CADTH

CADTH COMMON DRUG REVIEW

Patient Input

ECULIZUMAB (Soliris)

(Alexion Pharma Canada Corp.)

Indication: Neuromyelitis optica spectrum disorder

CADTH received patient input from:
Multiple Sclerosis Society of Canada

March 16, 2020

CADTH

Patient Input Template for CADTH CDR and pCODR Programs

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

The Multiple Sclerosis (MS) Society provides programs and services for people with MS and their families, advocates for those living with MS, and funds research to help improve the quality of life for people living with MS and to ultimately find a cure. The mission of the MS Society is to connect and empower the MS community to create positive change. Since 1948 the MS Society has contributed over $190 million towards MS research. This investment has enabled the advancement of critical knowledge of MS, and the development of a pipeline of exceptional MS researchers. In addition to supporting Canadians affected by MS, the MS Society of Canada provides support and services to people living with allied diseases, including neuromyelitis optica spectrum disorder (NMOSD). The patient input contained in this report is to support the review of the first drug approved for people living with NMOSD in Canada. Prior to the approval of eculizumab, patients diagnosed with NMOSD were treated with off-label agents such as azathioprine and rituximab. The approval of eculizumab for NMOSD is a significant therapeutic advancement for the NMOSD community.

1. Information Gathering

The MS Society launched an online survey from March 3, 2020 to March 13, 2020 posted to the MS Society website and Facebook sites, in both English and French. The survey was targeted to people diagnosed with NMOSD and those affected by NMOSD. People living with NMOSD and their loved ones were asked to provide feedback related to their quality of life and experience with the drug being reviewed. In total only 11 responses to the survey were received. All respondents were female (100%) and just under half were between the ages of 35-44 (41%). Approximately 25% were between 55 and 64, 16% between 25 to 34 and 8% between 45 and 54 and 65+. One-third of respondents (66%, or 8 respondents) are diagnosed with NMOSD, and the remainder self-identified as caregivers (33%). Of the caregivers, only two commented they assisted with activities of daily living (ADL).

The MS Society of Canada supports people affected by NMOSD through the Quality of Life Grant program (equipment) and via information and referral. Based on the survey comments, respondents appear to be Canadian however country of origin was not a survey question.

2. Disease Experience

Neuromyelitis optica spectrum disorder (NMOSD), is a rare autoimmune disorder of the central nervous system (CNS) where antibodies can damage the spinal cord and/or optic nerves during attacks. It is a demyelinating condition, meaning, it damages the protective myelin sheath around the nerve fibres. Attacks to the optic nerves produce swelling and inflammation that cause pain and loss of vision while damage to the spinal cord **causes weakness or paralysis in the legs or arms, loss of sensation, and problems with bladder and bowel function.** Due to the similarity of symptoms, NMOSD was previously confused with multiple sclerosis (MS) however NMOSD is less common than MS and attacks can be more severe than MS attacks. Disease modifying therapies for MS are not beneficial in NMOSD and in fact, when administered, have shown to cause NMSOD disease to worsen. NMOSD follows a relapsing-remitting disease course: during a relapse, new damage to the optic nerves and/or spinal cord can lead to accumulating disability. There is no progressive phase of the disease as in MS. NMOSD is most commonly seen in women (ratio 4:1) between the ages of 30 and 40. However, it has been diagnosed in preschool aged children and adults over 60. The cause of NMOSD in the majority of cases is due to a specific attack on the aquaporin-4 (AQP4) water channel located within the optic nerves and spinal cord.

With each attack, an individual living with NMOSD will accrue additional disability, which has an significant impact on every aspect of daily life including a negative effect on independence, their family, community, employment, and ultimately society. When asked how a diagnosis of NMOSD has impacted their lives, most discussed the debilitating nature of the damage caused by attacks affecting their vision and mobility.

- ***I've lost vision completely in both eyes, have poor sensation in many parts of the body after full paralysis, lost bladder and bowel function, and wheelchair bound... Oh, and suffer from residual symptoms caused by the damage from al the attacks I've had, which includes nerve pain and spastic muscles…***
- ***It (NMSOD) has changed my life completely. I am no longer able to work as a nurse. I suffer from residual symptoms that affect my functioning on a daily basis. My husband no longer works. He gave up his job when I was diagnosed as I needed the help at home. My life will never be the same again.***
- ***My eyesight has been permanently damaged.***
- ***Lowered my energy and productivity both personally and work related. Increased illnesses like colds, flu. Generally impacted my ability to participate in life events.***
- ***Some days just non functional due to eye pain. More time off work for appointments.***

- ***I am currently to the point where I am struggling to get funding for a power wheelchair. Without it, it's almost impossible for me to leave my house because of the level of pain & injury I'm dealing with. Injury is due to long term steroids.***
- ***Hard time walking / Restrictions on my walking.***

Four of the respondents reported living with NMOSD for two to four years, followed by five to ten years (two respondents). One respondent had been living with NMOSD for between 11-20 years and one was newly diagnosed, having lived with NMOSD for less than two years.

3. Experiences With Currently Available Treatments

Up until the approval of eculizumab, standard treatment for NMOSD involved intravenous steroids, and additional treatments to remove antibodies (intravenous immunoglobulin or plasmapheresis/plasma exchange). In addition, immunosuppressants are used off-label to help prevent further attacks though with varying levels of therapeutic benefit. Symptoms such as neuropathy, pain, stiffness, muscle spasms, bladder and bowel control problems can be managed with various medications and therapies. The following were identified as treatment plans by the respondents:

- ***Have just been doing naturopathic treatments now like taking natural supplements, acupuncture, cupping, and chiropractic adjustments because Since 2006, have been off preventative treatments because I was sent to a long term care home thinking there was no more to do for me at the time...***
- ***Rituximab, Lyrica, Aventyl, Baclofen***
- ***Rituximab***
- ***In a open label medical trial; med-immune***
- ***Rituxan, IV Solumedrol***
- ***Am in the process of switching to Soliris. Have been on Cellcept, Imuran & rituximab & daily prednisone.***
- ***Apo-azathioprine***

When asked if the current therapies were effective, four respondents reported that they were effective, one reported no perceived effectiveness and three did not know. The overarching theme was that the currently available therapies for NMOSD were stopgap medications.

- **So far so good, but not sure if this is something that will work forever.**
- **The steroid side effects are so bad.**
- **I have not been stable or able to come off daily steroids.**

Improved Outcomes

The approval of eculizumab to the market is a significant milestone, as the first treatment targeted to people living with NMOSD. Untreated, the burden of disease and increasing disability impacts all areas of a person's life including but not limited to: employment stability or loss, family income, increased need for assistance or caregiving, loss of independence, isolation, cognitive decline and increased mobility challenges. Eculizumab has the ability to reduce attacks and accrued disability and therefore fills a significant therapeutic need that has been unmet in NMOSD treatment to date.

4. Experience With Drug Under Review

None of the respondents had experience with eculizumab however one respondent had received approval to initiate treatment.

5. Companion Diagnostic Test

Data on companion testing was not requested as part of the survey however a list is provided below.

- Receive immunizations according to current immunization guidelines.
- Receive meningococcal vaccines prior to, or at the time of receiving SOLIRIS. Administer vaccinations for the prevention of infection due to Neisseria meningitidis, Streptococcus pneumoniae and Haemophilus influenzae type b (Hib) according to the National Advisory Committee on Immunization (NACI) guidelines.
- Receive 2 weeks of antibiotics if you immediately start SOLIRIS.

6. Summary points

- Up until the approval of eculizumab, there were no treatments specifically indicated for NMOSD (globally). Current treatments for NMOSD were used off-label and had varying levels of therapeutic benefit to reduce relapse rates.
- Eculizumab is the first treatment indicated specifically for NMOSD with statistically significant evidence demonstrating a reduction in relapse rate and relapse-rate hospitalization.
- Eculizumab fills a significant therapeutic need that has been unmet in NMOSD treatment.
- Treatment with eculizumab has the potential to allow people living with NMOSD to remain in the workforce, sustain family and social roles and responsibilities longer, improve their quality of life, decrease the need for caregiving (family caregiver or paid caregiver) and reduce the financial burden to health and social systems.

Appendix: Patient Group Conflict of Interest Declaration

No help was received from outside the MS Society to collect, analyze data or complete this submission, or used in this submission. The following companies have provided the MS Society with financial payment over the past two years. No company has interest in the drug 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
EMD Serono				X
Hoffmann La Roche				X
Biogen				X
Novartis				X
Sanofi-Genzyme			X	
Pendopharm (Pharmascience)			X	

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: Jennifer McDonell
Position: Senior Specialist, Programs & Services
Patient Group: Multiple Sclerosis Society of Canada
Date: March 13, 2020