Mattias Bankel – General Manager, Head of Nordics & Baltics, Amicus Therapeutics

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Amicus Therapeutics is a biotechnology company at the forefront of advanced therapies to treat a range of devastating rare and orphan diseases. Mattias Bankel, Head of Nordics and Baltics, shares his journey of establishing and developing Amicus's presence in Sweden and other Nordic countries, furthermore his commitment to ensure that rare disease patients have access to the right diagnosis and treatment.

Mattias, you have taken on the task of establishing Amicus's presence in the Nordic and Baltic regions from scratch. What have been some of the challenges you have faced along the way?

Challenges differ depending on the country. In Sweden, the Dental and Pharmaceutical Benefits Agency, known as the TLV (Tandvårds-och läkemedelsförmånsverket), approved reimbursement of Galafold for Fabry disease in November 2016, six months after European Medicines Agency (EMA) approval, marking one of the first major reimbursement decisions of the treatment in the European Union (EU). Within each individual Nordic and Baltic country the challenges differ, some countries have a higher number of patients than others, the reimbursement process can be faster or slower, but overall within the region we are doing remarkably well. Our strong performance has been driven early on by Denmark.

How many people suffer from Fabry disease in Sweden?

It remains unclear how many people are afflicted by Fabry disease in Sweden. According to the National Board of Health and Welfare, there are between 60 and 70 diagnosed patients. In Norway and Denmark, countries with about half the population of Sweden, there are about 100 patients diagnosed in each. In Sweden as in other countries globally, Fabry disease is thought to be underdiagnosed so there is potential for growth with an increase in awareness and diagnostic initiatives In order to identify people living with Fabry disease, we are supporting healthcare with projects to improve the diagnosis rates.

How are Fabry disease patients benefiting from this new therapy compared to the standard of care?

Galafold is indicated in EU for long-term treatment of adults and adolescents aged 16 years or older with a confirmed diagnosis of Fabry disease and who have an amenable mutation. It is an oral small molecule pharmacological chaperone with novel mechanism of action that represents groundbreaking innovation. It was the first new treatment for Fabry disease in more than a decade, as well as the first precision medicine for patients with Fabry disease who have amenable genetic mutations (estimated to be 35%-50% of diagnosed population today). It is also the only approved oral therapy for Fabry disease, designed to restore α-GAL A enzyme activity in patients with amenable mutations. Galafold goes where patients can go – one capsule every other day. The most commonly reported AE is headache, and the full prescribing information can be found on the EMA website

How did Amicus manage to receive reimbursement so quickly in Sweden?

At Amicus Therapeutics, we believe that our medicines must be fairly priced and broadly accessible. Receiving reimbursement from the TLV was a straightforward process, because we applied at a similar price and for the same patient pool treated by the two drugs already approved and reimbursed by the system. Galafold priced at parity to previously approved products delivers the value of an innovative, precision medicine option and won’t have infusion-associated costs, which could save the healthcare system money.

Since there was no cost increase involved, we believe the decision was quite easy to make for the TLV. For example, they are able to save resources as they no longer need a nurse to perform an infusion and can free up infusion chairs.

Since Galafold is part of the reimbursement system, patients who were previously treated have access to the medicine across the country. However, in the case of previously untreated patients, physicians first need to receive approval from the home region of the patient before starting treatment. There are three physicians treating Fabry disease in Sweden, one in Gothenburg, one in Stockholm and one in Uppsala and the three of them conduct regular phone conferences.

In 2018, Amicus doubled the revenues from Galafold from $36.9M to $91.2M. What role did the Nordic region play in this outstanding performance?

We are doing really well in the Nordic region and our strong performance has been driven early on by Denmark. At the time of launch, we switched patients that had been receiving Galafold in the Phase 3 clinical study to reimbursed Galafold after approval and reimbursement.

Thanks to early launch access in the Nordics, the region has been a major contributor to the global success of Galafold to date.

How would you compare the ease of market access between Sweden and Denmark?

Both Danish and Swedish systems are efficient to work through meaning that patients can generally get access to innovative medicines relatively quickly.

Since two of his children were diagnosed with Pompe disease in 1998, John F. Crowley has devoted his life to developing a treatment. What promise does Amicus's drug candidates hold for Pompe disease patients in the country?

Our Pompe disease drug candidates show great promise based on the Phase II data reported recently. We aim to make a difference with people living with the condition around the world. Locally, we hope sites in Sweden become involved in the ongoing Phase 3 clinical study.

Our fairly priced and broadly accessible approach also applies for Pompe disease, but it is premature to discuss any specifics. . . At Amicus we understand the importance of partnership with

the reimbursement authorities and will be ready to have productive discussions on the value of our therapies at the appropriate time.

Other rare diseases companies such as Shire conduct clinical trials in Sweden. Does Amicus have plans to increase its clinical development footprint in the country?

We hope to involve Swedish patients with Pompe in a Phase III study for Pompe disease. Generally, Amicus is very keen on opening many clinical sites where we can reach a broad set of patients and physicians in the clinical setting. This mindset is different from other companies I have worked with. It is part of the patient perspective of Amicus: if we can advance science by enrolling patients in trials, it does not matter where they come from. Other companies might refuse to perform clinical trials in the Nordics because they think the uptake will be slow, or the price will not be attractive, but at Amicus, it has never been a debate.

What do you see as the strengths of Sweden’s clinical ecosystem?

The unique strength of Sweden’s healthcare and clinical research ecosystem lies in its national quality registries. We would like to leverage this asset in order to identify undiagnosed Fabry disease patients. For example, there is clinical data pointing to the possibility of multiple sclerosis (MS) patients being misdiagnosed and having Fabry disease instead. Some studies say the proportion might be as high as 5 percent and Sweden has blood samples collected from 20,000 MS patients. Because Sweden is quite far ahead when it comes to digitalization of health data, it is one of few countries where AI technology or machine learning may also be possible to aid in diagnosis. Many European countries do not have national registries. Instead, registries are located at the hospital or regional level. This represents a strong competitive advantage of Sweden.

While 26 European countries have already introduced a national plan for rare diseases, Sweden still does not have its own plan. How are you working with other stakeholders to push the government in introducing a national plan for rare diseases?

We know that the patient association Rare Diseases Sweden is asking the Government to address this. It would be of great benefit for patients to have some clarity on how rare diseases should be managed in the future.

Globally Amicus is dedicated to patient advocacy. In Sweden, how does Amicus try and be the voice for patients?

We have a dedicated patient advocacy team that works closely with patient associations globally and within Sweden. Whenever we talk with physicians, we bring the patient perspective. While physicians understand the disease clinically, they do not always understand how it affects people on a more personal level. We try to bring that perspective to the table, not only in our interactions with physicians but also with politicians, regulators and all the key stakeholders.

On a more personal note, what makes you so passionate about working for a company so dedicated to the fight against rare diseases?

I became involved in rare diseases ten years ago. What first attracted me to rare diseases was the business side of the equation. Across all therapeutic areas, it is the most challenging and one where it is possible to make innovative business deals. But my motivation quickly evolved when I started meeting people living with Fabry disease. They told me their life stories, how they went from one physician to the next in order to receive a proper diagnosis. Fabry disease is a slow progressive disease which makes it difficult to diagnose. They knew something was wrong with them, but physicians could not put a name on it. This is where we as a company can make a big difference. We can support them through their journey, from identifying the symptoms to receiving a proper diagnosis and getting access to a therapy. It does not necessarily have to be our therapy, but they need to receive some kind of treatment or other support. Through these screening projects we are doing in Sweden, only a fraction of the patients we find will be amenable to our treatment. We are not screening patients to put them on Galafold, but because we want to help them get the right diagnosis and receive the treatment and support that they deserve.

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