

Slow Move Towards Orphan Drug Policy Leaves Patients in the Dust

By Marissa Decina

In their battle against time, a slow and steady trek towards orphan drug policy will not win the race for patients with rare diseases in Canada.

Though a plodding pace proved favourable for the tortoise of Aesop's fable, the same approach applied to establishing a national orphan drug policy is creating a path with no end in sight for Canadian patients seeking treatment, their advocates say.

Orphan drugs are drugs which treat rare disorders affecting no more than 650 to 1,000 people per million, by general guidelines. Since 1992, the Canadian government has been attempting to establish a formal definition, in order to establish policy that would accommodate the funding of these very expensive drugs within the public health plan.

It came close when Private Members Motion (M-426) was introduced by Donald Bell, former Liberal MP for North Vancouver, and was passed by the House in 2007. Given a year to report on the bill, the government let it die on the floor; and now, 17 years from its starting point, Canada is still without a policy.

Brenda Drabek understands waiting all too well. Drabek and her husband have been waiting 20 years to provide their son, Michael, with treatment for his Hunter Syndrome (Mucopolysaccharidosis II), a rare enzyme-deficiency disorder that progressively damages the heart, bones, joints, respiratory system and, sometimes, central nervous system of its victims. Though diagnosed at an early age, now 27 years old, Michael has yet to receive treatment for his debilitating disorder.

Until the emergence of the enzyme-replacement therapy, Elaprase, there was no treatment available to Michael. Approved in the U.S. by the FDA in 2006, Health Canada did not approve the drug until a year later, in 2007.

For the past two years, the Drabeks have been denied access to Elaprase by the Saskatchewan health ministry, with no sign of concession in sight. This is because Canada's current healthcare system makes no special provisions for funding the very expensive orphan drugs used to treat diseases like Michael's.

Presently, the federal government determines whether a drug meets safety and effectiveness standards, but it is up to provincial governments to decide whether a drug is worth being publicly funded.

Decisions to fund drugs are then based upon recommendations received from the Common Drug Review, an independent agency that measures drug cost-effectiveness. The heart of the problem, according to Kirsten Harkins, president of the Canadian Society for Mucopolysaccharide and Related Diseases Inc., is that a lack of national orphan drug policy puts orphan drugs through the same review process as drugs for common diseases.

"Drugs for rare diseases will never be cost effective because they only treat a very small number of patients," says Harkins. "If you hold it up to that kind of criteria, nobody with a rare disease would ever get treatment."

In Michael's case, the Saskatchewan health ministry refused to fund Elaprase in accordance with the negative recommendation of the Common Drug Review, on the grounds that the drug's clinical trials were too inconclusive to determine whether it would be beneficial to patients.

“Often these regulatory boards say that the data is not conclusive enough; but it’s very, very difficult to get data that is conclusive when you are conducting a trial with, say, 40 patients as opposed to 4,000 patients,” says Harkins. “This is one of the ways I think patients with rare diseases end up getting discriminated against. In the end, it is a lack of understanding and ignorance on the part of the people making these decisions.”

The Drabeks say they are devastated by their province’s denial of funding. Without it, the drug is too expensive for the family to afford, especially since Brenda is the only fulltime provider of the household.

“We have always felt like we were reaching for the impossible; and now that there is something out there for our son, we still can’t obtain it,” Drabek said, in a telephone interview, “It’s like someone has pulled the rug out from under you.”

Drabek says, it’s especially difficult knowing that some provinces are funding patients with Hunter Syndrome despite the Common Drug Review’s recommendation. In British Columbia, the wait is over for Mucopolysaccharidosis sufferers, like Harkin’s son, Nicholas, who has been receiving weekly treatments for MPS I since 2003.

“We were quite fortunate, here, in Vancouver,” says Harkins. “But, there needs to be equality across the board. You are treated very differently depending on where you live within Canada if you have a rare disease; and that’s not fair.”

Alberta is another province with a framework to accommodate drugs for rare disorders. However, citizens must be residents of the province for five years before being eligible for the program, prolonging the wait for treatment if patients from out of province decide to move.

Without provincial funding, the high cost of orphan drugs renders them nearly useless to patients outside of provinces like Alberta.

Ashraf Ghadban, a board member of the Canadian Organization for Rare Disorders (CORD), says the average annual cost of an orphan drug runs in the hundreds of thousands of dollars. His son suffers from Neimann Pick disease, a rare lipid storage disorder, which affects only approximately 2,000 people in the world.

In the most severe of cases, children with Neimann Pick disease do not make it past two or three years of age because fat stores build up in their brain tissue. This makes timely access to treatment of the utmost importance.

Ghadban says there is currently no treatment for his son's disorder, the mildest form of the disease, but a research team based in New York will likely have a licensed drug in the next three to four years, at an estimated treatment cost of \$200,000 to \$400,000 per year.

“Can my wife and I afford that? Absolutely not,” says Ghadban. “If there is no national policy by that time, we’ll have to move. When it comes to your kids, you’d do anything for them.”

With purchasing the drugs privately being out of the question, and time being of the essence, patients and their families are left to lobby the government individually in the hopes of receive funding for their treatments.

Harkins says a lot of patients or family members end up writing to their MPPs, arranging meetings with health critics, approaching the local media, and picketing outside government

offices to get their message out. But, she says, they are the last people who should have to advocate for themselves.

“Having patients have to advocate, and go to the media, and push in that kind of a way is very unfair. Some people have the energy to do that, and some people really don’t,” says Harkins. “You can imagine – your kid is diagnosed with a disease – that’s bad enough. But then to find out that there is a treatment for it, but that you can’t have access to it is almost more than some people can really deal with.”

Harkins says it’s shocking that Canada has gone so long without policy, when so many of its counterparts in the developed world have already put orphan drug policies in place. The United States was the first country to implement orphan drug policy in 1983 with its Orphan Drug Act. More than 25 years have passed without development of a comparable Canadian plan, but countries of the European Union, and others, like Japan and Australia have followed the Americans’ lead.

“I was in Taiwan,” said Harkins, “and even they have a wonderful rare disease policy, which they developed and implemented within three months. Here we’ve been working on it for years.”

For a country that has prided itself on taking care of its citizens, Harkins says, it is high time Canada lives up to its own standards. To bring equality to all Canadians in the healthcare system, she says, it is imperative there be a national orphan drug policy put in place without further delay that provides for all citizens no matter their condition or place of residence.

“These diseases are progressive disorders, so patients have had to wait while their diseases enact irreversible damage on their bodies,” says Harkins. “A five-year-old getting treatment now could have started when he was two or three.”

Harkins says time is of the essence, and implementing a national policy would give patients a sense that the government cares about them and their families.

“It’s really important to know that the hope for a better future is attainable,” she says, “Even if it’s not for your own child but for another child that may be born in the future with that disease.”

After years of waiting, there may finally be a light at the end of the tunnel for Canadians suffering from rare diseases.

Dr. Durhane Wong-Rieger, president of CODR, disclosed in a telephone interview that Health Canada has recently drawn up a draft orphan drug policy, which it released on December 1, 2009.

If approved by the government, the policy will adopt many of the same standards used by the European Union, which are very advanced in terms of how they fund, and ultimately, make available, orphan drugs to the public, she says.

“In terms of meeting top international healthcare standards,” say Wong-Rieger, “this orphan drug policy is just the starting point for Canada.”