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Chair

Mr. Bill Casey

Standing Committee on Health

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● (0910)

[*English*]

The Chair (Mr. Bill Casey (Cumberland—Colchester, Lib.)): We'll commence our 119th meeting of the Standing Committee on Health. We're going to continue our study on barriers to access treatment and drugs for Canadians affected by rare diseases and disorders.

Today, we have two panels. The first panel has two organizations, and then at 10:10 or so, we're going to have the Department of Health come in.

In our first panel, we have Erin Little, the president of the Liv-A-Little Foundation. Welcome. From the atypical Hemolytic Uremic Syndrome Canada, we have Mary Jane Vowles and Caryn Vowles, board members. Welcome.

I'm going to invite Ms. Little to make a 10-minute opening statement.

Ms. Erin Little (President, Liv-A-Little Foundation): Mr. Chair, members of the committee, thank you very much for having me speak to you about how we've been directly affected by life with the rare disease cystinosis.

I would also like to personally thank MP Ben Lobb for caring about our ongoing issue since the first day that our medication access became an issue. We wouldn't be here today if he didn't have an interest in our family's battle.

I'm the president and co-founder of the Liv-A-Little Foundation, which was founded in 2013, two years after our daughter was diagnosed with the rare, genetic, metabolic disease cystinosis at the age of one.

Liv-A-Little Foundation is committed to supporting the advancements of treatments and ultimately a cure for cystinosis, by educating, promoting and funding progress. I'm also a board member for the Cystinosis Research Foundation in Irvine, California, where they are currently funding the most progressive research in our cystinosis community. We are proud to partner with them to fund global research, some of which is proudly Canadian.

My most important role, however, is being Olivia Little's mom. Olivia has cystinosis. Since the day she was born, I have taken my role seriously, and even more so when we received the devastating news that she had a life-altering disease with a life expectancy of 27 years young. I committed myself to caring for her and advocating for her until she can one day take that role on herself. When she was first diagnosed, I believed that advocacy meant parenting her with her father, collaborating with her pediatric nephrologist and medical team, and providing her with proper nutrition, a healthy environment and a strong sense of normal, in spite of living with a rare incurable disease.

I had no idea that in addition to all of that, I would have to fight for medication access here in Canada. On July 4, 2011, we nearly lost Olivia due to acute kidney failure. We had already been in and out of the hospital three times a week, tirelessly pursuing answers to her failure to thrive since she was three months old. That day, July 4, we got lucky that the final doctor ordered blood work.

We were told to go to the hospital immediately and be prepared to stay for a few days. We hoped for and expected a quick recovery, but those few days turned into a month-long stay at the Children's Hospital in London. When we left, we did not have the healthy child we expected. Instead, we had a diagnosis with no cure, a grocery bag full of medications that I could barely pronounce, eye drops required hourly, and a heart full of information, sorrow and anxiety.

We learned that cystinosis is a rare, incurable, metabolic disease affecting only 75 to 100 Canadian children and young adults, and approximately 2,000 worldwide. In patients with cystinosis, cells cannot release the amino acid cysteine from their cells. In people without cystinosis, proteins degraded with the lysosomes of cells are transported from the lysosome to the cell's cytoplasm via specific transporters. The cells of those with cystinosis have defective transporters, causing the cysteine to crystallize within the tissue. The crystal buildup eventually destroys all the body's organs, including the kidneys, liver, muscles, white blood cells, eyes and central nervous system.

Without specific treatment, Olivia, like all those with cystinosis, will progress to end-stage renal failure by an average age of nine years old. In the past, this meant childhood death. Now these patients can receive renal dialysis or renal transplantation. However, even with successful renal transplantation, these children go on to develop abnormalities in their organs.

It is with enormous gratitude that we learned about the drug cysteamine, which slowed the progress of cystinosis by removing the cysteine from the cells. Cysteamine is the active ingredient in Cystagon, which was the first treatment of cystinosis and still is one of the only two treatment options today.

However, in order for the drug treatment to be effective, it must be taken every six hours. Although this has led to a much better future for these children, cysteamine is not a cure. When we administer it every six hours, we're always reminded that while the progression is slowed significantly, cystinosis still progresses in Olivia's body.

We adjusted to our new normal and all of its side effects, including interrupted sleep, constant medical preparation, and attention to Olivia's growth and eating along the way. This normal became routine, and our daughter thrived under the regime of electrolytes and Cystagon, which was primarily a life-sustaining medication to slow the unrelenting progress of cystinosis. This routine is hardly normal, but it worked well and she was healthy.

On November 7, 2017, we received a letter that introduced another level of fear to this rare disease situation. It was a letter which stated that here in Canada, Olivia could no longer access her life-sustaining treatment of Cystagon. This letter arrived five months after the new drug Procysbi—claimed to be the same drug as Cystagon but merely administered differently—was approved by Health Canada. The price tag for Procysbi, however, came at a hundred times the cost. When questioned about this extreme discrepancy, the response was that this new drug is a breakthrough drug.

Health Canada provided Procysbi without considering that they were replacing the drug Cystagon that was both physically effective and cost-effective. The company that produced it, Horizon pharmaceuticals, entered the Canadian market with an extremely overpriced drug.

In the U.S., Horizon already raised the price of Procysbi by 9.9% in January 2018, with another anticipated increase in January 2019. Pharmaceutical companies are allowed to increase the price of drugs by 10% per year in the United States, and Horizon is certain to go as close to its margins as possible.

I do support building healthy relationships with pharmaceutical companies and want new drug advancements for children and adults with diseases and illnesses, both rare and common. If a drug can enhance the quality of life for our fellow Canadians, we need to find a way. However, we need to hold companies to high standards of ethics, as well. Health Canada seems to have placed high standards on drug efficacy without considering the integrity of these companies that are benefiting on the backs of vulnerable populations.

To my knowledge, Horizon has one fellow Canadian employed here in our country, which is not contributing meaningfully to our local economy. With a drug at the price of Procysbi, we should expect the company to contribute to our local economy as well as mandate that it conduct research and development to improve life among the rare disease population.

In our capitalist democracy, being a for-profit company is expected and acceptable, but we must have higher expectations for pharmaceutical companies than price gouging patients and, more importantly, our taxpayer dollars. By approving Procysbi without an effective and all-encompassing understanding of the company, its ethics and history, Health Canada made a decision that will have an enormous effect and impact on cystinosis patients and Canadian taxpayers.

This is a policy issue. Policies are made for people, and not the other way around. If a policy is going to remove choice, security and health from Canadian citizens, then it is a policy that should be changed, and errors made by that policy need to be rectified to protect Canadian citizens.

On a more personal and immediate note, if our family made the switch from Cystagon to Procysbi, our original costs of $14,590.80 per year for Cystagon would now be over $300,000 per year for Procysbi, all of which the Province of Ontario covers for patients with cystinosis.

Adding Procysbi to the list of available cystinosis treatments would be a win for everyone, because the case of each is so different. However, Procysbi was not added to a list of treatments. Procysbi replaced our current treatment entirely.

Procysbi is not the same drug, although Horizon would like us to think it is. Its administration and dietary restrictions are only two challenges patients face when they switch.

Cystagon has been an effective medication in our case. While it is administrated every six hours and is taxing on our sleep and overall quality of life, Olivia's health has been unbelievably stable on it. She experiences very few side effects. We are extremely proud of the track record, as creating and maintaining her diet to minimize constant vomiting and headaches is very involved and tricky for us. She has been so stable, in fact, that we haven't had to adjust her Cystagon treatment since August 2015. The medication she takes, along with our constant compliance to the administration, is doing its job. When the time is right for Olivia, Procysbi should be available as an option. Remaining on Cystagon should also be an option.

The bottom line is that the patients with cystinosis and their families should be the ones selecting treatment in collaboration with their nephrologists. No one knows their children better than the parents, and no one knows how the children respond to treatment or the impact of treatment on the family than the parents. They should have primary decision-making ability in the treatment for their child, or in the case of adult patients, for their own treatment.

It seems that our Canadian system eliminates the power of choice for the parents, for which Health Canada says it advocates. Even Canadian doctors and medical specialists, who have been licensed by our government and have given oaths to provide best for their patients, are not given the authority to choose patient treatment for the patients they know so well.

When Procysbi was approved and Cystagon was so abruptly removed from Canada, and our letter of cancellation was issued, our doctor was shocked, because she had not been informed about the approval of Procysbi, and did not necessarily feel it was the best choice for her patients. When our nephrologist spoke with someone from Health Canada, giving verbal medical reasoning for Olivia to remain on her current treatment, she was denied that choice, leaving us terrified about what to do next. We were stunned that someone, however highly educated, sitting in an office, who did not know cystinosis or our child, was able to make a decision overruling our child's physician. As Olivia's primary caregiver and someone who trusts our doctors and medical system, I was disgusted that our physician was not trusted to make the most important decision for her patient.

We, as Olivia's parents, have adjusted every aspect of our lives to take the best care of our daughter, keeping her healthy and out of the hospital, and a broken policy and someone who does not know the first thing about cystinosis was able to make a life-altering decision against our will.

I'm not saying we have the answers. In a perfect world, there'd be no disease. In a perfect world, cystinosis would not exist. Until that time comes, though, let us focus on perfecting what we do have, and correcting policies that put pharmaceutical companies before patients and policies before people.

All lives matter. There has to be a way to correct the mistakes made last year, and if there isn't currently a way, then it's time to pave one.

● (0915)

Again, I would like to thank everyone for inviting me to address the committee. On behalf of our organization and the cystinosis community, we are grateful to see the rare disease community on a potential pathway to better the lives of those with rare disease.

The Chair: Thank you very much for sharing your story. It's certainly quite a tale. You've been through a lot.

Now we'll go to Ms. Vowles, for a 10-minute opening statement.

Ms. Mary Jane Vowles (Board Member, Canada, atypical Hemolytic Uremic Syndrome Canada): Thank you very much.

My name is Mary Jane Vowles and I'm one of the volunteer members of the board of aHUS Canada.

My daughter has aHUS, also known as atypical hemolytic uremic syndrome. This ultra-rare, life-threatening disease is a disorder of the immune system that can damage or destroy any organ by creating blood clots that stop the normal flow of blood to the organ.

At six months of age, my daughter developed flu-like symptoms. The pediatrician on call diagnosed her with the flu. The next day, there was blood in her urine. I took her to the family pediatrician, who diagnosed aHUS, and she was admitted to hospital.

Over a week, she received several red blood transfusions, appeared to stabilize and was discharged. Two weeks later, the flu-like symptoms returned. The same pediatrician was on call and claimed the first diagnosis was incorrect and repeated that she had the flu. The next day my own pediatrician sent us to SickKids.

In the next few days, Caryn's kidneys shut down and a nephrologist diagnosed aHUS. They installed a central line, followed immediately with dialysis, and then plasmapheresis. Her blood pressure was out of control. Medicines didn't work. After two weeks, they were able to cease dialysis and weaned her off plasmapheresis, replacing it with treatments of plasma infusion.

After six months, she was discharged, returning weekly and then bi-weekly for infusions of plasma. Many attempts were made to increase the span to three weeks, but each attempt failed and the aHUS recurred, requiring readmission to the hospital and plasmapheresis. She frequently had reactions to the plasma and went into anaphylactic shock many times.

When Caryn was in grade 8, she developed antibodies to that plasma and again was hospitalized for six months. After many unsuccessful attempts with treatments, they finally succeeded, using IVIG prior to plasma. She began peritoneal dialysis.

In grade 11, Caryn began a trial of eculizumab, also known as Soliris, injected every two weeks. It was sponsored by Alexion. Life was good.

At 18, she was transferred to the adult patient world, with a nephrologist from Credit Valley Hospital. She continued to receive the eculizumab treatments through SickKids.

In Caryn's second year of university, she fell, rupturing the tendons in both of her knees. In emergency, she received dialysis in hallways, despite protests.

She had successful surgery at Credit Valley Hospital. After a few weeks, she developed pains in her stomach while here in Ottawa and was diagnosed with aspergillus. She was airlifted back to her nephrologist at Credit Valley Hospital.

In the meantime, her father had been convinced to have the cost of the eculizumab covered by his health insurance plan. This had been done for several treatments. The insurance company would not cover the cost when she was admitted to hospital, and Credit Valley Hospital refused to cover it. At $750,000 a year, the family budget could not afford it. The nephrologist at Credit Valley knew nothing about aHUS and the hematologist there refused to see her or look at her case.

The experts in that adult field were at Toronto General and Saint Mike's. Both hospitals refused to admit her because of the cost of the drug. After a battle, the chief of staff at Credit Valley facilitated a transfer to TGH under the condition that they would not treat her with eculizumab. Instead they would use a detergentized blood to minimize reactions and prevent recurrence.

She was in hospital for five months, had many allergic reactions, and some were severe.

In January 2014, the aHUS recurred. She was put back on eculizumab under her father's plan. In May 2014, she no longer had insurance. Alexion, the drug company, agreed to continue the eculizumab on compassionate grounds. It continues to do so.

Caryn had to go on to hemodialysis. She's often in extreme pain as a result of the dialysis and has debilitating headaches.

Caryn is now 25. She has a degree in biomedical engineering from the University of Guelph, where she will shortly complete her master's degree in applied science. She has been accepted with a scholarship to complete a Ph.D. in biomedical engineering at Queen's.

Caryn's success story is only possible because of eculizumab.

● (0920)

Recently there have been other success stories in young patients treated with eculizumab as soon as diagnosed and they've been able to recover completely and resume absolutely normal lives. One 12-year-old was admitted to SickKids' coronary unit, vomiting and enduring internal bleeding. He suffered a blood clot and was put on oxygen. He received blood transfusions and plasmapheresis. His kidneys failed, requiring dialysis. Once diagnosed and treated with Soliris, his health improved. His kidneys recovered completely.

The onset can be very different for patients, and the age of diagnosis can vary. One patient was misdiagnosed with another ailment, lost kidney function, and then had a transplant and seemed to be doing well. Soon he became ill and also lost the kidney. Further testing showed he had aHUS. He has had multiple relapses, is on dialysis now, and has been approved under the Ontario government new format to receive the drug. The administrative team at the hospital are delaying. There's no guarantee the government will fund the drug continually following the transplant. A recurrence would lead to kidney loss and perhaps death.

In March 2013, after its extensive testing process for safety, quality, production and efficacy, Health Canada approved eculizumab. The Province of Quebec began to fund the drug immediately. Other provinces awaited the CDR report from the Canadian Agency for Drugs and Technologies in Health. Five months later, the CDR recommended provinces not fund the drug due to its high cost and lack of evidence of efficacy, criticizing the lack of using a placebo on a control group. This was purposely not done, because aHUS can be lethal. aHUS Canada questions why a drug that science supports, that is very safe and effective, was not recommended for these treatments.

Under the leadership of the deputy minister of Ontario public drug programs, meetings were held with aHUS Canada to create possible solutions. Headway has been made in some areas and patients are receiving the drug for transplants. Though most provinces now fund the drug, the issue is that patients are now being removed from the drug arbitrarily without scientific support or doctors' recommendations.

There needs to be a separate program that evaluates health technologies for rare diseases, as they so differ from common disorders. The CADTH should have a rare disease review in addition to its common drug review and the pan-Canadian oncology drug review. Just as PCODR was created due to unique needs, so should RDR. Without this change, rare disease therapies will be evaluated against the same criteria as common diseases, and this is unfair. The same robust statistics will never be available in rare illnesses because of the low number of patients, and that also will increase the cost of the therapy. A different viewpoint is needed.

Four problems face patients. The first is the difficulty of a quick and accurate diagnosis of the illness. We live in a vast country where not all patients can reach a major city for that diagnosis and treatment. The second is timely access to a drug for a disease that can permanently damage organs and cause death within days. Third, as long as specialists do not have decision-making capability for the dosing of eculizumab, patients remain at risk of recurrence. Finally, the cost of the drug needs to be addressed.

A world expert doctor from SickKids has offered a solution that seems plausible for the treatment of aHUS. He made the suggestion for Ontario, but I believe it should be considered as a Canadian option. We need a centre or hub here in Canada where blood can be tested for illness promptly and results returned to the physician. The centre would have at least three expert doctors in the field who are involved in current studies and research. Tests for the illness could be done promptly and effectively. Upon confirmation of a diagnosis, these experts would make medical treatment decisions, instead of provincial governments. This could prevent strokes, heart disease and kidney failure and reduce costs for hospital stays, dialysis, patient home support and disability payments.

● (0925)

Through this centre, patients' response and well-being could be monitored and drug treatments could be decreased in dosage or frequency as deemed beneficial. This model would follow one that is working in England.

Last, if Canada, as a country, negotiated the drug cost, prices could be further reduced. Other drugs are currently being developed, and research is promising, but at this point the only drug known to treat aHUS and change the lifetime prognosis of patients is eculizumab. It's time for Canada to recognize those with rare diseases and find solutions that are appropriate for 2018. We need a country-wide plan so we do not discriminate against the minority who have a rare disease.

Research has come a long way in 25 years. When Caryn was diagnosed, the statistics were that there were 100 patients in the civilized world with atypical HUS and over half of those had died. Recent research suggests that aHUS affects about 200 patients in Canada. It is possible to change the outcome for patients with aHUS.

I trust I have successfully addressed the barriers that patients with aHUS face, suggested recommendations, and stressed the need for action. Eculizumab must be made available to aHUS patients through public funds.

I thank you very much for listening to us today.

● (0930)

The Chair: We thank you for sharing that with us.

I'm going to start our seven-minute round of questions with Mr. McKinnon.

Mr. Ron McKinnon (Coquitlam—Port Coquitlam, Lib.): Thank you, Chair.

I'm going to start with Ms. Vowles.

You mentioned the suggestion of a national centre, a hub for blood testing. I was wondering if you could speak more about how that might work. Would all children get tested as a matter of course, or how would one determine when to use this service as a system?

Ms. Mary Jane Vowles: There are certain symptoms of blood breakdown and the mechanisms in it that are different in aHUS from in other illnesses, so the blood would be sent to the centre and they would be able to diagnose aHUS, because it's very difficult to diagnose.

Caryn, do you want to add to that?

Ms. Caryn Vowles (Board Member, atypical Hemolytic Uremic Syndrome Canada): There are a bunch of tests. I think one of them is for ADAMTS13. If you were having problems, that would also distinguish between ITP and aHUS. There are some tests that would go with those symptoms, and you'd send this test in and it could determine which disease you have.

Mr. Ron McKinnon: This hub concept is not specific to aHUS. Is that correct?

Ms. Mary Jane Vowles: That would be correct. You could have other rare diseases that could also be covered by this hub. The situation we were looking at was more for diseases that would be related to kidney or nephrology.

Mr. Ron McKinnon: It would require the physician to know that they didn't know, right? It seems in both these stories there were cases in which one physician made a diagnosis and was quite comfortable with that. In your case, it was a diagnosis of flu. If they're confident in that diagnosis, they're not going to go the next step to get blood tests, right? I guess I'm wondering how—

Ms. Mary Jane Vowles: That would be correct, but I would say that once the illness persists and it's not looking like the flu, they would then take that blood and they would treat it like your blood or my blood or regular people's blood and they would test for all those other possibilities and when those possibilities aren't there, then they would send it to this centre.

What currently happens is that blood is being sent to the States, and it's taking much longer to get back the results, and so by that time it's not possible to diagnose. With the way the provinces work the funding, you have to put the funding request in and it takes days for them to come back with it. If you've already spent days getting your blood tested too, it's much more difficult, whereas this would do the two things simultaneously.

Mr. Ron McKinnon: Right now you can get that testing done in the United States, but this basically takes that service and reimplements it in Canada for Canadian domestic use. Is that correct?

Ms. Mary Jane Vowles: Right, and because the illness is rare, it also puts together the facts on current research. Some of the provinces are suggesting that you do not need eculizumab quite so regularly or that we should remove the eculizumab, and so once the diagnosis is there, if we're going to start increasing the distance between the treatments of eculizumab, we need to have a blood testing facility that's going to be accurate and quick so, should the illness recur, there would be instant access to the eculizumab again.

Mr. Ron McKinnon: I'm also interested in the high cost of these drugs.

I direct this to Ms. Little as well as Ms. Vowles.

Is there anything afoot to reduce the high cost of these drugs? As it goes forward, the drug has existed for a longer period and the company has had an opportunity for a return on its investment. Is there any evidence or thought that prices will go down or that there can be some relief to that?

● (0935)

Ms. Erin Little: Speaking in general about the States, which is the closest to us and we're the most involved with them, I know that Horizon pharmaceuticals owns the patent for the new drug Procysbi. It has a seven year...and now they've added another three-year extension. Basically, for the next 10 years the price will continue to go up.

I can't speak to that in Canada because I think once the price is set, it's set. In the States, they will continue to increase it to make a profit, which these companies are known for—the rare disease market. It's a niche market when you have 2,000 patients, or in Canada you have 75 to 100 patients. You need their drug. It's a product that our kids couldn't live without.

I would really like to hear Horizon pharmaceuticals answer the question of why it's so expensive and what they can do to reduce the cost. Logistically, it's not a drug that should cost a lot of money to make.

Ms. Mary Jane Vowles: In Erin's case, we don't hear of any upcoming credits or bonus sales going on for the drug cost. The cost is the cost. However, in our disease case there is current research going on with, I believe, three other companies to produce other drugs that look promising to be cheaper. They're just not available yet. Down the road they may work just as well as the eculizumab but eculizumab is the only one currently available.

Mr. Ron McKinnon: I take it from both your testimonies that one of the problems here that needs to be addressed is the ability to have drugs approved in Canada for use, particularly for the unique circumstances of rare diseases. Is that correct?

Ms. Erin Little: That's correct.

Mr. Ron McKinnon: Those are my questions.

The Chair: Mr. Lobb.

Mr. Ben Lobb (Huron—Bruce, CPC): Thank you very much.

I have a question for both parents who are here.

Erin, how much per year is the cost for the Cystagon?

Ms. Erin Little: We are just under $15,000 per year.

Mr. Ben Lobb: Who pays for that? Does the province pay for that?

Ms. Erin Little: Under the inherited metabolics disease program Olivia's Cystagon is covered. With the new approval, Procysbi is as well. Our access to Cystagon is through SAP right now. Currently, we have to apply every three months for Olivia to stay on this treatment as it's flawless for her. That is covered under that program.

If our family were to switch to the new drug, in Ontario specifically, or if any other families switch to the new drug, it would also be covered under the same program—the new drug, as well.

Mr. Ben Lobb: It's $300,000—

Ms. Erin Little: Yes.

It's roughly calculated from the price that we had to search tirelessly for. That is what Olivia's dosage would come to.

Mr. Ben Lobb: Okay.

Mary Jane, does the province pay for the drugs that Caryn takes now or do you have insurance?

Ms. Mary Jane Vowles: Caryn is currently under Alexion.

She started in grade 8. When she started on the drug, she was part of a child project with two kids from SickKids and six children from other countries. When Alexion went back to giving her the drug compassionately, it was based on my fighting with them over a promise they had made that they would continue her on the drug. They have her on that now, but she could be dropped at any time, just as they dropped her the other times.

Mr. Ben Lobb: Are you aware of any other people in the country taking this drug who are covered, or is it all under the compassionate label?

Ms. Caryn Vowles: Some are covered by the Ontario government. They have a whole bunch of outlined rules, and if you don't meet certain criteria, they take you off the coverage. One of the main criteria problems is that after a transplant they can cut coverage after, I think, six months or something, and if someone's illness were to reoccur, they would lose the transplant.

● (0940)

Ms. Mary Jane Vowles: The illness reoccurs, and whenever the illness occurs, it's death. You're always on death's doorstep as it reoccurs.

Mr. Ben Lobb: Erin, for Olivia, how many pills per day does she have to take to maintain a quality of life?

Ms. Erin Little: She is probably at 50 pills a day to maintain her health.

I want to add, if I may backtrack—sorry—to what the issue is here as we talk about pharmaceutical companies coming here, and as Mr. McKinnon said. You asked about the cost. The pharmaceutical companies are two steps ahead of us. The other downfall is that when it comes to these rare disease markets and there are only 75 patients to sell something to, I wouldn't start a business to sell something to 75 people.

What they'll do is they'll come in under SAP as well. The pharma companies want to offer that compassionate usage of their products until they are approved so that we have something rather than nothing, but then, in all the fine print, they say that once a patient comes under their compassionate care, they remain there. Even when it's approved, the insurance companies won't pick up the cost because the company has already agreed. If a company comes in and offers our daughter a new product and, say, 35 people go on SAP, they don't have many patients to continue to build a business offer.

I think it's important to add to that, because it's another really big issue with pharma companies wanting to work with us as well as trying to build a business, make a profit and potentially hopefully continue to give back to rare disease communities.

Mr. Ben Lobb: Caryn, how many do you have to take?

Ms. Caryn Vowles: Pills?

Mr. Ben Lobb: Yes.

Ms. Caryn Vowles: I'm down a lot, but I do hemodialysis at home every day.

Ms. Mary Jane Vowles: Caryn's pills are for high blood pressure. The actual eculizumab is every two weeks. There's an infusion of eculizumab every two weeks.

Mr. Ben Lobb: I think it's important to highlight the fact that, as I'm learning as the process goes, if not for the parent advocacy for their child, the future is almost certain for the children as they are impacted by this. Maybe both parents could talk briefly about your own personal commitment—I'm not saying it's a sacrifice—to your children to make sure.... As for some of the sacrifice, maybe that's meant your own career that you had in mind.

Ms. Erin Little: I'm grateful that we're here today. I know that having patients come here and speak is relatively new to this kind of situation. I guess I really never knew what advocacy meant until I started in the role of doing it. It takes time away from my children, but I'm grateful to be here. I'm grateful that you guys are willing to listen to a different perspective that's not a medical or educational one. It's more about experience. At the end of the day for all this stuff, we're the ones who are impacted, yet nobody understands the full impact.

We came from Port Elgin. It's a trip to get here. We had to leave our kids and entrust them to somebody else, which I'm okay with, but at the end of the day.... I couldn't be more proud to do it, but we shouldn't have to do it. There's not enough listening to the patients.

I understand. If you took one patient from each rare disease, you would have thousands of people and you couldn't listen to all those people, but it's about listening to the people who are actually affected, not just the players. I have no skin in the game. I'm here away from my family. I am actually losing to be here today.

I think it's important to find that voice of the patient population and integrate that with what we're doing with Health Canada and policies and procedures in making a system that works for us. I know that it can't be perfect for everybody, but it has to work in a better way than it is today.

Ms. Mary Jane Vowles: I've been a single mom since Caryn was three. I had to keep my job; it wasn't a choice. Caryn is one of three children.

All those months she spent in hospital, I was there with her every night. I left work. I drove to SickKids. I would get up in the morning at six o'clock and head back to work. I did that for months on end.

That affected my boys. I found out more about that this weekend than ever before. They talked about the take-home meals they used to endure. We were referring to Little Caesars. They had buckets of pasta. I haven't had buckets of pasta in years, since she's been on this. But they remember that. They remember the Toonie Tuesday, because that's what they got on Tuesdays. I love cooking, but I wasn't able to do that when my boys were young.

I know this illness gave me a lot of strength that I didn't know I had. Part of the illness over Caryn's years was that there was no clear-cut treatment, no clear-cut answer. There were points when there were things happening where....

For instance, there was one apheresis machine in Toronto, and it got wheeled from Toronto General to SickKids in the underground tunnel. It could do two treatments a day. They decided after they'd started Caryn on it, and she was six months.... Maybe it was the first one they'd ever done this way. It was very scary, although I didn't know enough to be afraid. They wheeled it back. They did it for five days in a row. Then they decided that another patient needed it, and she wasn't going to get the machine. I was going to have to talk to the media and show my blue-eyed, blonde-haired little girl. In the end, the machine came back to Caryn. I have no idea what happened to the other patients who were supposed to be on it.

Through that, we, together.... When I say we, the doctors and I worked together on a plan that was agreeable to both of us in stretching out the treatments on the apheresis machine and trying whatever together. Together, we created research through this illness.

I don't mean to sound smug or smart ass, but I was the one who linked the nephrologist at SickKids to Dr. Kaplan at the Children's Hospital of Philadelphia at the time, so they could find out more about the research. All there was about Caryn's illness back when she was six months old was one chapter in a textbook, which they gave me to read. It was way over my head, but I learned to understand it. We've come a long way since then.

Certainly, lots of treatments are working, and the doctors know a whole lot more than they did when progressing through her illness. When she was diagnosed back in the day—I remember we could go on the Internet—there was one other case, and it was of another blue-eyed, blonde-haired little girl, but she did not survive the illness, the aHUS, the way Caryn did. Most kids, as much as they survived, were left vegetables. We've moved beyond that.

● (0945)

The Chair: I have to end it there. Sorry.

Mr. Davies.

Mr. Don Davies (Vancouver Kingsway, NDP): Thank you, Mr. Chair.

First, the voice you're bringing to this committee is every bit as valid as what we hear from the medical side, and I want to commend you on the information and the education you're bringing to this committee. I think you probably know more about the system and about the conditions you're dealing with than many people in the health care system. I want to thank you for being here and sharing your story. It takes a lot of courage and sacrifice, as you've said.

I want to start by trying to understand the finances here.

Ms. Little, how much does Cystagon cost per year?

Ms. Erin Little: For our daughter, we're just under $15,000 a year.

Mr. Don Davies: How much does Procysbi cost per year?

Ms. Erin Little: Her dose would switch to potentially $300,000, because it's a cost per pill.

Mr. Don Davies: Yet if I understand your experience, Cystagon was working very well for your daughter, but the system wanted to switch her from Cystagon to Procysbi at many times the cost. Do I understand that correctly?

Ms. Erin Little: That's correct.

Mr. Don Davies: Did anybody from Health Canada or the system explain to you why they wanted to move your daughter from a drug that was costing $15,000 a year and was working to one that you didn't know whether it would work and would cost $300,000 a year?

● (0950)

Ms. Erin Little: This is where it comes down to the bureaucracy of business and Health Canada. It was basically because the new drug was approved. SAP is there for special access when there are no drugs currently available in the country to take.

Procysbi was approved in July 2017, and we went to get our next dose filled in November. That's when we got our letter of cancellation, strictly based on rules around drugs coming into the country and having exclusivity, because they went through the process. That is the reason they wanted to switch us to Procysbi.

The other thing with Procysbi is that the ingredients of the drug are the same as Cystagon. It's the same drug. The only difference between Cystagon, which Olivia is currently on at $15,000 a year, versus Procysbi, is that it's enteric coated. They enteric coat it so that you only have to take it every 12 hours versus every six hours.

I know when you have children, the dream is to sleep. I gave that up when I had kids to begin with, but at the end of the day, they call it a breakthrough drug even though the ingredients are the same. This drug would not even be on the market if it weren't for families like ours and others across Canada and the U.S. who were fundraising to make this drug. We funded this drug to happen to begin with, which is a real kick if you're somebody like us who just wants the best for your child.

This drug, although it has the same ingredients, is slowly released over 12 hours, so you only have to take it twice a day versus six times a day. But in the fine print that nobody else reads is the fact that, with this drug, I have to limit my eight-year-old, who already has a hard enough time eating food, because the medication, the same ingredient, makes her nauseous. On top of that, we have to limit her food intake for eight hours in a day, an eight-year-old. I bet most of you couldn't sit around this table and limit eight hours of your waking time...to schedule your life around taking just one medication.... Olivia is on six other pills that have to be taken, too, which we also have to stagger.

She's on sodium bicarbonate. You can't take sodium bicarbonate with any kind of slow release because the bicarbonate would dissolve that drug immediately in her stomach and she would get a double dose, because with this new breakthrough drug, Procysbi.... I lost my train of thought.

Mr. Don Davies: Ms. Little, can I come back to that? I want to just follow up with the same kind of question for Ms. Vowles.

I'm trying to get a handle on the price of eculizumab versus Soliris.

Ms. Caryn Vowles: They're the same drug.

Ms. Mary Jane Vowles: They're the same drug. They just have a different name.

Mr. Don Davies: Okay, so that is the same drug.

A 2015 article in The Globe and Mail said this:

> Canadians with atypical hemolytic uremic syndrome (aHUS), a life-threatening genetic disease that damages vital organs and affects fewer than 100 people in the country, often require treatment with the drug Soliris at a hefty price tag of more than $700,000 a year. The cost spurred the federal Patented Medicines Price Review Board to call a public hearing into why the manufacturer, Alexion Pharmaceuticals, charges more in Canada than in other developed countries

Do you have any information to share with this committee on the outcome of that PMPRB hearing? Did they determine that the price of Soliris is excessive?

Ms. Mary Jane Vowles: I do not know.

Mr. Don Davies: Does it cost $700,000 per year?

Ms. Mary Jane Vowles: Yes, my understanding is it costs $750,000.

Mr. Don Davies: Ms. Little, I'll go back to you, and whenever you get your train of thought just pipe in.

Ms. Erin Little: Okay, yes.

Mr. Don Davies: You mentioned that your family is forced to reapply every three months to continue to receive the life-saving drug. In the past, your family had to apply every six months, and it was all but guaranteed you'd receive that approval, which is no longer the case.

Can you outline why that changed the application process that was imposed by Health Canada?

Ms. Erin Little: Prior to all the changes, I didn't even know they had to apply every six months; honestly, I thought it was once a year. I would call the pharmacy when we were headed down to the clinic and I would just say that Olivia needs a refill on her prescription and there were no questions asked. It would be there, and every three months we would get it refilled. When everything came into place with Procysbi, they just changed the way we have to apply to every three months. Why, exactly, we were never given a reason. There are speculated reasons around the issue that they have to be cautious because there is a marketed drug in Canada so they have to be cautious on how and who they give access to Cystagon to.

We have clinic in two weeks, and I messaged down because I'm extremely proactive, because this drug Cystagon now comes from the U.K., so I have to worry about it being imported on time, getting stuck in customs and so on. These are all things that I choose to take on as a parent and worry, because I take such great care of my child. There are other parents who are really whimsical about it, and that's fine, but they're in situations where they have run out of drug and then they have to look within our community to help them until they get their supply because they have to go through this process. The disease isn't going away in three months. I wish it would. My understanding is that it's just a business; they have to watch.

● (0955)

The Chair: Now we go to Mr. Grewal.

Mr. Raj Grewal (Brampton East, Lib.): Thank you, Mr. Chair.

Thank you, witnesses, for sharing your stories.

As my colleagues have already said, I think everybody around the table can agree, across party lines, that when we hear more of the patient experience, it definitely gives us an opportunity to try to go back to the table and fix some of the—I hate to say it—more common sense things that need to be fixed in government bureaucracy.

Ms. Little, your story is one we hear too often, not just in the medical scenario but in a lot of scenarios in which the government makes things more complicated than they need to be when people are just trying to help their children or help vulnerable people in our communities.

I wanted to ask, along Mr. Davies' line of questioning, about the cost of the two drugs and the fact that this new drug has this time release formula in it and, because of that, it's become so much more expensive. You may not have the data, but how many people have this type of rare disease in Canada? I know it impacts children, mostly.

Ms. Erin Little: In Canada, there are between 75 and 100 patients.

Mr. Raj Grewal: There are 75 to 100. What about in the U.S.?

Ms. Erin Little: In the U.S. there are roughly 500.

Mr. Raj Grewal: We're looking at a very small number for any pharmaceutical to be developing a drug, because it's not going to be profitable, no matter how many patients there are, when you're playing with such a small pool.

The provincial government is subsidizing the full cost of the medication today. Is that correct?

Ms. Erin Little: In Ontario, yes.

Mr. Raj Grewal: Is that not the case across the country, though?

Ms. Erin Little: No, each province is different on who covers it and and how it's going to be covered. It's relatively new. They announced this within the past four to six weeks that it's going to be covered this way.

Mr. Raj Grewal: Okay, do most of the 75 patients live in Ontario?

Ms. Erin Little: No, we're spread out. There's one patient in Saskatchewan and a few in the Maritimes, and Quebec has the highest population of cystinosis; how many exactly, I'm not sure, but we do know that they have a large population there. I know, Dr. Midgely, who was here in front of you as well, has 18 patients. Our nephrologist has four of us in her clinic.

Mr. Raj Grewal: I don't like making assumptions, but I want to ask you this. Are you in contact with families that are going through the same thing in the U.S.? Do you have any comments on their experiences? Are some of the states doing a better job at this than we are?

Ms. Erin Little: Yes, in the States, where the biggest issue lies right now is.... Yes, they get it if they want it, if their insurance will cover it, right? Some patients pay $92,000 a month to be on this drug in the U.S. Yes, that's what it costs a month to be on this drug, but the insurance company covers it. But they have the choice. They have the choice to stay on one. In Canada, people will say to me.... The company that now owns Cystagon—it was bought recently by a new company from the U.K.—but they haven't applied. I can't be punished for somebody else's business decision, and that's exactly what is happening in our country.

This drug Cystagon has been around for 35 years and it is trusted. It works. It's an awful drug to want to be on. Some days I can't believe I fight for it. I should be fighting for a new drug. So that's the biggest thing.

In the States they have a choice and there are families for whom the thought of every 12 hours is appealing and they switch to this drug because the other fear was, what if we did want to switch Olivia? What if we said she's ready and we want to switch her?

Our fear is what happens if there are really bad side effects. It has happened in the States. Their white blood cystine levels go all over the place and it just causes an upheaval in life.

There are families in Ontario who have considered switching and they should have the right to switch as well. I'm not against it being here, but they're wondering what happens if they put their two-year-old daughter on this new drug and the side effects are so bad and they can't switch back. What if they don't let them switch back? That should not be something we have to worry about. We just want to keep our kids alive and healthy, and now we're worried because we want to try this new drug but we can't.

● (1000)

Mr. Raj Grewal: That's a fair recommendation.

My last question is for both Ms. Vowles and Ms. Little.

What's the one recommendation you would like to see the government follow to make your lives easier?

Ms. Mary Jane Vowles: Our personal life or the life of all aHUS patients?

Mr. Raj Grewal: Either or both, to be honest.

Ms. Mary Jane Vowles: I would like to see the drug funded so that at this point in life I don't have to worry. Caryn can have a transplant. She could get the missing part of her life back in place, and not have to worry that the drug company or the Province of Ontario will cut it out after three months or whatever.

We have a history of every two weeks; that's all we can handle. That's all her body has ever been able to handle, no matter what, and I would like her to have the security to go on and be a productive member of the research world, where she seems to be headed, and be able to do great things for all of you.

Ms. Erin Little: For us in this case specifically, and there's a lot that I don't know, but I'm also trying to raise and home school two children and take care of a sick one. There's a lot that I wish I understood more about, but from what I do see and have felt the direct effect of, with drug companies coming into Ontario, something needs to change. We need to make sure we're two steps ahead of the pharmaceutical companies that want to come here, because they're always two steps ahead of us. That is something we need to be extremely cautious about. I'm so grateful to live in Ontario where we do have coverage. If we were forced to switch, we could switch to this high-priced drug and our daughter would still be on it.

The other piece is that when we welcome these companies to come to our country, they need to provide more to us, especially when their price tags are so high. They should be building business here. We shouldn't be paying for these high-priced drugs and then all of their business is going to other countries. They need to give back to the rare disease community as well. They should be advancing... again, we need pharmaceutical companies for advancements for our kids. But it's actually embarrassing that these companies come. They don't have to produce their drugs here. They don't have to employ people here, and even in our situation with Horizon pharmaceuticals, our potential patient support is in the United States. They don't even understand how each province is different.

There's that part. We need to have higher standards for the companies that come in, and we need to make the process more cohesive. The fact that a drug was approved in June 2017, and the potential treatment was taken away from us at that time, and there was lots of back-pedalling and trying to fix things, and then the price was just approved, and how we were going to cover it, over a year later.... It's embarrassing actually.

The Chair: Okay, thanks very much.

Now we have time only for one question, one question and one question.

We're going to start with Ms. Gladu, for five minutes.

● (1005)

Ms. Marilyn Gladu (Sarnia—Lambton, CPC): Thank you, Mr. Chair.

Thank you to the witnesses for sharing their stories. I'm going to summarize what I think I heard.

First of all, it's ridiculous that you have to apply to the special access program every three months for a drug that's prescribed by a doctor for a lifelong condition. That has to be fixed.

Second, it's ridiculous that CADTH is approving a drug that is 20 times the price and is the same exact chemical. Yes, you get something for technology of slow release, but being able to prevent you from continuing on a drug that was working I think is ridiculous as well. That's something that should be addressed in the recommendations.

Then, I think there is the question of funding and how we are going to be able to afford this. I agree that people need these drugs to live, but if I do a little quick calculation for the 27 life expectancy years for cystinosis and the more expensive drug, that would be $810 million. For the drug for aHUS, that would be $4.5 billion, if we think that people could live 30 years.

That is a huge amount of money, and as we see more and more solutions for rare diseases, we're going to have to come up with a way to fund these.

Would you agree that those are things we need to address?

Ms. Mary Jane Vowles: For sure.

Ms. Marilyn Gladu: Very good.

Does the Horizon price have something to do with the recent renegotiation of NAFTA? Was this the company that was involved there and wanted to extend the coverage? I think they used to get 10 years of coverage on the patent and now they have lowered it to eight years. Is that Horizon, or is that a different company?

Ms. Erin Little: I can't say.

Ms. Marilyn Gladu: You don't know. All right.

The last thing I want to ask is about the PMPRB process.

I think it was you, Mary Jane, who talked about the PMPRB when Soliris was being approved there was something they did that was not good. Can you tell me again what it was that they did that wasn't good and what we should do to fix that?

Ms. Mary Jane Vowles: What happened was that it was approved by the federal government, by Health Canada, and then when it went to the provinces, it was decided by CADTH that it wouldn't be approved because it was too costly and wasn't effective.

All the research said the drug worked. That was why Health Canada approved it. Why do we have two levels of government where one is undermining what the other said? If it's effective and Health Canada has approved it, how come a province can decide that no, it's not effective? Clearly all the research supports that it is.

The Chair: Mr. Ayoub.

[*Translation*]

Mr. Ramez Ayoub (Thérèse-De Blainville, Lib.): Thank you, Mr. Chair.

Ms. Little, thank you for your testimony. Let me reassure you. Earlier, you said that you took time away from your family to come and testify. I imagine it's the same for you, Ms. Vowles. This is time well spent, because it is very important for the committee to hear from families and to know what they are going through on a daily basis.

My questions are mainly for Ms. Little because I have had less time to research the Vowles family.

Ms. Little, while consulting your foundation's website, I saw that some of your supporters are pharmaceutical companies. It seems to me that you are the one funding the research to find a drug or a cure; that's what you said. How do you see your relationship with these companies that are both supporters and suppliers? Isn't there some contradiction between these two aspects?

● (1010)

[*English*]

Ms. Erin Little: That's interesting. We don't take pharma money to run any part of our organization.

We host a golf tournament to fund research, and for our tournament in 2017, Horizon pharmaceuticals was a sponsor of ours. This was prior to the issue and what I have learned over the past year. This year they did reach out to us and wanted to sponsor our event, and I did not reply because I will not take their money.

I don't know where you read that we do take pharma's money because—

[*Translation*]

Mr. Ramez Ayoub: No, I haven't read that anywhere; I just read on your website that you have supporters. I saw the list of pharmaceutical companies. I was just wondering what your relationship was with them.

[*English*]

Ms. Erin Little: On the Liv-A-Little website?

Mr. Ramez Ayoub: Yes.

Ms. Erin Little: I'm not even aware that it must read that way. Thank you.

[*Translation*]

Mr. Ramez Ayoub: The fact is that this is about seeking funding for drug development. I may not have understood what you said, but I had the impression that your feeling was that you were responsible for part of this research on a certain drug or cure, in particular. Is that correct?

[*English*]

Ms. Erin Little: Yes. Horizon Pharma purchased what was Raptor Pharmaceutical. It was Raptor Pharmaceutical within the U.S. with the Cystinosis Research Foundation, which I'm a part of.

The family organization directly funded Raptor to bring Procysbi to the market, and Raptor then sold that piece to Horizon Pharma.

That whole research and development that was done was brought forward because of Raptor Pharmaceutical, which was then sold.

[*Translation*]

Mr. Ramez Ayoub: Do you want to make a comment, Ms. Vowles? No? Okay.

We are talking about rare diseases. There are few people affected in Canada, but a little more in the United States. How do you view research competition within the G7 countries, particularly with respect to large countries developing drugs?

There is competition between these countries, but the treatment and accessibility are not the same. In a country the size of Canada, there are several cases. As for you, Ms. Little, we're talking about 75 or 100 patients. How do we manage, on a global scale, to offer a service and do this research in such a way that it is profitable not for companies but for patients?

[*English*]

Ms. Erin Little: It's interesting because what we're finding, actually, when we do start to research rare diseases is that it is unfolding and is involved in helping other diseases. There's a drug library that is progressing.

Let's say that they take Cystagon and they see if it can help other drugs. So, within these rare diseases.... I understand that it would be really hard for these companies to invest a lot of money specifically into cystinosis, but when you can nail down a disease with the exact genetic mutation, it gives a whole new understanding to the body, to what's going on and to how we can actually apply it to other diseases.

That's another world that needs to stop being exclusive to themselves, too, and that needs to share more so that we can grow together and heal multiple diseases. It is possible, and I know that within our own disease community, with regard to cystinosis, it's helping with Huntington's disease. There's another disease that I can't think of. Within just one small community of 2,000 worldwide, you can potentially help, so if that other disease group is another 15,000, it can slowly spread and multiply.

● (1015)

The Chair: Thanks very much.

Now we're going to Mr. Davies for three minutes.

Ms. Mary Jane Vowles: May I answer that question? Is that possible? I can make it very short.

The Chair: Go ahead. Make it very short.

Ms. Mary Jane Vowles: The expert is here in Canada. We've brought him here from Germany, and he is studying atypical HUS. The results are coming here, and Caryn is part of that research. It is related to some other illnesses as well, but he is here at SickKids working with a doctor at Toronto General and one at St. Mike's on a lower level.

Thank you.

The Chair: Thank you.

Mr. Davies.

Mr. Don Davies: Ms. Little and Ms. Vowles, do you happen to know if the research that was done behind the drugs that you're relying on was done in public universities or in Canada at all? Do you know where the research that formed the basis of these drugs was done?

Ms. Mary Jane Vowles: Do you mean the actual test sites, where they would have done the tests?

Mr. Don Davies: No, I mean the actual research.

It's my understanding that a lot of research on the chemistry and the molecules is publicly funded, often at universities, and the intellectual property is then often commercialized by the pharmaceutical industry, as opposed to the initial research being done by the pharmaceutical companies themselves.

Do you know where the basic research into the molecules that form the basis of the medication was done?

Ms. Erin Little: For us, for cystinosis, the research is being done in the States. We have doctors up here doing some research, new research, but everything has been funded directly by patients, because our rare disease population gets no public funding.

I can't think of it off the top of my head with Cystagon, but it was originally developed by a doctor, Jerry Schneider, in California. That's all I can answer to that.

Mr. Don Davies: Ms. Vowles, do you know?

Ms. Caryn Vowles: Initially, I think the research to do the actual drug was done by Alexion, but since then, research has been done at different hospitals. I don't think any has been done at any of our universities. Most is done outside of Canada, other than at the place my mom mentioned. Dr. Licht is the expert there.

Mr. Don Davies: Okay.

I have very little time left.

I want to end with you, Caryn.

I want to give you, as the patient and the person most affected by this policy, the last bit of time here to tell our committee what you think we should do. If you were the prime minister, and you could make a decision as to how we could change our system to help patients like you, what would be your recommendation and advice?

Ms. Caryn Vowles: I think you have to help people across Canada and not just in certain provinces. I know our board works at trying to help all the different provinces. Yes, we do it in Ontario and Quebec, and I think we're working on it elsewhere, but if a pharmacy comes in and works with only one province and offers one price to that province and a different price to another province.... If you try to do it across Canada, you will have more patients to go for, and you can drop the price that way, because you are now negotiating for more people.

The Chair: That's a good place to end.

I want to thank the witnesses for their testimony. It is very personal and very helpful.

I'm sitting here, Caryn, thinking that you must have not only good medication but also a good dose of persistence and determination by the sound of things. You just finished your biomedical engineering degree. You're working on your master's, and you plan to have a Ph. D. I'm sure we haven't heard the end of you. Thanks very much, and congratulations.

Thanks to all the witnesses.

We'll suspend now while we change witnesses.

We're bringing in the Department of Health officials, and I'm sure we're going to have some good questions for them.

Ms. Mary Jane Vowles: Thank you very much.

The Chair: Thank you.

● (1015)

(Pause)

● (1025)

The Chair: We will reconvene.

Before we start with our guests, I want to let everybody know that the unanimous consent motion for the correction of the premixed drink report will be tabled in the next few days.

As well, tomorrow, Mr. Roland Lescure from France, representing French residents overseas, will be visiting. He wants to talk to members of the committee about our role in the cannabis process. He's going to be available tomorrow at 3:15 p.m., for an informal chat, if anybody wants to participate.

We don't have a location yet. It might be in room 105 or room 107.

If anybody is interested in meeting with the gentleman from France about the role of the committee in the cannabis process, he wants to meet with us. I intend to meet with him. Anybody else who wants to come along can let me know.

Ms. Marilyn Gladu: What time was that again, 3:15?

The Chair: It's 3:15.

Ms. Marilyn Gladu: We have votes at three o'clock.

The Chair: Do we, tomorrow?

Well, we'll have to change it. It just came up and we just threw that time forward.

Ms. Marilyn Gladu: Maybe it could be right after the votes.

The Chair: If anybody is interested, let me know. We'll organize when and where.

We have members of the Department of Health here today. There are no opening statements, just questions.

We have Catherine Parker, director general of the biologics and genetic therapies directorate, health products and food branch; Karen Reynolds, executive director of the office of pharmaceuticals management strategies; and Dr. John Patrick Stewart, director general of the therapeutic products directorate.

We're going to start our questions with Mr. McKinnon.

Mr. Ron McKinnon: Thank you, Chair. I'll be splitting my time with Ms. Sidhu.

We've heard a lot of evidence certainly around rare diseases where there are trial drugs, and once they get approved, the price goes through the roof, or when another drug becomes an approved drug, the previous drugs that might be cheaper end up not being available anymore.

We're also hearing about the need for people on the special access program to reapply for coverage on a three-month basis.

I'll open it up to all of you to answer the questions, if you can.

What can the department do to streamline and facilitate access to these drugs for rare disorders?

Ms. Catherine Parker (Director General, Biologics and Genetic Therapies Directorate, Health Products and Food Branch, Department of Health): Dr. Stewart will answer that.

Dr. John Patrick Stewart (Director General, Therapeutic Products Directorate, Department of Health): A regulatory framework exists. We can't compel sponsors to come in and apply for market authorization. We can certainly encourage them. We can explain the process, facilitate, and provide incentives. However, ultimately it's a company's decision whether they market a product in Canada.

You probably heard over the last few testimonies that it is sometimes challenging to get products into market. We try to facilitate the conditions that would bring these products, through providing the framework for clinical trials, which supports clinical trials on rare diseases, and a process for priority reviews and notice of compliance with conditions, which I think we spoke about when we were here the last time, that incentivizes companies to come to market.

Once they're approved by Health Canada to be on the market, it's for other players within the system to decide on price and whether the provinces decide to ultimately fund it. It's beyond our role in that process. Maybe Karen can speak a bit about it.

As we talked about last time we were here, the special access program is one that is unique. It provides, on a probation basis, based on a request from a physician, access to an unapproved therapy that has not gone through the regular scrutiny for evidence around its quality, safety and efficacy. It's for the physician to explain why this therapy is the best choice for the particular patient in front of them, why it's a serious and life-threatening condition, why other available therapies, if they exist, have been considered and are not suitable, and evidence that supports its use.

Because special access requests are authorized in those kinds of conditions, they're not authorized for long periods of time. Typically, it's three to six months.

There can be a unique situation where a product receives an authorization, but it takes a company, once they get their approval, several months to get their labelling together and then actually put the product on the market. There's a period after a product gets market authorization where it's still not available, so the special access program will continue to provide access under that.

We shorten the time period because we know ultimately the product will be on the market. If there's an alternative product at the same time being accessed, we would shorten that as well, because we anticipate that most practitioners would transition to an approved therapy once it's available on the market.

● (1030)

Ms. Karen Reynolds (Executive Director, Office of Pharmaceuticals Management Strategies, Department of Health): I can speak a bit in terms of price.

As you're likely aware, the only federal lever that is able to exert any authority over price of patented medicines in Canada is the Patented Medicine Prices Review Board. I'm sure I answered a question related to the modernization effort from one of your colleagues when we were last here. They have authority under the Patent Act and its regulations to set what are termed “non-excessive prices” for patented medicines, and they do that based on the regulatory tools that are available to them.

That being said, as I'm sure you're also aware, Canada pays some of the highest prices in the world for patented medicines. We're third among the OECD countries, so it is acknowledged that prices of patented medicines in Canada are high.

The only other mechanism to bring prices down for Canadians is through negotiating mechanisms, largely through the pan-Canadian Pharmaceutical Alliance. That, as you're aware, negotiates prices for Canada's public drug plans. Prices would only be negotiated for those drugs that have received a positive recommendation for a formulary listing from the Canadian Agency for Drugs and Technologies in Health, better known as CADTH. That would not necessarily apply to all of the drugs that you're speaking about.

Ms. Catherine Parker: I would just add that we are working very aggressively with the list of drugs that are on special access, especially for the rare diseases, to bring those into some kind of authorized state.

In working with the companies, it's a matter of would it take to get them to file for approval of a product in Canada. If they have filed to another jurisdiction and they have the medication approved there, we will use the assessment reports of those regulators, the decision that regulator made, to help us get those products off SAP and into a marketed state, so that the patients, families and physicians don't have to go through the special access program.

Mr. Ron McKinnon: Thank you.

I'll go now to Ms. Sidhu.

Ms. Sonia Sidhu (Brampton South, Lib.): Thank you, Chair.

Thank you, all, for being here.

In rare diseases sometimes there's a lack of scientific evidence. What type of evidence does the department consider in its regulatory review?

We heard before from a panel that said Canada needs a drug review policy. Can you comment on that?

Ms. Catherine Parker: I can certainly comment on the fact that we treat every drug for a rare disease uniquely. We meet with the manufacturer of that drug and we go over what kind of data they have available, what kind of data they are capable of getting, what kind of data may have been generated elsewhere in the world. We negotiate on a case-by-case basis what will be the requirements.

Every drug is different, and even in the rare disease area, you could be dealing with a drug for two or three patients versus one drug for 100 or 200 patients. It may be a very rare disease in Canada, but not as rare in other parts of the world, so there may be data from other types of trials.

I can't say this more earnestly: We treat every, every drug case by case. We agree on and design an approach to that drug with the manufacturer that suits the needs of that patient community and the data they are capable of obtaining. We use conditional approvals. We use priority review. We use reports from other regulators. We use literature information. We take all the information....

We like to refer to it as the totality of the data, but it is unique to each product.

I don't know if Dr. Stewart wants to add to that.

● (1035)

The Chair: Actually, the time is up for that question.

Ms. Gladu.

Ms. Marilyn Gladu: Thank you, Chair.

Thank you, witnesses. It's good to see you again.

It's clear from the testimony we've been hearing that when it comes to rare diseases, people are in the situation where they need the medication and they will die without it. It also seems true that the better experiences they've had are when they're involved in clinical trials and they're able to get access to the drugs through that.

I'm interested to hear what you think about the right-to-try legislation that has been introduced in the U.S. Basically, when you have people in this situation, it's a very, let's say, efficient fast lane of drug approval for giving clinical trial approvals and letting people try things that may save their lives.

What do you think about that?

Dr. John Patrick Stewart: Access to clinical trials is often when we're doing investigational testing because there are certain requirements on the sponsor to have a well-designed protocol, that the risks are mitigated to the degree possible, that patients are informed and that you have REB approval. In the development of drugs, we encourage access to be through a well-designed clinical trial.

Having said that, there are challenges when patients with rare diseases may be distributed randomly or very widely in small numbers across the country. We work with sponsors to encourage access to those individual patients or specific patients. Failing that, there are other options, like open label trials or compassionate access programs, where individual patients, under the design of a protocol, can get access to a drug that may not actually be in the larger trial that's ongoing.

When trials finish, then there's also a concern about ongoing access. Again, there are opportunities, if the sponsor is prepared to continue to provide access, so that patients that are responding to the product can continue to get access through an open label extension or compassionate access. We encourage that until such time as it's market authorized.

Under the right to try, right to try can mean different things. It can also mean that a patient who wants access to investigational therapy where there may be varied or no evidence around its efficacy wants access to that drug. When we did the SAP renewal, the thinking around the special access program, we did a consultation back in December of last year and January this year. It was one of the questions we asked. There were health professionals, health care system workers, patient support groups and associations. By and large, there was very little support for the right to try.

Some of the reasons we heard were typically about, when you're talking about right to try you're talking about a serious or life-threatening condition, and almost unanimously, you require a health practitioner to be involved in that care. The special access program allows that to happen. A health practitioner can evaluate an individual patient, look at the products available, look at what evidence there may be, credible or not, to support the use and come forward with an application. It's also something in jurisdictions elsewhere that there's not a lot of support for. In fact, most manufacturers have commented that they don't want their investigational products necessarily being accessed in that way. They would rather that it be in its early development or a properly designed trial, where you can control for variables, and the evidence is usable to move forward with the support of market authorization.

Cathy, do you have any other—

Ms. Marilyn Gladu: That's very good.

We heard testimony that, when the PMPRB takes a really long time to come to price certainty, there's a fear that will discourage people from bringing their drugs to our country. We've probably talked about that before.

Have there been any reconsiderations to the modernization that's happening with PMPRB?

Ms. Karen Reynolds: As I think I mentioned last time, the department published proposed regulations to modernize the PMPRB in the Canada Gazette, part I almost a year ago, in December 2017. The proposal hasn't been finalized. We continue to consider the results of that consultation and engage with stakeholders on key issues related to the proposal.

● (1040)

Ms. Marilyn Gladu: As well, we heard that, once people come to the end of their clinical trial, there is a gap of time before they receive approval. There was a recommendation from one of the witnesses that if the trial was completed with no negative consequence, it should have instantaneous approval so they can continue to take the drug.

Are you aware of this situation and can it be fixed?

Ms. Catherine Parker: I think this is what Dr. Stewart was talking about.

Yes, absolutely, there is the opportunity for patients to continue on treatment after the clinical trial. That's through an extension protocol or an open label type of situation.

However, it is all dependent on the manufacturer being willing to continue to provide. That's the reality. We do require that they file a protocol as to how the patients would be treated, but we do a very efficient and timely review of that. Much of this timing and this gap is influenced by the manufacturer and what they are willing to do.

Ms. Marilyn Gladu: Thank you.

The Chair: Mr. Davies.

Mr. Don Davies: I don't know if you heard any of the testimony of the patients who were just here, but one of the mothers of a daughter who has a chronic rare disease testified that Health Canada makes her reapply every three months for the drug that her daughter needs to save her life. Why would that be?

Dr. John Patrick Stewart: I would assume you're talking about the special access program and applying for it.

As for the three months, I can't speak to specific requests as that's confidential, but typically a special access program request, if it is for a chronic illness or a longer-term use, would be approved for six months, and then it would need to be renewed again. The thinking, as I mentioned earlier, is that these are unapproved therapies and the regulations require the requesting practitioner to report on the use of the product. So, it's there.

In our special access renewal, we are looking at this from a client service perspective on an ongoing basis or a situation where it is a well-established therapy and the reason it's not on the market is that the manufacturer hasn't come to the market in Canada. The product may be approved in other jurisdictions. We will extend that to a longer period of nine months....

Mr. Don Davies: My understanding from the testimony we received was that it used to be every six months, but Health Canada has now made them apply every three months.

Dr. John Patrick Stewart: I think that might have been in the context that—I'm thinking it probably happened—as I mentioned earlier, when a product.... I'll speak specifically about a situation with Cystagon and Procysbi, which were two products—

Mr. Don Davies: Those were the products.

Dr. John Patrick Stewart: —for use with cystinosis through the special access program.

One of the products, an extended use product, Procysbi, applied for market authorization and received market authorization in June 2017. At that point we were still getting requests for both of the products through the special access program, knowing that it typically takes a manufacturer about three months to get their labelling in order and get it on the market. Knowing that in three months there would be an approved marketed therapy for this condition, it made sense that both requests for Cystagon and Procysbi were reduced to three months.

Our assumption was that most, if not all, patients would transition to the approved therapy. In fact, in the spring when this product was being reviewed and being announced as coming on the market, there was a lot of support from treating physicians who were involved in this disease group as well as some of the patients who were out advocating that it was great that Procysbi was coming to market. Our anticipation was that, in three months, there wouldn't be a need for as many requests, if any, through the special access program; hence, we reduced it to the time period we thought would be required for a product to be accessed.

Mr. Don Davies: Try to help me understand this, too.

The testimony we received was that their daughter was responding very well to Cystagon. I understand it was under the special access program. The cost was $15,000 per year. Their family was compelled to transition to Procysbi, which costs $300,000 per year, and the only difference—it's the exact same molecule—is the coating that affects the time release of the medication.

To lay people sitting here, that sounds absurd and ridiculous. Why would we have a policy that drastically increases the cost of the medication for no real difference in therapeutic value and, in fact, maybe, from this family's experience, a diminution in therapeutic value? Help me understand that.

● (1045)

Dr. John Patrick Stewart: Sure. I think it's important to point out that the situation of Procysbi and Cystagon was rare. It's very unusual that you would have two products coming through the special access program for one rare disease or that you have two products with a very different price.

The consideration under the regulations for the special access program is that we verify whether it's a serious and life-threatening condition. Have the products that are available on the market been considered, tried and failed or considered and not available or not suitable? Is there evidence on its use, safety and efficacy?

When Procysbi came on the market—it's the same molecule; one was extended release and one was immediate release—there was no clear medical reason at the time to say that Procysbi wouldn't be a suitable alternative. The special access program does not consider cost in its review. If you're looking for the reason why a product may be unavailable, cost is not considered. In fact, we're often not aware of what the cost is.

When Procysbi came on the market, the program had no idea at all what the price was going to be. It's not a conversation we're involved with. It's not part of the statutory purpose in the Food and Drugs Act that the special access program consider cost. In fact, their concern would be, if we went in that direction, that it would have an impact on market authorizations in general. It would introduce an unpredictability in the country in the sense that, if an innovator company wants to market a product and goes through the costs of doing research and development, the cost of marketing and the cost post-market, and there is a possibility that the special access program will provide access to a cheaper product that has not gone through extensive safety, quality and efficacy, you might destabilize whether innovator companies will come to Canada, because they have no guarantee of a secure market. It's not something that we—

Mr. Don Davies: But coming from the reverse end, if we're setting policy as a government and we're saying to patients that we're going to force them to take a drug that doesn't work as well, that costs way more than the drug they want to take and it's the same molecule—and it's paid for by the taxpayers—can you not see that Canadians would have some real concerns about how this program is being managed if that's the result?

Dr. John Patrick Stewart: You bring forward some very important considerations, some of which are beyond the mandate of the special access program.

Mr. Don Davies: I'd like to move to pricing.

Dr. Joel Lexchin testified to the committee. He said:

> ...the drug companies will not open up their books to reveal their R and D costs for new medications. There's a figure of $2.6 billion that's bandied around about being the cost of getting a new drug to market. That kind of figure is based on confidential data that won't be released. If drug companies want to prove that they need to charge these significant amounts of money that they do for new drugs, then they should prove to Canadians, to insurers, that those prices are actually justified, but so far they haven't.

To what degree are a drug company's production costs revealed to the government and considered in PMPRB reviews on excessive pricing? Do they open up their books and reveal their true cost of production to you?

Ms. Karen Reynolds: Your question is in relation to the mandate and the work of the Patented Medicine Prices Review Board. Unfortunately, I'm not in a position to comment on what information is made available to the board when making their price determinations.

As you may know, Mr. Davies, they sit at arm's length to the department. They're quasi-judicial. We don't have a view into the information that they receive. Officials from the board would be better placed to respond to your question.

The Chair: I'm afraid we're going to have to end our questions for today. We're a little beyond our time.

I just wanted clarification on the Cystagon issue. Cystagon was prescribed and was working, and now a new one is also approved. Does that mean that patients can no longer take Cystagon, or do they have a choice?

Dr. John Patrick Stewart: The practitioner has the option to submit to the special access program for Cystagon. It is still approved today. It was never not approved. But a practitioner has to present a request that explains the medical rationale for why the available therapy is not right for that patient.

There are currently in the order of about 64 patients receiving Cystagon through the special access program.

● (1050)

The Chair: It's still available.

Dr. John Patrick Stewart: It's still available. It was never not available.

The Chair: Thank you very much, everyone, for attending. I'm sorry we had a shortened question period, but it was very effective.

The meeting is adjourned.