SURVEY QUESTIONS:

1) **What do you perceive to be the current challenges and barriers facing expensive drugs for rare diseases?**

   ALS Canada is in a unique position to comment on the challenges and barriers facing expensive drugs for rare diseases given Health Canada's recent approval, in October 2018, of edaravone – making it one of two approved ALS drugs in Canada. We are soon anticipating the Canadian Drug Expert Committee's reimbursement recommendation. Given what we have observed and heard from Canadians living with ALS since becoming aware of the manufacturer's application to Health Canada, some of the most significant challenges and barriers are the numerous and disparate processes and timelines that factor into approval and reimbursement decisions and the lack of transparency particularly with respect to reimbursement. We also anticipate the potential for reimbursement decisions to vary by province, resulting in inequitable access.

   These challenges and barriers therefore have a direct impact on Canadians who in the case of ALS have limited treatment options. Due to the progressive nature the disease, which increasingly limits mobility as well as the ability to communicate verbally, people living with ALS often measure time by loss. Within Health Canada's 180-day priority review period, approximately 500 Canadians living with ALS died.

   Given our interest in seeing more therapeutic options available for Canadians living with ALS, we also see the opportunity to explore how improved processes related to clinical trials and pre-market access can make Canada a more appealing market for ALS therapies.

2) **From your perspective, does the proposed supplemental process address some or all of the current challenges encountered with complex/specialized drugs, including drugs for rare diseases? Why or why not?**

   The ALS Society of Canada is pleased to see that the proposed supplemental process better aligns the various government agencies involved and encourages more data sharing and input from patient groups. We believe additional clarity is required in defining "unmet need" within the early screening criteria as this can be open to interpretation. Relatedly, ALS is a heterogeneous disease that progresses differently in different people – the reasons why are not understood, but in anticipation of more therapies becoming available it may be necessary for people to be able to access different treatments in order to find the one that is effective for them.

   We also note that the proposed supplemental process does not address the lack of transparency associated with pCPA negotiations and timelines. Even with a supplemental process designed to establish a more consistent funding approach across the provinces, more transparency is required.

3) **What role could you or your organization play in working with others to achieve the stated objective of the proposed supplemental process?**

The ALS Society of Canada would welcome the opportunity to work collaboratively with other organizations on achieving a proactive, consistent, fair and transparent process in order to ensure timely and equitable access to therapies. We would look to be an active participant in discussions and would explore how we could engage our community in the collection of real world evidence. As a member of the Health Charities Coalition of Canada (HCCC) and the Canadian Organization for Rare Disorders (CORD) we work collaboratively with other health charities and organizations in support of better access to care and therapies, increased investment in health research and improved health policy.

4) Please provide your perspective on real world evidence (RWE) and how it could be incorporated into the proposed processes.

ALS Canada believes that real-world evidence is valuable to factor into decision-making as part of the proposed supplemental process and we are open to providing input into how it could be incorporated. We believe it is important for standardized tools to be developed, and for individuals with lived experience to be collaborators in developing these tools so their needs and preferences are accounted for and valued.

Recently through the ALS Canada Research Program we funded a study aimed at developing an index of health-related quality of life for people living with ALS. Conventional measurement tools tend to focus on the physical factors of the disease and ignore the value of a potential treatment on quality of life. Through this study, participating volunteers will identify specific aspects of their life that have been affected by ALS. Each participant will rate how they are doing for each domain and prioritize the domains where they see the most need for improvement. The tool will attach an economic value to each factor, allowing the researchers to calculate an overall score that incorporates gains in one health area with losses in another. Gathering information about cost-effectiveness during clinical trials could help measure the value of therapies on the quality of life of someone living with ALS and be used during the drug access pathway.

5) What challenges and/or opportunities do you see in obtaining and using RWE?

There are two opportunities we see in obtaining and using RWE.

1. **Standardization**: We acknowledge that each rare disease is unique unto itself and that one standardized tool would not facilitate the accurate collection of RWE, however establishing standard parameters to be customized for each disease area could help ensure that the data obtained is unbiased and representative of the entire disease population.
2. **Disease registries**: There is an opportunity to enhance existing disease registries to play a role in the collection of RWE. For example, the Canadian Neuromuscular Disease Registry (CNDR) currently collects data on the clinical features of ALS and other medical impacts of the disease upon registration, but collecting RWE could be incorporated into the process. Additionally, establishing a Canadian ALS Registry would further support the collection of RWE, as well as result in more efficient clinical trial recruitment and advancements in our understanding of ALS in Canada.

6) What is your perspective on having a national review panel to review patient cases? How do you believe this will impact access to EDRDs?

The implementation of a national panel to review individual patient cases has the potential to increase consistency of funding decisions across jurisdictions, but additional clarity must be provided on who panel members will be, the criteria for becoming a panel member and to whom will they be accountable.

The panel must also be comprised of individuals who can maintain objectivity throughout the decision-making process. This could be a challenging position for anyone to be in.

7) In considering the proposed process, have we missed anything?

Based on the principle of patient partnership developed by HCCC, we believe the proposed supplemental process design and implementation should be developed and monitored in partnership with patients to ensure the right medicine gets to the right patient at the right time in a cost-effective manner. The integration of the patient perspective throughout the planning and execution of the process would ensure that patients' health outcomes remain a central focus. For example, patient groups must be involved in the establishment of the screening criteria, as well as defining the specific eligibility requirements for concurrent submissions, in order to ensure barriers to equitable access to therapies are not unintentionally put in place.

There also needs to be increased transparency throughout the entire process and clarity on where a specific drug is within it.