Progression is about 20% over 10 years, and maybe even lower than that. Now all of a sudden, the treatment paradigm is changing. But with this slow progression, there were no treatments. And so now there are a number of new treatments that have been approved and that are in the pipeline to be approved.

What's going to fundamentally change is products are going to be sequenced after each other. Because these products, they kind of wear out a year later. There are recurrence rates anywhere from 60% to 80%, so quite significant. They will be sequenced. The patients don't progress, but they recur.

The analogy that I would use for this is a little bit like the multiple myeloma market. When Revlimid was launched, it was a $1 billion market. You add a dozen plus new products, it's a $20 billion plus market as product are going to be sequenced. I think the same thing is going to happen here. The bladder cancer market is about a $2 billion market. It's projected to be a $20 billion plus because of the reasons I just talked about.

Etzer Darout: Right. And sort of with that, can you just highlight -- obviously, it's sort of a space where competition is growing. If you can, highlight Detalimogene's differentiation relative to what we see from other molecules. And maybe also describe the mechanism of action and how that relates to the differentiation here.

Ron Cooper: First of all, as I said, we're the only non-viral gene therapy. By itself, it is super differentiated and super unique, and that confers a lot of interesting benefits. I think the way to think about that first of all is, let's flip it around from the customer perspective. The majority of the patients are with community urologists. When you ask community urologists what do they want, what do they need -- obviously, they need products that have efficacy. They need products that are highly tolerable. Because these patients are generally, and this is underappreciated, are 75 to 80-year-old smoking men with comorbidities. It's tough to get them on treatments. They don't really want to bother with this sort of slow cancer, so tolerability is important.

But the third thing that's important are some of the non-clinical benefits. How easy it slides into their practice. Does it consume resources? When you think of Detalimogene, it's probably the only product that delivers all 3. It delivers -- our preliminary data shows that we have a CR rate that's very competitive, good efficacy. We have probably best-of-class tolerability, very low discontinuations, very low treatment interruptions.

But in particular, community urologists, because we have a non-viral approach, our product can be stored in a regular freezer for many years. In a regular fridge, for many months. There's no special handling. If you compare this to some of the other products that require special handling, that require pre-washes, that require decontamination of rooms, that require hoods, that require procedure rooms, we don't have any of those things. Detalimogene is a really unique product because it provides the efficacy the doctors want, probably best-in-class tolerability, most of the patients don't discontinue. Most of the patients don't have treatment interruptions. And probably best-in-class handling and ease of sliding into those practices.

Etzer Darout: And with that, when you think about your go-to-market strategy and how you plan to reach those community docs, is there any education that needs to go on, given that it's a slow progressing disease? How are you thinking about that?

Ron Cooper: Well, I think first of all, from a go-to-market strategy, we think about geographies first. And in the U.S., we believe that we'll need 40 to 60 sales representatives, which is very manageable for a company like ours, so we're ready to go. In Europe, our go-to-market strategy, we're still evaluating. I and a lot of other members of our team have a lot of experience in Europe, and that will be a financial decision whether we go on our own or whether we partner. And the rest of the world, we'll partner.

Then, within the U.S. itself, how do we think about this? Well, within these, community practices are often organized in what are called LUGPAs which are a collection of practices. And within a LUGPA, there are usually a handful of doctors that are really focused in on bladder cancer. We will be targeting them. We will also have to really think differently about our targeting from how the practices work. And I'll tell you why. Because in the U.S., the number one specialty that does buy and bill is oncology. Number one.

The second one is urology. This is a big source of revenue for their practices. But when you segment the practices, some practices are very sophisticated in what they do and they do a lot of buy and bill. Some practices would really like to do that, but right now, the technologies that have been -- the first generation products are just clunky for community urologists. And Detalimogene is a next gen product, so it's really going to slide in well there.

And then there's a third group that really haven't explored buy and bill and do a lot of referring. We will be first of all targeting the first two groups. And we think we can really activate the community urologists because we have a next gen product that really fits their needs, as I talked about earlier.

Etzer Darout: Great. And then maybe with the registration as a study, you first announced data in 2024 for LEGEND and the amended protocol for the study. Can you talk about maybe a little bit more around that amendment, what it entailed, and sort of walk us through now and then the next data update and what we can expect from that?

Ron Cooper: Yes, so the journey for us in our pivotal cohorts, the pivotal study is the LEGEND program. Cohort 1 is the pivotal cohort. When it was designed, it was designed by a CMO who was an oncologist. And we just realized we were going to be a urology company, better have some urologists here. And the team figured that out, which is terrific. But urologists were like, okay, why are you making this harder on yourselves? You're not even applying standard of care for these patients. We had some good data, initial data in 2024, but then we made 3 pretty significant protocol changes that brought us in line with standard of care.

The first one being is, before patients even got into the study, the T1 patients, the T1 patients are of higher risk. Standard of care is to resect their T1, that lesion, and then do it a second time. And we weren't doing the second one. Now, they all have to get that second resection.

The second thing is, as patients got to 3 months, if you had a little Ta growth, a papillary growth, we said you're out of the study. And the doctor said, it's just a little growth, there's nothing happening with CIS, we just cut it out. Well, that's standard of care, so now we allow them to do that.

And the third thing that we do is, we ask for biopsies before patients come on. Before, doctors would look and say, yeah, it looks like it's progressing. But sometimes with

immunotherapies, the bladder gets red so it's a much more objective or subjective approach. You take that and you wrap it around the fact that our, again, with a different approach of doing the study, we brought in a very strong clinical operations team that if you take those protocol changes mixed with better execution, better follow-up, there you go.

And the results that we shared late last year are really compelling and much better frankly. And then what we're expecting is a data update at a major congress in the spring of this year. That's planned. And there, what we hope to do is really focus in on the primary endpoint, which is complete response at any time, so it's 3 and 6 months for us. That will be locked in because we have about 125 patients, and most of those patients would have reached their 6-month timeframe, so it's a pretty exciting time.

Etzer Darout: Good. And I guess the obligatory question these days is alignment with the FDA and the protocol change. And I guess as well is, how are you kind of thinking about the pivotal readout and whether or not, again, the FDA, you and the FDA are aligned with that. And again, also how does that compare to peers if you will in terms of their pivotals?

Ron Cooper: Well, the FDA has been terrific with us. Now remember, when you think of the FDA, very rarely do they have guidance in there. They recognize the need in NMIBC. The guidance thread they originally put out on 2018 and then reiterated draft guidance in 2024 said if you do 100 patients or so open label, you'll get a complete approval. And what's nice is that a number of products even recently have got that. It's pretty rare in all of the things that they do, and I call that rock solid.

We've had a good dialogue with the FDA, and the FDA has provided us a number of important designations. We have FastTrack, we have RMAT, we have CDRP, and I don't think they give those to companies unless they are serious about working with us to bring this product across the line. We've had very constructive dialogue with them. We've shared our preliminary data with them which has resulted in some of these special designations. I think we feel pretty good about being on track for working through the filing in the second half of this year.

Etzer Darout: Great. And how does the protocol compare to other programs, some BOND programs, SunRISe? How does the protocol -- how do you compare to those?

Ron Cooper: Well, I think I'm always -- I always caution people to make comparisons. First of all, we do not have access to their protocols, so we don't know for a fact. But from their publicly disclosed information, what you see is many of the changes that we've done seem to be similarly done with them. But I always caution people to compare against these particular -- I know it's human nature to compare, but first of all, the patient population, including criteria, patient populations are very heterogeneous. NMIBC patients are very different. I believe the protocols are different.

And then in these studies of 100 patients or less, you can see a lot of swings. I believe that we're roughly aligned with others. But again, I would just caution individuals to make comparisons between the studies. I mean, even though it's human nature, because there are different patient populations often and often different protocols.

Etzer Darout: Great. Yeah, and then maybe another change if you could comment on, is there a meaningful difference, or maybe the reason driving the change from complete response at 12 months to sort of complete response at any time? Like, what's -- is there a meaningful difference there? And then maybe what was the motivating factor for that change?

Ron Cooper: That is just sort of standard discussion. We started in a particular area, we started to talk to the FDA. And the FDA said, look, this is the primary endpoint for all the other products that we've approved, so they would expect us to align. Primary endpoint is CR anytime, and the key secondary endpoint is duration of response.

Etzer Darout: Okay. And how, with the potential for sequencing of these different therapies, I guess how do you see the NMIBC space evolving over time? And where do you think your program fits into that paradigm?

Ron Cooper: Well, again, I believe this is a real transformation that's going to occur. First of all, you have to think about the customer groups. You think about, where are the patients? Over 80% of the patients are in the community. About 17% from our market research are in the academic centers. And the academic centers, these are places where they remove bladders and they're really focused on efficacy and they have the resources to use some of these new technologies. Some of the newer products that are approved and the products that have come along actually sit very well there.

But these new next gen products where we lead with Detalimogene are actually ideal for the community. And right now, most of the new products just don't work very well for the community flow. What we see is, in the community, that patients will be diagnosed because that's where the patients are. Well, they'll start with BCG if they can get them.

Then a non-viral approach like Detalimogene, because it's just so easy in their office. We'll have a product sitting in the fridge. Literally, anyone in the practice can take it out of the fridge, mix it with water, regular catheter, instill it into the patient, 5 or 10 minutes they're out. That's a lot simpler than a lot of the other technologies. We believe there's going to be a transformation with these next gen products, and some of these other newer products will continue to be used in the smaller specialty area.

Etzer Darout: Got it. And for Detalimogene, do you think about opportunities for the program beyond BCG unresponsive NMIBC with CIS? Where else can this program go?

Ron Cooper: I think what's really exciting about our, first of all, our platform, is our platform can be applied in many areas. Our technology can be applied in many areas. Detalimogene specifically, we've already started a couple of additional cohorts in patients that are naive to BCG, those that have been exposed to BCG, and those that have papillary only and don't have CIS. Those cohorts are ongoing.

And we're also looking at some of the other areas as well. Because one of the nice things about our product is that being a non-viral gene therapy, it's the only one. It can be -- being a non-viral product and not being an LNP, the cost of goods in manufacturing this is actually relatively low. That allows you to go to a wider range of diseases. And because the handling is a lot easier as well, it also allows you to go to a wider range. The potential for Detalimogene outside of its first indication is pretty significant.

Etzer Darout: Right. And you just talked about manufacturing. You're participating in the CDRP pilot program. Maybe you can describe what that is. And then also, again, as you kind of talked about before, the differentiation of Detalimogene from just historical NMIBC programs that have sometimes struggled with manufacturing.

Ron Cooper: Yeah, really a great question. First of all, we're very honored that the FDA granted us CDRP. It's a pilot program, one of 9 companies. It's given on the basis of great innovation but also clinical data and clinical need. And to your point, when you think about in the old days, complete response letters were for clinical reasons, now they're mostly for

manufacturing. And it's been more so in this category.

Effectively what this means though, rather than taking Module 3, the CMC module, and kind of throwing it over the fence when we file, we actually start engaging in an early dialogue with the FDA. We open the kimono and we say, here's what we're planning. They give us feedback, more of this, a little less of that, etc., etc. And that will significantly reduce the risk of CRLs.

Now, we always felt because A), we've been manufacturing at scale, we've pretty much finished our PPQ batches, our validation batches, so we'll be writing Module 3 very soon. And because our product takes readily available ingredients and there's no -- it's not a virus, it's not an LMP, we lined up with a product that has relatively low cost of goods. And because it's not a virus, the handling is a lot easier as well. It makes it very competitive. I think CDRP is a real help for us and certainly reduces any risk that we might have on the manufacturing front.

Etzer Darout: Great. And what type of feedback have you gotten from key opinion leaders in bladder cancer around Detalimogene, as well as how it compares to competitors?

Ron Cooper: Yeah, well first off, I actually never consider them to be competitors. These are complementary medicines. And the reason I say that is, if there was a product that had a 12-month complete response rate of 80% to 90%, that would be a competitor. That would be the number one product that's being used. But most of these products have an anytime CR rate around 2/3, somewhere in that range, and the products have a 12-month rate of 20% to 40%. Which means patients are going to recur. All these products are going to be used, they're just going to be used in different places and in different sequence.

We just believe that when you think about the needs of the community urologists, the first-gen products they're not really using very much, because they just don't fit their needs. They're just too clunky to use. These next gen products where Detalimogene is leading, I think really fits to where the patients are and it's going to transform, be able to start the transformation of this marketplace.

Etzer Darout: Yeah, right. And then obviously, for your upcoming data disclosures, if you can just talk through what good looks like in terms of benchmarks or hurdle rates. When you think about what is a good dataset, what is not a good dataset as you think about these upcoming disclosures?

Ron Cooper: Well, these are experiments, so you never know exactly where you're going to land up. But I think in our spring conference, if we -- that's where we'll be focusing on the CR any because most of the 125 patients would have hit the 6-month timeframe. And if you look at the range of products in that, they range from sort of 50 to 60, 70 range of CR anytime. Let's see where we land up. We had a CR rate of 60 anytime, 63 in the preliminary. If we're within that range, I think that sounds good to me.

And then in the second part of the year, we expect to have the 125 patients, the majority of them, would have reached the 12-month timeframe. And again, it's an experiment, and the range there is 20 to 40. We think we're going to be competitive on both ends. I think that we feel pretty confident, this being an immunotherapy, in our durability as well.

Etzer Darout: Great. I was assuming beyond the CR, obviously sometimes you kind of have trade-offs in efficacy, safety, and convenience as well as just think about other parts of this dataset beyond CRs is that you're going to be looking at and trying to educate investors about?

Ron Cooper: Yeah, that's a very good point. I almost skipped over probably one of the most important things is the tolerability. Again, think of these patients, 75 to 80-year-old smoking, comorbid men. They don't want to have a hassle. And if you look in our clinical trials thus far, we have discontinuation rate in the very low single digits. We have treatment interruption rates very low, where other products are in the 40s and above. The tolerability for us is going to be very competitive. And that's going to be important.

And I think what Wall Street is missing is that particularly for where the patients are in the community, these newer products are just too difficult for them to use. To get into their practice flow costs them too much money and too much resource. Our product is going to differentiate so nicely, it's going to just slide right into their practices. In fact, it's going to be easier than what they've been using in BCG, so that's going to be a big benefit.

Etzer Darout: Yeah. And it looks like we are up on our time, but Ron, thank you so much for your time today. Thank you to our listeners and we'll be back soon with our next session.

Ron Cooper: Great. I appreciate the opportunity. Thank you.