Written Submission for the Pre-Budget Consultations in Advance of the 2023 Federal Budget

By: ALS Society of Canada
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RECOMMENDATION:

- The Government of Canada invests **$35 million over five years** to expand CAPTURE ALS from a pilot initiative to a world-leading platform. Expanding CAPTURE ALS will for the very first time, enable Canadian and global researchers to effectively study why people experience ALS symptoms and progression differently. This essential knowledge will help identify treatment targets, support earlier diagnosis, strengthen global clinical trials, usher ALS into an era of personalized medicine and develop Canadian infrastructure to attract further private-sector medical R&D investment.

Canada as a World Leader in Health Research

The Canadian healthcare system is highly regarded by countries around the world, as is its vibrant and innovative health research sector. Yet, more strategic investment is required to create an environment that enhances R&D investment and fuels vital scientific discoveries that will make a meaningful impact on Canadians.

The Government of Canada has a unique opportunity to play a major role in the global fight against ALS. **ALS Canada strongly recommends investing $35 million over five years in CAPTURE ALS**, **a world-leading Open Science platform that unites patients, physicians, and researchers in academia and industry to revolutionize the field of Amyotrophic Lateral Sclerosis (ALS) treatments**.

ALS is a devastating, terminal neurodegenerative disease that progresses with startling swiftness. It affects approximately 3,000 Canadians at any given time, and a thousand will die from ALS every year. Four out of five people living with the disease die within five years of diagnosis.

CAPTURE ALS has been designed to significantly contribute to the global effort to understand ALS and will enable Canadian and global researchers to study why people experience ALS symptoms and progression differently, aiding in earlier diagnosis of the disease. Additionally, by openly sharing the information gathered with other worldwide ALS initiatives, the essential knowledge collected by CAPTURE ALS will position Canada as a significant player in the field. It will also help identify new treatment targets, strengthen global clinical trials, bring us closer to an era of personalized medicine in ALS, and develop Canadian infrastructure to attract private sector clinical R&D investment.

Given the substantial unmet need for ALS, foreign governments are realizing the significant impact that can be yielded from investments that may be considered modest compared to most treatable diseases. For example, in 2021, the United States Congress passed a bill (ACT for ALS) to make $100 million available each fiscal year from 2022 to 2026 to fund ALS research and pathways to provide early access to investigational clinical trials. Similarly, the UK Government provided £50 million in funding for innovative ALS research.

Yet, in Canada, we continue to see no strategic investment in ALS research through the Federal Budget. As a terminal, progressive disease, ALS does not have the same capacity to raise funds as treatable diseases with a survivor population and large investment from big pharma. While current fundraising mechanisms allow for support of seed funding for innovative ALS research, without financial support from the government for larger, transformative initiatives like CAPTURE ALS, we will never reach a comparable, self-sustained cycle of funding and impact.

CAPTURE ALS is ready for this investment. It is launched, firmly established in the Canadian ALS community, and immediately scalable. In the end, the biggest thing that can dampen its impact is a lack of statistical power that can easily be overcome with financial support. Now is the time for Canada to lead in ALS.

The Problem of Heterogeneity in ALS

ALS paralyzes people because the brain can no longer communicate with the body's muscles. Over a short period, as the living wires that connect our brain and muscles, called motor neurons, break down, someone living with ALS will lose the ability to walk, talk, eat, swallow, and eventually breathe.

ALS is a very heterogeneous disease, meaning the disease varies significantly from person to person, including where symptoms first appear in the body, age of onset, rate of disease progression, the involvement of genetics and environment and much more.

In the past five years, there has been a significant acceleration of therapeutics being approved for ALS in Canada. By understanding why ALS differs in person, we have the opportunity to keep this momentum going by unlocking promising therapeutic targets and advancing new treatments effectively through clinical trials for people living with the disease.

Like cancers and other disease areas with significant treatment options, ALS needs to get to a place where new therapies can treat each person's unique form of the disease – treatments will likely not be a "one size fits all" solution. Currently, ALS clinical trials cannot effectively study individuals based on factors such as symptom onset or progression rates because there is not enough meaningful data to make these distinctions, which leaves researchers unable to match the right people with the right treatments. This prevents researchers from conducting accurate assessments of innovative therapies. The biological signatures collected through CAPTURE ALS will be used to match people for clinical trials more effectively and monitor responses to specific treatments.

CAPTURE ALS

CAPTURE (Comprehensive Analysis Platform To Understand, Remedy, and Eliminate) ALS represents a natural evolution of Canada's long-time ALS research efforts. A $35 million research investment over five years will transform CAPTURE ALS into a nationwide effort that will play a critical role in the global understanding of the disease and effectively tackle the heterogeneity of the disease, the most critical remaining hurdle in the development of new treatments for ALS.

In 2021, CAPTURE ALS was awarded a competitive $2.8 million Brain Canada Platform Support Grant, which included contributions from ALS Canada and two pharmaceutical companies. This funding enabled the project to begin as a pilot initiative in early 2022. Led by a group of world-renowned ALS experts, the pilot study will examine 100 people living with ALS and 25 healthy controls. Recruitment began earlier this year at four ALS centres of excellence in Edmonton, Toronto, Montréal and Quebec City.

However, while the pilot study will provide an important contribution to the global effort to understand and treat ALS, it is not enough to make the significant impact needed to accelerate timelines for a disease population that has an average lifespan of two to five years after diagnosis. With a $35 million investment from the federal government, CAPTURE ALS can immediately expand the platform to 10+ ALS research-ready Canadian sites and increase recruitment to 1,000 individuals with a far more comprehensive analysis of each person's disease.

How does CAPTURE ALS work?

CAPTURE ALS has merged several existing Canadian research programs into a single powerful resource for studying the disease. The platform provides the systems and tools necessary to collect, store, share and analyze substantial information about ALS, creating the most comprehensive biological picture of people with ALS ever attempted in the field. Using advanced analysis methods, CAPTURE ALS will combine all the data collected from people living with ALS and healthy individuals to identify unique subtypes of ALS and will provide a way for all patients in Canada to meaningfully contribute to global ALS research. Data collected through CAPTURE ALS represents each participant's biological 'story' of how ALS has affected them.

CAPTURE ALS will support ongoing collaboration within the Canadian ALS research community and connect to international initiatives. Through Open Science, these datasets and samples will be shared with academia, industry, and complementary consortia across Canada and worldwide. As an underfunded, terminal disease, there has never been the capacity to study people living with ALS effectively, to learn from what their bodies can tell science.

CAPTURE ALS involves three main stages:

1) **Data Collection:** Over multiple visits, data from neurological exams, cognitive and speech tests and MRI scans, as well as biological samples such as blood, cerebrospinal fluid, stem cells, and post-mortem brain and spinal cord tissue, will be collected to monitor disease progression. Information on other variables, such as risk factor exposure, socioeconomic status and race, will also be collected.

2) **Creation of Biosignatures**: Using a combination of established and cutting-edge scientific expertise, biological samples will be analyzed. Modern machine learning techniques will create a "fingerprint" or biosignature for each participant. The biosignature samples will be de-identified for privacy.

3) **Increased Impact of Future Research**: The collection of samples and data from each participant creates a permanent resource to study longitudinal (over time), well-characterized human cases of ALS, which is very limited globally. After CAPTURE ALS analysis, the remaining samples will be stored in a national ALS biorepository network to enhance future studies and harness emerging technology for future research.

Furthermore, CAPTURE ALS engages regularly with a Patient & Partner Advisory Council that helps guide the program to remain mindful of the community it serves. The success of this initiative requires collaboration from all stakeholders in the ALS community, which makes the program so unique and exciting.

Expanding Beyond ALS

Federal government support of CAPTURE ALS will contribute to many other investment areas, including rare diseases and genomics. As CAPTURE ALS is collecting biosignatures from Canadians, including whole genomes, it complements the $40 million federal investment made in the Canadian COVID-19 Genomics Network to coordinate a COVID-19 viral and host genome sequencing effort across Canada. A better genetic understanding of all Canadians will evolve these closer to effective use in medical care for the general population.

CAPTURE ALS will also complement the Government of Canada's April 2020 Strategic Innovation Fund (SIF) of $600 million to support clinical trials. CAPTURE ALS creates additional confidence in Canada as a marketplace for the life sciences sector to invest in research, manufacturing, and development of new treatments and vaccines. The platform will demonstrate Canada's experience in biocollection and storage and showcase that Canada is a favourable environment for health research investment.

Conclusion

ALS is a grossly underfunded disease with a real opportunity to be far more treatable in the near future with more support for initiatives like CAPTURE ALS. And while Canada is recovering from COVID-19, Canadians are not recovering from ALS – nor will they ever until meaningful investments are made in increasing clinical trial efficiency and accelerating ALS therapy development.

If funded by the Government of Canada, CAPTURE ALS will make a transformational difference in ALS research by fundamentally changing how new ALS treatments are developed. It will also encourage investment from the private sector and industry, enable people living with ALS to live longer and with a better quality of life, provide opportunities for people living with ALS to contribute to research and support a more robust economy by reducing the burden on our health systems. **Therefore, we urge the Government of Canada, in Budget 2023, to invest $35 million over five years in this critical initiative.**

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Appendix 1: CAPTURE ALS Financial Details

The ALS Society of Canada is requesting an investment in CAPTURE ALS totalling $35 million over five years. Please find financial details below:

ITEM	UNITS	UNIT COST	TOTAL COST
National Project Manager (federal funding to begin in 2025)	1	$120,000/year	$600,000.00
Patient Outreach Manager	1	$100,000/year	$500,000
Partnerships and Sustainability Manager	1	$100,000/year	$500,000
Grants and Communications Writer	1	$80,000/year	$400,000
CAPTURE ALS Research Coordinators and Assistants (four coordinators are covered by the existing grant until 2024)	Coordinators (15) Assistants (6)	$75,000/year $65,000/year	$6,675,000
Software and Data Team	3	$165,000/year	$2,475,000
Students & Postdocs – Data Analysis	Ph.D. student (6) Postdocs (4)	Students - $25,000/year Postdocs - $55,000/year	$1,850,000
Clinical Setup and Infrastructure (four site set-up covered by existing grant until 2024)	15 sites	$12,500/1 year/site	$787,500
Clinical Data Capture, Imaging, Biospecimen Collection and Brain Banking	900 ALS + 175 control	$6,600	$7,095,000
Whole Genome Sequencing	900 ALS + 175 control	$1,600	$1,720,000
iPS Motor Neuron and Glial Preparation, Differentiation and Omics	250 ALS + 50 control	$15,400	$4,620,000
Wearable Technologies, In-Home Assessments	900 ALS + 175 control	$3,200	$3,440,000
Biomarker Analyses	900 ALS + 175 control	$4,000	$4,300,000
Total			$34,962,500

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