CADTH

CADTH COMMON DRUG REVIEW

Patient Input

satralizumab (Enspryng)

(Hoffmann-La Roche Limited)

Indication: Neuromyelitis optica spectrum disorder

CADTH received patient input from:

Multiple Sclerosis Society of Canada

November 12, 2020

CADTH

Patient Input Template for CADTH CDR and pCODR Programs

Name of the Drug and Indication	**Enspryng (satralizumab)**
Name of the Patient Group	**Multiple Sclerosis Society of Canada**
Author of the Submission	[redacted]
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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 a second drug approved for people living with NMOSD in Canada. Prior to the approval of eculizumab, and more recently satralizumab, patients diagnosed with NMOSD were treated with off-label agents such as azathioprine and rituximab. The approval of satralizumab for NMOSD is another significant therapeutic advancement for the NMOSD community as it offers an additional therapeutic choice, which can be self-administered once monthly for people living with NMOSD.

1. Information Gathering

The MS Society launched an online survey from October 1, 2020 to October 31, 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. The survey was also shared with the Guthy-Jackson Charitable Foundation in the United States (charitable organization that support people living with NMOSD as well as funds research). Responses were received from patients living with NMOSD within North America. 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 37 responses to the survey were received. Most respondents were female (86%) and ranging in age from 55-64 (40%), 45-54 (27%) and 35-44 (27%). Approximately one-third of respondents (68%, or 25 respondents) are

diagnosed with NMOSD, and the remainder self-identified as 'other' (one caregiver and four respondents diagnosed with multiple sclerosis). Of the 'other' group of respondents, none provided comments.

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.

- ***Greatly. Once a very active person I no can no longer work, drive and am mostly housebound.***

- ***Blindness and leg weakness. Unable to perform certain recreational activities as I could once do in the past. Need aids at workplace in order to perform job duties. Cannot stay in certain temperature settings for prolonged periods of time (e.g. heat). Break times more frequent and it takes longer to recover.***

- ***I definitely struggle every day with pain and fatigue but I'm healthy.***

- ***I'm unable to work, I'm constantly in pain. The past five years has been very difficult.***

- ***Majorly. I am now a power wheelchair user. I have broken multiple bones from long term steroids to treat my NMO. I have permanent spinal cord damage & severe chronic pain.***

- ***Serious impact. I remain positive but it's made a massive impact. I have poor bladder control, can't walk unassisted and am currently on sick leave from work.***

- ***Had to quit work.***

- ***Limited daily activities and work. Slowed my life down significantly.***

- ***Bowel & bladder problems make it difficult to go out, can't work, I'm anxious about flying so I no longer travel as I once did. Problems with light touch make swimming or boating or playing with grandchildren in or near water impossible. I used to sail and I swim daily.***

- ***I can no longer work. I can't walk more than 1km before my body starts falling apart. My whole body has pins and needles constantly.***

- ***Dramatically. I gave up my career which was a huge change.***

Six respondents reported living with NMOSD between two to four years, fourteen of the respondents reported living with NMOSD for five to ten years (14 respondents), three respondents had been living with the disease for eleven to twenty years. Only two respondents had been living with NMOSD for less than two years.

3. Experiences With Currently Available Treatments

Up until 2019, 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:

Rituximab (13), steroids (6), azathioprine (3), Soliris (1) and symptom management medications such as gabapentin and tizanidine, as well as non-medicinal strategies including chiropractic care, acupuncture, exercise, nutrition, supplementation, naturopathic medicine.

When asked if the current therapies were effective, fourteen respondents reported that they were effective, two reported no perceived effectiveness and seven did not know. Most respondents were taking off label rituximab which would account for those who felt their treatment was effective. Approved effective therapies for NMOSD are required as treatments used off label will not be covered through private or public plans.

- ***We need these drugs covered so we have options.***

Improved Outcomes

The approval of satralizumab to the market is another significant milestone, as a second treatment option targeted to people living with NMOSD offered as a monthly self-administered subcutaneous injection, which is a lower frequency dosing schedule compared with the only other treatment approved for NMOSD (eculizumab, delivered as infusion in a specialized clinic every two weeks). 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. Satralizumab has the ability to reduce attacks and accrued disability and allows individuals to have the freedom to remain at home to receive their therapy once monthly, and therefore fills a significant therapeutic gap that had remained unmet in NMOSD treatment to date.

- **I appreciate that Enspryng will be more convenient to administer and would like to switch to this product if possible.**

- **So good to have other treatment options.**

4. ### Experience With Drug Under Review

None of the respondents had experience with satralizumab.

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.
- Neutrophil counts should be monitored 4 to 8 weeks after start.

5. Summary points

- Up until 2019, 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.
- Satralizumab is the first self-injected treatment indicated specifically for NMOSD.
- Satralizumab fills a significant therapeutic need that has been unmet in NMOSD treatment and allows patients to self-administer their therapy at home, once monthly.
- Treatment with satralizumab 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 industry 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	
Britsol-Myers Squibb			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.

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Date: November 9, 2020