

ALS Society of Canada

Speaking Remarks, Standing Committee on Health

INTRODUCTION:

- Mr. Chairman and honourable members of the Standing Committee on Health, thank you for the invitation to appear before you today.
- We appreciate this opportunity to address barriers to access to therapies, it is a reality our community is dealing with right now and a growing risk we see for the future.
- I am here representing Canadians affected by ALS.

ABOUT ALS:

- Carol Skinner is a young woman and a strong advocate for the ALS Community, some of you have previously met Carol. We invited Carol to be a part of today's proceedings, but she had to decline and sends her regrets. You see, ALS is a terminal illness, robbing Carol of her mobility, ability, and independence.
- To be here today...
 - Carol needed her personal support worker to come in extra early to attend to daily routines that most of us take for granted... brushing our teeth or putting on our clothes
 - Tasks further challenged by the early meeting time and muscles that do not respond as readily as it might later in the day
 - Carol's husband Travis, needed to take the day off work to physically support her attendance, resulting in lost wages in a family already burdened by a costly disease
- Carol's reality underscores that ALS is not just a terminal, neurodegenerative diagnosis for 3000 Canadians who are currently living with the disease. This disease impacts many more people. The emotional, physical and financial costs are devastating. And each year, 1000 more Canadians will be diagnosed, and they and their loved ones will find themselves on the journey with a disease that in the course of 2 to 5 years, will take away the person's ability to eat, speak, move and eventually breathe as the motor neurons die. An equal number of families will mourn the death of their loved one from this relentless disease.
- Only 5 to 10% of the people have a hereditary link, and in those families, the disease devastates in each generation. For the rest of us, we each have a 1 in 400 chance in the course of our lifetimes of receiving this diagnosis.
- The current situation, in which there is almost nothing in the way of therapeutic options, leads to urgent desperation, as people consider any options that provide hope, including those that place them at greater risk and financially drain their resources and challenge the healthcare system to support the fallout.

- With this context, for the remainder of my remarks, I ask that you consider what it would be like to be paralyzed by ALS, try not moving a muscle for the next eight minutes.

RECOMMENDATIONS:

As we consider access to therapies, we have three recommendations for the committee to consider. They are:

1. Create an environment that makes Canada a country of choice for new therapies, from research and development and clinical trials through to new drug submissions.
2. Coordinate, streamline and increase transparency – associated with the processes and timelines that enable patients to access drugs, both before and after market access is granted.
3. Work with provinces and territories to address the inconsistencies that currently result in inequitable access across Canada.

GUIDING PRINCIPLES:

These recommendations are grounded in the following principles:

1. **Equity.**
Every Canadian should have equitable and consistent access to high quality treatments that are appropriate to their individual needs.
 2. **Timeliness of access.**
Canadians should be able to access the treatments they need in a timely manner.
 3. **Affordability.**
All Canadians should be able to afford both the treatment and the equipment needed to administer the treatment.
 4. **Patient partnerships.**
As a member of the Canadian Organization for Rare Disorders, who you will be hearing from next, we broadly support the call for extensive stakeholder engagement and a strategy for the management of rare diseases.
- These recommendations and principals are a result of the experiences in our community and our engagement with organizations like Canadian Organization for Rare Disorders (CORD) and Health Charities Coalition of Canada (HCCC).

BARRIERS:

- I would like share with you some of the specific barriers faced by Canadians living with ALS when it comes to access to treatment and drugs.
- These are just a few examples, but they are far from the only ones.
- Norm's situation demonstrates **the lack of a streamlined process in clinical trials and pre-market access**, which results in physical, emotional and system costs.
 - Norm participated in a clinical trial in which there were no adverse events. Once the clinical trial had concluded, the company was willing to provide the drug, but an application had to be made to Health Canada for an open label extension.
 - The process of approval took over 6 weeks. While this may not sound like a long time, for someone living with ALS this could mean the difference between being able to still walk or use their hands or even speak.
 - During this gap in treatment, Norm's disease progressed with loss of function, resulting in two significant falls, causing injuries that required hospitalization including epidurals to manage the pain from a back injury.
 - Finally, after having to act as a liaison between two different decision makers that were not directly communicating, Norm was granted an open label extension.
 - When safety of the therapy is not a concern, this type of delay and regulatory inefficiency is not acceptable as it creates uncertainty and unfairly and unnecessarily impedes access to therapies.
 - In comparison, in the US, when the FDA approves a clinical trial, as long as there is no safety signal, the open label extension is immediately available following the conclusion of the clinical trial protocol. This process avoids a potential gap in treatment, which in Norm's case, caused injury and the lost function cannot be regained.
- **Right now, we are also seeing how the lengthy timelines and lack of transparency in Canada's regulatory and reimbursement processes affect patient access**
 - In May 2017, the FDA approved Radicava as a treatment of ALS in the United States.
 - After considerable lobbying of the company by patients, ALS Canada and Health Canada, it took almost an entire year, until March 2018, for the same drug to enter the Canadian regulatory process.
 - Currently, the drug is under priority review by Health Canada and given the 180-day timeline, a decision is imminent.

- In the 17 months since the FDA approval, those in our community who can afford to do so, have utilized Health Canada's Personal Importation process and paid out of pocket to import the drug. This method of access is not in the spirit of equitable access within a universal healthcare system and it has put people at risk.
- We are also very aware that even if a Notice of Compliance or Notice of Compliance with Conditions is granted by Health Canada, it will not mean the treatment is immediately covered through public drug plans.
- We expect CADTH - Canadian Agency for Drugs and Technologies in Health may provide reimbursement recommendations to provinces and territories by the end of the year. And it will be some time after that before actual decisions are made.
- With no defined timeframe and no transparency in the process, companies may find the lack of clarity is not worth the business risk to consider a Canadian marketplace which means Canadians will not have access to new therapies.
- In the 180 days of Health Canada's priority review period, 500 Canadians will have died of ALS. How many will die awaiting a CADTH decision? And after that, how many will die awaiting availability through publicly-funded drug programs. We are dealing with a community that measures time by loss, of their own function and by the number of members who will die during a process.
- Access issues do not end once the patient has the drug in hand – **it ALSO means creating a system where patients can have the treatment administered equitably, regardless of where they live or their financial means.**
 - Even though many in our population were able to access the recent drug through their own initiative, many faced challenges getting it infused.
 - This drug requires administration through an IV, 10 days out of 14, followed by 14 days without, and then the cycle begins again.
 - Provinces had different policies and approaches to managing the infusions and many people had to pay an additional cost to have the drug infused by private clinic or nurses.
 - In some provinces the situation was so dire, when the health care system wouldn't support the drug administration, people had the drug sitting on their kitchen table, but were on Kijiji looking for anyone who would agree to infuse. This put an already vulnerable and desperate population in a risky situation that could end of having a higher cost to the healthcare system due to adverse events.

- Our concern as we look to the future is that even with the pCPA process designed to establish a consistent funding approach across the provinces, we will continue to see differences in not only reimbursement decisions, but in standards of practice.
- Of course, a new, effective treatment cannot improve the health outcomes of Canadians **if the drug is delayed in coming to Canada or ultimately does not launch in the Canadian market at all.**
- It is easy to dismiss this concern by saying that it is up to the drug manufacturer to decide if they want to include Canada in their market access plans. But if innovative treatments do not come to Canada at the same time they launch in other countries, we will have a system that is greatly at odds with the concept of universal healthcare.
- Canada – with its relatively small population – must become a more competitive player in attracting manufacturers to bring their therapies here throughout all stages of the therapeutic pipeline.

CONCLUSION

- The examples I have shared with you today are based on limited experience over the last 18 months. But this has also given us a real-time window in which to identify and consider the barriers that a rare disease population encounters.
- There are more ALS therapies on the horizon. We do not wish to see the challenge of the last 18 months repeated as other therapies come forward. We cannot leave a desperate and vulnerable population without hope, when they can see it across a border, but don't have the physical or financial means to access a therapy that could save their lives.
- One thousand Canadians are dying each year of ALS. How many more must die before our Canadian healthcare system responds to the needs of the Canadians who are unfortunate enough to be diagnosed with this disease?

QUESTION AND ANSWER PORTION:

Mr. Ben Lobb: I'd like to ask Ms. Moore a question in regard to the clinical trials. Any member of Parliament who's been doing this long enough, whether it's in your social circles or out and around, we've all met people who have had ALS. It's terminal, and it's pretty tough to see that take place. When it's in a situation like that, what do we have to do to get it from six weeks to virtually immediate?

Ms. Tammy Moore: It would be a simple alignment, the same as what they have with the FDA in the U.S. When the clinical trial protocol is approved, it automatically gets open label extension. Unless there has been an adverse event, or something to signal safety issues associated with it. There should be no reason that a separate application has to go through for an open label extension once the clinical trial protocol has been met. It seems like a simple solution, I'm not certain.

Mr. Don Davies: Thank you. Ms. Moore, ALS Canada's website says that, "There are no significant sources of Canadian ALS research funding other than ALS Canada, resulting in a need for directed support from other sources like the federal government." How much funding does the federal government currently direct towards ALS research?

Ms. Tammy Moore: There is nothing specifically directed towards ALS research in Canada. We were fortunate in the past to be able to secure matched dollars through the Canadian Brain Research Fund with Brain Canada in partnership, but those were matched dollars, and the only reason we had those dollars available was because of the ice bucket challenge. Other than that, we are a Cinderella disease, and we can't get out of the cycle of that loop.

We provide for gaps in the healthcare system. Our societies across the country work in a very strong collaborative model, and we are filling a gap within the provincial healthcare systems to provide hospital beds, wheelchairs, ceiling lifts, ramps and things so that people can stay in their homes—and out of the healthcare system—where they are best cared for. As a result, we're grassroots fundraising, and we're now back to the fundraising levels we were at prior to that one anomaly, so we are back to about \$2 million that we have that we can direct towards research. That \$10 million that we had secured from Brain Canada wouldn't be the same opportunity that we have going forward, because we don't have a massive pool to draw from.

Aside from that, our researchers have the opportunity to apply to programs like CIHR, but because we are a relatively small population, both in terms of number of Canadians living with ALS as well as the number of researchers, we have a very small opportunity for success within CIHR, and so once again, it becomes this Cinderella disease. How can we possibly break this cycle using population-based research funding models, or population-based research support?

I would even challenge, as we're talking about the support of clinical trials, we're talking about registries. The Canadian Institute of Health Information does not get down to the level of ALS when looking at a neurodegenerative disease, so the data collection on ALS within Canada is done by societies like mine in a partnership across the country where we have donor-funded volunteer-based organizations in P.E.I. with three volunteers supporting the people living with ALS in their province, who are trying to help collect data to support advocacy efforts and to support clinical trial information. We need other systems in place to support rare disease and diseases like ALS.

Tammy Moore, CEO ALS Canada

Thursday, September 27, 2018 - 9:45 a.m. to 10:45 a.m.

Ms. Sonia Sidhu: Thank you, Mr. Chair. Thank you for being here and thank you for your advocacy. I have one question. I know families who suffer with ALS. Can you tell me, are they going through any social isolation? We heard barriers, we heard challenges. What kind of support system is there? Are there any support systems out there? Anyone can answer.

Ms. Tammy Moore: Absolutely. Thank you very much for your question. Yes, you can imagine that as a loved one becomes gradually paralyzed, there are the very physical mobility aspects around it and that is where an organization like ours—and in your riding it would be our organization—would help to fill a gap left by the health care system. These people are in their homes and what is happening is that as they're losing their independence, it's requiring supports, as I mentioned, hospital beds, wheelchairs, ceiling lifts, things to be able to get people in and out of their homes even. If those aren't in place, then they're even further isolated, but again, as their care needs increase, our health care system isn't keeping up with that, so often a caregiver, someone within the family, will have to stay home. Mr. Davies had asked about the costs associated with it. We had done a study and we know that the costs associated with ALS is between \$150,000 and \$200,000 in the course of the two to five years that someone will live with this disease. We're talking about loss of income as well. The social isolation becomes even more significant, as well as the financial constraints, so our population is making very hard decisions: Do I bother modifying my home for the six months I'm going to enjoy it that I'm going to have to take out of the equity in my home, out of my child's education fund to be able to support this or, unfortunately, will I have to make other choices about how I'm going to live out my final days as a result of those hard financial considerations. So social isolation is an important aspect that we also help to support. Again, we are a donor-funded organization that has people in our communities around the province. We work hand-in-hand with the ALS clinics, but once somebody is diagnosed, they're immediately signed up with our societies and our people will go to their homes and help them to start navigating their journey. We're providing psychological support groups so that they're able to be with people who understand what they are going through. There are many supports like that that we have in place to be able to, but we're a donor-funded organization. Should a charity have to fulfill this role in our society?