



## **BRIEF FROM THE ALS SOCIETY OF CANADA**

August 2018

### **RECOMMENDATION:**

That the Government of Canada invest \$35 million over the next 5 years in CAPTURE ALS, a groundbreaking, inter-disciplinary, foundational project that will build capacity for research internationally and across Canada.

## ALS Canada

The ALS Society of Canada (ALS Canada) is pleased to participate in the pre-budget consultation process and appreciates the opportunity to share with the members of the House of Commons Standing Committee on Finance our thoughts on ensuring Canada's competitiveness. This submission presents a way the federal government can help Canada to compete internationally as a country of choice for pharmaceutical investment and health research, thereby encouraging economic growth in the Canadian market.

## CAPTURE ALS

**CAPTURE** (Comprehensive Analysis Platform To Understand, Remedy, and Eliminate) **ALS** is a foundational project that will leverage a \$35 million research investment over five years to allow researchers around the world to study underlying disease pathology in order to develop personalized medicine and determine drug efficacy in different patients. This project will make Canada a key contributor to landmark medical discoveries and Canadians one of the first beneficiaries.

The project, and the development of infrastructure involved, will build upon the unique capacity and collaboration within Canada and amongst international researchers that has been fostered in the ALS community. It will solidify Canada as a leader in strengthening open channels of communication and data sharing amongst researchers and academic institutions (open science), as well as a country of choice for future neurological research and development.

CAPTURE ALS will connect to and enhance international research initiatives, accelerating the development of treatments. It also provides a means to build on existing research investments by agencies like the Canada Brain Research Fund (supported by federal funds from Health Canada) to their full potential, elevating and expanding them to have true impact.

In addition, CAPTURE ALS will provide open sources of data and resources that:

- Provide Canadian and international scientists unprecedented access to the biological data of people living with ALS.
- Increase and accelerate the development of treatments for ALS, making clinical trials more safe, efficient, and effective for Canadians.
- Provide Canadian researchers with competitive advantages by providing biological resources and imaging infrastructures.
- Increase Canada's impact on developing effective treatments for ALS and other diseases.

With a unique, multi-cultural population, a strong health care system, and a collaborative research community, Canada is poised to play a leading role in the design of research initiatives like CAPTURE ALS that will support the country's industry and innovation. The infrastructure of the project is applicable to many types of disease groups and will provide a model for researchers and health care.

## **Ensuring Canada's Economic Competitiveness**

CAPTURE ALS will be led through the Montreal Neurological Institute, which is working towards becoming the world's first open science research institution. It will be delivered through six ALS clinics at institutions across Canada, with ALS experts who are committed to starting now. This provides the Government of Canada not only an opportunity to support current ALS research, but the chance to build the future competitiveness of Canadian researchers and health institutions across the nation.

Neurological disease is a growing economic burden on society that will be increasing to unmanageable levels in the years to come<sup>1</sup>, creating the vital need for disease-modifying therapies. The implications for neurological research, combined with the cross-Canadian, collaborative, open science structure of CAPTURE ALS, builds the foundation to make Canada a country of choice for global pharmaceutical companies to run their trials and extend their economic footprint in the Canadian market. This will also ultimately create an environment where Canadians will have earlier access to groundbreaking treatments both in trial and through Health Canada applications.

It also leverages existing partnerships amongst various sectors of the Canadian economy, including the charitable sector, leading research institutes and health clinics, creating a platform primed for expansion with the support of private sector partnerships and funding.

Lastly, a person diagnosed with ALS today faces a tremendous financial burden: the disease has an average cost to a family of \$150,000 to \$250,000.<sup>2</sup> In addition to costs associated with treatment, care and equipment needs, there is also income lost when patients and family caregivers stop working. When the causes of ALS are better understood, and treatments developed, ALS will no longer result in a large loss of productivity.

## **Why ALS? Why now?**

Amyotrophic Lateral Sclerosis (also known as ALS, Lou Gehrig's disease, or motor neuron disease) is a terminal disease that gradually paralyzes people because the brain is no longer able to communicate with the muscles of the body that we are typically able to move at will. As the connection with muscles of the body breaks down, someone living with ALS will lose the ability to walk, talk, eat, swallow, and eventually breathe. Approximately 3,000 Canadians are living with ALS at a given time.

Each year approximately 1,000 Canadians die from ALS. With no cure and few treatment options available that have a significant impact on the progression of the disease, 80% of people with ALS die within two to five years of diagnosis. CAPTURE ALS is an opportunity to change this devastating reality.

ALS Canada's vision is to see a future without ALS. This vision can only be realized through a commitment to research. Most of the leading researchers and clinicians in the field believe we are getting significantly closer to understanding how ALS is caused, which will ultimately lead to treatments for this fatal disease.

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<sup>1</sup> GBD 2015 Neurological Disorders Collaborator Group. Lancet Neurol. 2017 Nov;16(11):877-897

<sup>2</sup> Gladman, M. et al. Amyotrophic Lateral Sclerosis and Frontotemporal Degeneration, 2014 Sep;15(5-6):426-32.

While this is encouraging, more needs to be done to find the answers. And given the rate of disease progression, understanding the disease requires gathering information from individuals while they are still living. Unlike chronic, non-terminal diseases, grassroots fundraising cannot support a necessary project like CAPTURE ALS. Even the funds raised through the Ice Bucket Challenge were only enough to support creation of the pieces that now need connecting through CAPTURE ALS and the ALS research community is in danger of losing the momentum gained from that incredible initiative, as funding returns to pre-Ice Bucket Challenge levels this year.

### **ALS and Frontotemporal Dementia (FTD)**

Funding CAPTURE ALS will allow the government to act on the National Dementia Strategy, established in 2017. Almost half of people living with ALS will develop some level of frontotemporal dementia (FTD). Understanding and treating ALS will undoubtedly create or assist in creating treatments for FTD.

Frontotemporal dementia (FTD) is one of the most prevalent types of non-Alzheimer's dementia. It primarily affects the frontal and temporal lobes of the brain, the areas most associated with personality and behavior. Common symptoms include behavioural changes, social understanding, difficulties with speech and language, and other neuropsychiatric disorders<sup>3</sup>. It is estimated that up to 50% of people with ALS demonstrate some signs of FTD, 35% develop strong symptoms, and 15% show a clinical diagnosis of pure FTD alongside their ALS. The connection between the two diseases is not fully understood yet, but there is significant and irrefutable research linking the two, and CAPTURE ALS will be critical to this understanding.

On June 22, 2017 the House of Commons passed Bill C-233, *An Act respecting a national strategy for Alzheimer's disease and other dementias*, which calls on the government to develop a multifaceted approach to fighting Alzheimer's disease and other forms of dementia. CAPTURE ALS will significantly benefit FTD research and will help accomplish the Government's goal.

### **M-105**

Parliamentarians witnessed firsthand the tremendous impact of ALS following the diagnosis of the Honourable Mauril Bélanger, MP who died less than a year later. As a result, there was considerable momentum for ALS awareness and on April 5, 2017, the House of Commons adopted Private Members Motion M-105, stating:

*"That, in light of the death of over 1000 Canadians each year, including the tragic loss of the Honourable Mauril Bélanger, former MP for Ottawa—Vanier on August 16, 2016, as a consequence of Amyotrophic Lateral Sclerosis (ALS), the House: (a) reiterate its desire and commitment to, in collaboration with provincial and territorial stakeholders, combat ALS via research and awareness; and (b) call upon the government to play a leadership role in supporting ALS research, and to support national efforts to find a cure for ALS at the earliest opportunity."*

CAPTURE ALS is the national effort to find a cure for ALS and for the government to play a leadership role in supporting ALS research. The project does so by building capacity to ensure the competitiveness of the Canadian health sciences industry, helping Canada become a leader in innovative, interdisciplinary and international research.

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<sup>3</sup> Strong MJ et al. Amyotroph Lateral Scler Frontotemporal Degener. 2017 May;18(3-4):153-174.

## Additional Details

Understanding the nuanced differences (or heterogeneity) of human ALS remains one of the last significant barriers for global ALS research and one that must be overcome in order to effectively treat ALS. Yet, Canada has the scientific infrastructure, innovative environment and world-renowned research talent needed to study this now.

**CAPTURE ALS** is a project that will use cutting-edge technology and foundational elements to capture the unique biological elements of individual people with ALS before the disease takes their life. Unlike traditionally funded, hypothesis-driven research projects, CAPTURE ALS guarantees meaningful and impactful discoveries about the disease. By analyzing whole genome sequences, proteins, gene expression, epigenetics and biochemical metabolites, researchers will be able to determine a unique biological fingerprint for each person with ALS and understand how this relates to the individual's disease course, making future personalized medicine and treatments possible.

CAPTURE ALS involves four main stages:

1. **Clinical deep phenotyping.** In this stage, people with ALS across Canada will make multiple visits to one of six ALS clinics. The progress of their ALS, their rate of functional decline, and their breathing capacity will be measured. They will undergo cognitive testing and brain imaging, and blood and cerebrospinal fluid samples will be taken.
2. **Biobanking.** Blood and cerebrospinal fluid samples will be de-identified for privacy, and stored at the Montreal Neurological Institute, where they become an open science resource. At this stage, cells from blood samples will be used for creating induced pluripotent stem (iPS) cells, which can be turned into any type of cell in the body, allowing us to study the nuances of each individual's disease. Creating iPS cells from every person living with ALS effectively creates a permanent resource to study individual cases of ALS.
3. **Whole Genome Sequencing.** In addition to being used to create iPS cells, blood samples will be used for whole genome sequencing, which ultimately provides a readout of every DNA base pair making up a human. Management of the scientific processes associated with whole genome sequencing would be done by Genome Canada, a federally-funded organization with the necessary expertise and a strategic vision to advance the economic competitiveness of Canada through stronger precision medicine efforts. Whole genome sequences would be additionally shared with Project MinE, an 18-country collaboration (including Canada) to create an open science database of 15,000 ALS genomes.
4. **Analysis.** The CAPTURE ALS team of researchers will turn iPS cells into motor neurons, astrocytes, microglia, and muscle cells, which can then be studied and comprehensively analyzed using multi-omics. Multi-omics is a set of techniques that will create a biological fingerprint for the person with ALS whom donated the sample, and the big data generated will be analyzed using Canada's world leading expertise in machine learning and artificial intelligence.

## **Financial Breakdown**

The \$35 million investment covers the entirety of the CAPTURE ALS program. A more detailed budget can be provided on request.

Government leaders need to step up to invest in Canadian research and international collaboration to globally fight for a future without ALS.