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Monday, May 30, 2016

Chair

Mr. Bill Casey

Standing Committee on Health

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● (1530)

[*English*]

The Chair (Mr. Bill Casey (Cumberland—Colchester, Lib.)): It being 3:30, I will call the meeting to order.

First of all, I want to thank our presenters for coming back. I know you were cancelled before, and I was afraid you were going to be cancelled again today. The votes tonight aren't until about 6:30, I think, but I apologize for that. We feel badly when we have to do that.

We very much appreciate your coming back and we are looking forward to your presentations. We feel that the information you will provide today will perhaps be some of the most important information we're going to get.

With that, each presenter has 10 minutes to start. In our first round of questions, the questioners have seven minutes each, and in the second round they have five minutes. If you could keep your presentation to 10 minutes, we'd appreciate it very much.

We're talking about a national pharmacare program. Anything you can put in that light would be most important to us. We're interested in hearing from you.

The Canadian Organization for Rare Disorders can go first.

Dr. Durhane Wong-Rieger (President and Chief Executive Officer, Canadian Organization for Rare Disorders): Thank you very much.

My name is Durhane Wong-Rieger, and I am the president of the Canadian Organization for Rare Disorders. Presenting with me today will be Maureen Smith, the secretary of our board.

On behalf of the three million Canadians who suffer from a rare disorder, we want to thank you for the invitation to share our perspectives on national pharmacare with a focus on rare diseases. We're mainly going to talk about the regulatory aspects, and about access gaps for rare disease patients, and some of the key aspects of a national pharmacare program.

Before we start, I'd like to give you a brief background on the Canadian Organization for Rare Disorders, also known as CORD, and to share with you a bit of our personal experiences related to rare diseases.

CORD is a registered charity. It serves as an umbrella organization for about a hundred rare disease groups in Canada. Most of these groups are kitchen table organizations run by volunteers. Most of them are run by people who are not only affected patients or parents, but also hold down other jobs in order to pay the rent. I want to stress this point in order to correct some of the erroneous impressions, which have been fuelled by some of the media outlets and by critics, that these volunteer organizations are heavily subsidized by the pharmaceutical industry. These allegations are not only unfair and untrue, they're harmful and hurtful to the patients and families who donate thousands of hours and thousands of their own dollars to running these groups for their cause.

In the same regard, at CORD we are even more challenged in terms of resources because we don't want to fundraise among our member groups. We can't do the fundraising there. Unlike academic institutions, we can't even apply for a grants from institutes like CHR, even though we at CORD contribute to the research programs.

Our two main sources of funds are educational programs that we run and our educational conferences and grants that we get. Most of them are provided by the pharmaceutical companies, but to put it in perspective, CORD is a lean organization. We only have two part-time staff, and I'm one of them. This is CORD, and we rely heavily on volunteers like Maureen Smith in order to do our work.

Our recent efforts, as some of you know, have included implementing Canada's rare disease strategy, because rare disease is about a lot more than just drugs. This strategy was developed and led by CORD, but we had input from patients, clinicians, researchers, policy leaders, and the private sector. We have five key goals: improving early detection and prevention, because if we can diagnose it early then we can prevent many of the harmful effects; providing timely, equitable, and evidence-informed care; enhancing community support at all levels; providing sustainable access to promising therapies; and promoting innovative research.

As an aside, I'd like to thank all the members of Parliament who wore the yellow scarves for the launch of our strategy for Rare Disease Day.

I don't usually talk about my personal experiences, but I have two children, both of whom were born with rare diseases. I have a husband who has a genetic cardiovascular disease called familial hyperglycemia. He also has Parkinson's disease, and he has a rare motility disorder.

I am a psychologist by training and taught at a university for almost 30 years. During that time, I was doing a lot of patient advocacy on the side. It was my experience working as a volunteer with the Hemophilia Society during the tainted blood crisis that convinced me that I could probably do more good by leaving the university and focusing on patient advocacy full time. I imagine a lot of you can relate to that, because I'm sure you have left other careers to have a bigger impact as a public official.

I'm going to turn it over to Maureen, our board secretary and a board member, to share her story.

● (1535)

Mrs. Maureen Smith (Board Secretary, Canadian Organization for Rare Disorders): Thank you, Durhane.

[*Translation*]

As a Canadian with a rare disease called congenital panhypopituitarism, I thank you for giving me an opportunity to testify before you today to discuss issues that are important to me.

My health deteriorated for 20 years because no research was being done on my condition, which is extremely rare. However, there was a drug approved by Health Canada that could have helped me. But the cost of the medication could not be refunded through insurance plans in my case, as there were no clinical trials on my specific condition.

During that time, I was monitored by numerous specialists and had to be frequently hospitalized. Finally, after more than 20 years, I was able to participate in a clinical trial and gain access to medications. I am now healthier. Unfortunately, my experience, which has been marked by significant difficulties in accessing treatments, is typical of what other Canadians with rare diseases are going through.

Should Canadians with rare diseases have to wait 20 years to gain access to the appropriate treatments and care people with common diseases take for granted?

I think it is time to implement policies and measures to improve the quality of care and treatments available to Canadians with rare diseases. Dr. Wong-Rieger will have some recommendations for you.

[*English*]

Dr. Durhane Wong-Rieger: Thank you very much, Maureen.

Let me continue by talking about our regulatory process and how it could be improved to facilitate access to orphan drugs.

I think Maureen's predicament stems from the fact that Canada is still the only developed country without an orphan drug policy. The biggest downside of that is that companies are not motivated to conduct clinical trials here in Canada. The U.S. passed an orphan drug act back in 1983, the European Union followed suit in about 2000, and other countries as well.

In reality, only about 50% of orphan drugs get authorized for use here in Canada. That may not be a problem, unless you're a patient or a parent who's waiting for a drug and has a debilitating disorder and are trying desperately to get something before it's too late.

To address these barriers and to facilitate patient access, we urge the federal government to implement as quickly as possible the draft federal orphan drug regulatory framework that has already been developed by Health Canada. Within this framework was a definition of rare disease as a condition that affects fewer than 1 in 2,000 people.

Even in cases where treatments are approved in Canada to treat rare diseases, patients experience important difficulties in accessing them. We know that most Canadians experience gaps in drug coverage, but for patients with rare diseases it's exacerbated. Our survey in 2015 showed that 1 in 3 patients with rare diseases could not access an appropriate drug treatment that was, in fact, in Canada.

Every country is struggling with how to address rare diseases and orphan drugs, but sadly, I think Canada is doing worse than most other developed countries to provide equitable and sustainable access.

Very briefly, the experience of patients who have Fabry's disease illustrates the delays in accessing orphan drugs. As you may know, in 2004 Health Canada approved two products to treat this disease, which is a debilitating condition that leads to organ failure and early death. Unfortunately, because they were using a very traditional cost-effectiveness assessment, the Canadian Agency for Drugs and Technologies in Health recommended that these drugs not be covered by the public drug plans. As a consequence, patients with private drug insurance were all able to access the medicines, but the patients covered by the public plans could not. It took about two years for the governments to set up a managed access program to fund the drugs. Very recently, another report confirmed the clinical effectiveness of these therapies.

What did we learn from this example and many others like it? Access to drugs for rare disease in this country is not effectively serving the patients. The problem is that the health system isn't saving any money by doing it this way, because the gaps in patient access to the right drugs lead to increased morbidity and poorer quality of life, and also to increased costs for the families, the health care system and, ultimately, the Canadian economy, as Maureen's story and those of thousands of other patients illustrate.

CORD supports a national pharmacare program, but we feel it should be guided by a set of core principles, including equity, quality of care and treatments, patient involvement, the incentivization of development and marketing of new therapies, and collaboration and coordination. We don't really care whether the program is administered federally or is a pan-Canadian federal-provincial-territorial approach, but we do believe that it has to have the following key characteristics: first, access to medicines according to a single set of eligibility criteria; second, flexibility and exceptional adjudication measures to ensure that the unique circumstances of each patient can be addressed, and addressed fairly; and third, a national pooling across all drug plans to ensure equitable and affordable access across the country.

I would be remiss not to address the prices of orphan drugs. We think that drugs for highly specialized diseases for small patient populations will, no doubt about it, cost more per patient than common drugs. There are some obvious factors involved, as you can imagine, including the higher cost for research and development of these drugs, as well as the smaller size of the patient population. To put that in perspective, rare diseases are more costly to diagnose, they're more costly to treat in hospitals, and they're more costly to follow by family physicians and pediatricians. They are simply more costly because they're rare, and they're severe and debilitating. Does that mean they're less worthy of treating, just because they're costly?

I think Pope Francis said it very well when he called for the globalization of empathy, which should be used to value all human lives, including people with rare diseases.

I want to conclude by saying that patients with rare diseases are really wanting to work collaboratively with all stakeholders. We want to make sure that the drugs are used appropriately and responsibly, and we also want to make sure that the prices in Canada are fair and—very importantly—sustainable.

● (1540)

We know that patients with rare diseases have extraordinary, unnecessary barriers to getting the appropriate care and treatment. We really encourage the federal government to do its part in helping address these challenges by implementing the orphan drug regulatory framework, and by working with the provinces to put in place a coordinated, national approach or program to ensure timely care and appropriate and equitable access to rare disease therapies. We think all Canadians with rare diseases deserve the same quality of care available to patients in other countries and patients with common conditions.

The Chair: Thank you very much.

Mr. Herder, welcome back.

Prof. Matthew Herder (Associate Professor, Faculties of Medicine and Law, Health Law Institute, Dalhousie University, As an Individual): Thank you for the opportunity to appear before you today.

My name is Matthew Herder. I'm a lawyer and an associate professor in the faculties of medicine and law at Dalhousie University.

My research focuses on laws, policies, and practices around pharmaceutical drugs. My publications and testimony before this very committee in 2014 helped contribute to a number of important changes to Canada's Food and Drugs Act, known as Vanessa’s Law.

I want to begin by registering my support for national pharmacare. It is not a panacea; however, it is highly preferential to the status quo and increasingly needed as more targeted, personalized therapies enter the Canadian market.

Building from my research brief, which I submitted to this committee, I will focus on the set of issues or challenges posed by the pharmaceutical industry's shift to more personalized therapies or precision medicine. In short, the range of drugs developed and marketed under the banner of personalized medicine both underscores the case for and helps define some of the essential features of national pharmacare.

Today I want to make three points.

My first point is that while personalized medicine remains a work in progress, the pharmaceutical industry has shifted its focus to more targeted therapies. In the last two decades, large-scale genomics and related research initiatives have generated a wealth of new information about the molecular underpinnings of human disease. But the process of discriminating between information that actually helps to prevent, diagnose, and treat human disease versus misinformation is just getting under way.

Researchers and drug companies are increasingly integrating genomic and epigenomic information into their drug discovery and development platforms. There are hundreds of such personalized therapies currently in development, despite the uncertain clinical value of many of the biomarkers that have been incorporated into these interventions.

This trend dovetails with industry's increasing focus on rare diseases. Indeed, many of the personalized therapies that have come on the market qualify for extra incentives that countries, such as the United States, have put into place to encourage research into rare diseases.

Pharmaceutical companies have allocated an increasing share of their resources toward rare diseases. For example, nearly a half of the novel therapies that were approved by the United States Food and Drug Administration in 2015 fell into that category. Why? This is because industry has figured out that the development of such therapies, also known as orphan drugs, can be as profitable, if not more profitable, than drugs that address more common conditions.

Developing orphan drugs tends to be faster and cheaper, because fewer patients are eligible to participate in trials. Once a rare disease drug is approved, it faces little to no competition in the marketplace, enabling companies to charge premium prices. Indeed, price tags of $200,000 to $300,000 per patient per year are increasingly common for rare disease therapies. As a result, the development of more personalized, targeted therapies, incorporating genomic and other sorts of biomarkers, account for a growing share of both public and private expenditures on prescription drugs in Canada.

My second point is that our national pharmaceutical regulator, Health Canada, is allowing these drugs on the Canadian market despite limitations in the evidence about the safety and effectiveness of those drugs.

For most of their existence, pharmaceutical regulators, such as Health Canada, have approached their task in a binary manner: should a drug be on the market, yes or no? In recent years, however, the approach has become more dynamic, with regulation theoretically continuing across the life cycle of a drug, both before and after it's on the market. This new approach to regulation is often called “adaptive licensing”. While adaptive licensing makes sense in principle, it may be a double-edged sword in practice.

We've long known that the evidence generated in the course of clinical trials has its limitations. Real-world use of drugs, without careful control from a research team, is when a drug's true safety and effectiveness profile becomes more evident. For that reason, no one thinks that ongoing, active monitoring of a drug's safety and effectiveness after it's been approved is a bad idea. However, if the promise of ongoing study, in effect, lowers the bar for approval in the first place, we run the risk of letting a lot more unsafe, ineffective drugs on the market.

The stakes of that gamble are especially high in the case of rare diseases. Because these drugs focus on small patient populations, a number of the standard procedures used by researchers to ensure that the results of their studies are robust are often not followed. Consequently, as two U.S. physician researchers recently put it, these sorts of studies run a greater risk of "identifying benefits that are not real or missing risks that are."

● (1545)

In such instances, ongoing, rigorous post-market evaluation of a drug's safety and effectiveness is essential. Thankfully, Health Canada, following the passage of Vanessa's Law, has a number of new legal powers that should assist with that task; it can finally compel companies to carry out post-market studies and share information.

However, and here's the rub in terms of pharmacare, there's a fundamental mismatch between Health Canada's move to adaptive licensing and the provincial and territorial infrastructure concerning drug coverage. The existing patchwork of payers and health technology assessment bodies is poorly equipped to process and act upon new information about a drug's safety and effectiveness in real-time.

That brings me to my third and final point.

Our current patchwork of health technology assessment bodies and public and private payers lacks the capacity to handle the shift that we're in the middle of, namely, toward personalized, targeted therapies. As I've just said, evidence about a drug's safety and effectiveness should increasingly span both the pre- and post-market phases of a drug's life cycle. Yet few, if any, private insurers in Canada have the capacity to evaluate this information on an ongoing basis. It is doubtful whether a number of provinces have that capacity either. A national pharmacare program or formulary should in principle have greater institutional capacity to do so, and to establish key information-sharing channels with our national regulator, Health Canada.

Similarly, our current system lacks the capacity to negotiate more nuanced drug coverage deals with companies. Because of the tenuous nature of the evidence produced with respect to personalized therapies during pre-market testing, and the deep divide between standards of safety and effectiveness applied by Health Canada and the question of cost-effectiveness that public and private payers are focused upon, there is a growing need for more sophisticated reimbursement contracts.

One model is called coverage with continuing evidence development, or CED. Another is performance-based risk-sharing agreements, or PBRSAs, where payment for a therapy is contingent on observing benefits in patients following treatment.

Again, both public and private payers in Canada generally lack the ability to collect and analyze information on an ongoing basis, thus negating the value of those kinds of more sophisticated contracts with companies.

In contrast, a national formulary could do so, and in turn adhering to a much more evidenced and performance-based approach to drug coverage. Given the incredibly high-priced nature of many personalized rare disease drugs, maximizing value for money is imperative, and national pharmacare has a much better potential for doing so. Plus, the prices of these new targeted therapies need not be so high. The pan-Canadian drug pricing alliance only represents about 40% of Canada's drug purchasing power. National pharmacare representing all Canadians would substantially increased our bargaining capacity.

Finally, and perhaps most importantly, our present patchwork of health assessment bodies and payers lacks the capacity to handle the increasing politics of drug coverage. Our national drug evaluation body, CADTH, adheres to an evidence-based approach in making recommendations about which drugs to cover. But that's just it: CADTH only makes recommendations. Payers remain free to make their own decisions, so drug companies and patient groups train their attention on those governments, politicizing drug coverage decisions through media stories and playing a game of divide and concur with provincial payers.

A national formulary situated at arms-length from government, and relatively free of industry influence, offers the potential to de-politicize drug coverage decision-making. We know that drugs are notoriously difficult to delist, but given the limited nature of the evidence at any given point in time about a targeted therapy, the power to stop funding a drug or renegotiate payment structures depending on observed benefits is integral to a well-functioning drug reimbursement system.

If a national formulary is to have any meaningful ability to exercise that kind of power, its independence from government and industry alike must be protected.

Yes, industry and patient groups should be heard as part of that decision-making process, but they should do so under strict conditions of transparency to the Canadian public and without compromising formularies' ability to negotiate and act upon performance-based drug coverage agreements.

● (1550)

To conclude, personalized medicine's laudable goal is to enable more precise decision-making about which drugs to use. It remains a work in progress. National pharmacare designed as an evidence-based, independent, and transparent institution has the potential to to improve Canada's capacity to make more precise decisions about which drugs to pay for. It's imperative as more and more high cost targeted drugs of uncertain benefit enter the Canadian market.

The Chair: Thank you very much.

Dr. McCabe.

Dr. Christopher McCabe (Capital Health Research Chair, Faculty of Medicine and Dentistry, University of Alberta, As an Individual): I'd like to start by thanking you for the invitation to speak today.

You probably notice from my accent that I'm from the U.K., and much of my experience and insights into a national pharmacare program I have derived from 20 years' experience as a health economist advising the NHS on paying for drugs. I have now been in Canada for five years.

I hope I'll take considerably less than the 10 minutes that you've allocated.

I wish to make three points with regard to national pharmacare.

The first is the cost-savings capacity of a national pharmacare program. We just heard from Dr. Wong-Rieger and Dr. Smith on how the absence of effective pharmaceutical therapies impacts not only the health of the individual, but also the costs they impose on the health care system.

This is not unique to rare diseases, as exactly the same story is in play for those people who cannot afford to fill their scripts for chronic common conditions or infections. These people also will turn up at the emergency department and may be admitted, but impose significant costs on the health care system, which, as you all know, come from the public budgets. In effect, the taxpayer picks up the tab for the health management of the avoidable complications of these conditions.

I think that's both a financial and a social equity argument for national pharmacare. We could hopefully reduce the inequality in access to high-quality care and the inequality of health by addressing the needs of those people who can't fill their scripts due to socio-economic reasons.

I'll move on to my second point, which is about precision medicine. I lead two large research programs funded by Genome Canada around personalized and precision medicine. Whilst the number of technologies currently on the market is small, the number in development is very large. The last time we looked at this, we identified 167 precision medicine technologies in clinical trials globally.

They are coming and are defined by a couple of key characteristics.

First of all, they arrive at market with a really quite immature evidence base. There's very little we can do about that in the clinical trial setting simply because the nature of precision medicine is that it identifies smaller subsets of the population who can benefit from the technology, and so the level of evidence that you can generate in the clinical trial phase is inherently less than for conventional medicines designed to treat common diseases.

Secondly, the nature of the test technologies—the defining characteristics of these—is such that it is much harder to generate convincing evidence. You need much larger trials to generate strong evidence on the performance of these tests that is generalizable to all the populations.

We are looking at a situation where regulators are going to be having to make decisions about proving these new technologies on much more uncertain evidence.

Dr. Herder referred to the Vanessa's Law and how it creates the facility for adaptive licensing so that we can move some of the evidence-generation process into the post-market phase. That is only feasible if there is a sister organization with a national remit that's responsible for reimbursement and that can take the baton from Health Canada and actually deliver national access so that you get the necessary volume of uptake of the technology to generate the evidence. In my briefing I describe the case of Prochymal, which was probably the first truly innovative technology to be put forward for a conditional licensing approval, and it went nowhere because it couldn't get reimbursed.

● (1555)

If, as I think is appropriate, our innovation or industrial strategy in this sector is seen as adaptive licensing, making Canada an attractive place for inward investment in the pharmaceutical industry.... Vanessa's Law and those facilities that it gives the regulators are a necessary condition, but a national reimbursement authority of some form is probably a sufficient condition. So there are important industrial policy implications to trying to move forward in this space without a national pharmacare strategy.

The third point I would raise returns to the orphan drugs and orphan disease community. A lot of those same issues I've described for precision medicines are pertinent for the orphan disease community. I think it's important to recognize that in creating a national pan-Canadian formulary, there is the opportunity to raise the standard of care for these people. Because there are very few with each individual disease, though they are very large in number in the entirety of the diseases, the clinical expertise in the packages of care they require for good outcomes is rare as well.

By having a national pharmacare organization that can contract with specialist centres, we would be able to ensure the quality of care that's provided, and to have specialist and more rapid diagnostic facilities. As a result, we could build a package of high-quality care around the drugs that were implemented. I think that would be beneficial both to the patients and the health care systems by avoiding those diagnostic odysseys that these patients go on, consuming large amounts of resources in doing so. It would also be an opportunity to negotiate for all Canadians affected by those diseases and thus an opportunity to reduce the total budgetary impact.

For each individual orphan drug, the budgetary impact is small, but with literally hundreds of orphan drugs coming down the line, the total budgetary impact is large. You can get an orphan drug designation with 17,000 patients affected in Canada. If it's $100,000 per patient, per year, you can quickly see how the total budgetary impact does not stay small and that there will be a need to manage the total budgetary impact of orphan drugs at the same time.

Thank you again for inviting me, and I hope they were useful insights.

● (1600)

The Chair: Thank you very much.

Dr. Tamblyn.

Dr. Robyn Tamblyn (Professor, Department of Medicine, and Department of Epidemiology, Biostatistics and Occupational Health, McGill University, As an Individual): Thank you very much for rescheduling.

I sent on a bit of a PowerPoint deck. I hope you have it. I will walk you through a few thoughts here.

I've decided I'm going to make just two points. One is going to be related to a plea, essentially, to consider a value-based formulary for Canada. The second is on our unused assets for post-market surveillance.

My colleagues have covered the rare-disease, designer-drug, genomic-based therapies better than I can, so I will not go in that direction. Let me speak to the first two points.

Presently in Canada there is a patchwork. If you're institutionalized, your drugs are free; if you're in the community, it varies by province as to whether or not your drugs are covered. All provinces set up a formulary, meaning those drugs we'll cover and those we won't. For the most part they're covering about 70% or 80% of the drugs that are approved for consideration in Canada.

They do so for what I call the vulnerable subpopulations, by and large—seniors, who use a lot of drugs; people on social assistance; and there may be some other very high-cost drug therapies, but this varies from province to province.

They can't afford it, because seniors are a big group, and so what they always do is attach what I call a copay to it. They say that you'll have to pay a part of this, because otherwise...we want to control the demand for something that may be misused or abused.

I want to take you down to an example of this. In this particular slide, I show you an example of a person who was 25 and was diagnosed as schizophrenic at age 24. This is an architect who had asthma. The person was put on risperidone. That drug costs $140 a month. The inhaled steroid for asthma is $120 a month. From time to time the person has sleeping problems, so that drug is lorazepam, and it costs $35 a month.

If you look at the cost sharing, which goes anywhere from zero to 35% across Canada, 25% was applied in Quebec. They have to pay the most for their anti-psychotic drugs, they have to pay the least for their sleeping medication, and somewhere in between for the steroid to control asthma. Tell me if that makes any sense whatsoever.

When Quebec put in place essentially an increase in that cost share, they saw a very rapid decline—that's shown in the graphic, figure 2 on the “Current Practice” slide—in the use of these essential medications. It was associated with an increase in hospitalizations and emergency department visits.

This is not really a surprise, in fact, because these drugs are essential to control chronic conditions, and they work. If they were being misused—say you run around and take insulin just on a whim—you would see no change in your health status, but if you really need it, you're going to get into trouble pretty quickly. That's exactly what they saw.

The argument I'm trying to make here is that we need to provide, if we want to make sense of what we're doing here, a value-based formulary that says, let's make essential medications that we know control disease free; let's make them available to our population. Let's negotiate the best prices possible in Canada, because we pay sometimes five times as much for an essential drug as other countries. Let's go after that, and let's be parsimonious.

Not every drug gets in—not the 70% that we're used to doing—but let's say the absolutely essential drugs that we know work and that keep people well and working in our community; let's make that our goal to start with. Then we can actually add the cost share to those that potentially could be misused, such as my taking a proton pump inhibitor because I ate too much at the last wedding I went to—that kind of thing.

This is what I'm pushing for. In fact, there are experiments now demonstrating that this actually produces better value for money—I think this is the key here, because we're all afraid that this is going to bankrupt us— and better health outcomes for the community at large.

That's my first case. I've actually suggested here that this value-based formulary essentially be free and that we attach the copays to drugs that could be used or misused in other ways.

Just applying that thinking, I want to make the case for parsimony. If you look at Canada, one of our biggest problems is uncomplicated hypertension, meaning that we're getting older, we're getting fatter, and we tend to have this problem.

What is Canada doing right now? I provide a bit of a breakdown on the slide called “Impact of Value-Based Formulary”. We're spending $2 billion on the treatment of uncomplicated hypertension—just that alone—per year.

● (1605)

If you look at the drugs that we're doing, they vary in price from $173 per person, per year, for a thiazide diuretic to $427 for a multiple drug therapy or for an ARP.

Does that make sense to you? Could we be smarter and say that we're going to give the thiazide diuretic free, and for all the other drugs you're going to have a copay attached to it? At least we'd know that we have an effective drug out there that could treat uncomplicated hypertension. If we did that, and even if we increased the use of thiazide diuretics by 25%, we would save $412 million a year. It makes sense for us to do something along that line.

That's not to say that it's without politics, but at least we have the evidence. We have the scientific evidence to support these decisions.

I will move on to my second point, which is that we're going to do this right and have a national approach to our pharmaceutical strategy, we need a value-based formulary first, and you must have a smart post-market surveillance system second. I am going to make the case here that Canada has some of the best assets in the world, which we've failed to use systematically. I think it's time we use them in a strategic way.

The first thing that happens when a drug gets approved is that it gets thrown out into the market, and people can prescribe it as they wish. If we want to look at off-label use, that was the origin of the unfortunate circumstances that lead to Vanessa's death. Approximately one in 10 drugs is going to be used off-label, meaning that the regulator didn't approve it for that indication. Of those drugs, not all of them lack scientific evidence, but the majority do. A little less than one in 10 will not have scientific evidence for the use of that indication.

It's a bit of a social experimentation that's going on. Sometimes that's a good thing and you discover things you wouldn't have normally discovered. The bad news is that when you are prescribing drugs without scientific evidence, you don't know what their effectiveness is, and you don't know what their risk is.

One of my colleagues did a study on this and looked at the rate of adverse events for off-label use of drugs without scientific evidence. It's quite a bit higher, as you can see in the graph for adverse events, because people are prescribing off-label. Are they doing this intentionally? Probably not. They probably don't know what the scientific evidence is or is not, and they may not have the most up-to-date information on that fact.

What do we do about that? One thing is that we'd better start monitoring off-label use, and we'd better start monitoring adverse drug events that are occurring in the population.

This next deck shows you the adverse drug event reporting requirement. What we know about adverse drug event reporting is that of all the adverse events that are reported, a small proportion, less than 5%, are recorded. Of all those that are occurring, very few are reported. Why is that? Well, it's onerous, quite frankly. No one pays anybody to do that, no one knows where the form is, etc. You can make it mandatory, which is the suggestion from the new regulations, but I think there's a smarter way of doing that.

If we look at our unused resources for doing this, first of all, we have a national health information organization that collects information, and could do so almost in real time, from everybody across Canada. It has agreements with all the provinces. It's called the Canadian Institute for Health Information. That's benefit number one.

Second, we have Health Infoway that invested heavily in creating data repositories for a population-based repository of all drugs prescribed and dispensed, all labs, and all diagnostic imaging.

Third, they've invested in electronic medical records. Now we have the digital information to start putting together a method of monitoring what drugs are being prescribed, what they are being prescribed for—meaning if we can monitor off-label indications—and when they are being stopped and changed because they're not working, or the person is experiencing an adverse drug event. We can capture all that information. Why? It's because we have these players in place. We've made these investments. We just need to pull it together into a national reporting system, and we have the talent in Canada to make sense out of that information.

Finally, we've just invested $50 million in having Infoway build a national prescription system. We can start capturing why this drug was prescribed, why it is being stopped or changed, and we'll know in real time. I've shown you an example of a screen that does exactly that. These are being built into electronic prescribing systems. If you stopped this drug, you need to let the pharmacist know you stopped it, because they're going to keep on dispensing it if you didn't tell them. The person stopped using it because they had an adverse drug event. And what was it? In this case, it was that the person had epilepsy, a seizure, from this medication. That can all be captured. You stop the drug; you need to tell people you stopped it, and that it was stopped for such and such reason, so that people won't re-prescribe it.

● (1610)

I'm thinking that we have all the pieces together and that it's simply a matter of trying to make use of all these pieces through a strategy that can be developed nationally for the country.

Thank you for your time.

The Chair: Thank you all for some incredible information.

Now we'll start the questions with a seven-minute round.

Dr. Eyolfson, you are first.

Mr. Doug Eyolfson (Charleswood—St. James—Assiniboia—Headingley, Lib.): Thank you all. This was all very useful information. I could not write fast enough to keep up with all that was really useful.

First, I have a couple of questions for Dr. Tamblyn.

We talked about an evidence-based formulary and about there often being alternatives that are more expensive but that don't do as well. We've had some criticism in the past, with some physicians and patients wanting choice even if the choice for a more expensive medication is not actually based on any improved outcomes.

Would you foresee a system wherein you could approve a drug for use, so that people had that choice, but not have it on the formulary? If someone has a proton pump inhibitor that is cheap, they can have it prescribed and it's paid for. If they really paid attention to the ad that said “Ask your doctor about Prevacid” and want that, they could get it, but they pay for it.

Is a system like that feasible?

Dr. Robyn Tamblyn: Yes. In fact, that's what I'm advocating. For chronic conditions for which we have very effective therapy, we can't afford to not let...just as we provide medical services for free, or hospitalization is free. We provide an equitable situation in our country for things that matter. We need to have that free.

Then you can attack the copayer, the sharing—it's always still available—under other alternatives that may be a bit more expensive, but for which something cheaper will do.

Mr. Doug Eyolfson: Sure. Do you know of any jurisdictions in which this is actually happening right now?

Dr. Robyn Tamblyn: Yes, there's a lot of experimentation going on in the U.S. and in the value-based formulary in the United Kingdom, where essential medications are free, and for the other ones you either pay completely out of pocket, which is currently what most of our population does.... They pay for everything out of pocket, unless they have private drug insurance.

Focusing on a value-based formulary, yes, is happening in both the U.S. and the U.K.

Mr. Doug Eyolfson: All right, thank you.

Another question is that when we talk about a formulary—you talked about off-label medications....

I should say, as a background, that I am a physician, so I'm familiar with much of that terminology, and I see medications prescribed for off-label uses.

Do you think there's a way that in a formulary there could be the indications for a prescription, so that you could say it's covered if you're doing it for the on-label indication but not covered if it's for the off-label? Is there a way of controlling that in a formulary?

Dr. Robyn Tamblyn: No, there is not really, not unless you captured the information at the point of care. That is what I'm advocating: you're going to build a national electronic prescribing system, and one of the requirements of it is that you indicate what the indication for treatment is. You can advise physicians at that point whether this is on- or off-label. I'm not sure they really know whether it is or it isn't.

It's there that I think you can capture it. Then you can actually do your conditional coverage.

Mr. Doug Eyolfson: Okay, good.

Mr. Herder, you talked about post-marketing research, about needing, once a drug is on the market, a system of surveillance to make sure that the effect you've seen is still there and that the efficacy and safety are still there. I agree that this is particularly in the case of the rare drugs for which you have low sample sizes. If the drug that has been marketed is found unacceptable and your surveillance shows it's not doing its job, are you foreseeing a mechanism whereby these drugs will actually be withdrawn, if something gets on the formulary, and you say this is not on the formulary because it doesn't do what it has promised?

● (1615)

Prof. Matthew Herder: In a word, yes.

Part of the challenge we face is that it's really hard in the current system, because of the patchwork that exists, to ensure that this occurs across the board and that therefore there is some level of fairness, and also that we're taking advantage of the kinds of opportunities Dr. Tamblyn highlighted to continue to evaluate and therefore make really informed decisions. In cases in which the evidence shows that over time a drug is not nearly as effective as perhaps Health Canada or the formulary thought, or is not as safe as we thought, or is being used on an off-label basis too much and therefore we need to end that kind of decision making, there might be instances in which coverage is withdrawn, yes.

Mr. Doug Eyolfson: For the question I just asked Dr. Tamblyn a second ago, do you know of any jurisdictions where they're doing this?

Prof. Matthew Herder: I think that's the assumption behind the jurisdictions that were mentioned, the U.S. and U.K., about moving in that direction, that it's necessary. I'm not aware of specific examples where it's been fully withdrawn pursuant to those kinds of agreements, but I think there's an increasing awareness that it's going to be a necessity because of the tenuous nature of the evidence at the time of approval and coverage. If we allow theses things to remain in perpetuity, regardless of what the evolving evidence tells us, that's a huge problem for safety and value for money.

Mr. Doug Eyolfson: In regard to the essential chronic drugs that are more widely used, one of the examples that is starting to come up in the medical literature right now is statins. A lot of money is being spent on statins. For anyone who doesn't know, these are the drugs that lower cholesterol. There's a growing opinion in the medical research that these do not improve outcomes at all and are not living up to the original therapeutic promise, but they're representing a high proportion of regularly prescribed drugs. Could such a mechanism withdraw an entire class of drug like that if it turns out that we're spending a lot of money on something that doesn't work?

Prof. Matthew Herder: It could potentially. The point I would make is that it could also elevate the standards for demonstrating improvement over existing therapies. What we see right now, unfortunately, is that a lot of new medications aren't terribly important for advancing care and health care outcomes. They are me-too drugs, and so on. I think this kind of body could encourage greater innovation that is in line with improved health care outcomes.

The Chair: Ms. Harder.

Ms. Rachael Harder (Lethbridge, CPC): My first question is for Dr. Wong-Rieger. I'm wondering if in Canada right now it is easier for patients with rare diseases to access the pharmaceuticals they need through a private or a public plan at the moment?

Dr. Durhane Wong-Rieger: It depends on the drug. When they're first approved by Health Canada, most of the private plans will make them available based on Health Canada's approval on the basis of safety and efficacy. They then have to go through CADTH and other negotiations to get on the public plan. So it depends. In the cases where the public plan makes them available, then they are accessible —sometimes in the same way and sometimes with more restrictions and criteria. Obviously, one of the benefits is that the reimbursement may be better because there isn't necessarily a private copay. There are more drugs that do not make it into the public plans, and sometimes it takes many years to get them into the public plans. The Fabry's example was one of the cases where for two years patients with private insurance were already benefiting, and it took us that much longer. It depends on the drug, the circumstances, and sometimes the criteria.

● (1620)

Ms. Rachael Harder: Would you be able to say more about one or the other at this point?

Dr. Durhane Wong-Rieger: The private plans will make any kind of second-generation innovative therapy available more quickly. The limitations come from the way in which the health technology assessments review the drugs for rare diseases. Like with Fabry's, they use a traditional method, and I think, as many of the other speakers have said, the level of certainty in terms of data is not there. They need a lot more post-marketing. They're going to be more niched even within that patient population. Not every drug works for everybody, so you have small patient populations.

What we have been able to do—and the private plans need to catch up with what the public plans are doing—is create the criteria for access. Similar to what everybody else has spoken about, and as Robyn indicated, within the rare disease access, no drug gets administered without good post-market monitoring. In fact, many of them are administered only through specialized clinics and very named physicians. It is not the case that anybody could write a prescription for a rare disease drug. In some respects, as I think Robyn said as well, we don't utilize all the resources that are available to monitor and track them. As Professor Herder was talking about, the limitations are definitely there, but it's not because we don't have the mechanisms. We do, but we don't employ them. I think those are where the challenges are. In some respects the public drug plans are providing appropriate access, but they're just slow about it. In many cases it takes a long time for them to set up the mechanisms.

Ms. Rachael Harder: As Mr. Herder referenced and you now are referencing, there are no results-based data, or very little in my understanding, with regard to making the decisions concerning orphan drugs and whether or not they would be included.

Mr. Herder, you said that when we make these formulary decisions going forward, they should not be politically based, but results based. If we don't have those results for orphan drugs, then how are we to make those decisions? Are you suggesting that orphan drugs should not be included in the formulary? If that isn't what you are suggesting, then I wonder where we draw the line in terms of what is going to be included and what is not going to be included in the formulary going forward.

I would ask this question of both Dr. Wong-Rieger and Mr. Herder.

Dr. Durhane Wong-Rieger: In many respects, we don't expect them to be included in formularies in the same way common drugs are. The formulary implies that almost anybody can prescribe it, unless the drugs are quite restricted. We believe there needs to be, for most of these drugs, managed-access programs. We believe they need to come in with, as Professor Herder was talking about, clear criteria for who gets access. We need to have start-stop criteria and know if the patients fit the criteria. What's happening is that in many cases these start-stop criteria in Canada are very impoverished relative to those in the rest of the world.

The other thing that's so important is that we need to be part of international monitoring. In this regard, I just came back from the European Congress on rare diseases, and I just came back from ISPOR in Washington and HTAi. There is not enough of such monitoring in Canada with these rare-disease drugs. We need to be much more in sync with what is happening in Europe—and I mean Europe even more than the U.S. This is the problem with our not having an orphan drug regulatory formulary. Our drugs don't come in at the same time. They come in with different conditions sometimes. We start to collect evidence, but we only have a small amount of evidence. We bring in the drugs on the basis of the evidence that Health Canada and the international community can approve in terms of safety and efficacy. We then continue to collect evidence and we continue to reassess. We reassess on an individual basis—

Ms. Rachael Harder: I'm sorry, I'm going to cut you off there—

Dr. Durhane Wong-Rieger: Okay, yes.

Ms. Rachael Harder: —just so Mr. Herder has an opportunity to respond to this question. Thank you.

Prof. Matthew Herder: If I indicated what you're suggesting, I might have misspoken. What I meant to say was not that we would adopt any kind of blanket rule about excluding these, but instead, in the case of orphan drugs, or drugs that target rare diseases, the evidence base is that much more limited at the time of approval by Health Canada. Ongoing monitoring to decide whether to pay for them and then continuously after that is that much more important. It's not that we would adopt any kind of blanket rule about in or out because they treat rare diseases, but rather the ongoing evidence collection is an imperative.

● (1625)

Ms. Rachael Harder: Robyn, you look like you have something to say to this. Yes? Would you like to weigh in?

Dr. Robyn Tamblyn: No.

The Chair: Mr. Davies.

Mr. Don Davies (Vancouver Kingsway, NDP): Dr. Tamblyn, I'll begin with you. You've conducted research to measure the impact of the number of hospitalizations caused by the fact that people do not pay for the drugs they needed. You've elaborated a bit on that. I have two questions. Can you quantify that for us, if you have any numbers, and would a universal system of the type you described assist in that regard?

Dr. Robyn Tamblyn: Yes, we did quantify it. You're going to ask me to remember the numbers, right?

Mr. Don Davies: Ballpark figures.

Dr. Robyn Tamblyn: It depends upon the sub-population we looked at. We were looking at those on social assistance and seniors. For seniors with cognitive impairment, it increased their hospitalization rate three-fold. For those on social assistance, the biggest hit was for people with severe mental illness, where their hospitalization rate within the six months of the introduction of this policy went up six-fold. It was quite devastating, especially when drugs work. If it's a statin, you have to wait awhile for things to happen. When it comes to a drug for mental illness, or when it comes to an asthma pump, you see things happen faster. We only followed this for nine months after the policy reform, to look at what the policy-induced non-compliance did to the population. I can only comment from that perspective.

Whenever people have looked at outcomes, when it comes to cost-sharing, number one, you always see a reduction in the use of essential medication when you do it. Number two, if you believe those medicines work—take the subset that do work—and you look in the short term for things where it works like your insulin pump, you're going to see complications happen quickly.

Mr. Don Davies: So it would be fair to say that were we to bring in some form of universal access for essential medicine that works, we would see significant savings in—

Dr. Robyn Tamblyn: Absolutely. There's no doubt in my mind that you would.

Mr. Don Davies: Okay.

Now, if I have your system correct—pardon my oversimplification—your suggestion is that in a universal system we provide, for free, access to essential effective medicine.

Dr. Robyn Tamblyn: Yes.

Mr. Don Davies: And then a copay for medications that are what...?

Dr. Robyn Tamblyn: They would be like therapeutic equivalents to treat the same thing. There are probably about six categories of medications to treat uncomplicated hypertension, so I would put the one that is the most cost-effective in the value basket and put the others in a copay basket. You can actually make a conditional listing on top of that, as suggested here, where the conditional listing is that "you show me that this person has that or has failed this kind of treatment before and therefore I'm going to pay for it".

That has administrative overhead on it in terms of someone having to manage that program. Payers in the U.S., for example, have a lot of those kinds of programs, and they change them practically weekly.

It's really to say this: can we at least get our value out of a system by giving free medication for absolutely essential diseases and the most cost-effective drug to treat that condition in that category? Yes, we agree that others could equally work, but we have something that will work and we want to pay and negotiate the best price for it.

Mr. Don Davies: Okay.

Dr. McCabe, when did the U.K. bring in their universal coverage?

Dr. Christopher McCabe: I believe it was with the establishment of the NHS in 1948.

Mr. Don Davies: In 1948? One of the raging debates going on between witnesses in this committee, I think, is whether bringing in universal pharmacare would cost us billions of dollars or save us billions of dollars. I'm wondering if there is any research in the U.K. on the experience of what happened there.

Dr. Christopher McCabe: It was a time of transition. Rudolf Klein did some work in the mid-eighties and looked at historical records. There was undoubtedly a transaction cost, because you had a culture shift. In the first couple of years, from 1948 through 1950 to 1951, if I remember rightly—it's a long while since I've read the book—you saw a real spike in prescribing levels. Anecdotally, doctors were filling their surgeries' dispensaries. There was a real spike, but it then settled down.

What we didn't have in the U.K. at that time was any mechanism for controlling. We didn't have a national formulary. We just had a legal commitment to pay for anything that a doctor prescribed. They got their prescription pads, they were used, overwhelmingly appropriately, and the health of the population definitely improved, but there was a very large spike that kind of shocked the system. Gradually, over time, mechanisms approaching formularies were developed.

Now we see the most recent version of that, which I think Dr. Tamblyn and Dr. Herder were referring to, specifically around cancer drugs, where new cancer therapies that are not clearly high value at the time of licensing will be entered into a special scheme called the "cancer drugs fund", and data will be collected on outcomes, typically for two years. At the end of the two years, if they haven't performed sufficiently well to be judged valuable by NICE, then they won't be paid for anymore.

There were some 130 cancer drugs approved under the previous situation with the cancer drugs fund, and NICE is going to be reviewing them to decide whether they are moved in. They are actually taking health technologies drugs off the market now.

● (1630)

Mr. Don Davies: That leads into my second question. Some people who criticize Canada's bringing in of a universal pharmacare system also argue that it will restrict the formulary, that patients will not get access to the drugs they need, and that it will restrict innovation. What was the U.K.'s experience in that regard?

Dr. Christopher McCabe: With having such a large domestic pharmaceutical industry in the U.K., it's obviously a major concern whether the restriction of access would actually impact upon investment and innovation in the U.K. I would observe first of all that the initial analysis done by the U.K. Office of Fair Trading established, as many others have, that there isn't actually a relationship between pricing behaviour and inward investment for the pharmaceutical industry. That claim isn't particularly strong.

Secondly, I think what is very interesting for Canada is that they worked to align the health system with the R and D process so that the U.K. continued to be an attractive place for the research around innovative drugs, because it could deliver high-quality patient-level information on the outcomes of patients receiving them and hard systems such as the NICE patient access scheme for negotiating those conditional access arrangements almost immediately upon licensing approval. The key economic role of the pharmaceutical industry in the U.K. economy has been maintained by aligning what the health system was doing with innovative technologies and the research capacity.

Mr. Don Davies: Are patients getting access to the broad range of pharmaceuticals that they need, that the doctors are prescribing for them?

Dr. Christopher McCabe: It depends on whom you ask. If you ask NICE, they will tell you they have turned down only two cancer drugs in all of their history. If you ask the cancer community, they will say they have turned down 40-odd. The difference is that NICE will count.... If we say yes to a specific subgroup that is not all clinically indicated, we are saying yes. The patient advocacy groups will tend to say that unless you say yes to every patient group that is named in the licence, you are saying no.

Overall, the access to cancer drugs and most innovative medicines is pretty effective. There are a few exceptions, but overall there is very good access at reasonable prices.

Mr. Don Davies: I am done.

The Chair: Mr. Fragiskatos, go ahead.

Mr. Peter Fragiskatos (London North Centre, Lib.): In your presentation, Dr. Wong-Rieger, you talked about kitchen table organizations, and I immediately thought of an organization in my riding of London North Centre, Bethanys Hope Foundation. You are nodding your head yes, so I imagine you know Dave and Lindey McIntyre very well. They have been active for the past 20 years, after losing their little girl, Bethany. She was seven years old when she passed. They have raised $4 million for research, and they continue to work very hard on this.

Can you tell me about the experience of organizations like this, families who are trying to raise funds and awareness, and trying to access treatment for their suffering loved ones?

The system—from reading the briefing notes for today, and knowing a little bit about the travails of families who experience rare diseases—seems very bureaucratic in Canada. It is a patchwork quilt.

Could you tell me about best practices from other jurisdictions? I know there is NORD, the North American organization, your counterpart, which looks at rare diseases on a North American level. You talked about the Orphan Drug Act in the United States. You talked about the European experience.

Are there other lessons to be learned there for Canada and Canadian policy-makers trying to deal with this question of rare diseases, either from the EU or the United States? We are talking about a national pharmacare plan. Rare diseases can be dealt with in a national pharmacare plan, I would expect, but do you have any ideas on that?

● (1635)

Dr. Durhane Wong-Rieger: As you say, there are so many good examples internationally, and Bethanys Hope is a wonderful group. They really are typical of many of our patient organizations that, as you say, are made up of parents who are doing fundraising, trying to raise awareness, and trying to develop a patient community so that if in fact research is being done or clinical trials are being done, they have patients who are identified and who can enter into that.

I will say that sometimes people misunderstand what an orphan drug regulatory framework could do for Canada. The biggest thing it could do for Canada is bring Canada into the research and development scheme.

We have researchers and small companies in Canada that are doing exactly what Bethanys Hope is raising funds to do. We have many examples of where in fact they actually discover the cause of a disease, and they actually discover a treatment that could be developed for that disease. In many cases, because we don't provide the facilitative support in Canada, these companies pick up and move elsewhere because there are more incentives to develop there, so Canada becomes only a buyer of drugs, not a contributor.

The other sad point is that many of our patients then don't get into the clinical trials. When we're talking about a progressive disease, you want to get your child or your family member in as soon as possible. In developing those communities, there are many examples in Canada, and many of these groups are also connected internationally, which is a big bonus, but quite frankly, that regulatory framework would help us support the research and development in Canada and engage more patients in terms of clinical trials.

At the pharmacare end, in Europe right now we're seeing the emergence of international programs. The EMA approves drugs for all of Europe, but each country there has been traditionally purchasing their own drugs, sort of like the provinces in Canada. What's emerging, in fact, are international programs. Belgium, Luxembourg, and the Netherlands are coming together to create international purchasing, and Austria and Romania and others are coming together to say that they need to work together at a European level, not just to get better pricing, which could in fact happen.... The pricing comes not because they're better negotiators, but because they can provide a better volume discount. If you can guarantee me *x* number of patients and you are willing to risk-share with us the introduction of these patients, we can actually introduce at a lower price and we can demand a lower price.

There's no upside for the patient in having very expensive drugs. We are very committed to bringing in drugs as cost-effectively as possible, and that means we can actually cover more people. We're also very committed, as I think both Professors Herder and McCabe said, to making sure that the drugs are available to patients as they work. But again, we have to work in collaboration. We have to work internationally. There are not enough patients in Canada with many of these diseases for us to know over a period of time, or at least know more quickly....

So I think it's very much the case. We have so many of these small patient groups that are working hard in Canada, and oftentimes collaboratively as part of international organizations to bring in these drugs. What we want to do is to give Canadians a fair shot. Right now, we're not doing that. I think that's the challenge. We're not doing it at the research and clinical trial level, and we're not doing it at the reimbursement level. If we would work together, we could do it better. If we would work internationally, we could do it really well.

Mr. Peter Fragiskatos: Would you point to a specific state that stands out for you? Let's take Europe, for example, where things are being dealt with effectively. Bethanys Hope is focused on metachromatic leukodystrophy. There is also Krabbe's, another rare disease, a sister disease. There are so many others. I emphasize these points because 8% of Canadians are facing a rare disease. If we can learn from a particular example, then I think we'll be better off as a country and we'll be doing our job as policy-makers.

● (1640)

Dr. Durhane Wong-Rieger: You've heard some of the discussions around coverage or evidence development. Germany brings in a drug, they negotiate a price, and then they get a year to actually demonstrate that they have the outcomes they've promised. France negotiates a price, they have a year in order to make that drug available, and they get a negotiated one, two, three, or five years to actually demonstrate the outcomes.

If you get the outcomes, you get to maintain the price. If you don't, you get to reduce the price. In theory, if you do better, you get to raise your price. I don't know if that has actually happened.

Right now, in terms of Belgium, the Netherlands, and Luxemburg, as I say, they are coming in with managed access programs. The Netherlands is a very good example. With Fabry disease, they brought in the program and monitored patients over a period of time. They then began to say that some of it was working and some of it was not working, and they said who were the patients for whom it might be working. The patient community had a heavy consultation role in trying to parse out that community to make some decisions on how it would work better.

If Canada were to work much more internationally in this realm, we could do so much better. We're a small country. As you say, the U.S. has a big population, and their conditions of access are a little different and they collaborate a bit differently in terms of which patients get access. I can tell you that some of the patients in the U.S. are getting very frightened, even with private insurance plans, because the private insurers basically are also starting to come up with differential schemes, and they don't know how to collaborate.

I was amazed at how many times people pointed to Canada and said, “You folks have a great model.” They said that we have the best potential for bringing in the very best program, based on some of the infrastructure we have and based on some of the monitoring we have. When we start to talk about pharmacare in Canada, people really do suggest that Canada could actually get it right. But we would have to do it internationally.

The Chair: We'll now move on to round two.

Mr. Webber, you have five minutes.

Mr. Len Webber (Calgary Confederation, CPC): I'll direct my first questions to Dr. Wong-Rieger and Maureen Smith.

A big frustration for me is the fact that our country runs on the silo system with regard to our health care system. We work within our provinces. You make perfect sense about collaborating internationally and collaborating amongst ourselves here in Canada, but of course we're not doing that, especially in your area of rare diseases.

We are currently debating a private member's bill in the House here on human organ and tissue donation, on a national registry in this country. I don't know where it will go, if it will succeed or not in terms of passing, but one of your points here is that you'd like to see a national registry created for all rare diseases. How do you see that helping you when we're in a system now where the provinces and territories tend to continue to hang on to that silo system?

Dr. Durhane Wong-Rieger: I think the pressure in terms of breaking that silo is happening organically. We've already seen the pan-Canadian Pharmaceutical Alliance recognizing that by negotiating together they can get not only better pricing but also better criteria in terms of making that happen. A patient registry for rare diseases is not something unique to Canada.

In Canada we have amazing resources in terms of being able to do that. We have some of the very best genomic sequencing labs in Canada. We can actually register patients according to identified phenotype, the physical features of a rare disease, and we can identify them in terms of their actual genetic sequence so that we can know what these patients' defects are that are leading to a particular rare disease. We have a great program in Canada, a matchmaking program where you can actually match up patients who have similar genetic sequencing that are defects and begin to say, “Oh, my gosh, these are families here”. I think we have a huge capacity in Canada to develop very rich registries. I travel to international conferences, and people point to Canada and say that we have the most amazing system and the greatest possibility of being able to build those registries.

We also have great collaboration among our specialists. We have metabolic specialists who work together. Most of our pediatric centres work together. We're big enough that we can have these, but we're small enough that our researchers, our clinicians, and our scientists actually know each other. For the most part, they actually like each other. They actually want to collaborate together.

We have all of that capacity. It means then that if we have a treatment coming up, patients who are already registered—we've talked about Bethanys Hope—can be entered into the clinical trials early on. We have seen examples of that already happening. I think the possibilities are huge here in Canada. We just have to be able to harness them. CIHR has done huge investments in terms of personalized medicines and supporting these kinds of disease programs. What we lack is the actual incentives for companies to actually set up that research and carry it to the next level here.

● (1645)

Mr. Len Webber: I was just going to mention that as well, the fact that these companies are not motivated to conduct these clinical trials due to the small population for rare diseases.

I have a quick story about a young constituent who came to me. This was a few years ago, when I was an MLA. She was just coming off of a clinical trial on a drug that was a godsend to her. It took her out of her bed. She had rheumatoid arthritis that was very severe for a young 21-year-old. She went through this clinical trial. It was a miracle drug for her. It got her back to work. It got her contributing to the tax system as a taxpayer, and she was happy and productive. Well, the trial ended. No more drugs for her.

The reason she was in my office was to ask if these pharmaceutical companies could do that, could take away a drug once they'd done their testing. It had not gone through the formulary, or hadn't been brought to the government yet, but they were ready and had done their research. Then they cut the drug off.

Dr. Durhane Wong-Rieger: We're doing more and more, especially in the rare disease community and with drugs in which there are no alternatives, to ask companies to set up bridging programs, to make guarantees that if your patient comes off the therapy and it's not yet approved, you continue to fund until there's an actual decision in terms of reimbursement.

We are also doing more in terms of insisting on crossover drugs. Take a small patient population with a debilitating disease. What if you're in the placebo group? How do we get patients to want to do that? We're asking companies to guarantee that there will be a crossover. Once the trial gets to a certain point, where it's clear that it's working, they have an obligation to take a patient off of placebo and to put them on the drug. With small patient populations, we can do some of that, but that's the right thing to do. We really reinforce that. They can't do it for life, but we really reinforce it until we get a good decision on the reimbursement.

The Chair: You are done?

Mr. Len Webber: I am done.

Thank you.

The Chair: Mr. Ayoub, go ahead.

[*Translation*]

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

I want to begin by thanking the witnesses for joining us and for their highly inspiring testimony, especially Ms. Wong-Rieger's and Ms. Smith's testimony.

Orphan drugs are very expensive; that's the norm. Those are the most obvious cases. How can those medications be assessed? My concern is really the assessment of those medications and their cost effectiveness. Of course, we would like something that costs a lot to be very effective. However, that's not often the case, and those medications will not be on the list. This issue has been discussed on several occasions.

What are the assessment steps for those orphan drugs to be put on a list? My question is for each of the witnesses. How is a ranking established to ensure that an orphan drug ends up on the list?

Mr. McCabe, I would like to hear your comments on this.

[*English*]

Dr. Christopher McCabe: In principle, conceptually, the comparison is the same as for a conventional therapy. The difference is about the level of certainty or uncertainty, the risk that what you observe in the trial is not actually going to be observed in practice when you roll it out to more patients.

The challenge of the orphan drugs to HTA is how to deal with that uncertainty. Intellectually, conceptually, the way to deal with it is to recognize the price, the value of that uncertainty. Health care is very much like laying a bet. We think something is going to work, but it doesn't work in everybody, so we take odds, like five to one, and we are willing.... If I were going to bet $5, I would probably quite happily do it on a hundred-to-one bet.

If I have to bet $10,000, I am probably not going to go more than 1.1 to 1. This reflects that uncertainty has a value, and there are mechanisms for calculating the value of the uncertainty.

What that information allows payers to do is negotiate down the price to reflect the uncertainty at the time of introduction, and as the evidence is accumulating, because the technology is on the market and being used, there is then the possibility for the price to increase to reflect the reduced risk, but actually you are buying something that doesn't work.

That is why we need, for these types of technologies.... Orphan drugs are one extreme, but also for precision medicines there is a large overlap. We are going to be moving into a world where decision-makers are going to have to be much more sophisticated. It is not going to be a simple yes or no. It is frequently going to be yes with conditions. Those conditions will reflect the nature and the magnitude of the uncertainty that is in play. Does that help?

● (1650)

[*Translation*]

Mr. Ramez Ayoub: I think so.

If I understand correctly, there is no uncertainty when it comes to more common diseases because there is scientific evidence to answer those questions. Normally, anything to do with medications is related to the scientific field.

[*English*]

Dr. Christopher McCabe: There is still uncertainty, but it is of a level where the cost of doing the research to reduce that uncertainty further, both in terms of health gain foregone from delaying general access and the direct cost of running the study, is less than the value of the uncertainty. It is just a bigger issue for rarer diseases, and that is why we have to [*Inaudible—Editor*].

[*Translation*]

Mr. Ramez Ayoub: What I am getting at is that the more research and investment there are, and the more data is gathered, the more the scientific community is able to find specific remedies and cure a disease that seemed incurable for maybe dozens of years. AIDS is one example of that, and there is now treatment for that disease.

That is one example of short-term choices to be made to be able to counter diseases that are now defined as rare and ensure that they will no longer be rare in the future. It's about the level of research, the sharing of information—we talked about the sharing of information internationally. It's about seeking out solutions and ensuring that everything is being put in place to share information on those issues. However, there are many silos in Canada—and my colleague mentioned this—but also around the world. What potential solutions should be considered to improve the situation and enable us to quickly make decisions in this area?

Ms. Smith, you can answer if you like.

[*English*]

Dr. Durhane Wong-Rieger: Can I just say what you hit on is exactly it, because we do clinical trials internationally. We know that with rare diseases we're not going to get all of the information from patients that are just going to be in one locale.

Post-market is the same thing. Why won't we assume that we can go to one locale, one province, one hospital, or one country even? We need to be collaborating internationally.

The challenge is that the access criteria are not the same internationally. Unless Canada is willing and able to collaborate.... Mind you we can adjust some of it scientifically, etc., but we need to be thinking about these as ongoing international environments in order to develop the monitoring. It means, in many respects, that collaboration must go from the beginning all the way through to the end.

[*Translation*]

Mr. Ramez Ayoub: Mr. Herder, did you want to say anything on the issue?

[*English*]

Prof. Matthew Herder: That kind of international collaboration will be a lot harder if we have multiple payers and health technology assessment bodies. I think it adds to the argument for a national decision-maker so that we can collaborate. You're seeing this already to some extent when a few European countries recently decided to join up to make decisions around rare diseases therapies for a whole bunch of reasons, such as improved bargaining power and to collaborate.

I think that makes the case for national pharmacare here, as well.

The Chair: Does anybody else want to throw their oar in?

Mr. McCabe.

Dr. Christopher McCabe: I'd like to reiterate that in those international relationships and through collaboration, a national pharmacare can not only contribute data, but that frequently with orphan drugs the companies control almost all of the evidence that's available. By having a national pharmacare that negotiates, you can negotiate not only price, but also the site of the data they hold and accumulate through their registries, to help Canada more rapidly get an understanding of the true value.

I think there are a lot of opportunities in what Dr. Wong-Rieger was describing, not just at the regulatory level but at the national pharmacare reimbursement and related research level as well.

● (1655)

[*Translation*]

Mr. Ramez Ayoub: Thank you.

[*English*]

The Chair: Mr. Carrie, you have about 25 minutes now.

Mr. Colin Carrie (Oshawa, CPC): Awesome. Thank you very much.

I could go on all day with something like this.

Professor Herder mentioned something about a national decision-maker. This is one of the things that I struggle with, and I know about all of the great work that you've done, Dr. Wong-Rieger, in the past on rare disorders, which I thank you for.

My colleague has mentioned private insurance versus the public purse, and we've had different advocates here in that regard. Some do want that public system focused on. I know that Dr. McCabe was with the National Health Service, and it would be great in Canada. I've always thought that we just needed one decision-maker to work that out because all these silos are a bit of a challenge.

My concern is, who decides, and what do they base that on? I've had constituents in the past... I remember when Remicade came out, which I think might have been the drug you were talking about. Some of these make a huge difference. Sometimes a private insurance company may make that more available, and it can be a life changing thing, whereas the national system may take a lot longer to get that.

How do you see a balance on this system, and who makes that decision?

Prof. Matthew Herder: Is this for me?

Mr. Colin Carrie: Sure, for both of you.

Prof. Matthew Herder: I think it's a great question.

I think there would have to be a lot of follow-up work about exactly how that body would be constituted, with representation from provincial decision-makers and other kinds of experts. We'd need to do a lot of thinking about how exactly that would look and how its independence would be protected from both outside and within government. I think a lot of careful work would be required.

The basic point to take away from my remarks is that there's a question of scale and capacity in Canada given the silos that exist. You see private insurance and some of the big ones in the United States—Kaiser Permanente comes to mind—that do have the capacity to critically evaluate evidence as it accumulates over time. I would venture to say that is not the case for private insurers and most provincial decision-making bodies in Canada.

We need to scale up for that reason and to improve our capacity both to negotiate better prices and to better appraise the information as it's coming, building on this approach I think CADTH has, but which is undergoing some rethink around these new kinds of rare disease therapies, as well.

We need to scale up and build capacity first, and a national body seems the right approach. Then we'd have to do a lot of careful work about how to constitute that with the appropriate representation. I should add that this feeds into the underlying legal issue around constitutional responsibility around health care, and that to some extent this would drive how this body would be created at the national level, as well.

Mr. Colin Carrie: Would you diminish the private sector insurance type of thing?

Prof. Matthew Herder: I see a public body as more able to strive towards key goals around fairness and access such that these really tough decisions.... Many of them will be challenging decisions. If you are a patient without any treatment options, you are going to consider that medicine essential, but the evidence base at that point in time and the price at that point in time may make coverage prohibitive.

I think if we are really thinking in terms of those public health care kinds of values, a public body is better suited to take on that tough decision-making process than a private one.

Mr. Colin Carrie: Dr. Wong-Rieger, would you agree with that?

Dr. Durhane Wong-Rieger: I would not disagree with that. Let's put it that way.

I think for patients, and especially with higher-priced medicines, innovative medicines, we have always looked to the private drug plans because they can make these drugs available to the patients quickly. What we want to do is make sure we have a public system that can respond appropriately.

I don't want to bring the private drugs down, but I can't disagree with the fact that we need to have, especially in this arena, a single system. That single system is going to be able to take the evidence and to develop a plan.

To be honest with you, we see the private insurers going that route as well. The two of us just came back from Washington. At an international society meeting on patient-reported outcomes, two of the biggest private insurers in the U.S. were talking about the HTA approaches they are bringing in.

We have met recently with private insurers in Canada who are looking at these kinds of managed programs.

I will say that I shudder a bit, because they do not necessarily have the expertise at their disposal in order to do as good a job as the national agency that we have.

As much as we may deplore CADTH, in terms of its coming up with right decisions in as timely as possible way, it stands as one of the best HTA agencies in the world.

● (1700)

Mr. Colin Carrie: You mentioned the potential with these registries.

Dr. Durhane Wong-Rieger: It has huge potential. We already see a company like Manulife saying, “We will wait for CADTH to make a decision before we fund some of these drugs.” Well, okay, are you going to fund CADTH to do that as well? Quite frankly, we don't think CADTH is looking at the same criteria as the private plans are, so if you are going to do that, you are going to have to expand it.

We would rather see a single body that can do that. There is no doubt about it. Whether there will still be private funders, I think that is a different question.

Germany has public and private plans, but they have one HTA system. They have one national registry. They have one monitoring system in order to collect that information.

At the end of the day, who puts their money into a plan in order to get the benefits from it is very different from who should actually have an overarching plan that would make the recommendations about drugs and how they are going to be monitored, and have—as I think everybody says—a combined registry that would allow us to track all that information.

At the end of the day, if there are some private plans that are going to be funding differently and people want to buy into those.... I don't know enough about it to make a real recommendation, but all the things we are saying.... I think having a national system that would actually do that is essential, especially for rare diseases.

Mr. Colin Carrie: Dr. Tamblyn, I see you want to get in on this.

Dr. Robyn Tamblyn: I can hardly wait.

Mr. Colin Carrie: I appreciate the work you guys have put into this, especially Prof. Herder. You did work on Vanessa's Law. I think a lot of it is the collection of data afterwards, the on-the-ground data you can collect with this.

The potential for registries, I think, is phenomenal in this country. What is your opinion on that, Dr. Tamblyn?

Dr. Robyn Tamblyn: Canada leads in terms of the post-market surveillance area, in terms of comparative safety and effectiveness of drugs. We are the world's leader in the analysis of that. We have the drug safety and effectiveness network, funded through Health Canada, which uses the repositories of population-based data in each and every province to answer questions about the concerns about the safety of drugs. This is something that is a huge success story internationally for Canada.

We don't have the richest data. We don't have patient-reported outcomes in there. I think we need that. We don't have information such as lab values and other such rich clinical data.

I guess what I am trying to say is that we are collecting that. We put it in repositories, and then we don't use it. We have the capacity. Now it is just the political leadership, I think, quite frankly, that we need.

We do have a central body that does a common drug review. Ontario and Quebec do their own review, I think more for political reasons than anything else.

What we don't have is the machinery for this proactive post-market surveillance system, a strategy that puts it in place and someone to lead it. Who is going to be responsible for that? We have all the parts. We just need the political leadership and the strategy.

This international network is absolutely going to be essential for rare diseases, but the other thing that is important to consider is that there may be some real benefit in some national role for negotiating prices. I think everybody would be appreciative of that, especially for what you might call the bare essentials. Absolutely, we could do so much better.

I forgot my last point, so I will just stop there.

Mr. Colin Carrie: That's good.

The Chair: Mr. Oliver.

Mr. John Oliver (Oakville, Lib.): Dr. McCabe, I had the privilege of serving on the Ontario Health Technology Advisory Committee for a number of years back when Les Levin was involved, and your cost-effectiveness research has been quite helpful.

You made a comment in your report about how we would start up. I have five minutes and three questions, just so you can time your answers. There is advice for us in this report as to how we would start up the formulary. I think you said a number would be grandfathered in. Is there another system like the one in the U.K. or another that you would recommend we adopt as our initial formulary while we sort through who would be developing...? This is for general-purpose drugs and not just for orphan or rare disease drugs.

● (1705)

Dr. Christopher McCabe: I'm quite a fan of what the Netherlands do, actually. I think the U.K. does lots of good things, but it also has a very long history, which constrains things it wants to do. It has a very, very big formulary, containing about 25,000 different things. The Netherlands kind of started later, and actually I think they've been pretty smart, so I would go look at the Netherlands. I also think IQWiG has been very clever, in Germany, and Belgium's system is good also. Those are the three that I would go look at, because I think all three of them are smart in different ways.

Mr. John Oliver: Do you feel they're fairly translatable? Could you take the formulary of the Netherlands and in some way start up with a formulary for Canada? If it needed to be fine-tuned, could CADTH handle a national formulary? I asked that question when they were here, and they said yes, that would be right in their bailiwick. Is that where you would recommend they be?

Dr. Christopher McCabe: I would agree. CADTH would be an excellent lead organization to put that together. Very briefly, what a health system pays for is an expression of a society's social values. You can't just pick up somebody else's, because it's about what Canadians value. There's going to be a large overlap, but you're going to have to be sensitive to social values around the edges. There are going to be some things you don't care about, and some of the things they don't care about, Canadians are going to care about. For the core things like essential medicines, I think you're there. With regard to the process for what comes in subsequently, I think CADTH is exactly the right organization. Knowing what I do about their direction of travel, I think they're only getting better in that realm.

Mr. John Oliver: Okay, thanks.

Coming back to the discussion of private-pay and orphan drugs and rare drugs versus a public system, you said in your report that a national pharmacare program would eliminate the huge variation in the time to access orphan drugs, implying to me that this would be a better, more robust model, and that there would be less of a dichotomy in how they come forward. However, you thought it needed to be tied into conditional licensing and a national reimbursement process. Could you just talk about that a little bit, and would you offer maybe a slightly different view than Dr. Wong-Rieger did on a private versus public system, and the market?

Dr. Christopher McCabe: I think in the orphan-drug space, the prices of these technologies are so high that relatively few people have the ability to sustain the premiums they have to pay. I think as a general principle, medicines for severe orphan conditions should be on the essential medicines list and should be provided publicly. Access to them shouldn't be a function of your socio-economic good fortune. The public should pay for them following very structured, clear criteria. It's not very good to use the routine data infrastructure to get the evidence and the clear clinically driven but value-informed basis for stop-starts with those technologies. If we don't do that, I think we're missing a real opportunity.

Mr. John Oliver: The other point you raised on orphan drugs, which I thought was really interesting, was that a national program could operate as a mechanism for raising the quality of care, because caregivers and treatment centres for those conditions, which know how to effectively manage them, are usually scarce, so if you tie the pharma component to those, you could get a great outcome. Are you aware of the jurisdictions that have done that? I get the theory of it.

Dr. Christopher McCabe: We did it in the U.K. It's probably the best example. If there's one thing I'm really proud of in the U.K., it's NHS' outstanding specialist commissioning program, which I think has genuinely been world-leading. They used it to drive up standards and reduce variations in the package, and therefore the quality of care. It's not right that we see families having to move to another province to get the best care for their disease. That's not what we want. I see one of the kind of fortunate benefits as being able to improve quality of care for these families. It's not the sole reason for doing it, but it would be foolish not to seize that benefit.

Mr. John Oliver: How would you set that up? How would you envision a national model in terms of rare diseases and orphan drugs?

Dr. Christopher McCabe: Informally, and correct me if I'm wrong, these networks kind of exist, but they don't necessarily get the resourcing support they need to really leverage the benefits. I think if you go to those informal communities and ask who the best people are and what optimum care would look like, and back that up with contracting and resources, I think they'll tell you what it should look like, but with national resources rather than provincial or territorial resources; it's much more feasible than in our current siloed world.

● (1710)

Dr. Durhane Wong-Rieger: Can I just add to that? I think that's an excellent point.

What's happening in Europe now is that the EU has just announced expert reference networks. These are grouped around 40-some conditions. Some of them are rare, some of them are not, but usually they're around different kinds of conditions. That's exactly the concept: it's within these...and they're right across Europe. They don't just work within the boundaries of one country, they actually span Europe. Clinics have to bid for them. They bring together all the experts from around Europe that would be part of it. Most of them are virtual centres, but there are actual physical sites for most of them.

To be honest with you, there's a great model for it. They have criteria, they have evaluation, and in order to qualify and to get the funding for them, they have to meet a very high standard. I think we could use that model in Canada. Even more importantly, as we talked about this past week, if we got our act together, and we actually had Canadian reference networks, we could actually make them part of an international reference network.

Again, we would have the opportunity to do that. We don't have to reinvent this. The models are there. They're built exactly, as Dr. McCabe says, on the kinds of networks we have, but in many cases, except for hemophilia and some of the others, they are much more informal than they are formal.

The Chair: Mr. Davies.

Mr. Don Davies: I think we should set up a national formulary right now, put these five witnesses on it, and be done with it.

Voices: Oh, oh!

Mr. Don Davies: To pick up on Dr. Wong-Rieger's last comment about models, a lot of jurisdictions in the world are struggling with the same issue. I think some of you have answered this, but I'd like to ask Professor Herder if there is a jurisdiction he would point this committee's attention to as a jurisdiction that would be particularly helpful for us to examine as a model for Canada.

Prof. Matthew Herder: This is not a novel answer, but I think it's really important to pay attention to the U.K. This is because of its obvious strengths as a national pharmacare example, and the kinds of things it's done that Dr. McCabe just mentioned, but also because of some of the tribulations associated with it. There have been challenges. When you concentrate decision-making in one place, the pressure on that organization to keep performing and make evidence-based decisions that aspire to some level of fairness will also increase. No system will be perfect, and I think there are important lessons about the institutional independence of NICE in particular that are worth paying attention to.

Mr. Don Davies: Dr. Tamblyn, I don't know that you've given us your suggestion about a jurisdiction that might be particularly instructive for us.

Dr. Robyn Tamblyn: I would agree with Chris's comments on the Netherlands and also the U.K. I think what's really interesting with the U.K. right now is that they've set up four e-health centres. Those e-health centres are actually taking clinical trials and linking them to population-based data so they can look at these longer-term outcomes. The comment about the statins was fascinating. They showed that the benefit of the statins, if you start earlier, increases with time, whereas if you start at 70 or 75, forget it; you're throwing good money after bad.

These are the kinds of things that are just fantastic. I can see more and more intelligent population-based models for actually getting the best value for your money coming from that environment.

Mr. Don Davies: I think I only have about a minute left, so I'll play Alex Trebek here.

As you were all talking, I was taking some notes about drivers of the system that if we get right, if we design a Canadian system, could be cost-savers. I have: cost-related non-adherence; a national formulary, with some bulk buying; perhaps exclusive market access for certain drugs; single administration or centralized administration instead of maybe thousands of private plans; better prescription practices; and better evidence-based evaluation and efficacy assessment.

Does anything there jar anybody? Are those basically the factors? Am I missing something big, or is anything wrong there?

Dr. Durhane Wong-Rieger: I think everything you've said makes great sense.

This is not something that's sorely missing, but it's something I'd like to really emphasize and that Professor Herder and Chris also talked about, and that is, when we bring in these drugs, we actually need to have a robust coverage with evidence to document the program and—

● (1715)

Mr. Don Davies: Yes.

Dr. Durhane Wong-Rieger: That means that a lot of these drugs come in with conditional approvals, and that's really important. I think the conditions, as you say, under Vanessa's Law, allow us to be able to hold companies accountable. But the other thing is to engage the patient community, because we get a lot of push-back that says, gee, what if we've started drugs, and we have stop criteria, and the patients don't want to come off them—

Mr. Don Davies: I'm going to interrupt you. Thank you for that post-market assessment, but I have a question. If we do those things, do you believe we can provide universal coverage for Canadians and save money over what we're paying today?

Dr. Durhane Wong-Rieger: Of course.

Dr. Christopher McCabe: What time period are you thinking in terms of the investment? Over a decade, I think yes, you will, but—

Mr. Don Davies: After the original investment, over time we will save money?

Dr. Christopher McCabe: Yes. Ten years from now, I think you would see real differences in the utilization of emergency care services by the socio-economically....

Mr. Don Davies: Professor Herder...?

Dr. Tamblyn, I think I saw you nodding.

Dr. Robyn Tamblyn: Yes. I wouldn't wait 10 years, but I'd get really parsimonious about what's going to go in the value basket that's going to be free. Go for the gold on that. Have an international strategy for the orphan diseases, and I think you can see it.

We have people on home care. They can't sustain the burden of the 15 medications they're on. They have to go into hospital in order to afford their therapy. We have 5% of the people in our country actually accounting for two-thirds of the health expenditures. Really, it's a payoff for people who are on a lot of meds, can't afford them, and can't afford to live in the community, yes.

Prof. Matthew Herder: Yes, absolutely. The best evidence we have shows you would save money.

Mr. Don Davies: Thanks to all of you.

The Chair: That concludes our normal round, but I'm going to ask the committee if they would like to have one round from each party for three minutes. We could fit that in.

Do we have—

Mr. John Oliver: I have that one bit of business for the committee.

The Chair: Oh, you do? Okay.

Mr. John Oliver: It doesn't necessarily have to interfere with that.

The Chair: Is it the committee's desire that we have one three-minute round for each party?

Some hon. members: Yes.

The Chair: Okay.

Ms. Sidhu, do you have a question? You haven't had a crack at this, so you have time for a question.

Ms. Sonia Sidhu (Brampton South, Lib.): I'm going to pass. My question has already been covered.

The Chair: Okay.

No questions?

Peter, you always have a question.

Mr. Peter Fragiskatos: Yes, I do have a question.

Dr. Tamblyn, this is what I didn't get to ask you during my round of questioning. Obviously, you're a strong proponent of national pharmacare. Is there a particular state doing it now that you would point to as a model that Canada can learn from in terms of cost savings and in terms of how they do it? That's a huge question, and I understand that.

I understand that the committee has been looking at this. I'm sitting in for a colleague today, and this is a fascinating issue. That's why I wanted to raise the point.

Dr. Robyn Tamblyn: In terms of principles, of getting the best bang for your buck for your investment, I wouldn't turn to a nation. I'd look at something like Kaiser. Kaiser manages in such a way as to get the best bang for their buck. If you can emulate their principles in a national pharmacare program, I think you would be in great shape.

Mr. Peter Fragiskatos: Thank you very much.

Mr. Don Davies: Mr. Chair, on a point of order, I don't know what Kaiser is.

Dr. Robyn Tamblyn: Kaiser Permanente is a mixture of a health insurance and health care delivery in the U.S.

Mr. Don Davies: Thank you.

The Chair: Dr. Carrie.

Mr. Colin Carrie: Dr. Tamblyn, I think that's a great idea on the adverse drug events. I mentioned Vanessa's Law and the safety component. What kind of doctor uptake and buy-in are you getting with this adverse drug reaction reporting? Do you have an update on how things are going with Vanessa's Law?

Professor Herder, do you?

Dr. Robyn Tamblyn: We have mandatory disease reporting for infectious diseases like malaria and such, and we still have a reporting problem, with 5% reported and 95% not reported, so I think mandatory reporting is not going to be the solution if we really want to have.... I want to keep track of all those adverse events, so here's the idea behind what I'm proposing.

We're already going to put $50 million into a national prescribing system, so why don't we make it a feature component to include drug stop orders and mandatory documentation on the reason for the stop order and the problem that ensued, which would be important for patient safety and important for our proactive monitoring system? That would be good for patient safety, and we will actually get information on a much faster, broader scale than we will only with adverse drug reaction reporting.

That's what I'm proposing. It does work, because it's important therapeutically and important for patient safety. We have an opportunity here.

● (1720)

Mr. Colin Carrie: Would patients have any input in there, or would it be just doctor centred?

Dr. Robyn Tamblyn: Actually, because we have a prototype of a system like this in Canada, for the most part, half the time a druggist stopped us because the person had already stopped it, and they come to their practitioner and say that it isn't working, that they had a big rash, or had uncontrolled nausea, and so they took themselves off it. So the physician dutifully records it so that they know that they shouldn't be prescribing that drug yet.

Mr. Colin Carrie: Yes, but the doctor uptakes to actually report that—

Dr. Robyn Tamblyn: Well, in fact, we could actually set it up so that patients could do the same thing, even better.

Mr. Colin Carrie: Dr. Herder.

Prof. Matthew Herder: Just to quickly update you on Vanessa's Law, it's fantastic on paper, but implementation has been incredibly slow thus far.

The power to compel drug companies to keep studying post market is in force now, and hopefully it is being used. The power to make information more transparent is there. Some of them are in force now; others require regulations. A law was passed in November 2014, but there are still no regulations from Health Canada.

A key part of evidence-based decision-making for our clinicians in practice and for the national formulary, if we ever get one, is more access to the information behind drugs. Vanessa's Law is being implemented too slowly in that regard.

The Chair: Mr. Davies.

Mr. Don Davies: I have one question. Some witnesses before the committee have proposed that we emulate the Quebec model, and others have critiqued the Quebec model. The two main criticisms are, number one, that the per capita costs for prescription meds are higher in Quebec than the rest of Canada. Second, there are equity issues between those who are covered by the so-called public system and those who have private coverage through their employers. I'm wondering if anybody has any comment on that.

Dr. Robyn Tamblyn: Quebec did face whether they should have a completely public plan or a public-private plan. It opted for the latter because of, obviously, a private insurance company lobby. That's one issue.

I think where they went wrong in Quebec—because I think their intent was absolutely fabulous, which is providing universal pharmacare—was that they took all the sick people, and everybody who wasn't sick got to go to the private insurance company. Then the private insurance companies got essentially everybody who was well and wasn't going to be using drugs to pay the premium. If private insurance had been regulated to take the mix and not just the well and active, thank you very much, but had the same mix as the public insurer, I think it would have been better.

The second is that Quebec does provide a very rich formulary. I'm not sure that they refuse very much, and then they put a copay on everything from insulin to your leisure drug of choice.

Mr. Don Davies: I'm from British Columbia. We have a different view, and it's not covered by any formulary currently, I don't think.

Voices: Oh, oh!

Dr. Robyn Tamblyn: I think you can be smarter about the formulary and be a whole lot smarter about those copays.

Mr. Don Davies: Thank you.

Does anybody else have a comment on Quebec?

Dr. Durhane Wong-Rieger: We get a lot of people who suggest that we should adopt something like Quebec did because they've got a very good *patient d'exception* program, so that if the drug comes in before it has gone through all the full review, you can have an individual patient that's adjudicated.

I mean that's kind of okay and it's kind of not okay, because sometimes we get stuck there as well. Patients are getting on one at a time. We don't develop the registry. We don't get good formularies. In some respects I think what we want to do is to bring the other programs up to being able to provide.

If you think about the adapted pathways and about what's happening in terms of collaborative HDA, at the time the drug is actually approved, we should be able to know, especially for rare disease drugs, which patient should be on it or not. We're not going to get better evidence after HDA looks at it. We will get better evidence after it has been on the market for a while, and then we'll have some other reviews.

We believe, and what we've seen happening in Europe under things like ADAPT SMART, is that there is a review ongoing while it's under review by Health Canada. At the time of approval, we can already begin to make some decisions about who should get access, who should not, and what conditions to continue to monitor.

The Quebec program works because patients can jump into it. We do not necessarily believe that that has the longest term benefit in terms of having a reasonable access for everybody.

● (1725)

Mr. Don Davies: Thank you.

The Chair: That concludes our official business. We're going to switch to committee business for a minute.

I want to say thanks very much to everybody, all the presenters, because you've provided us with a great deal of interesting information and in a way that we can actually understand. We do appreciate your time and appreciate your coming back.

Did you understand?

Mr. Colin Carrie: I just said, “That's hard.”

The Chair: Oh, it is.

Anyway, we really do appreciate your time and your contributions. Thank you very much.

Mr. John Oliver: On March 21, the committee passed a motion that the chair present report 13 and report 9 from the 2nd session of the 41st Parliament to the House and request that the government table a comprehensive response.

I was wondering whether the chair could give us an update on how that reporting is going?

The Chair: The chair hasn't done it yet, but the chair will do it next week.

Mr. John Oliver: That's perfect.

Thank you very much.

The Chair: The meeting is adjourned.