CADTH

CADTH COMMON DRUG REVIEW

Patient Input

eculizumab (Soliris)

(Alexion Pharma Canada Corp.)

Indication: Myasthenia Gravis (gMG), adults

CADTH received patient input from:

Muscular Dystrophy Canada

April 23, 2020

CADTH

Patient Input Template for CADTH CDR and pCODR Programs

Name of the Drug and Indication	Soliris® (eculizumab)
Name of the Patient Group	**Muscular Dystrophy Canada**
Author of the Submission	[redacted]
Name of the Primary Contact for This Submission	[redacted]
Email	[redacted]
Telephone Number	[redacted]

1. About Your Patient Group

If you have not yet registered with CADTH, describe the purpose of your organization. Include a link to your website.

MDC is registered with CADTH

2. Information Gathering

CADTH is interested in hearing from a wide range of patients and caregivers in this patient input submission. Describe how you gathered the perspectives: for example, by interviews, focus groups, or survey; personal experience; or a combination of these. Where possible, include **when** the data were gathered; if data were gathered **in Canada** or elsewhere; demographics of the respondents; and **how many** patients, caregivers, and individuals with experience with the drug in review contributed insights. We will use this background to better understand the context of the perspectives shared.

120 clients were interviewed with generalized Myasthenia (GMG). 70 caregivers were interviewed. 2 individuals in the United States were interviewed with (GMG)

3. Disease Experience

CADTH involves clinical experts in every review to explain disease progression and treatment goals. Here we are interested in understanding the illness from a patient's perspective. Describe how the disease impacts patients' and caregivers' day-to-day life and quality of life. Are there any aspects of the illness that are more important to control than others?

75% of clients reported debilitating chronic progression. This included chocking, slurred speech, impaired swallowing, breathing issues and disabling fatigue. These clients reported that they have had at least 5 admissions to hospital within the last 5 years with an average of 2 weeks admission for swallowing issues. 35% of clients reported to at least 1 admission to ICU for respiratory failure. 45% reported that they require in home support for all activity of daily living. 75% of clients reported that they were forced to exit from their employment due to progression of their muscle weakness. 25% clients reported that they are experiencing eye movement difficulty and are beginning to have speech issues. 15% of caregivers reported that they have had to exit from employment to be fulltime caregivers for their adult children affected by MG. 15% of clients reported that they were no longer able to take care of their children due to progression of muscle weakness resulting in their partner requiring to exit their employment leading to financial hardship. 35% of clients reported they were forced to sell home and move into subsidized rental housing.

100% of clients reported that they had experienced complications and they explained "that there are frequent exacerbations that they have no control over". 25% of caregivers expressed their challenges associated with "caregiver burnout". 25% of caregivers reported that they frequently see primary health care practionar related to caregiver stress related challenges and 12% reported that they have been admitted to hospital due to caregiver stress related challenges. 100% of clients reported that they are living in constant fear of the unknown. "Uncertain of their future, fear full of losing their independence". A caregiver explained "I have watched my daughter's health decline from losing muscle in her eyes to not being able to hold head and now needed to quit her job as she experiences constant crisis with her breathing, we in our 70's are raising her children".

4. Experiences With Currently Available Treatments

CADTH examines the clinical benefit and cost-effectiveness of new drugs compared with currently available treatments. We can use this information to evaluate how well the drug under review might address gaps if current therapies fall short for patients and caregivers.

Describe how well patients and caregivers are managing their illnesses with currently available treatments (please specify treatments). Consider benefits seen, and side effects experienced and their management. Also consider any difficulties accessing treatment (cost, travel to clinic, time off work) and receiving treatment (swallowing pills, infusion lines).

20% of clients reported that they are currently using corticosteroids and cyclosporine. 35% of clients have reported that both medications have assisted with decreasing exacerbations. However, medications did not have impact on their ability to work or live independently. 15% of clients reported that they are currently using azathioprine. Similarly, this medication assisted with decreasing the number of exacerbations but did not impact ability to work and live independently. .45% reported they have tried the medication and it was not affective.

55% of clients reported that they experienced a secondary health concern with corticosteroids that included hypertension and Type 2 diabetes. 65% of clients reported having nausea, fatigue and diarrhea with use of azathioprine and cyclosporine.

25% have the medication covered under their provincial funding program and 15% pay out of pocket due to being 65 years and older.

5. Improved Outcomes

CADTH is interested in patients' views on what outcomes we should consider when evaluating new therapies. What improvements would patients and caregivers like to see in a new treatment that is not achieved in currently available treatments? How might daily life and quality of life for patients, caregivers, and families be different if the new treatment provided those desired improvements? What trade-offs do patients, families, and caregivers consider when choosing therapy?

The most prevalent responses (in order) from our clients where the following:

1. Clients are willing to deal with side effects if the medication could decrease the "intensity" of the exacerbation. Current medications seem to be decreasing the number of exacerbations but not the impact "with each exacerbation our health declines". "Everyday, I am fearful of going into crisis". "I need another option, current treatments are not helping me".
2. Clients are willing to with deal side effects if they can stay home longer and not required to move to an institution to accommodate personal care needs. "I don't want to live in long term care, I am willing to deal with side effects, staying in my home is my priority". "It breaks my heart to see my daughter living away from her children, she is dependent on all of her needs".
3. Clients are willing to deal with side effects if they can experience less hospital admissions. "This disease impacts the health care system significantly" "When we need medical assistance it is serious" " A hospital stay for me is admission to ICU at least 3 times in the last year'. "I need very specialized help, 24 hour care for my frequent respiratory failures episodes".

6. Experience With Drug Under Review

CADTH will carefully review the relevant scientific literature and clinical studies. We would like to hear from patients about their individual experiences with the new drug. This can help reviewers better understand how the drug under review meets the needs and preferences of patients, caregivers, and families.

How did patients have access to the drug under review (for example, clinical trials, private insurance)? Compared to any previous therapies patients have used, what were the benefits experienced? What were the disadvantages? How did the benefits and disadvantages impact the lives of patients, caregivers, and families? Consider side effects and if they were tolerated or how they were managed. Was the drug easier to use than previous therapies? If so, how? Are there subgroups of patients within this disease state for whom this drug is particularly helpful? In what ways?

Individuals affected by GMG were interviewed in the United States as the FDA approved Soliris in 2017. These individuals reported the following:

1. Soliris decreased the intensity of elaborations which had better outcomes compared to other medications. This resulted in a significant decrease in ICU and overall hospital admissions.
2. Previous therapies were not suitable for individuals affected by GMG this provided another option.
3. Individuals reported seeing an improvement in their muscle strength and overall well being.
4. Individuals reported being more independent with their ADL's and not requiring the same level of support.

8. Anything Else?

Is there anything else specifically related to this drug review that CADTH reviewers or the expert committee should know?

It is clear from the interview process, that even though some clients had “similar overall experiences” with treatments, each client’s experience related to symptoms were very different. There was consensus that new options be available for clients who have not experienced positive outcomes with current treatments. In addition, overall clients could not describe any triggers for a crisis event. It came without warning and causes a great deal of anxiety. Lastly, clients expressed their concern not only about their own overall quality of life with frequent hospital admissions but also, the financial burden placed on provincial health care systems.

Appendix: Patient Group Conflict of Interest Declaration

To maintain the objectivity and credibility of the CADTH CDR and pCODR programs, all participants in the drug review processes must disclose any real, potential, or perceived conflicts of interest. This Patient Group Conflict of Interest Declaration is required for participation. Declarations made do not negate or preclude the use of the patient group input. CADTH may contact your group with further questions, as needed.

1. Did you receive help from outside your patient group to complete this submission? If yes, please detail the help and who provided it.

No

2. Did you receive help from outside your patient group to collect or analyze data used in this submission? If yes, please detail the help and who provided it.

No

3. List any companies or organizations that have provided your group with financial payment over the past two years AND who may have direct or indirect interest in the drug under review.

Company	Check Appropriate Dollar Range			
	$0 to 5,000	$5,001 to 10,000	$10,001 to 50,000	In Excess of $50,000
	0			

I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this patient group with a company, organization, or entity that may place this patient group in a real, potential, or perceived conflict of interest situation.

Name: Stacey Lintern
Position: COO
Patient Group:Muscular Dystrophy Canada
Date: April 23, 2019