Bob McLay – Vice President and General Manager, Sobi Canada

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Bob McLay, vice president and general manager of Sobi in Canada, discusses how innovation and collaboration with regulators and payers are helping Sobi to bring its medicines to Canadians suffering from rare diseases, while emphasizing the importance of an adequate rare disease framework to streamline this process in the future.

Bob, Sobi is a recent entrant to Canada and you have been heading the operations since 2016. What mandate was set for your tenure?

In Europe, Sobi has a 30+ year history in rare diseases, with its current business resulting from the merger of two Swedish companies, Swedish Orphan International and Biovitrum. Sobi came to the USA in 2013, subsequently opening an affiliate in Canada in 2015. North America is the largest economic region for pharmaceuticals in the world, presenting a significant opportunity for expansion, and Canada is a part of that strategy.

Before arriving in Canada, two of Sobi's products were already being sold through distributors. The two products are: Kineret® (anakinra) indicated for rheumatoid arthritis and Neonatal Onset Multisystem Inflammatory Disease (NOMID), a rare autoinflammatory disease; and Orfadin® (nitisinone) indicated for hereditary tyrosinemia type 1 (HT-1), an ultra-rare metabolic genetic disease that affects the liver and in many cases is fatal if untreated. My mandate was to steward

those two products through the regulatory and reimbursement system, as Orfadin was only available through Health Canada's Special Access Program. We had not applied for market authorization for Orfadin before, as the patient population is extremely small and Sobi did not have infrastructure in Canada to support a filing. With our establishment as an affiliate in Canada however, we quickly made the decision to go through regulatory approval, which we have now done.

For Kineret, Sobi invested in submitting a new filing to Health Canada to expand the label. The label for Kineret expanded to include NOMID, the most severe subtype of CAPS (Cryopyrin Associated Periodic Syndromes).

How have you progressed in terms of bringing these two drugs to market?

We have been able to gain approval for Orfadin capsules and for an oral suspension formulation that will offer a new option for pediatric patients. We also recently gained approval for once-daily dosing in patients who weigh 20 kilograms or more and who have undetectable serum and urine succinylacetone concentrations after a minimum of four weeks on a stable twice-daily dosage of nitisinone. We submitted to INESSS and CDR and were given positive approvals by both Health Technology Assessment groups. Expanding the label for Kineret has been completed and we are pleased to now have the ability to help pediatric patients with NOMID.

In summary, I would say we have made very good progress. However, this progress has only been achieved through Sobi's commitment to following the current regulatory and reimbursement pathways in Canada, and through great collaboration with regulators and payers to continue to advance through the process, which was not designed to accommodate drugs for very rare diseases.

Currently, there is no national rare disease strategy in place in Canada. Where does Canada stand in terms of its approach to rare diseases on a global scale?

Rare disease drugs are not like common drugs for pain management or blood pressure. It often takes years for patients to find the right physician or specialist and determine the diagnosis for their rare disease. Even if they are fortunate enough to put a name to the condition, finding appropriate treatments can be laborious, and the required drug might not be available or reimbursed. Moreover, the distribution of rare disease treatments is often more complex than for

other drug products. These products are not simply available in neighborhood pharmacies, but often have to be obtained from a specialty pharmacy and administrated by injection, several times a day, even in small children, which requires special education of physicians and families. There also may be dietary restrictions for which training is necessary. Expensive patient support programs are often required to ensure patients receive the necessary level of care.

A further complication is that it is impossible in the rare disease sector to conduct large, randomized, placebo-controlled studies. Rare disease populations are by nature small and often trials will be stopped early because the treatment effect is seen early, making it unethical to continue on with the study. This makes it difficult for regulators and the Health Technology Assessment community to effectively evaluate these products to the same level of accuracy or scrutiny of treatments for common diseases with more robust data.

This is why a rare disease pathway is important: to be able to look at individual rare diseases differently and to be able to acknowledge the challenging and unique situations of the various disease, treatments and research protocols. A one size fits all mentality does not serve anyone well in rare diseases. This is why many governments have implemented programs such as patent extensions, fee waivers and truncated review processes designed to incentivize companies to continue to invest in drugs for rare diseases and to get these drugs to patients as quickly as possible.

In 2012, the former government had issued statements that they were in the process of implementing a rare disease strategy in Canada. It has been many years of trying but there is still no legislation in place. Health Canada representatives recently acknowledged that a new regulation system was planned. Although this is encouraging news, nothing concrete has come of it yet. With the initiation of a study on how to implement a national pharmacare program, we are hopeful that this may represent an opportunity to finally have the rare disease community acknowledged and cared for more effectively.

It is essential for Canada to move forward with a strategy in rare diseases. We tend to rely on innovation from other countries’ research, leaving Canadian patients at the mercy of hoping that innovation comes to Canada. With a tough regulatory and reimbursement system here that was not designed to accommodate rare disease drugs, often innovation either does not come or is delayed by many years. Without a predictable rare disease framework and reliable funding model, innovation and investment will slow down or cease all together.

Statistics speak for themselves: only 60 percent of rare disease drugs make it to the Canadian market, and the approval processes takes six years longer than in Europe or the USA. Canada has some catch-up to do as an industrialized advanced country, to be at least on a par with countries of our size and economic abilities. Together with CORD and BIOTECanada, we are heavily advocating for better regulations.

Rare diseases typically require a more holistic approach to patient support. How do you collaborate with patient associations, especially in a geographically vast country as Canada?

By definition, individual rare diseases affect very few people. As such it is difficult for these patients and families to connect to each other or to other support networks. Often there is not much in the way of credible information to help guide them through the journey of diagnosis, treatment and care. Patient information and education is very important in comprehensively treating patients with rare diseases. Connecting with each other is also very important. Creating patient advocacy groups, however, is time consuming and difficult. CORD (Canadian Organization of Rare Disorders) has done a great job in assisting small patient populations in connecting and advocating on their behalf. As a drug manufacturer, we support the important work of CORD and patient groups where it is appropriate for us to do so. As a company operating in rare diseases, we are compelled by compassion. We fight for every patient.

What are Sobi Canada’s ambitions for the future?

Sobi has a 30+ year history of innovation in the rare disease space. Our goal in Canada is in line with our global strategy, which is to become a leader in rare diseases. We currently market 35 products across 67 countries, but there is so much more work to be done. There are an estimated 7,000 rare diseases identified in the world and less than 500 approved treatments. We are seeking to address these diseases through organic growth with our own internal pipeline, as well as external growth from partnerships and acquisitions of innovation. Our new global CEO, Guido Oelkers, has a track record of building and growing companies, and under his direction we look forward to helping more patients.

Despite the challenges you mentioned, how do you stay motivated?

Challenges can be overwhelming sometimes, but I have a great team here in Canada. We enjoy working together for a greater purpose of helping those affected by rare diseases. Patients are a key factor in our daily motivation. Patient stories are what allow us to remind ourselves that what we are doing provides value to Canadian families who need our treatments and support. We will continue to battle every day for patients. We know our work makes a meaningful difference in their lives.

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