Written Submission for the Pre-Budget Consultations in Advance of the 2022 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, self-sustainable Open Science platform. CAPTURE ALS will enable Canadian researchers to study why people experience ALS symptoms and progression so differently. This essential knowledge will help identify treatment targets, strengthen global clinical trials and develop Canadian infrastructure to attract private sector medical R&D investment.

COVID-19 Recovery: Canada's Health Research Sector

The COVID-19 pandemic has underscored the need for Canada to invest in and maintain a strong health research sector. From responding more effectively to future pandemics, to developing treatments and vaccines, and establishing foundational knowledge of diseases – a vibrant and innovative health research sector in Canada is essential.

The ALS Society of Canada (ALS Canada) appreciates the federal government's support of universities, health institutes and the life science and pharmaceutical sectors; however, the vital role of health charities in maintaining and strengthening the health research sector has been overlooked. For example, the commitment of $2.2 billion over seven years for the life sciences sector outlined in Budget 2021 and Canada's Biomanufacturing and Life Sciences Strategy failed to consider the health charity sector that funds more than $155 million of research annually.[1]

In particular, terminal diseases like ALS are significantly affected by this lack of funding as there are far fewer alternative research funding avenues to draw from. While current fundraising mechanisms may provide seed funding for innovative ALS research projects, without financial support from the government, ALS Canada cannot continue to fuel the vital scientific discoveries that will make a meaningful impact on people living with the disease. We must be able to support transformative research that will unlock the mysteries around the causes and progression of ALS.

As the government focuses on building a scientific foundation to support health research, as outlined in Budget 2021, **ALS Canada is recommending an investment of $35 million over five years in CAPTURE ALS.**

CAPTURE ALS is a critical piece of the global effort to understand and treat ALS and is an avenue for pharmaceutical investment in Canada. Federal government support for this platform will provide Canadians with better access to promising therapies and make Canada a primary country for pharmaceutical companies to market proven treatments. CAPTURE ALS also serves as an unprecedented opportunity for Canadians diagnosed with ALS to contribute to research in a new way that allows them to offer their unique form of the disease to science. The federal government has an opportunity to take a leadership role in helping us achieve a future without ALS through the support of CAPTURE ALS.

Heterogeneity in ALS

ALS is a devastating neurodegenerative disease that directly affects the lives of approximately 3,000 Canadians at any given time. A thousand Canadians will die from ALS every year – and 80% 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 of the body. Over a short period of 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.

Science has made great strides in better understanding how ALS is caused in laboratory cells and animals, which is vital to laying the groundwork for promising new treatments. However, ALS is a heterogeneous disease, meaning the disease varies 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. Understanding why ALS is different in each person is vital to unlocking promising therapeutic targets and advancing new treatments effectively through clinical trials to people living with the disease.

[1] Health Charities Coalition of Canada. "HCCC Vision & Values" Webpage. Accessed in July 2021. Available online: http://www.healthcharities.ca/about/vision-and-values.aspx

CAPTURE ALS

CAPTURE (Comprehensive Analysis Platform To Understand, Remedy, and Eliminate) ALS represents an evolution of Canada's world-class ALS research. A $35 million research investment over five years will transform CAPTURE ALS into a national effort that will aid 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.

CAPTURE ALS recently received a competitive $2.8 million Brain Canada Platform Support Grant, which included contributions from ALS Canada and two pharmaceutical companies. This funding will enable the project to begin as a pilot initiative in late 2021. Led by a group of world-renowned ALS experts, the pilot study will examine 100 people, with recruitment scheduled to begin later this year at four ALS centres of excellence in Edmonton, Toronto, Montréal and Quebec City.

However, while the pilot study of 100 people with ALS 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 expand the platform to 15 Canadian sites and increase recruitment to 1,000 individuals with a far more comprehensive analysis of each person.

How does CAPTURE ALS work?

CAPTURE ALS will merge a number of existing Canadian research programs into a single powerful resource for studying ALS data. The platform will provide the systems and tools necessary to collect, store, share and analyze substantial amounts of 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 patients with ALS and healthy individuals to identify unique subtypes of ALS.

CAPTURE ALS will also 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.

CAPTURE ALS involves three main stages:

1) **Data Collection:** Over multiple visits, biological samples such as blood, urine, 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 well-characterized human cases of ALS, which is very limited globally. The remaining samples after CAPTURE ALS analysis will be stored in a national ALS biorepository network to enhance future studies and harness emerging technology for future research.

Equally important, CAPTURE ALS provides 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. Furthermore, by including data captured through wearable technology and virtual visits, CAPTURE ALS will thoughtfully accommodate the most vulnerable in a time where in-hospital visits present a greater risk of exposure to COVID-19.

Accelerating ALS Drug Development

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 fingerprint data collected through CAPTURE ALS will be used to more effectively match people for clinical trials and monitor responses to specific treatments.

This will increase clinical trial efficiency, accelerate drug development, provide insights that will inform personalized medicine efforts in the future, and position Canada as a global leader in ALS research. In addition, this faster therapy development process will provide Canadian and global ALS researchers with the foundational knowledge to better understand the disease.

Solidifying Canada as a World Leader in Health Research

Initial funding secured through a competitive peer-review process primes the project for immediate expansion with additional funding. A $35 million federal government investment will enable CAPTURE ALS to secure additional partnerships with the private sector and life sciences industry, providing sustainable long-term support for the platform. This will in turn not only make CAPTURE ALS the benchmark initiative for ALS research, but will brand Canada as a primary country for investment through clinical trials and marketing of new treatments.

Making Canada a leader in ALS research and destination country for industry's innovative therapies will enable Canadians living with ALS to participate in the world's best clinical trials and provide faster access to therapies. Every day matters to Canadians living with ALS, so the impact of this opportunity is immeasurable.

Finally, federal government support of CAPTURE ALS leverages many other investment areas, including rare diseases and genomics. As a result, the lessons learned through CAPTURE ALS will revolutionize the future for Canadians diagnosed with ALS and reach far beyond its primary focus.

Conclusion

ALS is not an untreatable disease – it is an underfunded disease. And while Canada recovers from COVID-19, Canadians are still not recovering from ALS. But there is hope for people and families affected by ALS. Initiatives like CAPTURE ALS can create meaningful change for people living with ALS today and in the future. However, to achieve the greatest impact, CAPTURE ALS will require partnered financial support beyond the ALS community's current fundraising capacity. This is why the federal government must play a leadership role in investing in this foundational research platform.

If funded by the Government of Canada, CAPTURE ALS will make a transformational difference in ALS research. Perhaps most importantly, CAPTURE ALS will fundamentally change how new ALS treatments are developed. But this platform will also do much more. It will 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 reduce the burden on the Canadian healthcare system. We urge the Government of Canada, in Budget 2022, to invest $35 million over five years in this important initiative.

Appendix 1: CAPTURE ALS Financial Details

The ALS Society of Canada is requesting an investment in CAPTURE ALS totaling $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	$360,000.00
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 covered by existing grant until 2024)	Coordinators (15) Assistants (6)	$75,000/year $65,000/year	$6,675,000.00
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 (four site set-up covered by existing grant until 2024)	15 sites	$12,500/site/year	$787,500.00
Clinical Data Capture, Imaging, Electrophysiology, Biospecimen Collection and Brain Banking	900 ALS + 175 control	$6,600	$7,095,000.00
Whole Genome Sequencing	900 ALS + 175 control	$1,600	$1,720,000.00
iPS Motor Neuron and Glial Preparation, Differentiation and Omics	250 ALS + 50 control	$15,400	$4,620,000.00
Patient Oriented Research, Wearable Technologies, In Home Assessments	9900 ALS + 175 control	$4,320	$4,644,000.00
Biomarker Analyses	900 ALS + 175 control	$4,000	$4,300,000.00
Total			$34,951,500