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# Standing Committee on Health

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**EVIDENCE**

## Thursday, June 3, 2010

## Chair

**Mrs. Joy Smith**

# Standing Committee on Health

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● (0740)

[*English*]

**The Vice-Chair (Ms. Joyce Murray (Vancouver Quadra, Lib.)):** Good morning, everyone. I'm pleased to commence our meeting this morning on rare disorders. My name is Joyce Murray. I'm sitting in for our regular chair, Joy Smith.

I want to welcome all the witnesses and guests. I hope you've had a chance to fill up a plate and have a bit of breakfast.

We have a bit of a different format than is usual in our meeting, so I'll just touch on it for the benefit of the guests and the committee members. The format of the session is an interactive round table. We'll begin the round table with brief introductions from our guests. Then there will be an open discussion, questions and discussion, among the members and the witnesses. The clerk will be keeping a list of speakers, so please let her know if you'd like to speak. The chair will recognize speakers, so we're not all chiming in at once. And as you are all aware, there is translation, so use your earpieces if you need that.

Lastly, I'll mention that this is not my normal hair colour, for anyone who thought it was. It's actually about a campaign for a rare disorder. It's called Reddy for a Cure. It's about cystic fibrosis, in honour of Eva Markvoort, a young woman who died recently and who was, unintentionally, a spokesperson. I'm pleased to be part of the campaign in her honour to raise awareness of cystic fibrosis. That's why I mentioned it this morning.

With that, we will start with Peter Brenders. Please introduce yourself.

**Mr. Peter Brenders (President and Chief Executive Officer, BIOTECanada):** Thank you.

*Bonjour.* Good morning. My name is Peter Brenders. I'm the president and CEO of BIOTECanada. We're the national organization representing biotechnology companies in this country. There are 21 today working on rare disorder treatments. So I'm pleased to be here.

**The Vice-Chair (Ms. Joyce Murray):** Thank you.

Jean-Luc Urbain.

**Dr. Jean-Luc Urbain (President, Canadian Association of Nuclear Medicine):** Good morning. My name is Jean-Luc Urbain. I am the president of the Canadian Association of Nuclear Medicine.

I'd like to thank the committee for inviting me once again to testify on rare disorders and medical isotopes. We have significant issues, besides the big issue we have been dealing with over the last year. It turns out that we are now facing another significant crisis, due to the lack of authorization for isotopes that we need to treat patients with rare disorders. So I'm very pleased to be here this morning.

**The Vice-Chair (Ms. Joyce Murray):** Thank you.

Durhane Wong-Rieger.

**Dr. Durhane Wong-Rieger (President, Canadian Organization for Rare Disorders):** Good morning. My name is Durhane Wong-Rieger. I am the president of the Canadian Organization for Rare Disorders, which is an umbrella organization for a network of rare disorders organizations in Canada.

**The Vice-Chair (Ms. Joyce Murray):** Thank you.

Maureen Coleman.

**Ms. Maureen Coleman (President, Carcinoid NeuroEndocrine Tumour Society Canada):** My name is Maureen Coleman. I'm president of the Carcinoid NeuroEndocrine Tumour Society Canada. We're all over Canada. Our mandate is research, education, awareness, and support for our relatively rare cancer. We have a shortage of certain treatments, including isotopes. This will come out in discussion.

Thank you.

**The Vice-Chair (Ms. Joyce Murray):** Thank you.

Gail Ouellette.

[*Translation*]

**Ms. Gail Ouellette (President and Chief Executive Officer, Quebec Coalition for Orphan Diseases):** Good morning. I am Gail Ouellette, the director of an information and support portal for orphan genetic diseases in Quebec and president and chief executive officer of the Quebec Coalition for Orphan Diseases, which includes 20 rare disease organizations.

[*English*]

**The Vice-Chair (Ms. Joyce Murray):** Thank you.

Ladies and gentlemen, it's over to you.

Dr. Bennett.

**Hon. Carolyn Bennett (St. Paul's, Lib.):** I would prefer to hear more. We can't really have a discussion until they've put their issues on the table. So let's give them all a chance to actually tell their stories.

[*Translation*]

**Mr. Luc Malo (Verchères—Les Patriotes, BQ):** We should allow at least five minutes for each person to make a presentation.

[*English*]

**The Vice-Chair (Ms. Joyce Murray):** Other thoughts?

It makes sense.

Mr. Brenders, you—

**Hon. Carolyn Bennett:** I think it would be better to start with the people who are representing the patients and then go to the organizations. Is that...?

**The Vice-Chair (Ms. Joyce Murray):** It looks like everybody's in agreement with that.

Madame Wong-Rieger, would you like to frame the issues from your organization's perspective?

**Dr. Durhane Wong-Rieger:** Certainly. Thank you for the opportunity.

The Canadian Organization for Rare Disorders is the national network for organizations for rare diseases in Canada. We are affiliated with both the National Organization for Rare Disorders in the U.S.A. and with EURORDIS, the European Organization for Rare Diseases.

The issue is that in Canada people with rare disorders are not well served. About 1 in 12 persons in Canada has a rare disorder. This may be surprising, but it affects nearly 3 million Canadians. There are between 6,000 and 7,000 rare disorders in the world, and many are found in Canada. So it is in fact a significant public health problem.

The way health care is set up in Canada, rare disorders receive disproportionately fewer and poorer services. Most people with rare disorders have poor access to diagnosis, poor access to treatment, and poor access to care. Around the world what's been happening, especially in Europe, is that they have recognized this. In the European Union they have introduced a recommendation that all member organizations, all member states, should adopt a national plan for rare disorders. This is what we're asking for in Canada as well, to make sure that we are able to treat patients with rare disorders in the same way as we do patients with more common diseases.

Within that plan, we're looking for three main things. First, we desperately need a definition of rare disorders. We're one of the few developed countries that has no national definition of rare disorders. Most countries define a rare disorder as a disease that affects fewer than 1 in 2,000 people. This definition would allow us to provide comprehensive services to patients right across the country. I think it would open us up to having a regulatory framework.

We are probably the only developed country that does not have a plan for orphan drugs and rare disorders. This means that our patients receive disproportionately less access to new treatments derived from research and development. It means that we have a more difficult time getting clinical trials for patients with rare disorders. It means that, unfortunately, Canadian patients with rare disorders are among the last patients in the developed world to get access to new medicines.

So as they're watching—we have families who are connected internationally—they see patients in other countries getting access to drugs. We have not set up a regulatory framework that would provide incentives for companies to bring their trials and products to Canada. So we are disproportionately served. Our patients are diagnosed later and treated later. Many of them become sick or die, even when treatments are available right outside the country and available to other patients who are suffering from exactly the same diseases. So we need the regulatory framework.

Second, we need a national plan for rare disorders. Countries around the world are establishing plans for providing integrated, comprehensive services for patients with rare disorders in the same way as they would provide services for common diseases, while recognizing that rare disorders are different. It takes more to be able to provide that expertise, but it takes more by way of organization, not necessarily by way of funding. What we know is that we pay for patients with rare disorders already; we just do it poorly, and we do it inefficiently, and we do it in such a way that we oftentimes don't really begin to take care of them until they're much sicker than they need to be. So our care and our treatment is just as costly as it would be if we had a good plan. We just don't service it well; we don't provide the same benefits. I think we also need to make sure that we have a national, integrated, comprehensive framework within which we can make recommendations for the funding of treatments for rare disorders.

We thank the health committee—I think it was two and half years ago—for holding a review on the Canadian Agency for Drugs and Technologies in Health. At the time, we recommended that CADTH come up with an appropriate way of reviewing drugs for rare disorders. What this committee recognized was that the way these drugs were being reviewed was not serving patients well. Patients with rare disorders do not get the access to treatments that they ought to. Drugs are approved, but then there is no program to allow them to be funded. We urge this committee, then, to actually pick up on what you began three years ago and to make sure that that does happen.

● (0745)

I'll leave it at that, but I look forward to the discussion.

And by the way, I love your hair colour and I think what you're doing is perfect.

**The Vice-Chair (Ms. Joyce Murray):** Thank you.

The intent is that this is a round table, an experimental format that's a bit less structured than our normal meetings. So I'd like to invite the members, if you have comments and questions, to bring them up now, and then we can also have the other interveners give their five minutes.

Yes.

**Mrs. Cathy McLeod (Kamloops—Thompson—Cariboo, CPC):** I have a quick question. It might help frame the future discussion. The definition that's fairly consistent, is it only related to numbers or does it have any relationship to acuity?

● (0750)

**Dr. Durhane Wong-Rieger:** It really is based on prevalence, so it is defined as a disorder that affects fewer than 1 in 2,000 persons. But by their very nature, most rare disorders are in fact severe. Many are life-threatening, many are debilitating. So just by defining it as 1 in 2,000, you recognize that most of these disorders are in fact fairly severe disorders.

**Mrs. Cathy McLeod:** Thank you.

**The Vice-Chair (Ms. Joyce Murray):** Are there others among the guests who would like to comment?

Dr. Bennett.

**Hon. Carolyn Bennett:** I have met with Maureen Coleman and Dr. Urbain before, and I think the story in terms of "if you don't suspect it, you can't detect it".... The real plight is of patients with neuroendocrine tumours who are diagnosed late and then find that the radiopharmaceuticals are not available in this country. I think the story of having a patient from Canada, with Canadian-made isotopes...which by the way are not being made in Chalk River; they're available now, the yttrium and lutetium. The patient is being put on a plane to England to be treated, sometimes travelling on the same plane as the isotope, and then comes home to fight with the insurance people, just because the radiopharmaceuticals haven't been approved yet in Canada.

I would like to hear Maureen and Jean-Luc tell their story.

**The Vice-Chair (Ms. Joyce Murray):** Maureen, go ahead.

**Ms. Maureen Coleman:** Thank you for having me.

I was going to introduce the idea of our cancer, first of all. We are a cancer. We're sometimes not regarded as a cancer, but we're highly malignant, and it grows rapidly. It comes from the neuroendocrine cells in the body, and we need a variety of treatments.

When neuroendocrine cells produce too many peptides and hormones, they cause tumours to grow, and they can grow uncontrollably and you can eventually die from tumour load. One of the things that stops our tumours in their tracks is a variety of treatments, including radioisotope treatments, which are available right now in just about every country in the world. Lutetium and yttrium are available in Cuba, Bangladesh, India, Australia, Singapore, all over Europe, South America, everywhere. They extend our lives by many years. I've been a patient for 10 years. I haven't yet needed to use lutetium or yttrium, but many patients have.

One of the big problems is that not only do patients have to go out of the country, but they can lose their houses over it, because it's sudden. You're given two weeks' warning to go to England. The caregiver and the patient go out of country—with the isotope, possibly, on the same plane—and sometimes it's four treatments in a year. Once you take accommodation into account, it's $50,000. The money is very significant. Patients sometimes can't go because they can't afford to get on a plane. They just sit at home and wait for the end.

So I'd say about two-thirds of the patients who are approved can go and the others can't. They have to rely on less effective options.

One last thing. In Sweden, where they're quite common, the life expectancy is probably about four times as long as here. I remember Dr. Öberg at our Toronto conference in 2009 saying that it's 133 months or something for people once they're on isotope treatment, as opposed to 33 months in North America, in Canada, with certain isotopes.

Thank you.

**The Vice-Chair (Ms. Joyce Murray):** Thank you.

Dr. Carrie, you have a comment?

**Mr. Colin Carrie (Oshawa, CPC):** Yes.

Maureen, what is the reason they're not available in Canada? Has anybody applied through the special access program or anything along those lines? What's the rationale you've been given?

**Ms. Maureen Coleman:** They do apply through the special access program, though some people completely circumvent it. People with money just go out of the country. There's a high incidence of people with money going out of the country. They go through the special access program, but then I'm not sure how well educated physicians are on the special access program. And when they do apply, it's quite lengthy.

**Mr. Colin Carrie:** Seriously? What are we talking about?

**Ms. Maureen Coleman:** It can be lengthy. It could be months, and when somebody's quite sick, that's a long time.

**Mr. Colin Carrie:** It would be, yes.

**Ms. Maureen Coleman:** As important as that is, they can't afford to pay the airfare, so the special—

● (0755)

**Mr. Colin Carrie:** So the special access program...my understanding is it should be available in Canada.

**Ms. Maureen Coleman:** No, not in Canada.

**Mr. Colin Carrie:** So they apply for the special access program and they are getting denied?

**Ms. Maureen Coleman:** Some people will be. They get accepted to go out of the country. They don't get accepted for Canada, and that means they can't afford to go. So they can't take advantage of it because it's not regulated here.

**The Vice-Chair (Ms. Joyce Murray):** Dr. Urbain.

**Dr. Jean-Luc Urbain:** Colin, this is the key question. That's a very good question.

Let me try to frame this. You know we have been dealing with a major isotope crisis of molybdenum and technetium; they're 80% of the isotopes we use in nuclear medicine. Rare disorders need rare isotopes. It's very difficult to determine regulatory process, to have access to import into Canada what I'm going to call those rare isotopes. The way we're trying to cope with the system is we're trying every single path we can.

I'm going to give you a specific example. Three weeks ago, I got a phone call from Montreal, then from Halifax, and yesterday from Vancouver, to let me know that the special access program is now closing the importation of yttrium, which Maureen mentioned, just because the companies have not submitted a proper dossier to Health Canada to import those isotopes. The issue is that those companies cannot submit a proper dossier based on clinical trials in Canada. It would take 15 to 20 years. In other words, the processes with Health Canada are antiquated. What we have found is that it really depends on the person you're dealing with in a country of overzealous bureaucratic processes.

So in the case of neuroendocrine tumours, for example, that's something that we have had as a special access program for 15 years, and for 15 years we've been told we have to do the proper study, while, as Maureen said, the European Union has proper access to those drugs. There's a major disconnect between patient needs, Canadian needs, and the health care regulatory process.

One of the reasons why I really wanted to come here—and again, thank you for the invitation—is to plead with this committee to make sure that we put in place with Health Canada the proper processes for Canadians to have access to rare drugs or rare isotopes for rare disorders, besides the big picture, which is a totally different problem.

**Mr. Colin Carrie:** So you're saying Health Canada is demanding a Canadian study. It would be too small a study. Committee members would take 10 years to have that done. Is that what you're saying? They're not recognized in international studies or anything like that?

**Dr. Jean-Luc Urbain:** Yes. That's exactly the point. When you deal with cardiovascular disease or cancer, you can generate data very quickly. When you deal with rare disorders—and Durhane mentioned those numbers are in your information—it's virtually impossible. At the same time, we feel very bad for our patients, because we constantly feel it's a tennis table between the federal and provincial agencies. Federal agencies have regulations; provincial is basically reinforcement. Patients are trapped in between. At the end of the day, they don't get the care they need in the western world.

**The Vice-Chair (Ms. Joyce Murray):** Mr. Brenders.

**Mr. Peter Brenders:** Thank you.

I think Maureen's and Dr. Urbain's stories are great examples of a symptom of a challenge that Canada presents and that other countries don't have, which Durhane mentioned. If you think about it, Canadian patients are at a disadvantage. Researchers are at a disadvantage. Our health system is at a disadvantage, because we don't have the structure in the system. So what happens elsewhere in the world? It started with the United States in 1983 when they introduced an Orphan Drug Act, and it was called “orphan” because these diseases were deemed to be orphans. No one was looking after them. Rare disorders were basically neglected.

In the 10 years prior to 1983, there were only 10 drugs available in the whole world to treat a very limited number of orphan diseases. After the act came into place, you saw a whole upswing of research and development. Today there are over almost 400 products on the market to treat rare disorders and there are over 2,000 products in research and development. I mentioned earlier there are 21 Canadian companies that are doing it. But a lot of that research is all done outside of our country. It's done in these jurisdictions, like in the U. S., like in Europe and Japan, every developed nation except us, and we're in line with I think it's South Africa, Saudi Arabia, and India right now in not really having a defined structure to deal with orphan products to allow for the research, the development, the introduction, and the treatment. We're behind the times.

As I heard Durhane say, there are only two things that we need. One is we just need a regulatory structure to recognize these and create that support, because these are different from the sorts of common treatments that people see out there. Then we need a framework to allow for that coordination of diagnosis, treatment, and support of these patients. We need some federal leadership, which can be a regulatory change, a non-cost activity for us as a country to be able to put this in place and give that leadership down to the provinces that we're treating everyone the same. It's not new to us. We've talked about this for a number of years.

This committee has had some tremendous leadership in that area. Parliament has had it as well. Yet we continue to be stuck, and we're a little frustrated by that, especially as companies trying to introduce new treatments and wanting to make sure that Canadians can get early access in the trials, early access in the support, and that all that money we've been spending on primary research in the early days ultimately turns into products for Canadians.

I'm happy to take questions on that.

● (0800)

**The Vice-Chair (Ms. Joyce Murray):** Carolyn Bennett.

**Hon. Carolyn Bennett:** I think what we're hearing is that there's a huge opportunity here, that it is about rare.... The fact that it's rare means you're not going to get classical double blind trials in Canada. We've got to change the rules. I believe that if we can get it right for the rare diseases, we actually would get a better regulatory system for all diseases, meaning that we have been a bit blinkered in Canada with this idea that you have to have Canadian trials in order to go forward.

Maybe you could share with me what other countries are doing, because I thought the FDA had begun a system where you can have a committee of patients and health care providers looking at the international evidence and saying, this is good enough for me, for us, and this gets fast-tracked into Canada, or saying we don't like the way this trial was done, it doesn't look independent, it looks whatever, and that goes into the regular system. But it seems that we need to look at what you would seriously recommend we do from other countries so that we could quickly fix this. There isn't the expertise within that little office.

The idea that the difference between a drug and a radiopharmaceutical...the people in that office only know about drugs. They don't know about radiopharmaceuticals. It's now a real block that we as a committee I think could just suggest that it get done immediately with no money. With Fabry's disease, I think the feds, the provinces.... I was at the meeting where all the health ministers came together, knowing that the pharmaceutical companies were prepared to go a third, a third, a third and help pay for this. It was unevenly placed across Canada, Nova Scotia, and Alberta. All the health ministers said we should help with this, because it's not really fair and because there was almost an epidemic of diagnosis and a very expensive treatment.

I think what we want today is some advice. If we were to write a letter to the minister, if we were to be able to help do this, what would you want in that letter, and would you suggest a structure that has worked in other places? How did Cuba, Poland, Serbia, get around this such that their patients are getting access—on the neuroendocrine, but then also on the pharmaceutical as well?

**The Vice-Chair (Ms. Joyce Murray):** Ms. Leslie.

**Ms. Megan Leslie (Halifax, NDP):** Thank you, Chair.

Actually, I think it makes the most sense to have a response to Dr. Bennett's question.

Perhaps you can come back to me.

**The Vice-Chair (Ms. Joyce Murray):** Do any of our guests want to respond to that?

I think Ms. Wong-Rieger does and Mr. Urbain.

● (0805)

**Dr. Jean-Luc Urbain:** Carolyn, I think you're absolutely right. This is a unique opportunity for Canada. The way I see it, the unique opportunity is coming from the fact, as Gail said, that they call them genetic diseases.

We are in the era of the personalized human genome project and personalized medicine. These diseases—and don't take this the wrong way—are the perfect templates to look for a solution for common diseases in the future at limited cost. So they could be wonderful approaches to unroll personalized medicine in Canada, since we know for most diseases the genetic origin and mechanism, and the industry can develop drugs for diagnosis and treatment for those orphan diseases.

**The Vice-Chair (Ms. Joyce Murray):** Is there another answer to the question Dr. Bennett posed?

**Dr. Durhane Wong-Rieger:** I think Dr. Bennett is absolutely right. We don't need to re-invent the wheel on this.

Gail and I were recently at the European organization for rare disorders conference. There were 3,000 persons there and every country was represented.

People are putting together national plans to do this, and there is every opportunity for Canada to be a partner at that table, to actually follow the models that are being put together for national plans and look at the pillars they've already identified.

They've put out guidelines in the European Union for how countries should structure their plans. There are a dozen different models, so there's no one-size-fits-all. But there are blueprints to help you identify them.

First and foremost is what Peter talked about, and that is that we need that regulatory framework. It speaks to what Dr. Brenders and Dr. Bennett are saying. We have to modernize our Canada Health Act and the way in which Health Canada works so that we can take advantage of what is being put in place in other countries to deal with modern medicine.

The Canada Health Act is 50 years old, and in many respects, even the regulators can't do what they want to do. This is a tragedy because it results in all the kinds of inefficiencies we're talking about, unnecessary suffering, and people not getting treatment. As you say, it's difficult for companies to come to Canada to set up shop, do their clinical trials, or make these therapies available to people.

So you're absolutely right. First and foremost, we have to do the regulatory framework. I would say that in Canada we're a hair's breadth away from getting it. There are some draft regulatory pieces that have been put together. We have provided input on them to Health Canada. We know we're very close. It really needs the parliamentarians to say, “Yes, do it.” It needs this health committee to turn to the minister and the rest of Parliament and say we need to do this.

We believe that patients with rare disorders are different; therefore, we have to approach the problem differently. We have the models for how to do it. At the end of the day, it doesn't cost any more. As Maureen says very clearly, it can cost us a whole lot less and make it so much better for people if we do it right. The good news in being so far behind the rest of the world is that we can learn not only from other people's experiences, but we can take the best of the plans. I think we've got a lot of good blueprints.

As Dr. Bennett said, in many other countries, patients, researchers, clinicians, and the regulators are at the table to provide input into design of the clinical trials, the review of that information, and even on the next step in terms of how to make them available and accessible to patients.

Dr. Bennett said the solution that we had for Fabry's disease—that you had for Fabry's disease—was not the only model, but it was a very good model of how the feds, the provinces, and the territories can work together. I will wager that the feds played a leading role in making that happen, and I think we can.

So the blueprints are there. All of the things we need to do are there, and what you're hearing as to one specific disorder is exactly the case. This is the problem in many other aspects we get with all of the rare disorders, and we believe it as well. If we can fix this problem for rare disorders, we can do a heck of a lot better for many common disorders. We can get ourselves into a position of being one of the leading countries, because we're very close. We have lots of good stuff going on in the background. It's up to the committee and Parliament to make it happen.

**The Vice-Chair (Ms. Joyce Murray):** Thank you.

Ms. Ouellette and Ms. Coleman want to respond to Dr. Bennett's question. Then we'll go back to our list of speakers.

● (0810)

[*Translation*]

**Ms. Gail Ouellette:** The story of neuroendocrine tumours remains a story, and there are many like that in Canada. Unfortunately, it is often dealt with case by case. Fabry disease is another case that was dealt with to some extent. Often, the case was handled because parents or adults went to the media and managed to obtain a positive answer. But it is unacceptable that some people, in addition to being sick, have to go to the media to demand access to good medical care and treatments. There are many similar examples. Inequities happen all across Canada because each province offers different solutions.

For example, myosin is used to treat Crohn's disease. In our province, adults don't have access to it, but in our neighbouring province, in Ontario, adults can at least try this drug to see if it is effective, and can have an assessment done.

I could list a number of inequities like that. That is why we need a regulatory framework to standardize what happens in Canada with orphan diseases. The regulatory framework should address two issues: the development of drugs and access to them.

We know that drug benefits are under provincial jurisdiction. But, in terms of assessment, why redo the assessment of a rare drug, an orphan drug, in each province, when it is already something difficult to do? There must be a special way to review it. And that is what is going on at the moment.

We know that Quebec has excluded itself from drug review in general, but for orphan drugs, I believe in the need for a framework so that resources can be used better. We could do standardized assessments. We know that each province decides on the reimbursement, but at least the assessment would be properly done for orphan drugs, which are not the same as drugs for common diseases.

There is also the issue of developing drugs. I would like to give a positive example of an initiative that we could take more often in our country. In Quebec, a patient organization was able to have a company from California come to do a clinical study. Those people succeeded in turning the Montreal Children's Hospital into a site for international study. It is not an easy thing to do, but they managed because of those few patients with Morquio syndrome, who are actually more numerous in Quebec. In fact, that allows Canada to have expertise in a disease. That hospital could become a specialized clinic for that syndrome. It could become a site for phase 3 clinical trials for that drug. That is very beneficial for the patients, obviously, but also for drug research and development.

But, even if that drug has phase 3 trials in Montreal, we are afraid that we might be facing the same obstacles in terms of approval and access in Canada. There is also the question of quid pro quo in the research. Patients give their time and they travel to participate in the study. So, if they can't even have access to the drugs after that, it would be a real shame.

**The Vice-Chair (Ms. Joyce Murray):** Thank you, Ms. Ouellette.

Ms. Coleman.

[*English*]

**Ms. Maureen Coleman:** Speaking as a patient, I want you to know that patients in Canada and around the world are connected very much by the Internet, and through the Internet we read about clinical trials in other countries. We read about approved procedures in other countries. So we read about how successful certain isotope treatments are, we read the abstracts. Our support network throughout Canada...it's not so much that we're doing things like yoga. We don't really have the luxury of just being stroked. We are out there hunting for research, reading research, learning about research, and then we discover that what's happening over here is absolutely wonderful. What's happening in Sweden or Germany with a certain type of scan, say, the gallium scan, is wonderful.

In my case, I'm a patient with what's known as an undiscovered primary tumour. I was discovered with distant metastasis 10 years ago. I go through imaging. When I go through imaging, the imaging is okay, but it's not that great. It can't find my primary tumour. It might find 80% of primary tumours. If I lived in Sweden, it would find 99%; it most likely would find my primary tumour. The current thinking is that it's good to have your primary tumour removed. It offers a better prognosis for the future, but if you can't find the primary tumour, then that chance of having an improved prognosis is much reduced. I'm just basically walking around thinking, so far, so good, but wouldn't it be nice to know some of the uncertainty could be taken out of that?

Meanwhile, our community has created a worldwide network. I'm on a steering committee called the Worldwide NET Cancer Awareness Day Steering Committee, and we have representatives from about 15 countries on that committee. We met in Berlin, we're meeting in New York, and we're going to have Worldwide NET Cancer Awareness Day on November 10.

A website is going to be unveiled next month with basically information from patients around the world, so we will be able to read what is happening in every country with regard to this cancer and how patients are faring and what's happening in the medical world, what developments are occurring.

Our particular neuroendocrine site will be connected to that and discussions will be going across borders all the time. At my last meeting I sat next to the representative from Poland, so I learned about what was available in Poland, first-hand.

● (0815)

**The Vice-Chair (Ms. Joyce Murray):** Are there any thoughts about what could be in the letter to the minister?

**Ms. Maureen Coleman:** The key thoughts about what could be in the letter to the minister are this. Could we please relax the regulatory framework so that we can adopt some of those proven techniques, particularly the ones in Europe, maybe even in India? But we're close to Europe, so let's focus on Europe. Some of the isotope treatments that are used in Europe, the yttrium and the lutetium, and some of the scans, like the gallium scan, are so effective. Could we please look at them and adopt them, because they've worked year in and year out for many patients now without serious problems at all?

Thank you.

**The Vice-Chair (Ms. Joyce Murray):** Ms. Leslie, back to you.

**Ms. Megan Leslie:** Thanks, Madam Chair.

I really appreciate this discussion. I'm wondering if you can help me understand what we can do with regard to diagnosis. Are there policy responses or regulatory responses to enhance, to improve diagnosis, or does it logically follow that if we can approve treatment, diagnosis will become better?

**The Vice-Chair (Ms. Joyce Murray):** Ms. Wong-Rieger.

**Dr. Durhane Wong-Rieger:** Again, I think those are excellent concerns. If you look again in terms of what's happening internationally, I think it provides a lot of good commentary. As Maureen says, most of these are rare disorders, so you do have international expertise. You can't just rely on what's available in Canada. You do have to be connected internationally. The patients are connected internationally, and many of our clinicians, if they belong to international associations, are connected internationally. But our regulatory bodies and our approval bodies have also got to be connected internationally. It goes back to what Dr. Bennett is saying. We know we already have frameworks for approval in other countries. We've been talking about harmonization in terms of regulatory framework, so I wouldn't necessarily say we want to relax the regulatory framework—I understand what you mean—but I think what we want to do is to modernize it, and we want to be able to harmonize it so that we can take advantage of what is happening in other countries.

● (0820)

**Ms. Megan Leslie:** Harmonize internationally?

**Dr. Durhane Wong-Rieger:** Harmonize internationally; use international standards. We don't even have a definition that is harmonized. The diagnosis, then again, sometimes depends on having the knowledge and being able to get the right experts. What happens in many other countries is they have these centres of reference where if physicians suspect a disorder that might be of a particular type, they can actually send the tests and information, and sometimes the patient, to a centre to be diagnosed.

Many of our patients, fortunately, go to the NIH and they can get it, or they go to one of the centres in the U.S. Again, this is not acceptable for Canadians to just have that; we should have centres that are connected to centres that are international centres. So you have centres of reference where you've got experts, and they are virtual centres; it doesn't mean you have to build new infrastructure there.

The other thing that can happen in terms of diagnosis is, as you say, when you have treatments; that is in fact when you do stimulate a lot of diagnosis. There are lots of reasons—more is known about the disorder; there are more incentives for people to get tested for that particular disorder. Again, we need to be connected internationally, though, because as treatments become available, we do a better job in terms of diagnosis and we learn more about the disease.

The other thing we are doing a poor job of in this country—and we don't want to keep talking about the poorness of it—is newborn screening. I think as everybody has said, many of these disorders are genetic. In the U.S., even the most impoverished states will test for at least 50 newborn diseases with one single blood drop. Internationally, newborn screening is becoming one of the most important ways of identifying genetic disorders, many of which, in fact, if they were caught at birth, could be treated from birth without the devastating effects. Everybody knows about PKU, phenylketonuria, as one of the world's profound examples. We have many other disorders of that nature, which we do not test for. Except for Saskatchewan and Ontario, most provinces are identifying fewer than 10 of these at birth, and it just takes one drop.

We need to have a national reference centre, where once you've identified that, you can get the right genetic counselling and you can get the right genetic diagnosis. Again, it means that we need to be connecting these, nationally first and then internationally. The diagnosis is oftentimes difficult, but it is in fact improving tremendously because you've got these centres of expertise.

Unfortunately, Canada doesn't take part. And we can. Europe has its arms wide open. We go over all the time. They are telling us, “Come with us.” We're an affiliate in many ways. They're waiting for us to actually provide some expertise as well. We don't want to just be beggars at the table; we also want to be there with our own expertise. We have many tremendous pockets here; we just need to do a better job of taking advantage of our expertise nationally and internationally.

**The Vice-Chair (Ms. Joyce Murray):** Thank you.

We have two more of our guests who wanted to speak to Ms. Leslie's question.

Dr. Urbain.

**Dr. Jean-Luc Urbain:** Megan, this is also a very good question.

We love acronyms in medicine, so I'm going to use two of them.

One is PPPT, which is for predict, prevent, pinpoint, and treat. One is the predictions biomarkers, the prevention is basically genetic counselling, the pinpointing is diagnostic imaging, and the treatment speaks for itself. We do not have this in place in Canada. This is personalized medicine. We just don't have it. We are basically dealing with patients based on a very empirical model, which is that we treat symptoms. Since we don't have the means to do diagnosis, we cannot really queue related diseases.

The other thing I want to mention is in terms of the regulatory frame. Another acronym we like to use is the AAA approach. The AAA approach consists of availability, affordability, and accountability. Unless Health Canada takes that approach, I think we are going to run in circles for a long time.

To answer your question, I think there are models, but we have to put the framework on Health Canada to embark into 21st century medicine. As I said earlier, I think the processes now are very antiquated.

Thank you.

**The Vice-Chair (Ms. Joyce Murray):** Thank you.

Ms. Ouellette, can you speak to Ms. Leslie's question?

[*Translation*]

**Ms. Gail Ouellette:** To answer Ms. Leslie's question, I would say that we should expand on the second point in the letter to the minister; this is establishing a national plan for promoting the prevention, diagnosis and care of rare diseases.

I would like to give some examples from our patient associations and from our information and support portal. Many people call us or write e-mails to us to find out who the specialists in their diseases are in Quebec and in Canada. The situation is worse for people who live outside urban areas. Those people see a family doctor or perhaps a specialist once a year, but the doctor and the specialist do not seem to have in-depth knowledge of the rare disease in question. So the patients want to know if there is a specialist in Canada for that disease.

Patients will often find specialists outside the country through the Internet. I will give you another concrete example. A mother called us because her child has a chromosomal abnormality. She lives outside an urban area. She received the diagnosis for her child. She was told that nothing could be done for this chromosomal abnormality, the symptoms would be treated and she will be sent back to her region. On her own, she found a specialist in cytogenetics in France to help her. But there are cytogeneticists in Canada and in Quebec and she communicates through e-mail with a specialist from France. She even hopes to go there. It is absurd. All because we have not put our specialists in a directory for Quebec and all the provinces in Canada. We have expertise, specialized clinics and multidisciplinary teams for some diseases. We do not have enough, but the ones we have are not even in a directory.

Doctors have a hard time finding the resources, such as the resource centre for Fragile X Syndrome in Ontario, the clinic for tuberous sclerosis in Sainte-Justine or the clinics for Marfan syndrome in several provinces. They do not even know where to look for them. In Europe, and even in the United States, directories were created by patient organizations. In Europe, there is a tool called Orphanet where all that information is recorded for each European country.

So, a national plan that includes the creation of a directory would be very beneficial for care and early diagnosis. Shared practical guides, exchange and collaboration between health professionals would provide Canadians with better care. There are few patients in each province. There can never be a specialized clinic in every province for each of the 6,000 diseases, but there could be specialized clinics at the national level.

There could be what they call in Europe cross-border care, or if not, there are technologies like telemedicine or other ways to share knowledge. That is what patients are missing in our health care system, which is largely designed for common diseases.

If we had such a plan, all Canadians could benefit from it because the delay in receiving a diagnosis would be reduced, there would not be inappropriate treatments and we would not reach a critical point in care for those patients. There could also be support and social services that could help them, which would also lighten the burden on our health care system.

● (0825)

Almost 3 million Canadians have rare diseases, and many have serious, debilitating and fatal illnesses. We could take some of the weight off our health care system if we had a more standardized approach. It would already be a step forward to have them recognized nationally, with a definition of rare diseases that the provinces could follow. Working effectively together, we should adopt a plan that each province could implement individually without it being considered interference in their health care system.

Thank you.

● (0830)

[*English*]

**The Vice-Chair (Ms. Joyce Murray):** Dr. Carrie.

**Mr. Colin Carrie:** Thank you very much, Madam Chair.

I want to thank the guests today.

You've really opened my eyes on a few of these issues, particularly that three million Canadians suffer with these conditions. It certainly doesn't sound rare. I guess individually it's rare, but it's a significant part of our population.

I like what's been said around the table—I think Peter brought it up first—about the federal challenges on the regulatory issues.

We talked about drugs in different provinces and how it seems to be a patchwork type of thing.

In a previous parliament, one of our Liberal colleagues, Mr. Bell, had a motion. I think Madam Bennett would probably know. Some of the things we're talking about, considering internationally accepted standards and how Health Canada's work on a progressive licensing framework could provide appropriate support for the design of clinical trials for small numbers.... My understanding is that Health Canada started an initiative and began meeting with the provinces. Of course, the motion was dissolved when Parliament was dissolved, but my understanding is that the work continues.

However, I wanted to perhaps get Madam Coleman and Madam Wong-Rieger to comment. This is obviously something you've probably followed. My understanding is that some of the provinces have lost interest as well. Could you tell us about that?

Madam Wong-Rieger, you mentioned that we're close and some of the work has been done. You talked about modernizing the regulatory framework and harmonizing the regulatory framework. Where did the process end? What did you learn from it? What happened to it? Could you bring us up to date?

As Madam Bennett said, we should work on seeing what we can do to get things done. What happened there?

**Dr. Durhane Wong-Rieger:** We were certainly very pleased when Mr. Bell brought forth the motion. As many of you will know, Mr. Bell had a grandson who had a rare disorder and who unfortunately passed away from his disorder a couple of years ago, in fact, before the motion was actually introduced. He continues to speak and work in the area of rare disorders. The motion had called for a report to give us a status in terms of rare disorders in Canada. Unfortunately, when Parliament dissolved, the report was not in fact continued.

We know there is work on the regulatory front. I hope I'm not speaking out of turn or getting anybody in the bureaucracy in trouble by saying that we know work is taking place. We were very pleased because we had an opportunity to provide some input into that work.

I think if you were to ask for a status report in terms of the drafting of a regulatory framework that's taking place right now, you would be pleased, because we are very pleased. We think there are some very good people who have put together a regulatory framework in terms of what the Europeans have and what the U.S. has. It's the best of the best, I would say.

If we had it implemented today, we would be far ahead of the game. We could put ourselves in a position so that when Peter, Gail, and I go to international meetings and say we're from Canada and we deal with rare disorders, we could speak proudly. I think we sometimes duck when we're there. We honestly do.

I think it's very close. Work has been started. I think it needs some official recognition in order to move forward. There's a lot that's been done.

**Mr. Colin Carrie:** Could you update us on the federal, provincial, and territorial aspects of it?

**Dr. Durhane Wong-Rieger:** Yes. That's a whole different issue.

● (0835)

**Mr. Colin Carrie:** It is a different issue. It is important.

**Dr. Durhane Wong-Rieger:** The federal-provincial-territorial aspect that I think Dr. Bennett started to allude to was the whole issue around what happens once a drug has been approved, some of the collaboration around making it available to patients. As we've always said, the funding of medications and the funding of even treatments and other diagnoses are pretty much provincial jurisdictions, and I think that part has not in fact continued to progress.

I would say that, unfortunately, part of it is a debate that patients have no appetite for, and that is the question of who is going to fund it. At the end of the day, Canada is going to fund it. And as we say, whether it comes out of our left pocket or whether it comes out of our right pocket, we as patients don't really care. We really urge the feds, the provinces, and the territories to get together, because we need to have you all at the table in order to do a national plan.

We, as the Canadian Organization for Rare Disorders, have been going around the country hosting patient consultations to talk about how to get better access. We've been educating patients on what the regulatory framework is, what the funding framework is, so they can provide input. We have been very pleased that whenever we held our round tables, we've had the feds and the provinces at the table, so we know you guys can get along with each other. We do know that can happen. And we do know that at the end of the day you have a shared vision.

We want to say that we need to have you talking about, as Gail said, whether there is a review process that you folks are actually intimately part of with the Canadian Agency for Drugs and Technologies in Health. I think you fund 75% of that body. Give a nod to them to get on with coming up with a mechanism for the approval of funding for drugs for rare disorders, for which, as you said, the recommendations can be used nationally, so we don't have different provinces coming up with different solutions.

We were talking about centres of excellence and centres of expertise. Yes, you've shown that you can make those kinds of strategic plans. You've done it for cancer, you've done it for cardiovascular disease, and you've done it for diabetes. You've put together these national strategies and said you will provide the guidance, the leadership, and the support for provinces to work together collaboratively to make it happen, and to work with the institutions—

**The Vice-Chair (Ms. Joyce Murray):** Excuse me, Ms. Wong-Rieger, I have to interrupt because we're running toward the close of the meeting.

Ms. Coleman would like to make a comment, and then we'll have time for one last question. I ask the guests to keep their responses brief for the rest of the meeting.

Thank you.

**Ms. Maureen Coleman:** I would like to endorse everything Gail has said and everything Durhane has said, because in regard to networks of expertise across the country, what we lack is physicians who are aware of what is available in Canada or anywhere in the world. If somebody goes to a GP and.... For instance, it took ten years for me to be diagnosed. It takes the average person between five and ten years to be diagnosed. You run around to all kinds of people, but they don't know where to start looking. They don't suspect it, so they can't detect it.

So definitely we should have some kind of registry of physicians, and the existence of centres, the creation of specialized centres as well in Canada to treat our particular cancer is absolutely imperative.

**The Vice-Chair (Ms. Joyce Murray):** Dr. Carrie had a specific question about your perspective on the process of developing the framework and what your observation was as to where that went and what happened.

**Ms. Maureen Coleman:** Yes, sorry. When you talk about a framework, are you talking about a national...? Could you explain again, please?

**Mr. Colin Carrie:** From the comments I heard around the table, my point was that I think they have been made in the past. One of our colleagues, Mr. Bell, brought forth a motion, and my understanding is that Health Canada did start some collaborative talks with the provinces to see what we could do in that regard. I was wondering if you've followed that along and what you thought of that process.

What is your interpretation of what happened to it, how it fell off or it didn't fall off? I do understand, from what Madam Wong-Rieger was saying, that some of it has continued.

**Ms. Maureen Coleman:** Yes.

Well, Don Bell did speak at our international conference in Vancouver, where we had 35 physicians and about 230 attendees in Vancouver on May 14 and May 15. On May 14 he actually spoke for about 20 minutes, and he talked about his grandson and he talked about the process. He hoped that we would continue developing a rare disease policy in Canada. He is no longer in Parliament, but he would very much like to see this continued.

**Mr. Colin Carrie:** Do you think it would be a good idea, as Madam Wong-Rieger mentioned—

**The Vice-Chair (Ms. Joyce Murray):** Thank you. Due to a lack to time, we have one more round of questions.

Dr. Bennett.

**Hon. Carolyn Bennett:** I don't mind sharing my time with Dr. Carrie.

Obviously there are a number of pieces that come together. The people from the NeuroEndocrine Tumour Society and the Organization for Rare Disorders need to come together with a plan and put it in a letter to the minister. I hope that, *l'esprit d'escaliers*, anything you think about after you've left you make sure we know.

I would like to know from Dr. Urbain what it would it take to get yttrium and lutetium approved. Why is that in a special category compared to all the other isotopes we use?

● (0840)

**Dr. Jean-Luc Urbain:** Carolyn, it comes back to the three As I mentioned: availability, affordability, and accountability. I think it's very important that Health Canada develop processes, such as products like yttrium and lutetium, or that other medications be approved based on European clinical trials, for example. There's no need to reinvent the wheel.

**Hon. Carolyn Bennett:** Is that a regulatory change?

**Dr. Jean-Luc Urbain:** A good example is that last week a company requested that a generator be imported to Canada from Israel. It was denied by one branch of Health Canada. They sent the company to the special access program. The special access program is limited to physicians, not to companies. So basically they were run in circles.

**Mr. Peter Brenders:** To answer Dr. Bennett's question on whether it is a regulatory change, the answer is yes. We know there are a number of examples they're working on, which Durhane talked about, in terms of how you can define the product, how you would treat it, what kind of evidence and data you'd need to look at, and what type of protocol assistance could be provided.

These programs can be put in place; they're put in place elsewhere in the world. And it could be quite straightforward. It just takes interest and the will to actually do it. It takes someone to say Health Canada has great leadership.

For example, in terms of what goes in the letter, I was just reading CORD's plan for rare disorders. Your letter has her three points right in there. It's:

> Develop and implement a regulatory framework for orphan drugs and rare disorders, similar to those of the European Union....

That's the regulatory piece. We can add those supplements into the process, which would allow for easier introduction of new technologies for patients.

**Hon. Carolyn Bennett:** I think what I'm hearing, though, is that the feds have to do it in concert with the provinces. Whatever it approves, the provinces get stuck with the invoice. It's easier if you go forward together.

The other piece is the education piece. If you have a plan for rare diseases that would include educating family physicians, that needs to be in the letter too.

**Mr. Peter Brenders:** For sure. It speaks to her third point here:

> Establish a Canadian Plan for Rare Disorders that is based on international best practices....

That speaks to the network you build to share that expertise. And whether it's Health Canada centred, as in other areas, for instance, a committee for orphan products or rare disorders, or whether you're building it through our institutes or health research, it's a way to coordinate it.

It is federal-provincial, but from the regulatory point it's federal leadership. The provinces need to know we're all talking about the same thing. Today, across the country everyone is defining it differently, and it is creating a lot of confusion. That's where part of the problem comes in as well.

**The Vice-Chair (Ms. Joyce Murray):** Dr. Urbain and then Dr. Carrie.

**Dr. Jean-Luc Urbain:** I have a very quick comment. I think federal leadership is critical. Let me give you the example of London, Ontario, which has a neuroendocrine tumour program that patients were going to from everywhere in Canada. Now it's prohibited from doing that. We cannot see patients from out of province because of the funding issue.

I think the need for a centre of excellence is absolutely critical, and that has to come from the federal level, particularly for rare disorders.

Once again, this is a huge opportunity for Canada to redefine its health care system in the 21st century.

**The Vice-Chair (Ms. Joyce Murray):** Dr. Carrie.

**Mr. Colin Carrie:** I was just going to suggest earlier, Madame Coleman, that Madame Wong-Rieger mentioned there is good work going on. Her suggestion, I thought, was a very good one. Perhaps the committee could ask where that collaborative work ended off with Mr. Bell and get an update on what's happened so that we don't have to reinvent the wheel.

**Ms. Maureen Coleman:** I would be very interested in that.

**The Vice-Chair (Ms. Joyce Murray):** Thank you.

I really appreciate, to all of the guests, all of the suggestions, experience, and wisdom you have brought forward.

The meeting is over. I know that committee members will be here for the next few minutes to continue those conversations. Thank you, again, for taking the time to come and have a round table with us.

The meeting is adjourned.

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