Interview: Peter Zerhouni, CEO, Diamyd Medical, Sweden



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Could you begin by introducing Diamyd Medical and therapeutic focus to those of our international readership that are not yet familiar with your name?

Diamyd was recently listed on Nasdaq's First North list, a smaller growth list. We actually have quite a long history on the stock exchange, where we were on the big board previously in a group of companies from which we were spun off earlier this year to stand on our own.

We are a Swedish company based in Stockholm and are developing a diabetes vaccine to treat, prevent and cure type-1 diabetes. Type-1 diabetes is an auto-immune disease and accounts for about 5-10 percent of all cases of diabetes.

The problem with this type of diabetes is that the body's own immune system destroys the insulin producing cells in the pancreas. Without those, the patient cannot control his blood sugar, which causes diabetes and all the problems associated with the disease.

It is a chronic disease that typically occurs in childhood. Nobody knows what triggers this auto-immune attack, but we know that it has a genetic component. About fifty percent of the explanation of why a certain individual gets type-1 diabetes can be found in the genes. The other fifty percent is some sort of external trigger that has yet to be revealed.

What are the particular commercialization challenges & opportunities you face at this stage?

Type-1 diabetes is today treated with insulin. The body of a type 1 diabetes patient cannot make insulin anymore, so it has to be injected. Insulin is what keeps the patient alive; without it he will die within days or weeks. The problem with insulin is that it does not address the underlying disease process; it is not a disease-modifying treatment. It is basically palliative care – with good life-quality of course, but it does not stop the auto-immunity that actually causes the problem to start with.

That is where our drug, which we call a diabetes vaccine comes in. we call it a vaccine because it makes it easier to understand given the way it looks and how it is given, but the more correct medical term would be immune modulator.

We aim to teach the immune-system to leave the insulin-producing cells alone, and thereby preserve the body's insulin-producing capacity. It has been shown that, even with a modest, endogenous insulin-producing capacity, the common and severe complications from diabetes are reduced by 60-80 percent.

Diabetes is characterized by elevated and fluctuating glucose levels in the blood. This glucose causes damage to a number of organs in the body such as kidneys and eyes and causes cardiovascular disease, and it causes nerves to die which causes diabetes pain. With some of his own insulin-producing capacity preserved, the patient suffers much less from these complications.

Our aim is to preserve the body's own insulin-producing capacity, and thereby reduce the risk of complications.

When do you expect DIamyd to be on the market?

We did not get the results we were hoping for in phase III clinical trials testing Diamyd as a mono-therapy. That was done in recently diagnosed type 1 diabetes patients. The thinking now is, not just from us but from many stakeholders in the field of type 1 diabetes, that this disease will probably not be overcome by using a mono-therapy.

We are looking at combination therapies where we combine several different drugs that hit on the disease from different angles, with different mechanisms of action, where we definitely think that our mechanism of action, our approach, has the potential to become a key piece of the puzzle of a future solution to prevent, treat or cure type 1 diabetes and other forms of autoimmune diabetes.

Currently we have one clinical trial where we combine it with two other drugs, and one clinical trial where we try to treat earlier the disease process, actually before the subjects develop clinical symptoms with the aim to prevent the disease. We expect results from both studies in 2015.

As I said it is an autoimmune problem that destroys the insulin producing cells, and that process starts long before you have clinical symptoms. It is a gradual breakdown of the cells, and the symptoms only start when just 10-20 percent of the cells remain.

In the prevention study, we are piggy-backing on a population study in the south of Sweden, where they actually screen kids at birth for their genetic risk. If they have a high genetic risk, they are screened every three to six months for biomarkers that tell us that this autoimmune process has started. Then they are invited to be in the study with our diabetes vaccine, and the hope is that it will be easier to intervene in the disease process by coming in that early.

That is one study – as a mono-therapy in prevention or secondary prevention. Then we have a combination study in recently diagnosed patients. Once diagnosed, the disease has become so aggressive and complex that the one probably needs to hit on several different pathways to get a meaningful effect. We hit on one of these pathways with our approach, and we look to try different combination therapies.

To answer your question, it is not so easy to say when we can be on the market. We have already come a very long way development wise and if one of the ongoing studies comes out with the results we hope it can go fast. Taking into account that we were in phase III before, when it comes to having a large safety database, and we have an established manufacturing process. We made phase III material and were on our way of making commercial grade material together with the help of Johnson & Johnson that used to be our partner.

This means that we can be very quick from the time we know how we need to combine this drug with other drugs, or how we can use it in a preventive setting. From that point we can be much quicker than other projects. We have done all the late stage groundwork already. We can move from that point straight into phase III. If the phase III takes three years and then you allow two years for filing and registration, we could be on the market by 2020 at the earliest – if one of the ongoing studies comes out positive of course.

That is again based on finding the right treatment concept, and moving that into phase III and beyond.

Does the therapy have the potential to be used as a vaccine for the general population?

The biomarkers we have in the secondary prevention trial are so predictive of disease, that you could probably make the case that they are not healthy kids. You could say that they already have the disease but not the symptoms. Are they healthy? I think that people can agree that once the autoimmune process has started, that is when the disease starts; not when you can no longer control your blood sugar because you have lost so much of your insulin-producing cells that the system collapses. That is not a big leap to take. That is why you should have a drug to take when the disease process starts.

It is a bigger leap to treat the general population in a preventive manner, because even in the high risk genotype, you do not have that super-high risk of developing the disease. A person can have a

five percent risk, which is ten times the risk of the general population, but it is still only one in twenty.

I would say that intervention is the easiest; secondary prevention is absolutely possible and is in our current development plan. General population is not something we aim for at this point in time, but of course it would be absolutely amazing to achieve.

How does Diamyd's vision fit in the prevailing shorter term biotech business models – you have an idea, try to get a proof of concept, and partner with a larger organizationto finish the commercialization?

The traditional biotech model of developing the product to phase II and then partner it out – I do not know what you think about this model, but in Sweden at least it feels like it is broken. It is a time of change, and we will see if the model recovers or if a new model develops.

Historically, we actually did what a biotech was supposed or expected to do. We developed our product into phase III and then partnered with Johnson & Johnson. Then, we did not get the phase III results that we hoped for, and Johnson & Johnson returned the project to our former parent company. There was a lot of cash left from the deal, and we were thinking of how to optimally use this.

The business that we now have spun off did not require that much cash or capital.We have spun out the diabetes business together with a smaller part of the cash that was in the former parent company, about 50 million SEK.

How long will that last you?

With the current development plan for the diabetes vaccine, it should see us through the next round of clinical results. There are two clinical trials ongoing and the money should see us through those. We expect results in 2014-15.

But we have said that we want the company to be more than the diabetes vaccine. We want to build on our expertise, network and experience in this area to add more diabetes products to our portfolio. Recently we concluded a license agreement with UCLA for another molecule called GABA, to be used in diabetes and inflammation-related diseases.

In order to get money to do that, we are currently doing a rights issue through which we hope to raise another 21 million SEK to start development of that asset. That is very recent; June 7 was the first day to sign up, and this ran until the 20th of June. We have not pinned down a development plan for GABA until we know how much money we have.

It is part of our strategy of broadening the portfolio.

In an effort to dilute financial risk, we see partnerships become more crucial across the industry. Could you outline your partnership strategy in a bit more detail?

Our deal with Johnson & Johnson was one of the biggest biotech deals in Sweden ever, if not the biggest. We got 45 million USD upfront and agreed on another 580 million in milestones.

It would of course be great to do another deal like that, but you brought up the business model and the emerging business model for biotechs. Now we are thinking we may do more deals, but smaller ones.

We can divide by geographic region, by indication, or set up research collaboration, which does not necessarily mean the traditional structure with a big sum upfront followed by milestones, but rather a collaborative effort at an earlier stage from where you move forward together. It is more about building alliances than signing partnership deals.

What would be your final message to our readership?

We are trying to build research & development alliances around the diabetes vaccine and the active ingredient – a protein called GAD. We are open to receive proposals to work on that. We are also looking to expand our diabetes portfolio with projects, products or companies to really build upon our expertise and network in this area. We see ourselves as a hub for diabetes projects and products, where we can source projects and then find a taker –once we have developed it further.

We have the diabetes vaccine, which can become a key component of the future solution for type 1 diabetes; we have a track record of development – we have developed the diabetes vaccine from pre-clinical to phase III and concluded one of the largest biotech deals ever in Sweden; and we have the expertise and international network to place us as a hub in the diabetes sphere.

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