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Chair

Mrs. Joy Smith

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● (1530)

[*English*]

The Chair (Mrs. Joy Smith (Kildonan—St. Paul, CPC)): Let us begin.

I want to welcome everybody here.

We have Mr. David Lee, director of the office of legislative and regulatory modernization at the policy, planning and international affairs directorate, health products and food branch. Wow! Is that ever a long title, Mr. Lee—well deserved.

Also we have by teleconference, from the Canadian Organization for Rare Disorders, Dr. Wong-Rieger, president and chief executive officer. She is joining us from Geneva.

We're very honoured to have you here.

I'm going to begin with Dr. Wong-Rieger.

Doctor, you have 10 minutes for your presentation. Then Mr. Lee will take over from you. Do you understand that, Dr. Wong-Rieger?

Dr. Durhane Wong-Rieger (President and Chief Executive Officer, Canadian Organization for Rare Disorders): Yes. Thank you very much. And certainly many thanks for the opportunity to do this by remote teleconference.

The Chair: You may begin now. We look forward to hearing from you.

Dr. Durhane Wong-Rieger: My understanding is that we are talking about technological innovation and the treatment of rare diseases. I'd like to address a number of types of technologies and innovations that we feel are really revolutionizing the treatments of rare diseases and, certainly, the presence or the availability of these technologies, but also the application of them, especially in Canada. I think there are some things right now that we are doing very well in Canada, some things where the federal role I think has been especially strong, and then there are some ways in which it could be much better.

If we start by looking at the genetic research in terms of identifying the genetic causes of rare diseases, we see that about 80% of rare diseases do in fact have a genetic cause. Canada currently sits as one of the leading countries in doing this type of research. Certainly, I think it stems from the days when we were still identifying the genes that were in the human genome. Canada built up quite a good repertoire in terms of research technology, but also in terms of laboratories.

The challenge, I think, is that we have not been quite as good in terms of using this genetic knowledge to do screening so that we can identify individuals, from newborns all the way through to older adults, who may have these genetic defects, and certainly in terms of using that information in setting in place programs to prevent harm or to deal proactively with the results of those diseases.

Also, I think we can look at the research and development in understanding the causative pathways in these rare diseases. Really, what is the underlying mechanism by which these diseases happen? Again, Canada sits as quite a leader, I think, certainly very recently with some of the research funding that's come through the CIHR and Genome Canada, and then more recently with the funding that's come through with regard to personalized medicine.

I think the challenge again is our ability to use those to, in this case, develop treatments that are based on an understanding of that causative pathway. Canada, I think, has not really invested in the same way and has not made that as much of a priority. I think the challenge, then, is that we may understand how these diseases work, but we don't necessarily move into having the treatments for them.

I think Canada is beginning to.... I think David Lee is going to talk a whole lot about this. It's much to David's credit, as well as to Health Canada's credit, that we are beginning to develop a very good regulatory framework for the evaluation and the ongoing monitoring of drugs, devices, and other genetic therapies, including things like [*Inaudible—Editor*] therapies in Canada. The challenge we have again is with the application, that is, in making sure that these innovative technologies are really made available to patients in ways that are safe and monitored and, certainly, made available in a consistent way across the country.

Again, I think that where we have begun to do some things around pharmacovigilance post-market surveillance, we are probably not doing anywhere near the kind of job that's necessary, especially when we recognize how challenging many of these therapies are. I think that in many respects there's still a lot of hesitancy in Canada in terms of using the innovation that we in some respects pioneer here and that we have available. Certainly, I think there are a lot of challenges, because we are such a decentralized access environment, and we end up with the provinces and other jurisdictions that are now responsible for making these therapies available, not just from a purchasing and cost point of view, but in the overall management and monitoring of these therapies.

In some respects, this is where I think from a patient perspective we really would like to see some federal leadership. We feel that the federal government and Health Canada can play a huge role, not necessarily in terms of taking over the provision of therapies or the direct monitoring of therapies, but in providing that leadership role, as it has been in some selective cases, and also in providing for the provinces those kinds of guidance, like national guidelines, etc. Again, I think we've heard the provinces ask for this over and over again, so even within a federated environment, we think the federal government itself could play much more of a leadership role and much more of a guidance role.

I think the other thing we are concerned about in terms of some of the innovation coming in is what to do about therapies that are already in place.

● (1535)

I think Canada is probably very far behind other jurisdictions in being able to approve drugs that have previously been used off label but over time are actually indicated, and have demonstrated to be effective, for a rare disease. In Canada we have a very hard time bringing it into the regulatory framework to provide certainly an on-label indication, to provide the safety and guidance for it. So these drugs continue to be used off label in Canada, whereas in other countries they may actually now be part of the regulated and on-label indication.

One of the challenges for this is not only do we not have the good safety and effectiveness information; in many cases the costs are not reimbursed, because they're not considered to be on-label therapies.

Again, Health Canada could in fact take that role. I don't think they're unaware of it; I think this is something that will take a little bit of redoing. It's urgently important when we recognize that about 50% of the drugs that are used by Canadians with rare diseases are actually used off label. That means there's no regulatory oversight, no actual pathway in which they are managed, and a lot of them are haphazardly reimbursed.

Another area where we're playing a leading role right now is in what we call repurposing drugs—taking drugs that are already on the market or already developed and in many cases being able to reuse them for rare diseases, based on some similar pathways and mechanisms. In terms of risks, certainly, because the drugs in some cases have already been approved and are already in use, there should be in fact a much quicker and a less costly way of making those drugs now available for rare diseases.

We would like to see Canada take a deliberate role in that, in part because we are becoming one of the leaders in research on repurposing drugs—again, through the leadership from CIHR, from Genome Canada, and some of our universities and clinics. It behooves us to also then find a way to bring those onto the market quickly and make them available to patients.

That also speaks to extension for the drugs that are already on the market, oftentimes for rare diseases. As we begin to identify other diseases that have similar pathways, is there a way that we can more quickly make sure that these drugs are also going to be available for other patient populations without having to go back to the same level of clinical trials that the drugs in the original indication required?

We're really quite excited by the fact that Canada seems to be getting into the game here. I think we are looking at our regulatory framework and in some respects at possibly being one of the world leaders once it is in fact put into place, and we hope very soon. Quite frankly, I think Health Canada, certainly with the leadership right now, has been taking the best of what's available internationally and incorporating that into what we're doing. They're also looking into the future: where do we believe rare disease drugs, orphan drugs, will be, and what will they be like?

We're very much a leader, as we've said, in the repurposing of drugs, but also in terms of personalized medicines, which are really very close cousins to these orphan drugs, these drugs for rare diseases.

I think that's very exciting for us in terms of where Canada was just a few short years ago in this whole environment of technology and innovation with regard to rare diseases. We're now moving, I think quite rapidly, to taking what is I think recognized by other countries as well as a leadership position.

I think we're positioned very nicely between Europe and the U.S., and I think both jurisdictions look very much to Canada in terms of not only being part of the game, as we say, but also providing some really good bridging information and maybe in some areas also taking a leadership role.

I'll finish by saying that what we still don't have in Canada, to take full advantage of our innovative technology, is a rare disease strategy. In Europe, the European Union has mandated that every country in the European Union should have a rare disease strategy by the year 2014. Every country is moving towards that.

Canada needs to do that, because without that kind of rare disease strategy, we're not going to take full advantage of some of these innovations; certainly we're not going to do it in a consistent way; we're not going to do it in a way that gets, as we're learning, the most effect out of it; and we're certainly not going to be doing it in a cost-effective way.

Some of that would include things like having centres of excellence, where you can designate centres where you have leading researchers and clinicians who can provide not only the information on diagnosis and research and managing clinical trials, but also can serve as a resource to other clinicians and to other sites in terms of these rare diseases.

● (1540)

I think we need some other things that I think only the federal government can do for us well—a national newborn screening program, for instance, and a national disease registry. We cannot have provincial registries when we're talking about diseases in such small numbers. And to make some of these therapies, these technologies available without good registries—

The Chair: Thank you, Dr. Wong-Rieger.

I gave you a little extra time. We'll be asking questions of you, as well. Thank you so much for your presentation.

Dr. Durhane Wong-Rieger: Thank you.

The Chair: Now we'll go to Mr. Lee.

Mr. David Lee (Director, Office of Legislative and Regulatory Modernization, Policy, Planning and International Affairs Directorate, Health Products and Food Branch, Department of Health): Thank you, Madam Chair.

On October 3, 2012, the Minister of Health announced the development of a modern framework for orphan drugs. These are drugs used to treat rare diseases. This opens the way for increased Canadian research and development of these drugs and for improving Canadian patient access to treatment.

It's a pleasure to appear before the House of Commons health committee to explain more about how this proposed regulatory framework would benefit Canadians affected by rare diseases.

There are dozens of well-known diseases in Canada. They're well known because they affect the lives of many people. There are charities, associations, and support groups for people suffering from those diseases.

But there are thousands of people suffering from other diseases that most of us have never heard of. That's because they're so rare, they can affect fewer than 12 people in the country. At any given time, even internationally you can have very small numbers of patients.

While some of the rare diseases may affect only a handful of Canadians, in all, hundreds of thousands of Canadians are dealing with these conditions, and they need effective treatments. In Canada it's estimated by some that one out of 12 Canadians is affected by a rare disease.

The diseases are often linked to genetics, as Dr. Wong-Rieger suggested. They can have a very early onset. They can be diagnosed during childhood, often very young. And they're very difficult to study, to treat, and to understand how to regulate because of the small size of the population. It's scientifically difficult to tell how a therapy would work in that population and to pick up safety information.

Rare diseases can be serious chronic conditions—they last throughout the lifespan of the patient. They can be seriously debilitating or life-threatening. They often are life-threatening.

The drugs that demonstrate promise for treating these diseases are often referred to as "orphan drugs". It's a term that has developed globally, and the United States started that.

Today when a patient with a rare disease needs access to an orphan drug, because we don't have rare-disease regulations currently, it's not available in Canada. The patient's doctor, often a specialist in that disease, will obtain it through our special access program at Health Canada. But every time the specialist uses this option, he or she has to take time to write out a form to request in writing the allocation of the drug to the patient, and then the department can contact the manufacturer to release the drug to that patient. While this works—and it's what's operating right now—it is time-consuming, and each decision is made on a case-by-case basis. It's an unnecessary burden on the health care system.

Health Canada has also approved some orphan drugs as new drugs under division 8. That's our normal review provision for commonly marketed drugs. While this path has worked in the past as well, it's limited because it was not designed to address the unique challenges of rare diseases. It really doesn't pay attention to the data requirements, for example, that we would tailor in a new framework.

What is needed is a new regulatory framework designed to gather information used to treat small, vulnerable patient populations, specifically tailored to facilitate the development and approval of drugs meant to treat rare diseases, an orphan drug framework.

An orphan drug framework will level the playing field for Canadian rare-disease patients so they, too, can share in the benefits rare-disease patients in the U.S., and in many European countries with such frameworks in place, already have. In those countries and those jurisdictions, they have a lot of experience with rare diseases by now, and they've been very helpful in teaching us about that.

The orphan drug framework will allow Health Canada to approach the approval of these drugs in a flexible manner, recognizing that greater uncertainties may exist for orphan drugs, given the complexities of the diseases and again the small size of the patient population.

First, we're aligning with trusted international counterparts, the European Medicines Agency and the U.S. Food and Drug Administration. And I will say that they're very generous in their advice, opening up what has worked for them and not jurisdictionally. Both have had frameworks in place for orphan drugs for over a decade, and in the case of the States, more than two decades. International alignment of Canada's regulations will allow our scientists, Canadian scientists, researchers, and regulators, the ability to pool increasingly limited resources to help us to better understand these complex diseases and their treatments.

● (1545)

Second, drawing on the idea of life-cycle management, the framework will also allow us, the regulator, to more closely follow the safety and effectiveness of these drugs once they're brought into the market. This will be done by ongoing post-market data collection relating to the drug's safety and efficacy or effectiveness. This innovative approach complements the current pre-market focus with a more balanced, dynamic, and fluid set of regulatory interventions, and it will better serve the patients' needs while maintaining a strong safety oversight.

We want to make sure the design of our approach is very patient-centred. The patient's voice needs to enter into that regulatory process, so we want to enable patients to have a voice throughout the decision-making.

Because the proposed regulations will align with international frameworks, which is very important, it will be more commercially feasible for pharmaceutical companies to develop and then bring their drugs to market in Canada. This is because the international alignment gives drug manufacturers a more predictable, operationally less burdensome path to follow. What they follow in other jurisdictions will be typically sufficient here as well.

A more predictable regulatory path, with clear research requirements and flexibilities to enable international collaboration, also creates space in which Canadian research and innovation can thrive, and we're trying to pay attention to this in designing the framework. The framework will also provide for greater transparency to improve the gathering and sharing of information among patients, health care professionals, researchers, payers, and international regulatory partners.

Improved transparency is expected to result in more informed, evidence-based decision-making. It also brings with it increased public confidence in evidence-based research and the safety of research participants. This is because the broad sharing of research data accelerates the research, fosters data integrity, and increases accountability.

Most of all, the new framework will benefit Canadian patients with rare diseases by improving access to new and existing drug therapies that might have been harder to get or not available at all without these new rules.

As part of our work to better understand the impact that the orphan drug framework will have on Canadians, Health Canada has also met with many Canadian researchers, clinicians, rare disease representatives, and patients. I've done this often in conjunction with CIHR and other valuable domestic colleagues.

In response to what we heard, Health Canada, with support from the Canadian Institutes of Health Research, has launched Orphanet. There's been a lot of effort around that. It's a very important online resource, a global resource that offers a directory of specialized information for people with rare diseases and health care service providers. It includes information about specialized clinics, medical laboratories, clinical trials, and registries. Together the new framework for orphan drugs and Orphanet will create a better environment to increase access to information for patients with rare diseases and the new treatments coming onto the market.

In closing, the proposed framework is in the final design stages. We will soon be broadly targeting public consultation. Comments and feedback would then be gathered during consultation and incorporated into a final version of the proposal.

Thank you for inviting me to appear today.

● (1550)

The Chair: Thank you for being here.

Now we'll go into our seven-minute Q and A round.

We will begin with Ms. Davies.

Ms. Libby Davies (Vancouver East, NDP): Thank you very much to both our witnesses for being here today, particularly to Dr. Wong-Rieger in Geneva. I guess it's quite late there, right?

Dr. Durhane Wong-Rieger: Yes, it is.

Ms. Libby Davies: Thank you for being up late to be with us here, where it's still a sunny afternoon in Ottawa.

I realize that today we're dealing with a very specific issue, which is rare diseases, but nevertheless it does relate to bigger issues around innovation as well. I'm certainly no expert. Probably none of us is an expert on rare diseases, so the information that was provided today was helpful in understanding that a lot of people are affected by rare diseases in aggregate in our country.

I want to focus a little on looking at the new framework that's coming. I notice you didn't speak about the issue of drug safety. This is something that's currently very much before us. We've had a number of examples recently with drugs. We have one today whereby there's now a warning from Health Canada after we heard from the Americans about a month ago. This is a huge issue. I want to know if I'm correct that with some of these rare diseases, so many of the drugs are off label, as Dr. Wong-Rieger has said. I don't know all the ins and outs of that, but it sounds as if it's not a great regime and that it's much better to have things on label and within a regulatory sense.

For many of these drugs there could be increased problems in terms of adverse side effects, drug safety, and so on. Am I correct that there would be a higher incidence than in other “mainstream” diseases? I don't know what to call them.

The Chair: Mr. Lee.

Mr. David Lee: Thank you, Madam Chair.

In terms of safety, that's a very, very important aspect of the new rare disease framework. Recognizing that the food and drug regulations themselves are somewhat older, we do have powers on market tracing and finding out what's going in the market, largely through the reporting of adverse drug reactions. That also happens through delivery and special access. The manufacturer and the physician would have to report back in if there's an incident through that transaction. So if the orphan drug is going out into the market on a one-by-one request, we do see it. The problem is that you don't get a population view of what's going on. The new framework would be much more deliberate. Safety would be a matter of trying to look out and understand what it is you need to follow if you've got any particular concern. If you start to detect something, you can put in further tests and studies and make sure you really follow what's going on with the drug, including utilization studies. So you can follow if it's being used outside of the labelled indication, which means that physicians are prescribing it, notwithstanding that it hasn't been completely demonstrated with us.

Basically, it would be a much more advanced way to follow out safety issues. That's not to say that pre-market there's any less of a look. We really want to make sure that as the drug—

● (1555)

Ms. Libby Davies: Is there actually going to be post-market surveillance for safety based on this framework? Is that included in the framework?

Mr. David Lee: That would very much be included.

Ms. Libby Davies: Okay, thank you. I didn't actually see it spelled out in the brief in those terms. It was sort of alluded to, maybe, if you stretch it.

But it is clearly going to be part of that?

Mr. David Lee: It is very clearly. When we talk about life-cycle management, that really does get to the crux of following the drug, both from a safety point of view and also a benefit point of view.

We start monitoring right from the first introduction in human beings, so the first phases of clinical trial we're starting to already plan out how we will construct vigilance. That's the new scheme. The old scheme is a little bit more passive: you wait for something to happen and it gets reported.

Ms. Libby Davies: Why has it taken so long? We hear that in the U.S. they've had such a program for 10 years. I just wonder why is Canada so far behind on this.

Mr. David Lee: We're learning from the U.S. proposals. Functionally, within Health Canada, we also get the benefit of a lot of the tools that have been constructed by the U.S., for example, the REMS—so the plans going out into market to follow vigilance. We do get those filed. We also get them filed from Europe. There's been a convention that we've been part of through the International Conference on Harmonization on pharmacovigilance for Health Canada. We've implemented much of that. The thing is to follow in with the regulatory proposals. That's what this would do in the orphan drug setting. It would put in an ability to require a plan to go out into the market and follow the safety issues as a part of your licence. So the company would be obligated by law to make sure that they are tracing out and actively looking for signals where we need to.

Ms. Libby Davies: I have one other quick question.

We're studying innovation. I would imagine that one innovation in this issue is affordability.

Can you give us an idea, and maybe Dr. Wong-Rieger could give us an idea as well, of the costs of some of these drugs? Are they incredibly expensive? I just think about the commitments that were made in the health accords to have drug coverage, particularly in situations where people are paying astronomical prices. Can either of you give us a sense of what some of the costs are?

The Chair: You have a minute left.

Dr. Durhane Wong-Rieger: Overall, I think what we do recognize is that many of these drugs, because they are being developed for very small patient populations, can come in at a very high individual cost—though it's not true for all of them. I will give you a bit of a reality check. If we think about all the drugs that are now currently being funded for rare diseases, including some that you've heard about as being very expensive, the cost still amounts to only 0.7% of the public drug budget because the numbers are very small. A very good projection that was recently made by researchers in Europe, where there's much better drug coverage for these rare disease drugs than we have here in Canada, is that because of the rates of development, the numbers involved, and the fact that when you develop a drug for one of these rare diseases many of the patients come on right away—so it's not likely you're going to get a continuing increase in these patients—at most it would never be more than between 3% and 6% of the total. That was their estimate.

The Chair: Thank you, Dr. Wong-Rieger.

Now we'll go to Dr. Carrie.

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

Thank you to the witnesses for very interesting presentations.

I just want to follow up along with my colleague. We are doing technological innovation, and I think one of the innovations is actually data collection, data availability, and international cooperation. When I've talked to stakeholders, it seems, as Mr. Lee pointed out, that sometimes there are only maybe 12 people in Canada with a certain disease, so how do you study it? How do you bring this about?

Mr. Lee, you mentioned this online technological innovation, Orphanet Canada. I wonder if you could elaborate a bit more about exactly what that is, and if it could be modelled for other things? Is this online resource something that patients can load data into? Is it something that researchers can take something out of? Or is it something else? Could you elaborate a bit more for us?

Mr. David Lee: Thank you, Madam Chair.

Orphanet aggregates a lot of information about rare diseases. So it will list disease states, patient organizations associated with them, and trials going on. Medical professionals can use it; patients can use it. It's typical that, if you are starting up a study, for example, on a particular rare disease or you've identified something new genetically, then you would enter that information into that world database.

It started off in France, and more and more countries have joined into it, so it's becoming a very global effort. Now that Canada has stepped in, we too will be contributing great primary research that's being conducted here.

On the innovation side, I would also point out that one of the exciting parts of the new framework being proposed is the regulation of orphan drugs. After you have identified that an orphan drug is maybe effective for the treatment of a rare disease, one of the next steps is to go to the regulators such as the USFDA and the European authorities and talk to them about how to design your trial. How you research and investigate the drug is an important discussion because it's a very hard thing to design when you have such small numbers. Our statistical models are often different, and how we have to approach it is different. It's one of the common areas where more and more often those two agencies are trying to give aligned advice because, if you can pool together that international look at this small population of data, that's just better.

They've been inviting Canada to join in those discussions, which I think from a participation point of view is quite an opportunity. So we would be involved in these discussions about innovative design and trying to get Canadian study arms up and running here in conjunction with our regulatory colleagues. So I think it's an important moment in the proposed framework to recognize.

● (1600)

Mr. Colin Carrie: While we're talking about the framework, I was wondering if you could explain a little bit some of the challenges from a federal standpoint when you're doing these frameworks. We've heard a lot of different witnesses say that in Canada the provinces are responsible for delivering health care, and the hospitals track different data, and they get it agreed to that they'll share information with the federal government, and the privacy issues, and all that stuff. Could you explain to us some of the challenges with these regulatory frameworks and how you're designing this to get through that little minefield—or big minefield maybe?

Mr. David Lee: Yes, I've certainly learned that making regulatory frameworks is not for the faint of heart.

For a framework like this, at the federal level you really have to go out and first listen and understand the needs across country, because it is quite true that there is a lot going on in the hospitals. There's a lot of research going on. Often patients are appearing and they're very hard to diagnose. You have to do some international collaboration to even understand if the disease is there, and then all of a sudden you're looking for a treatment.

That will start to involve us at the federal level, but there are many levels in play. There are funding levels. Getting a small research project off the ground is very important. At early stages you're not thinking about regulations when you're in your lab, trying to innovate and identify whether a therapy will work. But it's very important to approach the regulatory aspects early so that when you're doing your studies, you don't misfire. You can start to innovate, but if you don't start to build a case to get on the market, your research is not going to translate out.

I've been doing more work with Genome Canada and CIHR to go out and talk to researchers about what they're going to be expected to do as they work up their innovations. So that's one level of federal participation where we have to look at and talk with a lot of colleagues. That's both primary care physicians, research physicians, and academic physicians.

But really, there is an international discussion as well. One of the really interesting things about rare disease is that it does attract a lot of international cooperation. So part of what we need to do is look at the needs of our patients, our researchers, our provinces, and also see what we can draw from the international context and bring together. That's a good federal role because, as I mentioned, with things like trial advice, if we're setting up a global trial, that affects all those levels, but we're giving the advice. So we're trying to find ways to do that and build a framework so that people can have a voice at those early discussions.

It's not easy to design because we don't have a lot of good precedence in Canada. But we do get help from our international colleagues who have designed frameworks such as these, and they have been giving us very important advice on it. Putting it together is quite involved.

● (1605)

Mr. Colin Carrie: Thank you for that.

Do I have a little more time?

The Chair: You're just about out of time. You have about 45 seconds.

Mr. Colin Carrie: Off-label use and the challenges here in Canada were mentioned, because you can have a physician giving a patient something that maybe it wasn't originally intended for but they're finding that it does have an effect.

What would be the regulatory challenges to allowing more of this off-label use of product to be recognized on the formularies for certain purposes, and as was brought up, perhaps for reimbursement for patients in that situation?

Mr. David Lee: Off-label is a very important concept in our realm. Label indications mean that you've come to Health Canada and you have demonstrated, as a company, that the product works and it's safe for that indication or that claim. So we put that on the label.

It then goes out into the prescribing environment, and we don't, as Health Canada, tell physicians how to prescribe. They need to—

The Chair: Thank you, Dr. Lee.

Thank you. You're way over your time.

We'll now go to Dr. Fry. Perhaps she'd like to continue this line of questioning.

Hon. Hedy Fry (Vancouver Centre, Lib.): Thank you very much, Madam Chair.

I am going to move into a different line of questioning. I want to talk about clinical trials. Given that the cohort group in clinical trials is so small because it's about rare diseases, there is a problem that I have heard about. People have come to me with this problem many times.

As you're looking at clinical trials, access to the clinical trial group is usually a very difficult thing. If you have two people in New Brunswick, for instance, they may need to come to Ottawa because there's a larger group in Ottawa, etc., and the problem they face is having somebody pay for them so that they are able to come to Ottawa and spend time here. There are costs for staying in a hotel, etc.

These costs of the clinical trials are often a burden for a lot of people, and I wondered if you would talk about how much greater that would be in regard to a rare disease, because we're really talking about small amounts going into one big place. That's the first thing.

Ms. Davies talked about drug safety. It is becoming a major problem for us here in Canada. What I like about your concept is that if, because of new communications technologies, we're suddenly going to work with places such as the United States and Europe now, and if it turns out that the FDA is doing a far better job of drug safety than we are, I would be prepared for the FDA to tell me that “this is a good drug to use in this kind of environment”. I think that's a great piece. I think it's good because we don't have to reinvent wheels and do that kind of stuff.

However, there is the problem of diversity, given that many rare diseases have a genetic component. Given that Canada has such a diverse population—very much unlike Europe in terms of ethnicity, race, and those kinds of diversities, which as we know do have certain DNA components and genetic components to them—and given that the United States also has, but has a difficult time breaking down that information because of their multiple insurance agents and privacy issues, how do you see us getting around that?

The final piece of the question may be more directed to the European Union. Dr. Carrie asked about how difficult it would be here in a federation where there are other jurisdictions, but I see the European Union mandating things for probably 50 countries that are all autonomous nations with their own things going on. They manage to do that relatively well, so maybe we could also learn about how to look at multiple jurisdictions and then come up with a good idea and some innovative ways of dealing with that. I'd like the presenter from Europe to answer that.

For the other questions on cohort size and trials and so on, could you please answer?

Mr. David Lee: Thank you.

Clinical trials are a key aspect of the new framework. This actually is a very international discussion as well.

A number of features will be uniquely Canadian. One is our geography. I think it's quite right to say that the disparity of where patients live and whether they have access to a trial site will be a uniquely Canadian factor. That's one of the reasons why we need good patient input and physician input at that front end when we're designing the trial site. We are reviewing the clinical trial regulations as well, to make sure that the definition of the trial site, for example, is not prohibiting being able to do some virtual work and reporting.

There's another important discussion that we're having, largely through our oncology researchers, our cancer researchers. They've talked about really being able to focus in the trial on the main things and really understanding the burden of paperwork and the innovation cycle, not dropping any of the safety, but really understanding what the basics of the trial need to be. We're having meetings to discuss our way through that. That, too, is also an international discussion. If you have multiple research ethics boards, and you have to file and file and file in each place that you're doing a study, that can be hard when you only have a few numbers.

In terms of that, we're really looking. Some of it's formative. Some of it is well defined internationally, but there are some new sciences gathering around trial size and being able to deal with small populations in regard to how you put that together pre-market and then how you follow it out into the market, in order to make sure that some of the assumptions you're making before you market are real in terms of both the benefit and the safety.

On the safety point, as regulators, it's more and more common that the moment we see a signal it's very important to make sure that globally we understand that. Within the framework, we're proposing to have fairly immediate updates, obligated in regulation, to tell us if there's anything is happening in any other jurisdictions. If you're running a trial or if you have a licence to sell in Canada, it would be a feature of the system to make sure that the constant flow of reporting on safety is really there. If in Canada we start to see safety signals when we get case reports, though, we need to feed that rapidly into the global understanding as well. We're all working together commonly.

Again, this is one area where there's a lot of cooperation among regulators. At least for the designation, it's the only place I'm aware of where the U.S. and the Europeans have a common application form. That's a really important thing from an innovation point of view.

● (1610)

The Chair: Perhaps we could also let Dr. Wong-Rieger make a comment because we're just about out of time.

Dr. Wong-Rieger.

Dr. Durhane Wong-Rieger: I would very much agree. From a clinical trial point of view, I think the importance of having this regulatory framework is that until now many of our patients didn't get early access to clinical trials because we didn't have a designation for an orphan drug so we weren't talking with Canadians at the same time. It meant that patients and clinicians didn't benefit from it.

The other thing I think, as Mr. Lee is saying, is that it's very important that lots of new flexible frameworks are being designed internationally, specifically for rare diseases. Quite frankly we are also part of developing and designing those clinical trials so that we can take advantage of small patient populations but also ensure that we've got the right balance of benefits and risks.

Right now, where we sit, I think we're going to be able to make sure that we have more patients involved and certainly better access to early treatments.

Hon. Hedy Fry: How much time do I have left?

The Chair: You don't have any more time.

Thank you, Dr. Fry.

Hon. Hedy Fry: Because of that, I didn't get the answer about how they deal with jurisdictional problems.

The Chair: Perhaps you'll have a chance later on, Doctor.

Mr. Wilks.

Mr. David Wilks (Kootenay—Columbia, CPC): That's about two days in a row you've done that, Chair, calling her doctor.

The Chair: It's just a suggestion, maybe you could become a doctor. You've done everything else.

Mr. David Wilks: I'll consider it if I find an extra 10 years in my life to do so.

Anyway, thank you very much, Madam Chair, and the witnesses today.

Dr. Wong-Rieger, according to Orphanet, those affected by rare diseases are psychologically, socially, economically, and culturally vulnerable, in part because they face challenges with access to quality health care, overall social and medical support, effective liaison between hospitals and general practices, as well as professional and social integration and independence.

Could you tell me what role the federal government could play in addressing some of the additional challenges that people living with rare diseases face?

Dr. Durhane Wong-Rieger: Certainly. The proposal Europe-wide, and certainly what we would love to see in Canada, is the notion of a national strategy. What I didn't mention is that part of a national strategy would include, for instance, the recognition, as you say, of some of the social and psychological challenges and specific supports for that. Also one of the big challenges is with people getting appropriately diagnosed. Sometimes it may take 10, 20, 30 years to get an accurate diagnosis of a rare disease, even though there may be experts in Canada who could. So part of it is educating GPs and pediatricians so that they're more aware of what these rare diseases are, recognizing the possibilities, and having specialists they can refer them to.

So again from a federal point of view, being able to support an overall framework that looks beyond just the drugs, as you're saying quite appropriately, is extremely important. We think that only the federal government can play that kind of a leadership role.

● (1615)

Mr. David Wilks: Thank you very much for that.

Dr. Lee, on March 5 of this year, Dr. Edwards of the Structural Genomics Consortium was before this committee, and he noted that the vast majority of biomedical research focuses on a very small number of well-understood proteins, often ones where there are tools readily available for their study.

He suggested that researchers and funding agencies should be less risk averse. In your view, is risk aversion, in the sense of uncertainty of financial benefits, a factor in the challenge to encourage research in the area of rare diseases, and is Health Canada able to help offset risk aversion by supporting research on rare diseases? Could you share some of the initiatives that might be of assistance to that end?

Mr. David Lee: Thank you for that question.

In terms of uncertainties, there are some uncertainties that Health Canada is able to address. I think others would be in the hands of entities like Genome Canada and CIHR, which are on the ground working with those who have proposed to conduct research.

We are keeping a very close eye on what's going on in the research community. One of the things we're doing is outreach for this new framework. With those involved in diagnosing and trying to find drugs to treat these diseases—and a lot of it is genetically based work—we're meeting with them early to try to understand what they're doing. We're doing that to try to take away uncertainties about what they need to look at for a potential regulatory filing in the future. You don't want the research to stall in the clinic. Eventually you want to achieve a market presence to make sure that you're translating your research into the most benefit for Canadian patients.

One of the uncertainties we can reduce and that can cause risk aversion is asking what the regulator needs. What is the pathway to taking this research into what the market will require? We're working on that part.

From a risk aversion point of view, I think that the costs of some failures, given the costs of development, are recognized by regulators. We do try to understand why we require data in the way that we do, because some of the expensive work is what you need to do to present the regulatory filing to the USFDA and Europe.

We do have some working groups and some initiatives worldwide. They are not only trained on rare diseases, but we're also trying to understand how to focus our data requirements better. We're on a group with the USFDA and Europe, trying to come to ground on that discussion. We have participants such as industry—a lot of very excellent scientists—trying to think that through.

It doesn't displace risk entirely, but it tries to bring more innovation to that research climate and how it might translate into the development of drugs.

Mr. David Wilks: How much time do I have left?

The Chair: You have about one minute.

Mr. David Wilks: Thank you.

I wonder if you could expand a little further on what Dr. Fry was speaking of or alluding to in her question. Could you go back to that for a second?

Mr. David Lee: Madam Chair, if I could clarify, is it the question on the cooperation, or the ethnicity issue?

Hon. Hedy Fry: Yes, the fact that unlike Europe, we have a proportionately larger ethnic and—

Is that the one you meant, Mr. Wilks?

Mr. David Wilks: Yes.

The Chair: We don't have much time, Mr. Wilks.

Ms. Fry does have another question, so she could bring that up herself and have more time, if she chooses to do that.

I'm going to have to cut you off in about 30 seconds because the time is running out, and I hate to do that.

Mr. Wilks, you are a very generous man. Are you finished your question?

Mr. David Wilks: I am.

The Chair: You did a great job.

I just didn't want to cut you off because you can't answer that in such a short period of time.

We'll now go into our five-minute rounds, and we will begin with Dr. Sellah.

Ms. Fry, you can be ready to go.

[*Translation*]

Mrs. Djaouida Sellah (Saint-Bruno—Saint-Hubert, NDP): Thank you, Madam Chair.

I want to thank our guests for joining us.

As it is stated on the Orphanet website, “There is no disease so rare that it does not deserve attention”.

Dr. Wong-Rieger, we know that 80% of those orphan diseases are linked to genetic factors. You also said that Canada is a leader in genome research, but it has unfortunately not been able to use that technology for a prevention or treatment program.

Why has Canada been lagging behind in that area even though we have world-renowned minds and scientists? As you said, we are almost 10 years behind the U. S. and Europe. Unfortunately, Canada now has to learn from that European and American Orphanet.

● (1620)

[*English*]

The Chair: Go ahead.

Mr. David Lee: Thank you, Madam Chair.

In terms of timing I will acknowledge that we are doing our best to catch up. I'm not sure it's—

The Chair: I was saying Dr. Wong. I should have said Dr. Wong-Rieger. I'm sorry about that.

Mr. David Lee: Pardon me, Madam Chair.

The Chair: I know she's on the telephone.

Dr. Wong-Rieger, I think it was directed to you, was it not?

Okay. Go ahead.

Dr. Durhane Wong-Rieger: Yes. Thank you.

Let me give you an example and it may help provide the answer. We have a very important drug that was discovered at the University of Montreal by a physician there, for treatment of a very rare bone disease. At the time the drug became ready for clinical trial, the company in fact moved to the U.S. to host those clinical trials, even though we had the first clinical site in Canada. At the time we did not have a designation for orphan drugs, and we didn't have a regulatory framework that would support and provide the kind of support that's necessary in order to foster a climate there. That's why the regulatory framework is so important.

That drug is now currently in late phase 2 clinical trials. One of the major sites is in Canada. But quite frankly, the benefits of that research and development are taking place in the States. That is one of the reasons why we have been pushing so hard to get this orphan drug ready for a [*Inaudible—Editor*] in Canada, to provide the supports, including the supports for how to design the trials; the critical inputs of those trials; as well as some of the research incentives, including in the U.S., and some of the supports in terms of rebates for the clinical trials, as well as future market exclusivity. It has been the case that we did not create an environment that was very supportive of it, and that was unfortunate. But if somebody will remember, back in 1996, Health Canada said that we didn't need it, that the drugs were being developed elsewhere and that Canada was able to live off other people's research and development. It has taken us, quite frankly, 13 years behind these [*Inaudible—Editor*]. But we are very happy. I think what we're going to get is very nice too, what we call coming to the front of the pack.

[*Translation*]

Mrs. Djaouida Sellah: Madam Chair, how much time do I have left?

[*English*]

The Chair: You have about another minute.

[*Translation*]

Mrs. Djaouida Sellah: Thank you.

Thank you for those clarifications, Dr. Wong-Rieger.

If I have understood correctly, you are asking the government to show some leadership and possibly create a regulatory framework, so that Canadians can at least benefit from the research carried out by our scientists.

[*English*]

Dr. Durhane Wong-Rieger: That is exactly right. As you rightly said, we have the intellectual capacity here, but this is just one example. Over and over again we see companies leave Canada for the U.S. and Switzerland. We have not really focused on the capacity that we thought in order to bring these drugs to market.

[*Translation*]

Mrs. Djaouida Sellah: Thank you very much.

[*English*]

The Chair: Thank you very much.

We will now go to Mr. Lizon.

Mr. Wladyslaw Lizon (Mississauga East—Cooksville, CPC): Thank you, Madam Chair.

Thank you, witnesses, for appearing before the committee.

Mr. Lee, I have a very simple question. Of course, I'm not very familiar with all of the aspects of rare diseases. In terms of numbers, what are we talking about in Canada? Can you give some examples of rare diseases and the number of people affected, whether *x* number per thousand or in gross numbers?

What are we talking about? What are the proportions?

● (1625)

Mr. David Lee: Madam Chair, I would defer on that question to my colleague, Dr. Wong-Rieger.

I will answer, though, that some of it is hard to measure. It will be inexact because diagnosing some of these diseases is very hard to do. We may not be picking up every patient in the country. But we think there are just under 7,000 diseases that have been identified internationally. How many Canadians have them? Again, there's not a specific count, but we do think, as I mentioned in my opening remarks, that there's reason to believe that one out of 10 Canadians is affected by a rare disease.

I don't know if Dr. Wong-Rieger would have more exact statistics on that.

Dr. Durhane Wong-Rieger: It isn't just that there are statistics. You're absolutely right that we would extrapolate to Canada the international figures, so you're looking at maybe 28 million Canadians who could be affected.

Very important as to why we need this regulatory framework and why we need to focus the research and development is that there are, as I think Dr. Fry was mentioning, ethnic pockets in Canada, and there are also geographically isolated pockets in Canada. So we are actually the host of some rare diseases that are most prevalent or most well identified in Canada. So unless we're doing the research on treatments for these diseases, these treatments aren't going to be developed elsewhere, and we offer a great opportunity to do the research here.

The bone disease I was talking about is prevalent among Amish communities. A small community outside of Winnipeg is actually the site for one of the clinical trials. So it's not just the sheer numbers but the recognition that many of these diseases will become prevalent because of the geographic isolation. We in Canada are actually home to some very unique rare diseases, or we have a large population, because of ethnic migration, of some of these rare diseases. So it's a great opportunity for us not just to deal with numbers, but also to deal specifically with some of those diseases that are either overrepresented or easily identified in Canada.

Mr. Wladyslaw Lizon: The reason I'm asking that question is that I had a friend who had a vary rare form of cancer—unfortunately he passed away—and from what I remember there were only three places in the entire world where he could receive treatment: one was in the United States; I think one was in Sweden; and one in Rome, Italy.

Mr. Lee, can you maybe give us some examples of new inventions in medicine that can be adopted to treat rare diseases in others, or do they have to be strictly developed for a particular medical condition?

Dr. Durhane Wong-Rieger: I think one of the things that's certainly happening in the rare disease community is that it's a very internationally linked community. So as you say, even though there may be only three sites in the world that can treat that particular disease, in fact many of these clinicians will know each other. Now, because we are also part of Orphanet and we're part of those international communities, we are also listing our clinical sites and listing our experts on there, and we have access to others. We have diseases, as David was saying, for which we may have only a dozen people in Canada with that disease, but they actually can be followed and supported by international sites—

The Chair: I'm sorry, something has happened with the connection, Dr. Wong-Rieger, and I don't know if we can correct it or not.

Dr. Durhane Wong-Rieger: I'm sorry, can you not hear me?

The Chair: You're back again. Okay, continue on.

Dr. Durhane Wong-Rieger: As I was saying, because of the international linkages in many cases if you have only, let's say, three sites internationally, we actually do have patients who are followed and have consults via international sites. Look at Sick Kids Hospital in Toronto, where they actually are providing consults to patients who are in other countries as well. So it is very important that we're now part of this large international community.

The Chair: Remarkable. Thank you very much for that answer.

We'll now go to Dr. Morin.

[*Translation*]

Mr. Dany Morin (Chicoutimi—Le Fjord, NDP): Thank you, Madam Chair.

Normally, when I take the floor at Standing Committee on Health meetings, I focus on issues of national scope. However, since we are talking about rare diseases, it is appropriate to also discuss individual cases.

In the municipality of Sainte-Rose-du-Nord, which is in my riding, there was a little boy named Miro Angers-Laurin. He had a rare disease called diffuse intrinsic pontine glioma. Only one or two such cases are detected annually in Quebec.

When the diagnosis was made, the child had only nine months left to live. Miro's family, which I know very well, decided to spend those nine months granting him all his wishes. That was a nice thing to do, but, during that time, they refused to allow therapeutic trials that had been proposed because they were not very conclusive.

Afterwards, they founded the MIRO foundation. That organization feels that it would be important to create and implement an international registry in order to learn more about that tumour and encourage research, since there aren't many such cases in Canada and the world.

We can also draw a parallel with other rare diseases. Would Health Canada be prepared to support that kind of initiative and collaboration? Dr. Wong-Rieger said that it would be important for Canada to become a leader in the area of rare diseases. The government would show leadership by working with our other international colleagues.

As a Health Canada representative, what do you think?

● (1630)

[*English*]

Mr. David Lee: Thank you.

Madam Chair, we at Health Canada would very strongly support the idea of cooperating in international registries. This is a very key aspect of the rare disease world.

How you enter very important information into registries is a key question. So coordinating internationally is a very important issue. That's why having these discussions among regulators about how to study drugs that are otherwise very difficult or impossible to study so that we can be very clear about the requirements is very important.

A new international consortium has been developed. It is actually chaired by a Canadian, Paul Lasko from CIHR, and I think we can be very proud of that. This consortium coordinates research of this kind, very small research, so we need to link countries together. There are over 30 countries in this consortium. Its purpose is to focus research and make sure we don't have redundancies. So lifting registries into the important study of both the disease and the drug and trying to learn as much as we can from them is a very important aspect of regulating.

Mr. Dany Morin: Thank you.

I would like to ask Dr. Wong-Rieger what she thinks of such an initiative.

Dr. Durhane Wong-Rieger: We are very supportive. The European Organisation for Rare Disorders, the National Organization for Rare Disorders in the U.S., and the Canadian Organization for Rare Disorders have actually signed a joint memorandum of understanding to say that we support international registries, and as David says, they need to have common elements.

The other thing I will mention is that the NIH, the National Institutes of Health in the U.S., has just in fact helped to launch a large genetic registries website called Registries for All. It's meant to be exactly for this purpose, to provide a common platform on which you can have individual genetic diseases registered. Patient access is available. We're very much encouraging all countries to go to a common platform so that the data may in fact be shared. Patient privacy can be protected. As David said, we're very supportive of it, and we're also very proud that Canada quite frankly is again one of the leading partners in helping to move this kind of an initiative forward.

[*Translation*]

Mr. Dany Morin: Thank you very much.

[*English*]

The Chair: Thank you very much, Dr. Morin.

I want to thank both the witnesses for their very insightful answers. It's a very unusual topic in some regards, and it's very useful.

Mr. Daniel, you're next.

Mr. Joe Daniel (Don Valley East, CPC): Thank you, Madam Chair.

Thank you, witnesses, for being here.

As we hear about all the processes that are going on and the framework you're putting together, it would seem that most of the remedial action is at the back end of the process. A disease has to be discovered, and then you react to that to try to come up with a medication to counter that.

My question is since this is all based on defective genes and so on that create these rare diseases, is there any innovation going on in technology—for example, there's a piece of equipment called GeneXpert that's being developed in Canada—to actually predict some of these things ahead of time?

Are you including any of that innovation in technology into the framework process?

● (1635)

Mr. David Lee: Thank you, Madam Chair.

Predictive technologies are a very important innovation in this space. Some of them will become regulated as medical devices, but some of them are a matter of hospital-based research. Identifying predictively when a disease is going to appear or what its symptoms and natural history might look like is very important work. It's frontier work. So if we were bringing it into the regulatory cycle, I think we would need to pay attention to two things. One is how it integrates with presenting treatments, and the other is how much validity and certainty we can get around these new technologies, because a misfire, predictively, could lead someone either to be without the necessary treatment or to have treatment they shouldn't have.

This is something that internationally many regulators are trying to get their minds around. A lot of very important discoveries are being made, but again, bringing them into the regulatory pathway is a matter of study. We want to make sure too that we don't overburden those innovations and remove a certain suppleness from them by requiring too much of the wrong thing. So understanding how you would validate that kind of predictive model in the regulatory cycle is another important discussion. We want to be very practical. We're having more and more discussions, especially in oncology, the area of cancers. A lot of progress is being made on identifying different types of cancers we haven't seen before.

So with regard to what we are to make of this—we're talking with a lot of our research specialists and others to develop technologies that detect those sorts of genetic variations—and to understanding how to bring that in are parts of a very important discussion.

We're also inviting our international regulatory counterparts. There's a lot of work going on in the United States on that as well.

Dr. Durhane Wong-Rieger: Can I can add a couple of things to what David is saying? It's very important.

First, it's not just looking for new technologies. I will tell you that there's an area in which Canada ought to be ashamed, namely, that we do not provide universal newborn screening. More and more countries are moving to that. We could actually detect, at birth, many more of these genetic disorders, many of which can be intervened in at birth to prevent the disease, or at least identify the disease before the family has another child with that same disease.

Yet there is not a universal program. Some provinces do a good job, doing more than 30 diseases; some provinces are still doing as few as four.

Second, because we don't have a national strategy around it, each province is in fact doing its own thing. Here there are two dangerous things. One is that you need a critical number in order to be able to identify these diseases, and even to know what to do with the test. Quite frankly, we're wasting money by having each province do it on its own. We don't need that many newborn screening sites in Canada. We could do a better job and have better use of our resources, as well as be more effective.

There's another thing that we're investing in—I think David knows this—and that's the very good research that's going on towards that whole area of genome sequencing that we know about. There is a prediction that in a very few years, that could become very practical. In one test, you could actually identify a whole host of rare diseases. We are at the forefront of that. How we will use it and how we will apply it and make it available—those will be our challenges.

So on the one hand, I think we're doing a shameful job. On the other hand, we're at the front end of the research.

The Chair: Thank you very much.

Mr. Joe Daniel: Do I have any time left?

The Chair: You have about 20 seconds.

Mr. Joe Daniel: Okay. That's fine.

The Chair: Thank you, Mr. Daniels. And welcome to our committee.

We'll now go to Mr. Kellway.

Mr. Matthew Kellway (Beaches—East York, NDP): Thank you, Madam Chair.

Thanks to our guests for coming today.

There are a few things I've heard today that have struck me. One of them is this aggregate total of rare disease, which actually strikes me as a very high incidence.

I think you said it was one in ten, Mr. Lee, or one in twelve, as per your paper; it's roughly the same.

The other thing is the easy and free acknowledgement that international cooperation and collaboration make so much sense, and in fact kind of follow naturally because of the small groupings and stuff.

I think, Mr. Lee, you said that in fact the thing about rare disease is that it attracts a lot of international cooperation.

In spite of all of that, to date we somehow seem to have resisted that natural attraction to international cooperation by just now—if I understand this correctly—bringing in this framework, or proposing a framework that will, in your terms, Mr. Lee, create space in which Canadian research and innovation can thrive.

I'm kind of stunned by the place we're at with all of this, frankly. In order to almost fight the natural attraction for international cooperation to be able to participate in that and help the 10% of Canadians who suffer from rare diseases, we somehow have resisted that opportunity, and are in the place we're in today.

Ms. Wong-Rieger, I want to ask you about the genome research you mentioned at the end of your last comments. Now that we seem to be on the precipice of a place in which Canadian research and innovation can thrive with this framework—if that's indeed the case—how do we connect this genome research into the issue of rare diseases? Is there an element of the national strategy you're proposing that does specifically that?

● (1640)

Dr. Durhane Wong-Rieger: It does all relate together. Certainly not all genetic diseases are rare diseases; however, about 80% of the rare diseases are genetic diseases.

One of the biggest challenges, quite frankly, is being able to identify and diagnose a disease. As we often say to families, when you have a disease or you have a condition that's not diagnosed, it's not that it's not diagnosable; it's just that we haven't been able to diagnose it.

So having some means of being able to diagnose a disease, and diagnose it fairly accurately, can then actually lead to a whole host of either preventive actions or at least supportive actions. That's the first step.

We do need to have these national centres of reference where we can then actually refer patients in order for them to get ongoing kinds of support and intelligence. So it's the first step towards it. But you're absolutely right that if all we're doing is diagnosing the disease and we're not doing any of the other kinds of work necessary, we could actually be leaving a lot of families in a lot worse condition than if they didn't know.

So it is part and parcel of an overall strategy, but as we've said here already, we know it has to be national. We should not ask each province to do this. It makes no sense to do that.

I go back to saying that we would love to have the dialogue. The next step beyond an orphan drug regulatory framework is to really talk about a national strategy, and I think it's a public health strategy.

Mr. Matthew Kellway: Now's your opportunity, actually. There will be other opportunities, I'm sure, but I'd like to give you the opportunity to take advantage of this one and perhaps elaborate a little more on what these centres of excellence might look like and might do. What institutionally do they look like? Where do they live? Where do we build them? Who participates?

Dr. Durhane Wong-Rieger: We have some already, so not to say that we're starting this without some knowledge. If you look at SickKids, for instance, they have a very good centre of excellence around pediatric cardiovascular diseases and rare diseases. There is a vasculitis centre there. In fact, the research projects that have just been funded by CIHR for emerging teams on rare diseases all actually constitute a centre of excellence. These centres have to be bigger than a single disease, obviously, so there are natural groupings of diseases that need to take place. They can be defined, certainly, by the cause of the disease; they can be defined by the whole host of organs that they actually impact, though many of these are multi-system diseases. So we actually have some of those in place.

What we would love to see is, in fact, a national consultation around that, bringing together the clinicians, the researchers, and the patient community. There's a whole format that the European Union has put together to host consultations around that discussion. Quite frankly, we want to get the orphan regulatory framework first, but we really would like to introduce that format into Canada so we aren't reinventing the wheel even in terms of how to do it.

The Chair: Thank you, Dr. Wong-Rieger. Thank you for those insightful comments.

We'll now go to Dr. Fry.

Hon. Hedy Fry: Thank you very much.

Dr. Wong-Rieger answered my question on the diversity. It's important, because I think that Canada has a huge role to play in terms of looking at transnational research. We have one major public administrator, really, to get all our data from, so the data is easy to share, unlike in the United States, which has the same diversity, but has to deal with, I don't know, 3,000 separate private insurance companies, and that makes it very difficult.

That's something that I feel. I don't know if you were looking at that within your framework, how Canada plays that kind of role, in terms of looking at the ethnic and the racial diversities, and how we can provide some transnational research for that in the international framework.

Mr. Carrie did ask the question, and it's something that we need to learn from others about how to do this. I think we used to do it well. The question is: how do you work around jurisdictional responsibilities to create something for all Canadians? If the European Union, as I asked in my question, has many autonomous nation states that they can set a framework for, why can't we in Canada learn from them? What can we learn from them? Is there going to be something we're going to try to learn from the European model of how this is done well?

Does anybody want to take that on?

● (1645)

Dr. Durhane Wong-Rieger: I don't think anybody would pretend, as you say, that the 27 member states of the European Union are all working in lockstep. There is something very important —and certainly rare disease patients are counting on it a lot—and that is the whole cross-border directive, that patients, if they cannot get the care in their own country, can in fact move and get the treatment from another country. I mean we're saying, "Good gosh, you can't even do that well across provinces in Canada". That's the first thing we could learn, namely, how we actually facilitate patients who are in one jurisdiction getting access to what may be the only centre of excellence in another.

Quite frankly, as you might imagine, if there were in fact a national strategy, the provinces would expect that it would come with some sort of incentives to make it happen. I always hate to talk about the big word, in terms of funding, but I think there would need to be some sense of how we would do that. The same as CIHR has done, we need to have the Genome Canada kind of funding. Orphanet is funded out of a national budget. I do think that one would have to talk about how you would actually be able to mobilize the funding so that it could be centrally managed and to make sure that there is, in fact, fair participation, and not just based on population and ability to pay. We know that some of the pockets of these diseases are not necessarily in the most populous cities or even in the most populous provinces.

Hon. Hedy Fry: That's a good point you make. We always tend to look at major centres of excellence in cities and tertiary centres tied to universities, and in some instances we may need to look at centres of excellence in rural areas as well. I don't know if that's something that you're thinking of, Mr. Lee, so that we don't only look at these centres of excellence, especially in rare diseases, being in universities or—

Dr. Durhane Wong-Rieger: Many of these are in fact virtual centres. If I look, for instance, at the hematological and rare blood disorder centre, while it's housed in Spain as one hub, it is fact a virtual centre. The collaboration in that case is from right across Europe. So there isn't actually a bricks and mortar site specialized just for that. They're different, special areas. Some testing, some diagnosis, some research is done at different sites, but they collaborate virtually. So it makes a rural site very feasible.

The Chair: We're virtually out of time.

I just want to thank both of our guests.

Dr. Wong-Rieger, all of your comments were extremely insightful, as were Mr. Lee's. This is a very important topic because it's such a small population. So the committee wanted to hear from you both.

Your idea about innovation and doing it virtually across the country.... I mean, we're in the day now of the Internet and things like that, which can assist us.

So thank you very much.

Dr. Durhane Wong-Rieger: Thank you so very much.

The Chair: The committee is dismissed.