

New Recombinant Lipase Compound that came out of the French Wine and Cheese Industry offering Hope to Patients with Cystic Fibrosis and Pancreatitis in the Digestion of Fats



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CEOCFO: Mr. Spoor, what is the concept behind AzurRx BioPharma?

Mr. Spoor: AzurRx BioPharma started with the premise that we want to look at non-systemic pharmaceuticals. Drugs are categorized into two really broad categories, large molecule and small molecule. Large molecules tend to be proteins and complex structures and it is really tough for them to be absorbed and there are often problems with having them formulated for delivery to patients. Small molecules are things like aspirin or Tylenol which are small chemicals are more easily absorbed into the blood from the stomach and then go throughout the body. AzurRx BioPharma wants to focus on things that are non-systemic, things that only stay in the parts of the body that you want them to go into. If you swallow our drug, it stays in the stomach and does not go anywhere else but the gastrointestinal system. Then it leaves the body after it does what it is supposed to do. Our origins are from research in the French wine and cheese industry, but at this point, we are focusing on making gastrointestinal drugs better.

CEOCFO: What is wrong with what we have now? Where is there room for improvement in the gastrointestinal industry?

Mr. Spoor: The company focuses on two major opportunities to improve patient care. The first is gastrointestinal and the second is anti-infective. Across medicine, I think there are always places where there is room for improvement. In some cases, the room for it is extraordinary and in some cases it is very minor. In our case, we are looking at patients with cystic fibrosis and patients whose pancreas has shut down. The current standard of care involves a massive pill burden that has problems with stability in the gastrointestinal system. We are busy trying to improve the standard of care for patients in a disease that impacts the thirty thousand patients with cystic fibrosis and chronic pancreatitis affects another 120 to 200,000 adult patients with pancreatic disorders. In those cases when the pancreas is not working, you need to have a way to give a chemical called a lipase to these patients. When we talk about patients who have a lipase deficiency this means they are missing the enzymes that help them digest fat. In the 1890s, the first way to serve these patients was when physicians and pharmacists started using ground up pig pancreas to treat patients with this disorder. The theory was that if the missing enzymes are from your pancreas, then you go to the closest omnivore to a human, which is a pig, to replace what is missing in these humans. The premise was very sound and it ended up working in these patients prompting other companies to develop their own versions of pancreatic extract. Amazingly enough, 130 years later, Abbvie, J&J and Allergan share a billion dollar US market of pig pancreas extract that is served, sold and used by all these patients. We think we can go and make a big improvement in the technology.

CEOCFO: *In what way?*

Mr. Spoor: When we speak with clinicians, they tell us that on the current standard of care, 50% of cystic fibrosis patients and 35% of the chronic pancreatitis patients do not have their fat absorption managed properly. If we zoom out for a second, what are we really looking at? You have three kinds of food that you eat; you have proteins, carbs and fats. When you eat your meals, in order to absorb the food, you have to digest it first. You are not going to swallow a cheeseburger and absorb a cheeseburger intact into your bloodstream. You have to digest the food first and break down the components of the cheeseburger into things your body can use, and that is called digestion. Once the components of your food are small enough, your body can take them in, a process called absorption. We want to help digestion so that absorption can actually take place. Your carbs are digested, mostly as a result of your saliva and the enzymes there. A lot of your proteins start being digested in your stomach with both your stomach acids as well as the enzymes produced by bacteria in your gut. Fats on the other hand need the enzymes from your pancreas, and the biggest group of enzymes that are missing are the fat digesting enzymes called lipases. When we look at patients with disease, the biggest issue they have is trying to get more calories into them. Patients with uncontrolled chronic pancreatitis are often very thin, they often have many bowel movements per day and they have trouble getting enough nutrition. They will often have discomfort if they eat fatty foods and they can take up to thirty or forty lipase pills a day. To reiterate the opportunity for us to really help patients, many patients are taking literally 25 to 40 pills a day and still not having their fat absorption managed properly. The chemical reason for this is because the pig enzyme is not considered acid stable. A pig's gut is less acidic than the normal human gut. While a human gut is acidic, patients with pancreatitis are more acidic than patients without pancreatitis. This creates a tough situation where you have very little lipase activity from the pig enzyme in the acidic environment of a human stomach. What's we hope to do is to bring patients from 25-40 pills a day down to 5-8 pills a day.

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CEOCFO: *Where are you in the process and what have you developed so far?*

Mr. Spoor: The evolution of the technology is fascinating. It came out of a research institute supporting the French wine and cheese industry. The French cheese industry is a big part of the local economy and there are estimated to be more than 360 different cheeses in France. In one of the cheeses, scientists found a yeast that produces an acid stable lipase. That technology came out of a group called INRA, which is a French research institute who then licensed that to us and to our corporate partners in France to develop a new pharmaceutical. AzurRx has a lab in France where we do a lot of our research development so we did a lot of preclinical work and safety testing, developed a formulation, and ramped up our production. We did a phase 1 A/B study back in 2010 and currently we are in the middle of a phase 2A study, looking at how to actually help patients with the disease.

CEOCFO: *Does it make sense to the medical community?*

Mr. Spoor: Generally in modern medicine, if you ask a physician if they are managing their patients properly, they will reflexively say they absolutely are managing their patients to the best of their knowledge based on the data that exists. But once you start to ask a few more questions, in any specialty, you realize where all of the opportunities are. My guess is that if I were to ask a physician right now if patients with cystic fibrosis or pancreatitis are being managed properly, the initial answer is probably that they are managed well with the standard of care. If you start digging and ask what it would mean for a patient if you could actually lower their pill burden from 25-40 pills a day every single day for the rest of their lives, if you could lower that to 5-8, it is a huge reduction. Asking patients if they would rather take 5-8 pills a day instead is almost a rhetorical question. When we did our market research, we found that if we gave physicians the ability to lower pill burden, 57% would actually start using it in their newly presenting patients. When we speak to patients, not surprisingly, they think that number will be much higher. The current standard care is a fairly large pill as well, a size 00 capsule, which is quite large. We can do a capsule that is three sizes smaller.

CEOCFO: *AzurRX recently closed five million dollar financing. What are you going to do with the money?*

Mr. Spoor: The money is used to continue our development of our clinical programs. Thankfully, in modern medicine, physicians and the FDA are very data driven. Our job as innovators is to prove everything with our data, which we are

trying to do right now. Our acid stable lipase is currently in phase 2 with two potential indications, chronic pancreatitis and cystic fibrosis. We have some interesting pipeline compounds as well that our researchers are working on under the theme of non systemic compounds. Our idea is that if you swallow drugs that are not absorbed, you lower the side effect chance. By focusing on something in the gut or bacteria in the gut, we think we can make a big difference in patients' lives.

CEOCFO: *Why is this an area that people have ignored?*

Mr. Spoor: I don't know if this has been ignored or if it has just been very hard to get success. Laisses are in your laundry detergent and break up fat, but the problem here is more complex. If you go back to a chemistry class, things can be at either a basic or an acidic, pH. How basic or acidic something is affects how an enzyme works. All the lipases that are using in industry in detergents and things like that, are all basic enzymes, not acid stable enzymes. The pig enzyme used to help patients now is not acid stable, it generally works better in a more basic environment. There has been a lot of trouble getting something that is acid stable and we think that AzurRx's product will be the first time that I have ever seen an acid stable lipase that can be used. The market is huge. It is now thought to be over a billion dollars in the US alone. The patient need is clear but to innovate to get to where we are has been hard.

CEOCFO: *What has been the response from the investor community?*

Mr. Spoor: When we meet with the investment community, we categorize them into everyday investors/patients and institutions. Retail and patients love the idea. There is usually an emotionally connection and people understand that we can make a big difference. A year or two ago, when we started speaking with institutions, there was certainly interest but they wanted us to try and validate this with more data. We have had an extraordinary amount of initial institutional interest this year, which has been quite encouraging. We have a small market cap compared to the billion dollar market we will compete in, which is probably why it seems like such an interesting proposition for people.

CEOCFO: *Why is AzurRx BioPharma so important?*

Mr. Spoor: There are two questions there, why it is important to pay attention to and why it is important to the medical system overall. For investors, everything is about price and risk/return. If you are someone who would look at where AzurRx BioPharma is, our market cap and the size of the market that we expect to massively disrupt, this should be something really worth people looking at. We have near term catalysts. We have trial data coming out, we have a clear path to approval according to published FDA guidelines. When we think about what we can do for the patients and the medical community, there is a really big factor that I have barely touched on. There is the whole idea of using animal products to create pharmaceuticals such as lipase. The current standard of care is dependent on pigs coming from slaughterhouses and so the FDA has a black box warning saying there is a risk of actually having something slip through the animal supply chain. There have been cases in recent history where disruptions from animal based products have been significant. Heparin had a massive recall about ten years ago due to problems with the porcine supply chain. Mad cow disease had a big disruption on the food supply chain. The risks can come from proteins, particles, prions or viruses. We are offering, for lack of a better term, a vegan product as we have a yeast based compound where we think we can reduce that risk of animal based contamination. The two halves of drug approval are safety and efficacy and while we think we have the potential for better safety we also think we can offer a lot of patient benefits from having a better response to their medications. If we can actually have people gain weight then they can manage their disease better and not have to always worry about how close they are to the bathroom. If we can lower their pill burden from 40 a day to 8 a day, we think we can make massive improvements in patients' lives and that leads to better patient outcomes, which leads to everyone being happier.

