Written Submission for the Pre-Budget Consultations in Advance of the 2021 Federal Budget

By: ALS Society of Canada

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RECOMMENDATION:

- The Government of Canada invests $35 million over five years to fund CAPTURE ALS, a first-of-its-kind Canadian open science platform to collect comprehensive biological data from 1,000 Canadians living with ALS. CAPTURE ALS will enable the world leading study of ALS heterogeneity, which will strengthen global clinical trials, accelerate therapeutic development, and encourage new private sector medical R&D investment in Canada.

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The COVID-19 Context

The COVID-19 pandemic has not only created an unprecedented challenge for Canada's healthcare systems, it has also affected organizations that are actively supporting vulnerable populations and Canada's health research sector. For example, Canadian health charities have seen a 50% drop in revenue due to COVID-19. At the same time, demand for services has increased and research initiatives have been put at risk, jeopardizing years of work and hundreds of millions of dollars of investment.

As a health charity that serves one of Canada's most vulnerable populations, the ALS Society of Canada appreciates the steps the federal government has taken to respond to the pandemic. Yet, there is more for the federal government to do to enable health charities to continue to provide critical services for Canadians and fund the research that will fuel vital scientific discoveries. While the federal government made a commitment of $450 million for universities and health institutes to mitigate the impact of COVID-19 on health research, this initiative fails to support the health charity sector that funds more than $155 million of research annually.

The federal government has an opportunity to take a leadership role in not only ensuring stability within Canada's health research environment but also in providing critical funding to support impactful and evidence-based research focused on developing effective treatments. In the case of ALS, without sustainable funding for ALS research there is a considerable risk of losing the opportunities and momentum achieved to date.

As the government focuses on building a scientific foundation to support research for health emergencies as the country recovers from COVID-19, ALS Canada is recommending they invest in CAPTURE ALS. CAPTURE ALS will not only improve the lives of people living with ALS, but will add to Canada's global leadership in health research, develop critical infrastructure that can be used for a variety of different disease areas, and encourage additional private sector medical R&D investment.

Furthermore, in alignment with the Health Charities Coalition of Canada (HCCC), ALS Canada supports HCCC's recommendation that following the same structures and principles of other government funding programs, the Government of Canada provide direct financial support to health charities of up to $28 million per month as health charities recover from the impact of COVID-19 so that they can continue to develop and offer programs to support patients, families and caregivers.

Heterogeneity in ALS

ALS is a devastating neurodegenerative disease that directly affects the lives of 3,000 Canadians at any given time. A thousand Canadians will die from ALS every year – and 80% of people of people living with the disease die within two to five years of diagnosis.

ALS paralyzes people because the brain is no longer able to communicate with the muscles that we are typically able to move at will. Over time 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.

Complicating matters further, the disease varies greatly from one person to another, including where symptoms first appear in the body, age of onset, rate of disease progression and the role of genetics. These varying degrees and paths of symptom progression is known in the scientific and medical community as the heterogeneity of ALS, and it is impeding the development of new treatments.

To understand ALS heterogeneity and unlock promising therapeutic targets, greater funding for ALS research is needed. Moreover, answering the unknown questions about the heterogeneity of ALS will create an innovative model that can be applied to other disease areas, like COVID-19 or cancers, and can help us more accurately diagnose, categorize patients into subgroups, and run productive clinical trials.

<ins>CAPTURE ALS</ins>

CAPTURE (Comprehensive Analysis Platform to Understand, Remedy, and Eliminate) ALS is the next frontier in ALS research and will address the heterogeneity of ALS. It is an innovative initiative that will leverage a <ins>$35 million research investment</ins> over five years for a national effort to tackle the most important remaining hurdle in the development of new treatments for ALS.

Led by a group of world-renowned Canadian ALS experts, CAPTURE ALS will be driven academically at 15 existing ALS centres of excellence across Canada. The platform will merge existing research programs into a singular and powerful resource for studying ALS data, supporting the collaboration that already exists within the Canadian ALS research community and connecting into other international initiatives to create a global network of human ALS resources and information, shared freely through open science.

CAPTURE ALS is an evolution of the Canadian ALS Neuroimaging Consortium (CALSNIC), an international research platform that facilitates multi-centre clinical and translational ALS research across Canada and the US. CALSNIC was previously funded in partnership with the Canada Brain Research Fund (with financial support from Health Canada) and CAPTURE ALS builds on this initial investment. It also integrates with the Montreal Neurological Institute's C-BIG (Clinical Biological Imaging and Genetic) Repository.

Using standardized methods, neurological, cognitive, speech, electrophysiological, and neuroimaging data along with biosamples (blood, urine, cerebrospinal fluid), will be collected over time from ALS patients and healthy controls. Through open science, these datasets and samples will be rapidly shared with academia, industry and complementary consortia across Canada and the globe. And through the convergence with C-BIG, the first open science neurological biobank in the world, it will provide opportunities to the world for access to the most comprehensive picture of a person living with ALS.

Equally important, CAPTURE ALS provides a way for all patients in Canada to contribute to global ALS research in a meaningful way. The data collected through CAPTURE ALS represents each participant's biological 'story' of how ALS affects them. Furthermore, by including data capture through wearables and virtual or in-home visits, CAPTURE ALS will thoughtfully accommodate the most vulnerable in times where in-hospital visits present greater risk of exposure to COVID-19. By providing biosamples, research participants are actively engaged in contributing to scientific efforts to understand ALS heterogeneity, which will play a direct role in informing new therapeutic targets.

CAPTURE ALS involves four main stages:

1) **Data Collection:** Over multiple visits, biological samples such as blood, urine, cerebrospinal fluid, stem cells, and postmortem 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 experiments, the biological samples will be analyzed. Modern machine learning techniques will then create a "fingerprint" or biosignature for each individual participant. The biosignature samples will be de-identified for privacy.

3) **Open Science Contributions**: The ALS biosignature data will be made available through an open science database to aid in collaboration with international ALS initiatives. Global scientists will be able to analyze the biosignatures further and add to our understanding of ALS.

4) **Promotion of Future Research**: The collection of the samples and data from each participant effectively creates a permanent resource to study individual human cases of ALS. The remaining samples will be stored in a national ALS biorepository network to enhance future studies and harness emerging technology for future research.

Accelerating ALS Drug Development

CAPTURE ALS will enable ALS researchers to collect an unprecedented amount of data and biosamples from people living with ALS. To truly understand the effectiveness of potential therapies and who might benefit from certain therapies, ALS researchers must be able to differentiate patients based on how their ALS begins and progresses. The data collected through CAPTURE ALS will be used to better qualify people for clinical trials and monitor responses to treatment, which will increase clinical trial efficiency and ultimately accelerate ALS drug development, positioning Canada as a global leader in ALS research. It will also give the Canadian and global ALS research communities the foundational knowledge to better understand the disease

CAPTURE ALS will also enable researchers to take a different approach to clinical trials based on disease heterogeneity, which will better demonstrate how disease progression is being altered, delayed, or even stopped by a potential therapy.

Canada as a World Leader in Health Research

Having access to comprehensive biological data and a centralized biorepository is of great interest to pharmaceutical manufacturers and investing in CAPTURE ALS will support an environment that will help to bring more industry-sponsored clinical trials to Canada. By supporting CAPTURE ALS, the federal government would be a leader in developing an open science data collection platform that would benefit people living with ALS both here and abroad.

As CAPTURE ALS is collecting biosignatures from 1,000 Canadians that would include genetic make-up details, 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. Better biological and genetic understanding of all Canadians will someday require these techniques to be applied to the general population. As with COVID-19, the government should start with the most vulnerable Canadians first, like those with ALS.

CAPTURE ALS will also complement the Government of Canada's April 2020 Strategic Innovation Fund (SIF) investment of $600 million to support vaccine and therapy clinical trials. As this SIF initiative is to be led by the private sector, CAPTURE ALS creates additional confidence in Canada as a marketplace for the life sciences sector to invest when it comes to researching, manufacturing, and developing new treatments and vaccines.

The platform will demonstrate Canada's experience in the biocollection and repository area and showcase that Canada is a favourable environment for health research investment, in both rare diseases and COVID-19.

Conclusion

As Canada recovers from COVID-19, CAPTURE ALS is a timely opportunity for the Government of Canada. An investment in CAPTURE ALS will elevate Canada into a world leader in the health research sector and transform the way we understand diseases, conduct clinical trials, develop new treatments for ALS and other diseases. It will reduce the burden of ALS on our health care system. It will lead to the discovery of new therapies. And it will create a more attractive market for industry and private sector investment, all the while complementing existing COVID-19 related health research commitments made by the government.

CAPTURE ALS is the best way for the federal government to better support Canadians living with ALS and we urge the Government of Canada to act on this opportunity by providing $35 million over five years toward CAPTURE ALS.

Appendix 1: CAPTURE ALS Financial Details

The ALS Society of Canada is requesting an investment in CAPTURE ALS from the Government of Canada totaling $35 million over five years. Please see financial details below:

CAPTURE ALS Financial Details

ITEM	UNITS	UNIT COST	TOTAL COST
National Project Manager	1	$120,000/year	$600,000.00
CAPTURE ALS Research Coordinators and Assistants	Coordinators (15) Assistants (6)	$75,000/year $65,000/year	$7,575,000.00
Software and Data Team	3	$100,000/year	$1,500,000.00
Students & Postdocs – Data Analysis	PhD student (6) Postdocs (4)	Students - $25,000/year Postdocs - $55,000/year	$1,850,000.00
Clinical Setup and Infrastructure	15 sites	$12,500/site/year	$937,500.00
Clinical Data Capture, Imaging, Electrophysiology, Biospecimen Collection and Brain Banking	1000 ALS + 100 control	$6,600	$7,260,000.00
Whole Genome Sequencing	1000 ALS + 100 control	$1,600	$1,760,000.00
iPS Motor Neuron and Glial Preparation, Differentiation and Omics	300 ALS + 50 control	$15,400	$5,390,000.00
Patient Oriented Research, Wearable Technologies, In Home Assessments	1000 ALS + 100 control	$4,320	$4,752,000.00
Biomarker Analyses	1000 ALS + 100 control	$3,000	$3,300,000.00
Total			$34,924,500