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

By: ALSAC – ALS / SLA Action Canada

- **Recommendation 1:** That the government develop a regulatory framework for Health Canada to transition from a passive system to a proactive framework, similar to Project Orbis, to approve ALS therapies for use in Canada once they have been approved by the FDA or equivalent regulators in other countries to ensure Canadians living with rare-terminal disorders have immediate access to life saving treatments.

- **Recommendation 2** That the government invest $10 million to launch a trial site for the HEALEY ALS Platform Trial in Montreal and fund an additional $5 million to expand trial sites to provinces that currently do not offer patients clinical trial options such as, British Columbia, Manitoba and Nova Scotia.

- **Recommendation 3:** That the government ensure the Patent Medicines Prices Review Board's (PMPRB) changes to the pricing Guidelines for the Amended Regulations encourage the availability of ALS clinical trials and therapies in Canada, and/or exempt ALS therapies from the new pricing rules.

- **Recommendation 4:** That the government provide $5 million to support Canadians with ALS who must travel outside Canada to access treatment and trials unavailable in Canada.

Background – About ALS / SLA Action Canada

ALS/SLA Action Canada is a Canadian patient-led initiative to improve access to innovative therapies for people living with ALS today. We believe there is, for the first time ever, hope for all people with ALS given that over 160 drug companies worldwide are working on ALS projects, with several promising clinical trials in their final stages, including one that appears to reverse the progression of ALS in some people.

Recommendation 1:

The federal government recently stated: "Canadians want access to the latest treatments for themselves, their families and their friends – especially for cancers, rare diseases and conditions with limited treatment options."

Project Orbis, an initiative between Canada, the US, Australia and other countries, currently provides a framework for concurrent submission and review of oncology products among international partners, and is an example of regulatory project that could be similarly designed for ALS treatments across multiple jurisdictions.

The Project brings together regulators from multiple countries to review cancer drugs concurrently so that patients can receive earlier access to innovative oncology medical products and further promoting greater uniformity in the standards of treatment across multiple jurisdictions.

The first Project Orbis collaborative review involved the US, Canada and Australian regulators who reviewed applications for two products and then subsequently issued simultaneous decisions in all three countries.

Achievements to date:

- **September 2019:** Canada, Australia and the US simultaneously approved two drugs as a combination immunotherapy and treatment of a specific form of endometrial cancer for patients ineligible for radiation or surgery;

- **November 2019:** Canada, Australia and the USA approved a drug to treat certain types of lymphoma and leukemia;

- **April 2020:** Canada, Australia, USA, Singapore and Switzerland approved the first new molecular entity under Project Orbis. It is a drug used in combination with chemotherapy drugs to treat patients with inoperable or metastasized advanced positive breast cancer who have received prior treatments. The FDA used its real-time oncology review (RTOR) pilot and was granted priority review, breakthrough therapy, fast track and orphan designations; and

- **June 2020:** Australia, Singapore nominated a Phase 2 drug to treat small cell lung cancer for Project Orbis consideration. Note: Project Orbis approval pending.

Our view is that since Project Orbis has proven that collaboration and cooperation among international partners is possible for accelerating the approval process for life-saving oncology treatments, it is certainly possible to develop a similar project for the ALS community and other rare disorders. With Health Canada's current engagement in Project Orbis, there is already a regulatory framework in place to help support the government's commitment to implementing a national Rare Disease Strategy.

In early June 2020, regulators from Health Canada, the FDA and the European Medicines Agency provided their respective views on issues related to accelerated approval pathways at the DIA Global

Annual Meeting. On Health Canada's participation in Project Orbis, Kelly Robinson, Director of Health Canada's Centre for Evaluation of Radiopharmaceuticals and Biotherapeutics said, "it's an opportunity … for new indications or new products to come to Canada sooner than they otherwise would," adding that the initiative also gives reviewers from the different agencies more opportunities to interact."

We would welcome the opportunity to collaborate with Health Canada to develop a regulatory framework to expedite approvals across jurisdictions to ensure that new products for ALS patients come to Canada, similar to Project Orbis.

Recommendation 2:

The HEALEY ALS Platform Trial is the first of its kind. A platform trial is an accelerated the path to new ALS therapies by testing multiple treatments at once, reducing the cost of research by 30% decreasing the trial time by 50% and increasing patient participation by 67%. Further, the HEALEY ALS Platform Trial is spearheaded by the Massachusetts General Hospital (MGH), which is recognized as the global leader in ALS Clinical experience.

On July 3, 2020 MGH and the Montreal Neurological Hospital (MNH) discussed a Memorandum of Understanding, for opening a trial site in Montreal. Direct patient correspondence with the leading neurologists from MGH and MNH have articulated that both the Health Canada submission and trial startup costs in Canada will be approximately $10 million.

24% of Canadian people living with ALS (pALS), (approximately 720 of Canada's 3000 pALS) do not have access to clinical trials within their respective province or territory. Meaningful change within the Canadian ALS landscape would see platform trial site expansion into British Columbia (where approximately 400 pALS don't even have access to a dedicated ALS clinic), Manitoba and the Maritimes. ALSAC is requesting $5 million in federal funding to facilitate this expansion and improve clinical trial access for patients across Canada.

Recommendation 3:

In a recent Letter to the Editor in *The Hill Times*, the President of Innovative Medicines Canada (IMC), Pamela Fralick stated:

> "Recent data from a range of sources confirms what Canada's innovative medicines industry has been saying for more than three years now: Health Canada's recent changes to the PMPRB will have a negative impact on patients' access to medicines, on investment in our life-sciences sector, and on the launch of new medicines and vaccines in Canada."

Ms. Fralick further articulated that "since the amended PMPRB regulations were published last August, IMC members have reported several drug-launch delays or suspensions, including for rare disease and oncology medicines.

The current Canadian drug approval process is longer than the expected lifespan of an ALS patient. Health Canada maintains a six-month review process similar to the FDA and EMA. However, Canada is not always pharmaceutical companies' first choice for launching therapies, and on average, there is a nine-month delay between a drugs' FDA approval and a subsequent application to Health Canada.

Canadian patients often watch larger markets like the US and the EU access treatment fifteen months before them. Following this, the provincial drug coverage process degenerates into a province by province approval process, decentralized and uncoordinated, failing to appreciate the life-expectancy of an ALS patient.

The fastest provincial drug coverage approval for an ALS medication to date, was 574 days in Quebec. **Between federal and provincial drug approvals, some patients wait 34 months to access new therapies.** In other words, if a scientifically validated cure is made available in the US tomorrow, and a Canadian is diagnosed with ALS the following week, that Canadian patient may not survive to access the therapy. They will most certainly have to pay for it and if they cannot afford the therapy, they, in all probability, will die before provincial coverage is made available. Canadians are fighting for the right to live.

Recommendation 4:

Canadians with ALS must incur many self-funded costs associated with identifying, locating and participating in potential treatments or therapies most often not located within Canada. The types of costs incurred include:

- Consultation with leading neurologists with international level skills and knowledge currently not found within Canada. These neurologists play leading roles in advancing ALS research and potential therapies that Canadian neurologists do not have. Neurologists in other countries have access to knowledge networks and communities that Canadian neurologists do not always have. This means that Canadians with ALS who want to get the best advice on treatment options most always have to pay for non-Canadian expertise; and

- Participation in trials inside or outside of Canada are not fully covered or not covered at all. Canadians with ALS may require the support of a caregiver during travel and trial participation that are usually self-funded. Multiple visits outside Canada for ALS trial participation is part of our lived experience to date, before COVID-19.

Conclusion

The global outbreak of COVID-19 has shone a spotlight on the importance of the research and critical investments required to bring new medicines and vaccines safely to market, and providing Canadians, specifically those living with ALS, with access to new, potentially life-saving medications and therapies.

We believe our recommendations described herein will support those living with ALS and other rare disorders as the federal government works to recover from the novel coronavirus pandemic.