



**Matinas BioPharma  
Corporate Update Conference Call and Webcast  
March 19, 2018**



**Operator:** Greetings and welcome to Matinas BioPharma Corporate Update Conference Call. At this time, all participants are in a listen-only mode. A brief question-and-answer session will follow the formal presentation. If anyone should require operator assistance during the conference, please press star, zero on your telephone keypad. As a reminder, this conference is being recorded.

I would now like to turn the conference over to Jenene Thomas with Investor Relations. Please go ahead, Ms. Thomas.

**Ms. Jenene Thomas:** Good morning, everyone. Thank you for joining us this morning for the Matinas BioPharma Corporate Update Conference Call and Webcast. Today's webcast will be accompanied by a slide presentation that can be found under the Investors section of the company's website, [www.matinasbiopharma.com](http://www.matinasbiopharma.com), under [Events](#) and Presentations.

At this time, I would like to remind our listeners that remarks made during this call may state management's intentions, hopes, beliefs, expectations or projections of the future. These are forward-looking statements that involve risks and uncertainties. Forward-looking statements on this call are made pursuant to the Safe Harbor provisions of the federal securities laws. These forward-looking statements are based on Matinas BioPharma's current expectations, and actual results could differ materially. As a result, you should not place undue reliance on any forward-looking statements.

Some of the factors that could cause actual results to differ materially from those contemplated by such forward-looking statements are discussed in the periodic reports Matinas BioPharma files with the Securities and Exchange Commission. These documents are available in the Investors section of the company's website and on the Securities and Exchange Commission's website. We encourage you to review these documents carefully.

Following the Company's prepared remarks, the call will be opened up for a question-and-answer session.

Joining me on the call today are Jerry Jabbour, Chief Executive Officer, Dr. Raphael Mannino, Chief Scientific Officer, and Dr. Matthew Wikler, an infectious disease clinician and member of Matinas' Board of Directors.

It is now my pleasure to turn the call over to Jerry Jabbour.

**Mr. Jerome Jabbour:** Thank you, Jenene.



Good morning, everyone. Some days just feel different than others, and today is a great day. Today marks a new chapter in the history of Matinas, a history that's seen us transform from a lipid-based omega-3 drug development company to a clinical-stage drug development company in the anti-infective space built on the foundation of a proprietary and potentially disruptive drug delivery platform technology.

2017 saw us successfully uplist this company to the New York Stock Exchange and reveal positive data in patients with our lead antifungal drug, MAT2203. It also saw us move into a state-of-the-art formulation development and manufacturing facility here in New Jersey and make exciting advancements on our cochleate delivery technology platform.

Now, with significant momentum at our backs and equipped with the invaluable feedback, guidance and support from the FDA on MAT2203, we stand poised to drive the Company forward into yet another stage of growth and value creation with more than an eye towards transforming ourselves yet again into a drug delivery platform company, focused on our own innovative products and supported with thoughtful and strategic collaborations in cutting-edge areas of science and medicine.

Our mission for this update call today is clear. We would like to provide clarity and convey confidence with respect to our path forward with MAT2203 following our FDA interaction in January while highlighting some of the recent developments we have made on our cochleate platform delivery technology, which we believe position us to capitalize on its broad applicability and potentially collaborate with well-respected, successful companies in a meaningful way.

However, before we turn to these updates, I would first like to take the opportunity to convey our gratitude to my Co-Founder and friend, Roelof Rongen, for all of his contributions to Matinas since we founded this company together in 2012. Roelof's passing of the proverbial baton is symbolic of our growth as a company and readiness to begin our next phase of value creation. As an organization, we share his pride in all that we have accomplished together to date and stand determined to capitalize upon our strong foundation to take the Company to new heights. I know that I am ready to lead this Company, and I have a precise vision of what needs to happen to take Matinas to the next level.

Companies evolve and change, and the process of transition is an important one. Change brings opportunity, but in our case, we stand to significantly benefit from continuity and my intimate familiarity with our team, our technology and our current strategic objectives.

And so, we start on Slide 4 with a clear two-part strategy in the near-term with the goal of generating significant value in our products and in our technology platform. For our lead



product, we will take full advantage of the feedback and acknowledgments provided by FDA, combined with customary drug development work, to position this important drug to enter a single Phase 2 pivotal trial as quickly as possible.

We believe the strategy that we will lay out for you on this call, has the potential to significantly reduce the risk, timeline and cost for the development of MAT2203. Most importantly, we believe it will lead to a potentially accelerated approval for the prevention of invasive fungal infections, or IFIs, in patients suffering from Acute Lymphoblastic Leukemia, or ALL, who are rendered immunocompromised by the therapies related to that condition.

In parallel with driving MAT2203 forward toward approval, we will take advantage of the breadth of the applicability for our platform to forge and cultivate meaningful strategic collaborations with reputable and well-known companies. These discussions are ongoing, and we look forward to being in position to share more with you in the coming weeks and months.

Later in the call, we will address some of what we believe are the differentiating characteristics of our cochleate delivery platform versus other existing drug delivery technologies and why companies are so interested in learning more and exploring ways to work together.

As with any great organization, people make the difference, and we are fortunate here at Matinas to have a talented and experienced team. One of the things we have tried to do over the last year to compliment our internal talent is to identify and attract experienced and well-regarded individuals to serve on our Board of Directors. Notably, on January 1st of this year, we officially added Dr. Matthew Wikler to our Board of Directors. Matt has a very special and distinguished track record of developing products, both clinically and from a regulatory perspective, for over 35 years. An infectious disease clinician by training, Matt is the former Deputy Director of the Division of Anti-Infective Drugs at FDA, the very division we interact with for our lead product candidate. In addition, during his time in prior industry at companies like The Medicines Company and Peninsula Pharmaceuticals, which was acquired by Johnson & Johnson, Matt has gotten 20 anti-infective drugs approved on the first try.

Since joining our Board, Matt has been instrumental in helping us to prepare for our first FDA interaction and then taking the learnings from that meeting and assisting us in designing an appropriate and thoughtful development strategy for MAT2203 to de-risk and streamline that program and position it for approval in a significant area of unmet medical need. I have asked Matt to join the call today to provide his perspectives on our technology and our lead drug and to outline for you our development program moving forward for MAT2203.

At this point, I will turn the call over to Matt.



**Dr. Matt Wikler:** Thank you, Jerry.

I thought it would be interesting to provide you with some perspectives that I had about Matinas and why I decided to join the Board of Directors of this Company.

First of all, I was quite impressed with the drug delivery technology, which I feel has some clinically important advantages. First of all, the drug is delivered through this technology specifically to target areas required for effect. Therefore, the drugs with small margins between the effect and safety can get needed concentrations where the drug is needed for effect while decreasing the concentrations in the plasma and other tissues, which could result in decreased toxicity or adverse events.

Also, the ability to deliver drugs through an oral route - many drugs, as you know, cannot currently be delivered orally and creates a great inconvenience and added cost to the healthcare system. And clearly, this could be a true benefit to patients in many clinical situations.

I was also impressed with MAT2203, which as you know, is encochleated amphotericin B. And once again, I think this meets an unmet medical need. Amphotericin is the gold standard product for the treatment of fungal infections. However, it's one of those products with a very narrow margin between efficacy and safety. And as discussed previously, it appears that MAT2203 allows for delivery of the amphotericin in affected doses to the relevant tissues with decreased plasma concentrations. And as a result of this, we believe this has potential to result in fewer toxicities.

Also, MAT2203 can meet a specific unmet medical need in preventing invasive fungal infections in patients with Acute Lymphoblastic Leukemia. Due to the various drug/drug interactions between other anti-fungal agents, amphotericin B is really their only option. However, these patients frequently require amphotericin for up to 90 days, and therefore, an oral formulation would be a true advance.

Also, I was impressed with what has been demonstrated to date with MAT2203. Clearly in animal studies, it's been demonstrated in proof-of-concept studies to both treat and prevent fungal infections as well as the fact there were no signals of any safety or toxicity problems.

So, for all these reasons, I felt this was a very exciting technology, something that can have some real clinical benefit to patients, and therefore decided that I'd be pleased to join the Board of Directors for Matinas.



Let me talk a bit about our FDA meeting on Slide 6. So we had an FDA meeting in January of 2018, specifically to discuss the development--the development plan for MAT2203. And this was a typical end-of-Phase 1 meeting. And at these meetings, one generally reviews the initial animal and human safety and pharmacokinetic data, gains the perspective of the FDA on how they interpret that data, and then gain perspectives and discuss how best to proceed the development program to ultimately lead to approval for the indication being sought.

At that meeting, the FDA acknowledged that efficacy was demonstrated in animals, and there was no evidence of toxicity or safety signals in the studies conducted to date. And were very productive in collaborative discussions on how to move forward with the next stages of development. And therefore we--we're moving forward with the clinical development plan and animal plans as noted starting on the next slide.

So, the next logical step the one takes whenever they're developing a drug, once they know that a drug is let--you know, works in animals, we understand some of the pharmacokinetic properties and we understand the safety profile of a drug, is to say what is the optimal dose for the treatment of a specific disease. And therefore, just like any normal drug development plan, our next step is going to be to determine what the optimal dose is in order to appropriately develop the drug and optimize the likelihood for success and to de-risk the product for the future.

So, our stage one, there'll be more robust animal studies that will replicate the specific types of patients that are going to receive MAT2203. And this data will be utilized in stage three, which I'll discuss in a moment, which will ultimately determine the optimal dose to be used in our follow-on studies.

In stage two, one utilizes the human PK data that's already been generated and based upon that data, pharmacokinetic and pharmacodynamic model will be created. And in stage three, one takes the model that was developed in stage two and incorporates the data from the stage one animal studies. And based upon all that data one can do various simulations, Monte Carlo simulations and evaluations, and determine what the optimal dose is. And therefore, we believe that--and this has been done many times before, that one therefore can get the optimal dose.

The folks who are doing these studies are the top experts--considered the top experts in the U.S. in fungal infections studies in animals and also the top organization that does pharmacokinetic and pharmacodynamic modeling in the United States for infections disease products. And actually, they're frequently they're called upon by the FDA to help them review issues and problems to help them understand pharmacokinetics and dynamics of these various products.



Of interest, companies who have developed drugs without going through this process have frequently failed because they've selected the wrong dose. And therefore, as I said, this is the next logical step and will help us to de-risk the asset and maximize our opportunity for success.

So, once we have the optimized dose, we'll then go into our Phase 2 pivotal studies, which will be adaptive design, and that's on your next slide. And the FDA and specifically this division understands that when you're dealing with these types of infections and for products that are being used for underserved populations, that--that it's important to try to find ways to get these products approved, realizing it's difficult to do some of the larger studies, that have been done in the past for drug approvals.

So, therefore, for these types of situations, the FDA has been more flexible in its approach. It just so happens that, as I said, the ALL patients are a limited population. They do not have a good practical alternative for the prevention of fungal infections due to the fact that there are these drug interactions with the other drugs we'll be receiving. And so, this drug and this use would fit into one of those situations. And based upon that, we believe that a single Phase 2 trial with adaptive design should meet the needs of the FDA to lead to a limited indication use for the prevention of invasive fungal infections in patients with ALL.

Our Phase 2 adaptive design pivotal trial will have two phases. The first phase will be a standard PK/PD and safety study in approximately 30 patients with either AML or ALL. And the purpose of that part of the study is specifically to get more PK/PD data at the optimal dose and to confirm that in the patient population, we're seeing the expected results based upon the modeling work that was done as well as confirming the safety profile is as we expect from our previous experiences with the product.

And then, once we have the results from that, we would then immediately go into our stage two, and our stage two would be an efficacy study of MAT2203 versus placebo and once again, we'd look at the PK/PD, the efficacy and the safety of the product. And the goal of this portion of the study is to statistically demonstrate superiority over placebo. And the placebo is being used, because as I mentioned before, there are no clear alternative therapies for these patients for this use.

There are some potential advantages to doing placebo-controlled trials versus non-inferiority trials. Frequently, the patient--the numbers of patients required are less. Non-inferiority studies obviously can take many more patients. And also, since there is no other drug to compare against, obviously, would want to do a placebo-controlled trial because we believe we can show a true benefit for MAT2203.



For this study, we're going to be utilizing a Monitoring Board, and they're going to be reviewing both safety and efficacy. And this is important, obviously, to look at safety of any drug, but in this particular case, it's also very important because they will also be looking at differences between the two arms in the study. And if they start seeing differences, they will determine whether or not those differences are statistical or not, and if they are, may make the decision that the study should be stopped prematurely because there are statistical differences, and it would be unethical to continue a study past that point and expose them to placebo.

Also, it provides us flexibility in the sizing of the study. So, let's say, we've--we're getting close to enrolling the number of patients we anticipated, we're seeing the kinds of differences we were hoping for, but it looks as if in order to reach statistical difference, you're going to have to enroll say another 20 or 30 patients. This way, you get that information so you don't prematurely stop a study that will ultimately result in showing statistical differences, but just missing that could because you haven't enrolled enough patients.

So, I think you can see that we've developed a logical plan to move MAT2203 forward in an efficient manner, which I believe optimizes the program for potential success and approval for the product in a limited indication of prevention of invasive fungal infections in patients with Acute Lymphoblastic Leukemia. And based upon my experience, I feel this is a logical next step and development plan for this product.

I'll turn it over to you now, Jerry.

**Mr. Jerome Jabbour:** Thank you, Matt.

Another extremely important element to our overall strategy and kind of a precursor to the work outlined by Dr. Wikler, is the formulation optimization that we're working to complete on MAT2203. We believe that by successfully improving MAT2203 in the ways that Dr. Raphael Mannino is going to describe in a few minutes, we're positioning MAT2203 to meet its objectives in the clinic and to become then a commercially successful drug, if and when approved by FDA.

Beyond that, I have also asked Dr. Mannino to provide some additional detail around our platform technology and some of the initiatives that we've taken to demonstrate the sort of data that will position our technology to potentially be widely adapted in exciting in cutting-edge areas of medicine and science.

So now, I'll turn it over to Raphael.





**Dr. Raphael Mannino:** Thank you, Jerry, and thank you, Dr. Wikler. That was a very, very nice description and summary of the clinical development program that we have in mind for the development of MAT2203.

And to reinforce what Jerry just said, consistent with what Dr. Wikler described, we have been in the process right now of taking our formulation, which has shown very good low levels of toxicity and efficacy, and moving it now toward a formulation which can be positioned to be in a direction of being a viable and very robust patient-friendly commercial product so that when we finish the trials that Dr. Wikler has just described, we have the product already online ready to go. In order to do that, we're taking the current product that we have, and we're making it more concentrated so there'll be a lower volume for the patients to take, and we're making it a much more tasty formulation with adding flavors and flavor enhancers and other types of controlling substances so that the material that the patients will take will be a much more patient-friendly material that they will be actually interested in taking, which will then help us with compliance because one of the problems with the amphotericin, especially the IV formulations, in previous studies has been patient compliance.

So, we're moving our formulation to be a formulation which not only is effective, orally bioavailable and low toxicity, but also one that will be something that will be very, very patient-friendly in order to enhance compliance.

We've already moved in that direction in terms of developing the processes for concentrating. We have batches that we have made, both in small-scale and large-scale. And over the next several months, we're going to be able to demonstrate how these formulations are working. We already know they work. We're going to be scaling them up, making GMP manufacturing processes already.

So, I think, we have a very, very robust plan both on the formulation side and on the clinical development side to move MAT2203 into this much more focused clinical development program.

One of the other things, though, that we'd like to talk about about the cochleate technology is the broad aspect, potential aspect of the platform. It's--if we can go to the next slide please. We've talked to you before about the aspects of cochleate technology and its ability not only to be delivering material parenterally and on mucosal surfaces, but also to be able to deliver drugs which cannot be currently given orally to make these orally bioavailable and efficacious formulations.

There are two very, very unique aspects to the cochleate platform that we need to continue to focus on. Besides the oral bioavailability properties of cochleates, the ability of a cochleate to



deliver a drug into the interior of a cell is critical to especially many of the newly being developed technologies in the biotechnology world.

In order to get things across the membrane of a cell, it's important that a drug, especially a drug like an oligonucleotide or an aminoglycoside, which is water soluble and highly charged, can get across the membrane. Other particle formulation delivery technologies accomplish this usually by destroying aspects of the membrane. When they do that, they cause damage to the membrane, and this can result both in not only damage to the cell but in toxicity in the animal when these things are used.

Over the years, we've been able to demonstrate both in animal models and in our human studies that cochleates have the ability to deliver material into the interior of cells without doing damage to the cell membrane. When we deliver oligonucleotides, for example, into cells in cell culture, 100% of the cells remain alive, and that's in great contrast to other oligonucleotide delivery systems where 50 to 60% of the cells may die in those studies.

So, we now have the unique advantage of being able to take material and deliver it to the interior of a cell. Other aspects of the cochleate technology, which we've talked about before, are--is their ability to not only deliver the drug, but prior to that, to be able to formulate the drug into this multilayered anhydrous crystal, which provides much greater shelf-life stability. We've been able to take very unstable drugs and put them into cochleates - for example, oligonucleotides - and show that they are very, very stable over time.

So, we now can have a product which is made of natural material, phospholipid and calcium, which we develop manufacturing protocols for, which can be scaled up in a reproducible manner, which is another problem for nanotechnology products is reproducible particle size in large-scale. The product inside the cochleate is now highly stable. You can give these formulations orally. And when they interact with the cell, they can deliver the material into the interior of a cell without causing cell damage or cell death, and therefore minimizing toxicity.

So, how do we believe we can develop this in a very focused way? Well, the area of the delivery of oligonucleotides, both in terms of siRNAs, in terms of DNA plasmids and the ability to deliver Messenger RNAs for expression of proteins on the interior of cells, is a clear area where, not only do we have potential, but we also have experience. We've been able to demonstrate in the past the ability to formulate siRNAs, DNA plasmids, and more recently, Messenger RNAs into cochleates at high efficiency and with imparting great stability to the oligonucleotide. We've been able to get close--greater than 90% encochleation efficiency in some of these studies with very long shelf-life stability.



Once again, when you take these formulations and you put them into culture cells, we can show not only delivery, but efficacy. We've also used both siRNA formulations and DNA plasmids in animal models, and we've been able to show expression, lack of toxicity and good efficacy. Once again, I'd like to say we can do these in the absence of toxicity to the cells.

In terms of our manufacturing capabilities, this is another advantage, which Matinas over the last year has made great progress in. We have the ability in our facility, our very modern facility, to not only make new formulations, but to scale them up to demonstrate stability and efficacy, to go to the commercial level production, to do the analytical work, to make the clinical trial material, and to put it out into both animal and human studies. We do this all in the one facility, and then that way, we have control of our fate. We can decide--we can make the process work. We are the experts on the cochleate technology, and we have the ability to go from formulation development to clinical trial material that is now in the clinic.

So, I believe with the team we have now and the resources, in terms of facilities that we have now and the progress we've made thus far, we have a very, very strong opportunity to develop both MAT2203 and develop the platform into other directions.

Great. Jerry, thank you very much. Jerry?

**Mr. Jerome Jabbour:** Thank you, Dr. Mannino. It's clear that you have an infectious enthusiasm for cochleate delivery. And, you know--but, let me be clear about one thing, because it's really nice to talk about coming attractions, and we think the science behind the applicability of the cochleate is very strong, and it's that sort of not only enthusiasm, but the data we've been able to generate, which indicates that this has the potential to be a true platform technology and something that can grow into other areas of science.

But, let me be clear that our focus in the short-term is on the development of MAT2203. The value associated with pushing this program forward into the area of unmet medical need of prevention of invasive fungal infections is meaningful, it's impactful, and the streamlined development program that Dr. Wikler has outlined for us, will put us in position to capitalize on that opportunity sooner rather than later.

So, while we have visions of transforming this company into a true platform company, we are very focused on unlocking the value in creating a gold standard amphotericin product that is free from concerns of toxicity and can meet significant unmet medical need.

And so I turn to Slide 15 now because what we've tried to do for you is create a roadmap. A successful company is able to outline for its investors and the market - what are the boxes we intend to check in the short-term which are going to unlock value. And so I want to highlight



some of those milestones and catalysts on Slide 15. And what's important and the takeaway here is that we have a number of different things in the short-term that you're going to be able to follow along with us and check the boxes.

Following our formulation optimization--and I want to be clear that that is not a restart, that is taking a product that has already successfully worked in patients at the NIH and making it a more viable, commercially viable, formulation especially for the leukemia population as we head towards a Phase 2 trial in what traditionally has been a sensitive patient population.

Let's take our product and improve it from approximately, you know, eight micrograms per milliliter to 30, and take our formulation from a substantial amount of product to essentially two tablespoons a day, and we're already there. That is huge, and that box is going to be checked and scaling up in the very near-term.

Then we're going to turn very quickly to taking advantage of the state-of-the-art dose optimization to give us the indication of which dose is likely to determine efficacy in our pivotal trial.

We will then have a strong presence and announce data at the three most important disease, infectious disease conferences this year - ECCMID, ASM Microbe, IDWeek. Our goal here is to continue to be a thought leader in the development of an--of anti-fungal drug and to demonstrate the difference that MAT2203 brings and the choice it's going to give to infectious disease clinicians that they've never had before.

Later this year, we will look toward another interaction with FDA to finalize our plans for our adaptive design pivotal trial with the goal of commencing that trial early in 2019. And the gap between the FDA meeting and the start of that trial is really all about the administrative processes around getting the IRBs at the main site locked in and ready to start that trial. But, the good thing is we already have established relationships with MD Andersen, with Johns Hopkins, with Duke University, with Cologne. Through the work of our internal team, a lot of those relationships are already there, and it will be a quicker start once we have a final protocol from our Phase 2 meeting with FDA.

And on the platform development side of things, we fully expect to advance and conclude on one or more strategic collaborations in the near-term as we look to expand the use of our delivery platform. This is truly an exciting time for the Company, and it is relatively easy to see the incremental value tied to our anticipated progress for the balance of the year.

As a team and as a Company, we are intently focused on execution and unlocking and expanding the value associated with our delivery technology.



We thank you for your time this morning, and we will now turn the call over to the operator for our Q&A session.

**Operator:** Thank you. At this time, we'll be conducting a question-and-answer session. If you'd like to ask a question, please press star, one on your telephone keypad, and the confirmation tone will indicate your line is in the question queue. You may press star, two if you'd like to remove your question from the queue. For participants using speaker equipment, it may be necessary to pick up your handset before pressing the star keys. One moment please while we poll for questions.

Thank you. Our first question comes from the line of Jason McCarthy with Maxim Group. Please proceed with your questions.

**Mr. Jason McCarthy:** Hi, guys, thanks for taking the questions. But, before my questions, Jerry, I just want to be the first to congratulate you on becoming the new CEO. I think you're a great guy and perfect guy for this role, you know, as the Company continues to build and you know I'm an anti-fungal guy, and I'm really looking forward to, you know, what you're gonna do going forward in leukemia and these other indications.

You know, a couple of questions --you know, I think something that gets lost, not by you guys but by investors, is how toxic amphotericin is. Maybe you can just discuss or review with us, you know, how much could somebody take over their lifetime of amphotericin versus what you can give with a cochleate and what you have given in patients going out to a year?

**Mr. Jerome Jabbour:** Great. Thank you, Jason. First, you know, thank you for your generous comments. Obviously, I'm honored and humbled to be leading the Company, but this is really all about the exceptional team we have here and the technology, which we believe can take the Company to the next level.

But you raise a great point on toxicity, and there's a reason why amphotericin is called amphoterrible. And traditionally, you've seen, you know, whether delivered intravenously as AmBisome or injected, you know, as Fungizone, you typically tend to see toxicity around Day 10 or Day 14. And that causes physicians to make choices about whether or not they're going to effectively, you know, treat a deadly invasive fungal infection or they're gonna subject the patient to irreversible kidney damage.

And over the course of therapy, you know, it's traditionally been thought that any amount in total over three grams can never be given to a patient. And I want you to keep that three gram number in mind, because in our NIH trial--and this is where, you know, we took three different



doses into patients - 200 milligrams a day, 400 milligrams a day and 800 milligrams a day, and the balance of that protocol was treatment, you know, for up to, you know, 54 days.

And so, when you think about just that protocol in and of itself, let's take--and you ladder, you know, those dosages over the course of that, you know, therapy time period, you're getting past three grams a day in essentially, you know, by a month. And what we've been able to do in that study in at least two patients who have been taking it for more than a year, one 400 milligrams a day, one 800 milligrams a day, we've smashed that kind of impression of the toxicity of amphotericin in that study where both, you know, the traditional levels associated with kidney toxicity especially have never been outside the normal guidelines.

And that's also remained true for our third patient in that study, who during her time in the study also showed no toxicity. And because of that safety profile and because of the meaningful lifestyle impacts, those patients have all elected to go into the long-term safety aspect of that study.

So, our ability to effectively give amphotericin for now more than a year, at least in two patients, and more than 90 days gives a lot of comfort as we are developing a drug for a patient population whose neutropenic period is between 84 and 90 days. So, the toxicity is probably the best aspect of the data we have from the NIH.

**Mr. Jason McCarthy:** Okay. And, you know, as you're starting to look at, you know, what types of leukemia you're gonna try to use this--use 2203 as prophylaxis, you're going to start with ALL-- Can you walk us through what other types of Leukemia that are--could potentially benefit from this? See, I know most therapies are gonna make patients neutropenic, anyway, but in AML, you might use posaconazole. There's some other options out there. But do you see this or the potential for 2203 to expand to AML, cLL, other types of leukemia because amphi really is just the better antifungal agent, in my view, versus what's out there?

**Mr. Jerome Jabbour:** Yeah, we certainly like the aspects of amphotericin, which would make it the primary choice for either an infectious disease specialist or a hematologist looking to treat patients in this area. And you're right that AML patients today do have a choice of posaconazole as a preventative treatment.

The reason we're focused on ALL is for a couple of different reasons. The drug to drug interaction associated with posaconazole doesn't make it you know, available to those sorts of patients. We are also purposefully designing a drug in the development program to yield exclusivity associated with both QIDP and Orphan and ALL qualifies for that, which would give us 12 years of exclusivity. That's very important, and we want to be able to give patients who



unfortunately are dealing with Acute Lymphoblastic Leukemia, the opportunity to not develop a deadly fungal infection, which unfortunately results in mortality rates of over 50%.

We do think there are opportunities for the use to potentially be expanded, but mostly because of either growing resistance to posaconazole in that patient population or advancements, for example, in AML, which could yield drug to drug interaction with that drug similarly to kind of the regimens that are in place on the ALL side.

But, we're intently focused on ALL. We think that that alone is a viable commercial opportunity on top of meeting a significant unmet medical need. But, down the road, do we think that once there is a positive experience in the ALL patient that it could be considered for broader use? That's absolutely possible but, we're definitely focused right now on developing it for the ALL population.

**Mr. Jason McCarthy:** Right. And just a quick follow-up to that—you know, while you could develop it for AML or some other indication, you know, what's your take on the use of antifungals in general, meaning if 2203 is approved even for, you know, a relatively small population in ALL, you know, my experience is that, I.D. docs, if there's an antifungal available, they'll use it for anything. You know, so there could be a significant amount of off-label use of 2203 if it does get approved even if it's initially for ALL. Your thoughts?

**Mr. Jerome Jabbour:** Yeah, you're starting to sound like some doctors I know at the National Institutes of Health who are so excited about the potential broad application of amphotericin. But, I want to be very cautious and careful about how we talk about potential uses of this drug. And whether or not it's used off-label is obviously within the discretion of the physician. But for us, it's important to establish that it's safe and effective in the patient population that we're targeting right now in ALL.

What happens in the future or a physician's level of comfort with the profile of this drug relative to other available antifungals, that will take care of itself. So, while I appreciate the fact that it would be potentially interesting to take the broadest spectrum antifungal drug, which is fungicidal and not fungistatic, and use it for a lot of different things, we're really only concentrating on meeting that white space on the map.

**Mr. Jason McCarthy:** Okay, great. Thanks for taking the questions. Congratulations, again, and my best to Roelof.

**Mr. Jerome Jabbour:** Great. Thanks, Jason.





**Operator:** Our next question is from the line of Robert LeBoyer with ROTH Capital. Please proceed with your question.

**Mr. Robert LeBoyer:** Well, let me also begin by congratulating you, Jerry. I think the Company's in very good hands now and also wish Roelof the best. My question has to do with first of all, the development timeframe for the drug going forward. My understanding from the comments is that you're going to be doing the protocol assessment for the rest of this year and then start in 2019? Am I correct on that?

**Mr. Jerome Jabbour:** Yes. I mean, obviously, protocol assessment is not what will take up the balance of our activities in 2018. But, it's those necessary ingredients - formulation optimization, dose optimization and interaction with the FDA hopefully in the late third quarter, early fourth quarter in agreement on that protocol and then, you know, the subsequent interaction with the leading leukemia centers in the U.S. and abroad that'll take up that balance. But, our--we anticipate commencing, you know, the first stage of this adaptive design trial, you know, late first quarter, early second quarter 2019, yes.

**Mr. Robert LeBoyer:** Okay, great. And one of the things that was mentioned was the collaborations and the potential for using this drug delivery system for other molecules. And that's something that I've always thought had potential and seems to be getting more attention. Are there any potential collaborations for 2018, or are these long-term things, anything that we should be aware of or expect maybe the rest of the year?

**Mr. Jerome Jabbour:** Robert, thank you for your question there. And obviously, that's a key strategic objective of ours. The timing of that is always very difficult to predict. But given the fact that the discussions we've had in these--with these companies and in these areas you know, have progressed, you know, we believe that it's certainly possible and perhaps even likely that, in 2018, we see one or more of these collaborations reveal themselves in some way, shape or form.

It's too early to comment on exactly when or what the nature of these will be. But what is clear, is that, in these areas that we're focused on, whether it be vaccines, or oligonucleotides, or antivirals there is a need for improved delivery of those molecules. And we're confident that, in the work we've done to date, we've demonstrated an ability to do those sorts of things, but we'll have to let those discussions play out, you know, before we can really comment them--comment on them in any meaningful way.

**Mr. Robert LeBoyer:** Okay. Yeah, that's consistent with my interpretation. It sounded like this has gone to a higher priority, maybe more active discussions from great potential to something





a little bit more tangible and a little bit closer on the horizon. So, thank you for that. And just lastly, are any updates on [MAT] 2501?

**Mr. Jerome Jabbour:** Yeah, great question, because again, that would be an asset, where we would demonstrate the ability for the first time to orally deliver an aminoglycoside. And so, there's still a high-level of interest and attractiveness to that market. And obviously, the comp to Insmed who's--who has successfully demonstrated the ability to deliver amikacin through an inhaled process, you know, despite some of the challenges of inhaled delivery and some of the adverse events, the value that has been unlocked in that category is significant.

So, 2501 for us remains an interesting asset, but we have to look at where our focus needs to be in the short-term to drive the greatest value, given the resources we currently have in the Company. And so, as we made an assessment of where we are and where we want to go in 2203 and where we are and what's likely to happen in terms of expansion of the platform into these other areas, you know, 2501 is there, but until we are a better resourced Company, we will do work necessary to move that forward, but we're not gonna undertake any major clinical work on that until we're funded. Now, I happen to think that could be a very important component of a financing, given the attractiveness of that market and the comps in that space and our ability to differentiate significantly from those other products in those other companies but for right now, intently focused on 2203 and strategic.

**Mr. Robert LeBoyer:** Okay, great. Thank you very much, and congratulations once again.

**Mr. Jerome Jabbour:** Thank you.

**Operator:** Our next questions is from the line of Raghuram Selvaraju with H.C. Wainwright. Please proceed with your question.

**Mr. Julian Harrison:** Hi, thank you for taking my questions. This is Julian on for Ram. Are you able to provide any guidance at this time on the cost of the pivotal Phase 2 trials for 2203?

**Mr. Jerome Jabbour:** Yeah, I--because we haven't really have those discussions yet with FDA to narrow in on things like patient population target, what are the statistics that will yield the number of patients we think will be required to demonstrate statistical superiority, it's hard to project it. We know essentially what the cost of patient is in this area, and it's anywhere from 75,000 to \$100,000 per patient.

But, as far as like total cost, that's kind of hard to kind of project at this point. But, we'll very quickly know that around the time that we meet with FDA in late--late in the third quarter. So, we're not far away from that. In the past, we've predicted, you know, something in the nature



of, you know, \$25 million plus. But, to be certain about that, we need to decide on the final protocol.

**Mr. Julian Harrison:** Sure, okay. And regarding [MAT]9001, have there been any recent updates? Is there any possibility of developing this drug candidate in-house?

**Mr. Jerome Jabbour:** Another great question, you know, but given all the different things we want to focus on and do, MAT9001 from an internal perspective is not a priority. But, we keenly are aware of upcoming data from companies who have competitive products in the space who may change the entire value perception associated, you know, with that omega-3 cardiovascular space and we like the fact that we're sitting there with head-to-head data against the leading product in the space, which shows that we are significantly more efficacious than they are perhaps giving us a dose advantage over them.

We'll have to see how that reads out. We have had active partnering discussions on MAT9001. We expect those to continue and perhaps intensify when Amarin releases its outcomes data later this year. But, for right now, it's not an internal source of focus.

**Mr. Julian Harrison:** Okay, great. Thanks for taking my questions.

**Operator:** Thank you. Ladies and gentlemen, this concludes our question-and-answer session. This will also conclude our conference for today. You may now disconnect your lines at this time, and we thank you for your participation.