Summary of Responses
Online Submission CADTH – Building Towards a Pan-Canadian Formulary

1. **As part of developing a framework, the panel recommended 6 guiding principles and accompanying definitions that would shape the overall system for a potential pan-Canadian formulary. Please refer to Table 1 in the discussion paper.**
    a. **Do you agree with the proposed principles and definitions? Please provide the reason(s) and suggested changes, if any.**

As a member of the HCCC, ALS Canada recognizes and supports the existing alignment between CADTH principles and those of HCCC.

We recognize that a sustainable and modern formulary, that places principles of patient need ahead of cost containment, is an aspirational goal to be achieved through the integration of formulary decision making within the greater healthcare system.

We recommend that point in time decisions influenced by competing principles of cost-effectiveness and patient need be viewed as opportunities for transparent patient engagement and issue identification for immediate or ongoing system change.

Additionally, we recommend that consideration be given to updating Principle 3: Effective and High Quality: An effective and high-quality national formulary is one that achieves better patient outcomes, comprehensive access to modern medicine and cost savings over what could be achieved by any one provincial or territorial formulary on its own. As access to innovative medicines are essential to Canadians affected by ALS, we note the importance of balancing efforts to lower drug prices with the value of making innovative medicines accessible in a timely and equitable manner.

2. **The panel recommended a 3-stage approach to creating a potential pan-Canadian formulary. Stage 1 is developing a process to create a proposed sample list of commonly prescribed drugs and related products. The proposed sample list is a starting point and is meant to be a proof of concept for the process. Part of the process involved comparing the listing status of each drug on existing public drug plan formularies and identifying gaps in access. The proposed principles were also applied when discussing each drug. A predefined assessment criteria was used by the panel to determine if a drug or related product should be included, flagged for additional expert consultation, or excluded from the proposed sample list. Please refer to Table 2 in the discussion paper for more information on the proposed assessment criteria.**
    a. **Do you agree with the proposed assessment criteria? Please provide the reason(s) and suggested changes, if any.**

As a member of the HCCC, we are commenting on this question, however we must acknowledge the barrier in doing so without understanding the entirety of the approach to connect the creation of the pilot list to a functional national formulary. For progressive diseases, like ALS, time is of the essence. Therefore, a full plan must be established in a timely manner to ensure the right treatments gets to the right patients at the right time.

In alignment with HCCC, ALS Canada recognizes the benefits to a staged approach and efforts that have led to the pilot list of products. We note that the success of the pilot will be determined by the impacts of any unmanageable changes to patient therapies and improved or loss of therapies.

We would also like to highlight that the creation of a national formulary is an opportunity to take into account information not previously considered as well as improve interfaces between formulary decisions and other aspects of healthcare. We recommend that in parallel to exercises that nominate products from existing formularies, a new process also be considered that re-sets past decisions based on current healthcare considerations and principles that support holistic patient care.

We recommend that a change management strategy should accompany any transition to a national formulary and a principle of avoiding any changes to therapy could/should/must be considered. We would like to emphasize that with such limited ALS therapies available, we do not want the national formulary to result in a removal of access to any drugs that are currently on existing formularies.

As a member of the HCCC, we recommend that principles should reflect opportunities to manage costs through modern procurement avenues/negotiations and include dynamic features to respond to marketplace cost changes.

3. **Related products (devices that assist with the delivery or administration of drugs and/or are necessary for the optimal use of drugs), primarily those for patients with diabetes, were assessed by the panel for inclusion on the proposed sample list. The panel felt strongly that the inclusion of related products on a potential pan-Canadian formulary should be explored because this could help improve patient access and could potentially improve adherence with drug treatment. In many cases, these related products are covered through different programs within the health system, which makes accessing coverage difficult for patients. As such, a potential pan-Canadian formulary could be an opportunity to streamline the process, provide simplified access, and ultimately help patients access these types of products. The panel noted the importance of having standard criteria to help determine which related products should be eligible for inclusion on the potential pan-Canadian formulary. This standardization will be particularly important when assessing new or emerging technologies that could be numerous and costly and might impact sustainability.**

3.a **Do you have suggestion(s) on a definition and/or criteria to determine the eligibility of related products that could be included on a potential pan-Canadian formulary? Please provide details.**

In alignment with HCCC, we support the creation of new or additional criteria that can address both immediate needs for complementary therapies but also support a scalable to whole of person therapeutic approach, especially given the heterogeneous nature of ALS.

3.b. Should related products be listed in the same list for drugs and have the same evaluation criteria applied to them (see Table 3 in the discussion paper)? Please provide the reason(s). Note that this question pertains only to evaluation of related products; there will be an opportunity to comment on the proposed criteria for evaluation of new drugs in question 6.

We support the inclusion of related products by making use of the most effective operational model (existing lists or new/dynamic methods) necessary to achieve the patient outcomes identified in the principles. In addition to the products, the methods of drug administration must also be considered within the criteria as many innovative ALS drugs are complex in their method of administration (i.e., intravenous infusion, lumbar puncture).

As a member of the HCCC, we support the changes to CADTH approaches that capitalize on this opportunity to establish a world leading HTA process inspired by the best practices of other jurisdictions while contemplating integration with national centres of clinical excellence and continuous/dynamic methods of incorporating modern medicine.

4. **Stage 2 involves scaling the process to add drugs and select related products for other health conditions to the proposed sample list. The proposed approach would follow the review steps described for stage 1 — considering the listing status from existing federal, provincial, and territorial formularies; utilization data; availability of generic or biosimilar for the drug molecule; information about safe use in pregnant and lactating women; and references summarizing available drugs and use in Canada. These considerations would be supplemented with literature reviews of pharmacotherapeutic areas that have been shown to improve health outcomes in people made vulnerable by systemic inequities (if available). Assessment would include reviewing the totality of the information.**
   The panel recommends that the proposed principles (e.g., universal and integrated) be applied. As part of the refinement, the panel suggests that products listed under specialized programs (e.g., cancer and special drug programs) be included. This is because product listing and eligibility, among other aspects, may differ across the country and a gap could inadvertently be created. The panel also suggests that therapeutic areas could be prioritized based on national health priorities. Further details can be found in the Stage 2: Expanding to Other Therapeutic Areas section of the discussion paper.

4.a Do you support the proposed approach to expand to other therapeutic areas? . Please provide the reason(s).
We recommend that the goal of achieving a truly universal and equitable national formulary is satisfied by achieving access to products that support all disease states and conditions.

We recommend that therapeutic areas not be compartmentalized for prioritization as many patients, particularly those living with comorbidities of ALS, will face challenges across multiple therapeutic areas.

We acknowledge the aim of developing a rare disease strategy with its own unique approach. However, we hope that one day ALS therapies are not considered under a rare diseases framework and that emerging therapies are taken into consideration under this proposed expansion.

4.b Should the remaining therapeutic areas be prioritized based on national health priorities?. Please provide the reason(s).

As a member of the HCC, we support on-going efforts and engagement to overcome any constraints that may be impeding a national formulary and that are contributing to the need to prioritize.

To ensure a transparent way of determining such priorities, we support and encourage further engagement opportunities to clarify and determine the planning and operational consequences of setting priorities.

5. **The panel explored alternative approaches to the first-in, first-out process for reviewing new products and indications for inclusion on a potential pan-Canadian formulary (see the Selecting New Products to be Considered on a Potential Pan-Canadian Formulary section of the discussion paper). The following options were explored:**
    - **Option #1: A prioritization model could be developed to align with Health Canada's priority reviews. This would allow for a predictable process for identifying products that represent a significant therapeutic advancement. Although this approach could support a seamless integration between regulatory and health technology assessment (HTA) processes, it does not address the inability to control when a submission is initiated.**
    - **Option #2: A clear and transparent scoring system that would prioritize new drug submissions could be created and applied (e.g., new innovative products that address unmet needs of a population could score higher and be prioritized on a review agenda).**
    - **Option #3: Opportunities to work together at an international level to review and prioritize products collectively could be explored. There have been international collaborations in several areas of regulatory and HTA processes. This could potentially save on resources and accelerate access for Canadians and international partners.**

      The panel encourages strong engagement and collaboration with all key stakeholders (e.g., patients, clinicians, industry, government, and HTA bodies) through all steps in the process and recommends the use of a transparent process.

5.a Which option could be adopted as an alternative to a first-in, first-out submission review process? Please provide the reason(s) for your choice.
As this consultation does not include a strategy for rare diseases drugs, ALS therapies would not be subject to any of these options. However, we support HCCC's recommendation for CADTH to explore and analyse the options further to determine feasibility.

5.b What criteria could be used to identify priority products?
We support clarity in the resource constraints that are contributing to the necessity of an option analysis and priority setting exercise.

We recommend consideration be given to changing operational approaches to allow for a dynamic method of resource management and consultation on those constrains and metrics that are driving the need to engage in a priority setting dialogue.

6. **To guide the evaluation of new drugs and new indications for a potential pan-Canadian formulary, the panel considered the following proposed criteria:**
    - **alignment with patient and societal values**
    - **clinical benefit**
    - **feasibility of adoption into health systems**
    - **value for money**

    The panel proposed 2 additional criteria — equitable access and additional considerations or long-term thinking — to enhance the deliberative process. The proposed criteria are linked with the guiding principles and provide the basis for decision-making with respect to the selection and evaluation of drugs for a potential pan-Canadian formulary. Please refer to Table 3 in the discussion paper for details on the proposed evaluation criteria for new products.

ALS is a progressive and fatal neurodegenerative disease. Therefore, equitable access to approved therapies is a critical element for people living with ALS. We consider the need for timely access in alignment with patient and societal values, as equitable access to therapies will play a significant role improving the lives of people with ALS and their families' quality of life.

As a member of the HCCC, we recommend that the feasibility of adopting a therapeutic should be viewed as an opportunity to include emerging therapeutics supportive of on-going improvements to patient care.

We are concerned that limiting inclusion of therapies on a National Formulary that are overcoming challenges with adoption (feasibility) will only further hinder their uptake and could undermine the adoption of new effective products and therapies, which are essential to those living with ALS and other rare diseases.

7. **The panel also provided recommendations on a deliberative process for using the proposed criteria and applying them in practice. Of particular interest, they explored ways to structure the deliberative process so that evidence from multiple disciplines and perspectives could be weighted. The panel proposed that evaluating and selecting products for a potential pan-Canadian formulary should involve an expert committee. Please see the Deliberative Process section in the discussion paper for details.**
    a. **Should the deliberative process include weighting of the evidence or a score for each criterion? If yes, how should weight be distributed among the proposed criteria?**

As a member of the HCCC, we recognize the importance of a governance model to a successful national formulary and support a governance model that is objective and takes into account authentic and regular patient input.

We recommend that patients and patient caregivers should be among the experts consulted to arrive at the score.

We support the use of a governance model that is inspired by and improves upon existing models of patient inclusion and HTA around the world.

8. **Current Canadian drug review processes generally focus on assessment of new products. There is a desire to ramp up formulary modernization strategies (e.g., reassessments, therapeutic reviews) and to re-evaluate existing listed products with emerging new evidence on a regular cycle (e.g., every 3 years to 5 years). This would likely increase the workload of stakeholders throughout the health system (e.g., clinicians, patients and patient groups, researchers, industry, regulators, and plan administrators).**
    a. **What measures could be put in place to ensure operational sustainability, with limited resources and time, including the ability of stakeholders to participate meaningfully in multiple processes (e.g., should there be a prioritization system for listed products to be re-evaluated or other criteria to determine eligibility for reassessment or therapeutic review)?**

We recognize that changes to existing processes will require the input of many different stakeholders.

In accordance with HCCC, we support an iterative process to manage a workload while engaging all groups necessary to achieve a successful National Formulary that is integrated within health systems and remains both sustainable and modern.

9. **Are there any other comments that you would like to share with us?**

We express our sincere appreciation for the opportunity to participate in this consultative process. We believe a National Formulary is an important component of a modern and patient focused healthcare system – and we applaud the progress made to date and look forward to continued engagement.

However, given the nature of ALS and the limited therapies available to this community, any new therapies for ALS would likely be managed under a rare disease strategy. As such, we have commented on this submission as a member of HCCC with the hope that ultimately ALS therapies will be considered under a proposed national formulary.

The current approach for rare disease therapies is fragmented across the country. We believe a rare disease strategy in which provincial, territorial and federal governments work collaboratively, is vital to ensuring timely, affordable and equitable access to ALS therapies to Canadians.

In agreement with HCCC, we propose the following considerations in support of a successful National Formulary within the greater context of achieving an integrated patient outcome-based health care system:

- Changing the Conversation – Recognizing that Patient Centric Policy is Cost Effective
- Treating Patients as a Whole
- Rising above Jurisdictional Issues and Creating Systemic Solutions
- Embracing Change as Part of Preserving Canada's Healthcare Legacy

We emphasize that patients must be engaged as equal partners in all consultations and decision-making processes that affect their care. While this consultation represents positive progress, it remains difficult to assess elements of a pan-Canadian formulary in isolation of the other three components that are core to its implementation – that is: terms of coverage, financing and clear decision-making authority.

As we recover from the impacts of the pandemic, we must take this opportunity to apply the lessons we have learned to improve the lives of people living with ALS and other rare diseases. With promising new ALS therapies on the horizon, now is the time act to ensure Canadians living with ALS can access innovative therapies in a timely and equitable fashion.

We look forward to providing continued comment on the outstanding elements that are relevant to the development of a national formulary. We thank CADTH for this opportunity and the important contribution they make to our healthcare system.