Patient Input for edaravone (RADICAVA™) 3586 for the treatment of ALS from Patients, Caregivers and Patient Groups

Prepared for

Members of the Drug Benefit Council (DBC)

DBC Meeting

Meeting held: TBD

Number of Eligible Questionnaires

Patients (13), Caregivers (6), Patient Groups (1)

Participating Patient Group

The following patient group, who met the criteria for inclusion in this report, provided input to the Ministry of Health (Ministry) for its review:

s.22

Source

Your Voice website: questionnaires posted from October 24, 2018 to midnight on November 21, 2018.

Conflict of Interest Declarations

Patient Responses

The patients who completed a questionnaire reported no potential conflicts of interest.

Caregiver Responses

The caregivers who completed a questionnaire reported no potential conflicts of interest.

Patient Group Responses

The patient group who completed a questionnaire reported no potential conflicts of interest.

The PharmaCare Drug Information Page

Patient Responses

12 of 13 patients who completed a questionnaire reported they had read the PharmaCare Drug Information page.

Caregiver Responses

All caregivers who completed a questionnaire reported they had read the PharmaCare Drug Information page.

Patient Group Responses

The patient group representative who completed a questionnaire reported they had read the PharmaCare Drug Information page.

Responses to Drug-related Questions

Patients and Caregivers Question 5, Patient Groups Question 9:
Describe how the medical condition or disease which the drug under review would be used for affects the day-to-day lives of patients.

Patient Responses

1. The answer is it changes as the disorder progresses. Ultimately I will be unable to speak, swallow, or [breathe] due to ALS.
2. My disease has changed, and will seriously shorten, my entire life. I no longer have a future (disease is terminal) and can anticipate a prolonged period of increasing disability and function loss, followed by death. Edaravone could slow down progression and provide me with more time with my family before I die.

3. Presently the disease has affected my hands and arms more so than my legs. I am no longer able to do many of the normal tasks that involve my hands. Those that I can still do require much effort. The strength in my arms is markedly diminished [due] to significant muscle loss. The strength in the lower limbs is less affected but present. My energy level is lower than normal and fatigue compounds any weakness that is present. Overall I am still independent in most activities.
4. If this medication does reduce symptoms by 30%, it would help me to be more independent for perhaps longer than it might without it.
5. [No response provided.]
6. It is claimed to slow down the rate of deterioration of a person with ALS.
7. Slow down the progression of ALS.
8. Possibly extend my life with ALS and a better quality of health.
9. ALS is a life changing disease. As the disease kills motor neurons, the muscles controlled by those neurons begin to atrophy and weaken. I first lost the use of my right arm, and as I'm right-handed this was a significant change. Next my breathing was affected, and I began using a BiPAP machine at nights to help me sleep. Recently my legs are weakening and we are having to consider installing a stair lift to enable me to access the upstairs. I use a walker now when I have to walk a distance. I am apprehensive about what this winter will be like as I feel the cold so much more keenly. I have lost about 30 lb and I'm down to 128 pounds now. When my left hand becomes cold it gets stiff and uncooperative. Almost all of the outdoor activities I used to enjoy doing are now impossible for me.
10. I was diagnosed with ALS on the 26 September 2018 at [clinic name redacted to ensure patient privacy]. Edaravone is believed to help delay the onset of the symptoms by 1/3(??). This would have a great impact on my quality of life. This medication may add more time for me to enjoy with my family and friends before the symptoms restrict my quality of life. This also gives me hope that a cure will be found in time. Thank You for this opportunity to express my opinions.
11. ALS has taken away my voice 99%. It is difficult to swallow. s.22 I am getting to the stage I can not dress myself. My right hand is so weak I can barely hold a glass. s.22 s.22 My mental capacity has diminished a bit. In general my overall well being has taken a hit!
12. ALS limits my ability to function normally every day. Loss of energy. Constant discomfort. I am unable to dress myself completely. I need support from my wife to do basic things like button shirts, pulling up zippers, putting on socks, pulling my pants up. Also for eating I need someone to cut my food up into smaller pieces as I am unable to use a knife. I know the muscles/nerves in my legs and chest are also starting to weaken. s.22 My life expectancy is 3 to 5 years.
13. I need a walker for balance. Can no longer climb stairs, swim, run, hike, ski, etc. Difficulty rolling over in bed. Poor volume of speech and slurring. Poor fine motor control. ex. cannot zip my coat. Fatigue. Cannot work.

Caregiver Responses

1. If started soon enough it might extend their life by 30%. Depending on the coverage they have they may not be able to afford it if it is actually priced at $190,000/year.
2. Hopefully [it] is slowing the progression of the disease.
3. Hopefully it would slow down the progression of the disease. Allow more time with family. If progression is slowed then [a] person can function at higher level for longer period leading to a better quality of life.
4. s.22 entering his fifth year with ALS. His ALS has advanced slowly up to this point, but the cumulative effects of growing disability are starting to take a toll. His respiratory function has declined by 50 per cent. He is still managing relatively well because he has the use of a bi-pap machine all through the night and during his daytime naps. Despite his breathing difficulties, however, he can still dress himself, and eat all his meals in the regular fashion. He can still talk and walk and work very part-time. This is a really significant time for considering a drug therapy that could slow down the progression of his ALS. We have learned over this past four years just how worthwhile life—even a handicapped life—can be. And we continue to hold to the hope that all of the research dollars being poured into finding a treatment or cure for this dreadful disease will pay off. Radicava is the most recent new treatment that persons with ALS can take, but it's way beyond our financial means. Hence our interest in PharmaCare's covering the costs of it.
5. This disease has completely altered our lives. s.22 is dependent on me for an ever-increasing number of activities of daily living...dressing, hygiene, house and yard work, driving, meal prep, cutting up food, opening jars etc. There is no baseline as the disease progression is unrelenting. We have been told to expect a period of profound disability followed by death in 3–5 years after diagnosis.
6. If effective it could prolong s.22 life. It would complicate life as we would have to give injection at home I would presume. We could never afford this drug.

Patient Group Responses

1. Endaravone (Radicava)is being used in other parts of the world and is felt could slow the progression of Amyotrophic Lateral Sclerosis (ALS) if taken at the early stage of the disease. Currently, the only other drug prescribed is Riluzole. Endaravone is another option that will provide hope to people living with ALS.

Patients and Caregivers Question 6, Patient Groups Question 10:
What drugs or other treatments has the patient used, either now or in the past, to treat the medical condition or disease which the drug under review would be used for?

Patient Responses

1. Riluzole, Vitamin D, edaravone IV, NAC. The edaravone made a noticeable difference.

2. [No response provided.]
3. Edaravone since April 2018. This was started shortly after diagnosis. My neurologist believes the progression of the disease has been slow, so I hope the drug has been successful.
4. Since being diagnosed with ALS I have not taken any drug for the disease.
5. Only edaravone.
6. I have not taken any drugs to treat ALS. I take other drugs to relieve symptoms.
7. APO-Riluzole: expensive with a very limited slow down of progression.
8. Gabapentin helps with nerve pain and cramping.
9. I chose to not take riluzole. Several people I know developed serious side effects from its use. It didn't seem to offer any significant benefits. Recent studies have shown that it prolongs the final stages of life, which makes me feel that I have made the right choice. I have been taking vitamin B 100 complex on the recommendation of my doctor, and it seems to help reduce the cramping associated with the disease. I tried coconut oil capsules as well, but that didn't seem to help. Other than that I have tried to remain as active as possible and eat healthy meals.
10. I have just been diagnosed on 26 September 2018, so I have not been put on any medication for ALS at this point.
11. No drugs have [been] available to me. All I want is a chance not to decline so that a cure can be found.
12. I am taking Riluzole and have no side effects. I am unable to say whether or not it is slowing the disease progression as my condition continues to worsen. It is a costly drug but my extended benefits cover 80%. I am currently involved in a drug trial through the s.22 No side effects and no benefit yet. One thing I do find helpful is 50:50 CBD/THC which helps me sleep (not wake with fasiculations) with no cognitive impairment. I have this through a medical prescription. I also find massage therapy relieves symptoms for a few days after a treatment.
13. I currently take the generic form of nuedexta. It helps a bit with my speech. I also take oxybutynin for bladder urgency.

Caregiver Responses

1. Riluzole is an accepted provenance therapy that is currently priced at less than $2500/year. Why/how can we allow a firm to charge $190k/year for edaravone? Outrageous.
2. None.
3. None.
4. There is only one other drug used by ALS patients and s.22 decided not to take it because of possible side effects and some skepticism about its efficacy.
5. He takes Riluzole which has no side effects, is covered partially by our extended medical plan. Unfortunately it is difficult to say how it may be slowing the disease. There is no cure. s.22 It is just looking at tolerability and dosing and again it is hard to say if it is making any difference. They are not investigating it as curative. We have to travel s.22 every two weeks for the study but we are desperate and will try anything reasonable that

might give us hope. Medical marijuana at bedtime gives him relief from muscle tension and allows for a deeper sleep. Massage weekly is expensive but does give him some relief.
6. [No response provided.]

Patient Group Responses
1. The only other drug that prescribed is Riluzole.

Patients and Caregivers Question 7, Patient Groups Question 11:
If the patient has tried the drug under review, please tell us about the effects they experienced.

Patient Responses
1. After 4 days being on infusion I had less fatigue, less muscle ache and less spasm/cramps, but after ending the first 14 days of it, I began to have increase back to baseline of those symptoms after being off the edaravone for 10 days. It appears to be helping me with the disorder. I am shocked that Canada is looking at paying $190,000/year for this med. I brought it in from Japan and it is also available through India at less than $10,000/year. Same company, same drug, same packaging. At some point we need to not be gouged by pharma because of my illnesses.
2. [No response provided.]
3. I have been on the medication since April 2018. There have [been] no side effects. As mentioned in previous question, the progression of the disease in the view of my neurologist is slow, so hopefully the medication has been effective.
4. [No response provided.]
5. Naffected [sic] part was not getting worse also not getting better.
6. No.
7. [No response provided.]
8. N/A
9. No, haven't tried it yet.
10. I have not.
11. No drugs available.
12. Have not tried it as it seemed too complex a process to access it and try to arrange for infusions at our local hospital.
13. [No response provided.]

Caregiver Responses
1. No, was not available.
2. No side effects noticed.

3. N/A.
4. s.22 hasn't tried this drug due to the prohibitive cost and the fact that it simply wasn't available or approved in Canada. That situation is changing now, so I expect he will reconsider trying it.
5. We have not tried it as we could not figure out how to access it or have it administered. There was only sketchy information from fellow patients on Facebook support sites, which of course is not a reliable source of information and medical advice.
6. [No response provided.]

Patient Group Responses

1. [No response provided.]

Patients and Caregivers Question 8, Patient Groups Question 12:
How can patients benefit from using the drug under review?

Patient Responses

1. Using edaravone has relieved symptoms, is expected to extend my life by 30–50%. Less muscle ache, fatigue, and spasms. Given me some well-founded hope.
2. [No response provided.]
3. Hopefully the medication will continue to slow progression of the disease, which in turn will improve my quality of life and prolong my independence. My sense of well-being is very important, but I do not expect improvement in my condition as such.
4. Relief of some symptoms, more independence.
5. Definitely improve[ment] in quality of life and well being.
6. It is supposed to extend my lifetime for an undetermined amount of time. It will not change the symptoms or improve upon anything. Only slow down the rate of deterioration.
7. A longer life.
8. Would be alive longer to spend time with my s.22 family.
9. I understand that edaravone can possibly reduce the speed at which ALS symptoms develop. I would welcome this! In fact, I would have loved to start using edaravone a couple of years ago when I still had a lot of functionality. But even in this middle stage of the disease, to retain some of the abilities that I still have would be fantastic. To continue to be able to use my left hand to feed myself and clothe and bathe myself would be wonderful. To continue to be able to eat and talk would add so much to my remaining years. I don't really walk now, I just sort of shuffle, but that was still good enough last Saturday to be able to join my s.2 on a short walk along a trail. Retaining these abilities would be a priceless gift.

10. Relief of existing symptoms or at the least delaying the onslaught of the symptoms, which would result in improvement in the quality of life. This is a very difficult disease to deal with; any kind of relief is very much welcomed. There is a cure coming down the road—hope to be here when it arrives.
11. Slow the progress of the disease. I hope that this drug affords a few more years to spend with my family. At the rate of my disease is progressing I will not be able to get around by spring.
12. I would hope that it would prolong my life or at least give me more time until respiratory failure. What we really want is a cure, but it seems to be off in the distant future and unfortunately not in my lifetime.
13. This new drug does not affect existing symptoms. It is thought to decrease the progression rate of the disease. I would certainly like to maintain my current level of function!

Caregiver Responses

1. If used at right time of disease process it can improve longevity and reduce suffering.
2. Slowing the progression of the disease.
3. All of the above. If symptoms are slowed the person is able to function at a higher level. Eating may still be possible instead of being tube feed. Speech may be possible, therefore communication so much easier. We can't have a conversation at all as his speech is unintelligible. Everything must be written or typed out. o frustrating and impossible to have an in depth conversation. Lack of speech is very isolating and leads to loneliness. Makes it hard to know what his needs are. Mobility would be better, independence could be maintained for longer period, less care needed from others. Mental health would be much improved. Emotions are affected by the disease itself and also the fact of having a terminal disease leads to depressions, labile emotions etc. Overall the quality of life would be so much better for a longer period of time, and allow more time, and a better time.
4. Slowing down the progression of disease has to be the major benefit for s.22 Radicava is not a cure, but it could buy him more time to live with hope and, possibly, to benefit from more effective treatments that could move ALS patients in the direction of a cure. I am not sure whether Radicava offers condition improvements, but that sure would be encouraging if it does!
5. Any chance at slowing the progression or hopefully eventually curing the disease would give us hope. There is no other alternative Disability followed by death over the course of 3–5 years is the natural course of the disease. Stephen Hawking was an outlier in many ways and folks who have him in their mind as the example of an ALS patient do not have an accurate picture of how relentless and rapidly this condition deteriorates.
6. [No response provided.]

Patient Group Responses

1. It provides an option, and we do not know if it will help but with only one other option, it provides choice. Imagine being diagnosed with a terminal illness and then told there is no drug available to try. This forces Canadians to take desperate measures and travel to other countries where therapies are available at great expense.

Patients and Caregivers Question 9, Patient Groups Question 13:
Are there any additional factors you would like PharmaCare to consider during its review of this drug?

Patient Responses

1. This drug helps some people but not everyone with ALS. It appears to be helping me and my family.
2. [No response provided.]
3. The literature suggests this drug slows disease progression, in an incurable disease. It has been well tolerated in my personal experience. With the PICC line in place and a minimum of training home treatment is quite easy. Hopefully by using the medication I can be less of a burden to my family and the healthcare system.
4. I would be willing to try this drug.
5. [No response provided.]
6. Using the drug involves attending a hospital for infusions for two weeks per month. If one lives in a rural area this means finding accommodation in the nearest city plus maintaining one's home. Terribly expensive and inconvenient. The [clinic name redacted] clinic has advised me that they have no way of accurately assessing whether the use of the drug has made any difference to their patients. That is the main reason I am not planning to use it.
7. [No response provided.]
8. [No response provided.]
9. I hope that PharmaCare considers the fact that this is only the second drug ever found to be beneficial for ALS patients like me. You chose to not cover the costs of riluzole, I'm not sure why. But edaravone offers much more hope to people like me, as it can reduce the speed of progression of the disease. With all the cancer treatments and other medicines that PharmaCare does cover, surely now is the time for a significant investment in the health of people with ALS. I totally understand the fact that this is a very expensive drug. Without Pharmacare's help most of the people with ALS will never be able to afford it. Please also consider the fact that I along with most people with ALS have had a healthy life and have asked little from the health care system. But when ALS strikes, we need all the help we can get.
10. I don't know how the medication will react on me (it is a unknown as with a lot of mediations). This drug seems to be an improvement on the other drug approved for ALS. The cost is very important; it's my hope that the average person with ALS will be able to afford the medication. My other hope is that I would be able to have the medication administered locally. Thank You.
11. All that I hope is that gives me a chance to live a bit more and if side effects are mild it is worth the risk
12. Currently there is no cure for ALS. At least with cancer, there is a possibility of remission or recovery. This is a relentless disease that is gradually shutting down all my muscles and will unavoidably result in my death in the next 3–5 years. Before that, I will experience progressive disability that affects every aspect of me and my family's life. If there is even the remote chance of a medication helping delay the disease progression, it should be made available to patients. We have no other options

13. This is currently the best available drug for treatment of [motor neuron disease]. Riluzole, which was developed over twenty years ago, is expensive and has very limited efficacy. Please give me some hope.

Caregiver Responses

1. The cost of $190k/year is outrageous. Tell them you will pay them 10% of that or they can get stuffed, we will encourage and facilitate people getting the med from offshore.
2. [No response provided.]
3. [No response provided.]
4. Right now I believe Radicava is given by infusion in a hospital setting. If a pill form could be developed, or a quicker way to deliver it in the home setting, that would be really something. Quality of life would really be enhanced as the patient could live a much more normal life, maybe even working some if symptoms allow.
5. [No response provided.]
6. [No response provided.]

Patient Group Responses

1. We encourage PharmaCare to proceed. We need to take a proactive approach to supporting people living with ALS.

Appendix A
Number of Eligible Questionnaires

In order for a response to be eligible for inclusion in this report, the respondent

- must meet the eligibility criteria in question one of the questionnaire;
- must answer all mandatory questions in the personal information and conflict of interest sections of the questionnaire;
- must answer at least one drug-related question; and
- must, if responding on behalf of a patient group:
  - identify the patient group they are representing; and
  - ensure that the patient group has registered with the Ministry of Health to provide input.

Patients

13 patients provided input to the Ministry for its review. These met the criteria listed above for inclusion in this report.

Caregivers

7 caregivers provided input to the Ministry for its review. Of these, 1 was excluded because they did not answer at least one drug-related question. We have included all information from the other 6 responding caregivers in this report.

Patient Groups

2 patient groups provided input to the Ministry for its review. Of these, 1 was excluded because they did not answer at least one drug-related question. We have included all information from the other responding patient group in this report.

Patient Input for nusinersen (SPINRAZA™) 3620 for the treatment of spinal muscular atrophy from Patients, Caregivers and Patient Groups

Prepared for

Members of the Drug Benefit Council (DBC)

DBC Meeting

Meeting held: TBD

Number of Eligible Questionnaires

Patients (9), Caregivers (7), Patient Groups (1)

Participating Patient Group

The following patient group, which met the criteria for inclusion in this report, provided input to the Ministry of Health for its review:

s.22

Source

Your Voice website: questionnaires posted from October 24, 2018 to midnight on November 21, 2018.

Conflict of Interest Declarations

Patient Responses

The patients who completed a questionnaire reported no potential conflicts of interest.

Caregiver Responses

The caregivers who completed a questionnaire reported no potential conflicts of interest.

Patient Group Responses

The patient group who completed a questionnaire reported no potential conflicts of interest.

The PharmaCare Drug Information Page

Patient Responses

The patients who completed a questionnaire reported they had read the PharmaCare Drug Information page.

Caregiver Responses

The caregivers who completed a questionnaire reported they had read the PharmaCare Drug Information page.

Patient Group Responses

The patient group representative who completed a questionnaire reported they had read the PharmaCare Drug Information page.

Responses to Drug-related Questions

Patients and Caregivers Question 5, Patient Groups Question 9:
Describe how the medical condition or disease which the drug under review would be used for affects the day-to-day lives of patients.

Patient Responses

1. This drug will hopefully stop the progression and I will continue to contribute to society.
2. It would be used to improve function which would help to reduce barriers in my life and burdens to care.
3. My day-to-day life is extremely limited in it's scope. I have SMA type 2 and it has taken my ability to do 99% of things physically and I must rely on caregivers to do everything like feeding, bathing, and dressing. This drug could help me gain some independence back, or at least prevent my condition from worsening and losing what little motor function I still have.
4. It would stop my progression and make me stronger. By allowing me to have access to this drug it will make me a more productive Canadian citizen. It will increase my ability to go to school, get a job and better Canada. Without this drug none of this can happen.
5. The medication could stop my condition from worsening.
6. Spinal Muscular Atrophy affects my every day-to-day life. Literally, every single moment. As someone with SMA Type II, I have experienced degenerative downfalls, and declines in comparing my mobility as a child to a s.22 I cannot roll over

on my own, I cannot transfer, use the bathroom, cannot use my legs or bear any weight, I cannot raise my arms at all. My list could go on to describe just how much SMA affects my every day, but it would be an essay. I have care aids to aid in in all personal, professional and recreational aspects of the life I lead.

7. My muscular and respiratory system are compromised and my quality of life is significantly affected. I fatigue easily and am not able to obtain regular employment. My social life is also affected and my disability hinders my ability to participate in activities with my peers.
8. Because of this disease I am completely dependent on other people. I require constant medical care, and it leads to many secondary complications. Most notably limiting my respiratory health. It also effectively shortens my life expectancy.
9. Spinal muscular atrophy affects every aspect of my day to day life. Physically I can barely move. I need help with everytcdom [sic] going to the bathroom, put on clothes, eating food, and rely on a machine to breathe at night. The last few years have been so rough s.22 even that was affected as I was even too weak s.22

Caregiver Responses

1. Nusinursen would improve the strength s.22 and provide more opportunity to participate successfully in more activities and independence. Also to reduce the level of daily care required over a life time.
2. This drug quite simply will save his life. His ability to breathe is becoming more compromised, any halting of motor function would be live saving. Also, if he loses use of his hand/finger function he will no longer be able to drive his wheelchair and he will not be able to communicate on his computer, which is his connection to the world.
3. For more mobility.
4. This drug can help SMA patients getting stronger having less [breathing] issue and getting more independent.
5. Spinraza has helped and continues to help s.22 on a daily basis. s.22 is part of the trial and has received 8 doses to date. Things that are not mentioned which I have noticed are her fine motor strength has improved so much that she can now s.22 s.22 She can grab, turn and hold a door knob and using her other hand to drive her power wheelchair back to open a door. Liquids needed to be thicken as she used to be at risk of aspiration. She now can drink all liquids without any thickening. She has more confidence and endurance.
6. [Patient's name] is s.22 young adult man. Despite his physical limitations due to Spinal Muscular Atrophy he leads a very full life with the help of his caregivers, family and friends. If he was able to gain back some of his muscle control and abilities this would improve his quality of life enormously.
7. s.22 old who was diagnosed with SMA at age s. and I am submitting this patient input on her behalf. [The patient] has below normal strength and stamina, particularly regarding mobility. She's precarious when she walks and falls quite frequently and can't go very long distances, she requires help with anything that requires bending knees, the squatting motion or any thigh strength. Getting up and down stairs, picking things up from the ground, carrying s.22 moving onto the toilet, and dressing herself is particularly taxing for her. When tired, her needs increase and s.22 needs to be with her almost at all times, as she gets frustrated with her lack of function. [The patient] requires fixed ankle orthotics at night to help prevent

contractures as she grows. Maintaining a healthy diet is incredibly important and is a constant concern as she functions best with consistent caloric intake throughout the day. This necessitates healthy snack options being available at all times and constant management of her balance in diet. SMA patients have particularly sensitive immune systems and the muscle deterioration accelerates if they get sick, including catching common colds/flus from others. We have to be extra mindful to keep s.22 very healthy and away from situations where there could be these types of germs. s.22
s.22

Patient Group Responses

1. SMA patients experience a continual decline in strength, abilities, lung function, and eventually may die from these losses. Their quality of life declines, their ability to hold jobs, maintain community and often have their emotional and mental health affected from this loss. The disease also greatly affects the family members negatively due to the heavy burden of care and worry that comes with this disease.

Patients and Caregivers Question 6, Patient Groups Question 10:
What drugs or other treatments has the patient used, either now or in the past, to treat the medical condition or disease which the drug under review would be used for?

Patient Responses

1. None.
2. [No response provided.]
3. There is no other effective treatments like this new drug. It is a miracle for those of us it can help.
4. I haven't used any other drug.
5. None.
6. There are no other drugs available to treat my disease. All interventions have been to aid in keeping myself as stable as possible (physio, respiratory maintenance i.e. bipap) but there are zero drugs or interventions to treat Spinal Muscular Atrophy.
7. Spinraza has been the ONLY drug to date that can treat SMA. From individuals who have received this drug, the evidence is overwhelmingly positive. Their quality of life has significantly improved and they have experienced fewer health complications caused by SMA.
8. There are no other drugs or treatments for this condition.
9. I have been doing physiotherapy my whole life and am still getting weaker. It's so hard to keep up with when you can't get any better, only a little less worse. I have tried so so many drugs for the pain and suffering associated with SMA, all of which just dull things; there hasn't been any other treatment until now to try for SMA itself.

Caregiver Responses

10. No drugs. Physiotherapy weekly.
11. No treatment has ever been available.
12. N/A
13. Spinraza is the only one he used and I can see the improve after treatment.
14. [No response provided.]
15. [The patient] is on a daily regimen of antibiotics, lung medication, creams, etc. They only help to make his day to day life tolerable. There is no other medication like Spinraza that would help him at this point.
16. s.22

s.22 Otherwise, she eats a healthy diet and takes Vitamin D.

Patient Group Responses

1. There are no other drugs or treatments that are or have ever been available for Spinal Muscular Atrophy.

Patients and Caregivers Question 7, Patient Groups Question 11:
If the patient has tried the drug under review, please tell us about the effects they experienced.

Patient Responses

1. No I have not.
2. [No response provided.]
3. [No response provided.]
4. N/A
5. I have not tried it.
6. I have not been able to access the drug under review.
7. [No response provided.]
8. [No response provided.]
9. N/A

Caregiver Responses

1. [No response provided.]
2. [No response provided.]
3. N/A
4. He can sit on the chair longer (from 15 mins improve to 1 hr). More independent, can be off bi-pap longer (from 7 hrs to 9 hrs) and less suction, less serious medical situation happened.
5. Please see my response to Question 5. In addition, since being on the drug trial s.22 has never been hospitalized (thankfully!). Whenever she is sick, we have been able to manage it at home to bring her back to health.
6. No, he hasn't, as it has not been approved for Spinal Muscular Atrophy Type 2.
7. Beginning day one after treatment, we noted significant increases in [the patient's] stamina, strength, quality of sleep, and confidence in independent movement. Examples include: Notable increase in comfort when sitting. Markedly less bracing with one or both hands or need to lean on her elbows. This allows her to engage in different activities which require both hands to be used, or even such things as sitting s.22 in the bath without fear of falling into the water. Prior to treatment, she originally needed help to lift her knee and move each foot when climbing our local s.22 climbing wall. 1 month after treatment she

climbed the same wall on her own, with no assistance. Two weeks following that, she climbed the same wall triumphantly, 7 times in a row! Again prior to treatment, [the patient] would walk about a fourth of a block before falling over and/or getting frustrated by her instability. She'd also get quite tired s.22 Now that the loading doses are complete, she frequently walks 2+ blocks fully unassisted and without falling. Her quality of life and confidence levels are soaring! [The patient] regularly goes to physiotherapy to help build on the success we are seeing with treatments, and when she is not in official therapy, we are both working with her to build strength through s.22 We have seen incredible improvements in her walking, climbing and stamina but we've also seen new skills developing that were not even present before. In particular, she has been beginning to learn how to bend her knees properly in order to go up and down steps on her own. She still needs quite a bit of reminding for proper form, but the fact that she keeps attempting to do it and we see progress if so wonderful. Hopefully one day with continued treatment and practice, she'll be able to walk up and down stairs safely on her own!

Patient Group Responses

1. The effects are dramatic and life changing. Patients are living longer, are substantially stronger, they have gained in their abilities. Patients of the different types and varying ages have experienced a marked improvement in their overall health and abilities. The younger patients are living longer and reaching physical goals that were not reached without treatment. The older patients are gaining physical abilities they had previously lost.

Patients and Caregivers Question 8, Patient Groups Question 12:
How can patients benefit from using the drug under review?

Patient Responses

1. Less Pain, better quality of life, better relations.
2. Improvement to activities of daily living and reducing the barriers in life.
3. Depending on how effective it is on myself, it could radically change my life. If it reversed my condition I would be able to reclaim a lot of quality of life and extend my life expectancy and resiliency in the face of diseases that would be life threatening (chest infections, pneumonia). There is no aspect of my life that would not benefit because my condition affects everything.
4. The drug would decrease my existing symptoms, improve not only my quality of life but also improve my productiveness as a Canadian citizen, improve my condition, improve my long term health. I will be a more productive citizen and contribute to society. Also the drug will decrease the cost of my medical needs yearly.
5. My quality of life would be better.
6. In Spinal Muscular Atrophy Type II there has been stabilization with Spinraza and there has been marked improvements, from tiny to larger, which for those living with SMA can mean everything. I believe, for myself, I could gain strength and more mobility to increase my independence (like rolling in bed, brushing my teeth, even lifting heavier glasses in order to drink water from, increased stamina) to improvements in lung capacity and power to cough, which would be a huge life-saver in terms of battling

anything respiratory and especially pneumonia. s.22 in terms of long term health and well-being, Spinraza would give me opportunities to continue to enjoy life and not in the shadow of fear of decline. I could live longer and with a better quality of life. For myself, s.22 and family. It would give me a real treatment, something to allow me fight SMA instead of always following it's decided path.

7. I would LOVE to be able to take Spinraza as I have heard numerous stories of positive impact. I am not expecting to gain any improvement to my current health and even if this drug would halt any disease progression, I would be elated. This would mean that I could pursue my current dreams and aspirations. I would not be crushed by my deteriorating health and could lead a more meaningful life.
8. This drug is my only chance to reverse or at least halt the degeneration caused by my condition. I think it can greatly stabilize my health, and potentially even extend my healthy lifespan.
9. In the last year alone I have lost the ability to use the bathroom independently and am close to losing the ability to eat independently, as well as many other things. I would hope the drug would allow me to regain some of these things. I think that experiencing hope for the first time in nearly s.22 years may help lessen the overwhelming depression that has been crushing me.

Caregiver Responses

1. Improve independence. Reduce care level.
2. Life saving, improve quality of life and again it is worth repeating it will extend his life.
3. [No response provided.]
4. Sitting longer and less rely on the medical equipment can help his life more independent. He is slowly getting his muscle function back. That also can help him to get a normal life.
5. Please see my responses in the previous questions. I truly believe Spinraza has improved s.22 quality of life, her condition, and overall long-term health and well-being. Her facial expression muscles are stronger. She can do a full smile showing her teeth! She has more energy and strength and does not get fatigued by the end of day. She can do more. s.22 s.22

   s.22 Her voice is much stronger. You can really hear her now in the house. s.22 and can now help out in the kitchen. She makes her own scrambled eggs, stirring the eggs all on her own. Our clinic visits to s.22 Hospital are much faster.
6. The Spinraza helped him gain muscle strength. This would improve his quality of life and specifically with his breathing as the muscle weaknesses compromises his lung health
7. Because of the Spinraza treatment she has received to date, [the patient] is stronger. She is more confident, more able to participate in everyday life, exploring her environment and learning to control her body. [The patient] and her family would benefit from continued use of Spinraza in EVERY way. Continued relief of her diseases progression, hope for continued improvements, improvements in day-to-day life provided by her increased function, and great potential to avoid further health effects of the

disease such as scoliosis, ventilation, hospital stays, etc. Spinraza has given our family hope for [the patient's] future, where previously there was only fear, grief and acceptance of an inevitable decline in function and quality of life. With continued medical treatment and physiotherapy, she has a chance to live not only a normal life but an extraordinary one.

Patient Group Responses

1. From knowledge of patients on treatment, we know these statements to be true:
   - Less time in hospital when ill.
   - Less likely to be hospitalized.
   - Less illness.
   - Quality of life is increased due to the ability to participate in school, community, family activities and jobs. Patients have more endurance, energy, strength to make this possible.
   - Patients have gained skills they haven't previously been able to accomplish or had lost due to the progressive nature of SMA. For example: s.22 patient with type 3 has improved balance, lift his arms up, play catch, has more energy, more ability to concentrate in school s.22 with type 2 is able to open jars with her hands, a skill she has never been able to do. She is able to work for longer periods of time as she has more stamina, less fatigue. She is able to manage self care in the way of catheter and food preparation. In the case s.22 s.22 She has gained the ability to roll, sit for much longer periods of time, she is improving with each dosing.
   - Patients who couldn't crawl, are crawling,
   - Patients who were on G tube are having them removed.
   - Patients are spending a much lower incidence of time in hospital.

Patients and Caregivers Question 9, Patient Groups Question 13:
Are there any additional factors you would like PharmaCare to consider during its review of this drug?

Patient Responses

1. No.
2. Does the drug allow for the patient to start to participate in the community again, can they start to volunteer. Not everyone can go straight back to school or work but the ability to leave your home and participate in the community is of huge benefit too.
3. There is no other treatments for SMA that are effective like this drug. Nothing else is available, access to this drug would be life changing. There are no other options.
4. If you only approve children for this drug, you are harming young adults in your society. Life does not have a price but if you deny a certain age group from the drug you are putting a price on a life. Please don't put a price on my life.
5. [No response required.]

6. This is the only treatment for SMA. Simply put, it would save and extend lives, including my own. That factor alone speaks to the crucial need for Spinraza coverage.
7. There is NO other drug to treat SMA. As an adult with Type 1 SMA who was told that I would not live past the age of three, this drug would provide me with immense hope. I kindly urge you to approve this drug for ALL ages and types as I deserve to have equal access to this drug!
8. THIS IS THE ONLY TREATMENT AVAILABLE. Please approve it for everyone with SMA. There are no other options.
9. This is a life saving treatment and withholding it is punishing patients to death. Me and many other patients are hanging on by a thread, and everyday that we have to continue to go without access to the drug is day where a part of us dies, and we only have so little to start with. Please don't let this go on any longer.

Caregiver Responses

1. Extend life Improve independence i.e., strength: being able to raise arms to brush teeth and comb own hair and bath. Reduce hospital stays and sickness related to hospital stays.
2. [No response provided.]
3. [No response provided.]
4. [No response provided.]
5. This drug does reduce our visits to hospitals. s.22 loves school and is able to attend more days in school. Side effects are acceptable. I think Spinraza has helped stop the progression of the disease and that means a lot.
6. Spinraza is the only drug that I am aware of for the treatment of SMA—so this is specific to this condition.
7. This drug is the only treatment available!! [The patient] has experienced zero negative side effects in her 6 months of use and massive positive results. These results have enabled her to attend a regular s.22 setting, and improves both her day-to-day and future outlook immeasurably.

Patient Group Responses

1. Because there are no other treatments for this disease and we have a small number of patients, please ensure that all of our patients are approved for this treatment, regardless of age or type. All of our patients are progressing daily, all would desperately wanting to improve their quality of life and longevity. The side effects are minimal, its safe and has proof of great benefit for all patients.

Appendix A
Number of Eligible Questionnaires

In order for a response to be eligible for inclusion in this report, the respondent

- must meet the eligibility criteria in question one of the questionnaire;
- must answer all mandatory questions in the personal information and conflict of interest sections of the questionnaire;
- must answer at least one drug-related question; and
- must, if responding on behalf of a patient group:
  - identify the patient group they are representing; and
  - ensure that the patient group has registered with the Ministry of Health to provide input.

Patients

10 patients provided input to the Ministry for its review. Of these, 1 was excluded because they did not answer at least one drug-related question. We have included all information from the remaining 9 patients in this report.

Caregivers

10 caregivers provided input to the Ministry for its review. Of these, 3 were excluded: 1 did not answer the mandatory questions in the personal information and conflict of interest sections of the questionnaire and 2 did not answer at least one drug-related question. We have included all information from the remaining 7 caregivers in this report.

Patient Groups

1 patient group provided input to the Ministry for its review. This met the criteria listed above for inclusion in this report.

Patient Input for tofacitinib (XELJANZ®) 3604 for the treatment of ulcerative colitis from Patients, Caregivers and Patient Groups

Prepared for

Members of the Drug Benefit Council (DBC)

DBC Meeting

Meeting held: TBD

Number of Eligible Questionnaires

Patients (2), Caregivers (0), Patient Groups (0)

Source

Your Voice website: questionnaires posted from October 24, 2018 to midnight on November 21, 2018.

Conflict of Interest Declarations

Patient Responses

The patients who completed a questionnaire reported no potential conflicts of interest.

The PharmaCare Drug Information Page

Patient Responses

The patients who completed a questionnaire reported they had read the PharmaCare Drug Information page.

Responses to Drug-related Questions

Patients Question 5:
Describe how the medical condition or disease which the drug under review would be used for affects the day-to-day lives of patients.

Patient Responses

1. I am on my way to clinical remissions on this drug. As I have exhausted all other available options and failed them, this is the only option I have going forward.
2. Control UC so I can go places without mapping the location of public washrooms available on the way.

Patients Question 6:
What drugs or other treatments has the patient used, either now or in the past, to treat the medical condition or disease which the drug under review would be used for?

Patient Responses

1. Enttvio, remicade, Humira.
2. 5ASA: Temporarily worked. Imuran: Strong side effects of nausea, lost of appetite and constant tiredness. Remicade: The C Protein climb to 221 making it impossible to move. Humira: The C Protein count started climbing and I stopped before reaching the same result as Remicade. Etrolizumab: Stopped working as my body got used to the medicine

Patients Question 7:
If the patient has tried the drug under review, please tell us about the effects they experienced.

Patient Responses

1. My bowel movement has come back to almost normal from 20 or more bloody diarrhoea.
2. I have been taking the drug for 12 days now. Bleeding stopped within 3 days. Diarrhea started after 6 days. I can plan ahead for a trip without mapping the location of public washrooms.

Patients Question 8:
How can patients benefit from using the drug under review?

Patient Responses

1. Relief and restoration of normalcy.
2. Once the temporarily side effects are done with I will have a quasi normal life without the constant scanning for public washrooms, and better night sleep.

Patients Question 9:
Are there any additional factors you would like PharmaCare to consider during its review of this drug?

Patient Responses

1. Easier to use, no infusion, no needle.
2. Being an oral drug instead of infusion or self injection there is less risks of infection. I have not been taking the drug for very long, so I can't give my experience with daily life yet.

Appendix A
Number of Eligible Questionnaires

In order for a response to be eligible for inclusion in this report, the respondent

- must meet the eligibility criteria in question one of the questionnaire;
- must answer all mandatory questions in the personal information and conflict of interest sections of the questionnaire;
- must answer at least one drug-related question; and
- must, if responding on behalf of a patient group:
  - identify the patient group they are representing; and
  - ensure that the patient group has registered with the Ministry of Health to provide input.

Patients

2 patients provided input to the Ministry for its review. These met the criteria listed above for inclusion in this report.

Caregivers

No caregivers provided input to the Ministry for its review.

Patient Groups

No patient groups provided input to the Ministry for its review.

Patient Input for efinaconazole (JUBLIA™) 3610 for the treatment of onychomycosis from Patients, Caregivers and Patient Groups

Prepared for

Members of the Drug Benefit Council (DBC)

DBC Meeting

Meeting held: TBD

Number of Eligible Questionnaires

Patients (2), Caregivers (0), Patient Groups (1)

Participating Patient Group

The following patient group, which met the criteria for inclusion in this report, provided input to the Ministry of Health for its review:

s.22

Source

Your Voice website: questionnaires posted from December 19, 2018 to midnight on January 16, 2019.

Conflict of Interest Declarations

Patient Responses

The patients who completed a questionnaire reported no potential conflicts of interest.

Patient Group Responses

The patient group who completed a questionnaire reported the following potential conflicts of interest:

The PharmaCare Drug Information Page

Patient Responses

The patients who completed a questionnaire reported they had read the PharmaCare Drug Information page.

Patient Group Responses

The patient group representative who completed a questionnaire reported they had read the PharmaCare Drug Information page.

Responses to Drug-related Questions

Patients Question 5, Patient Groups Question 9:
Describe how the medical condition or disease which the drug under review would be used for affects the day-to-day lives of patients.

Patient Responses

1. Onychomycosis (fungal infection of the toenails): have tried tinactin for forty years, never a cure but helped to stop spreading.
2. I suffer from toe nail fungus which appears to reoccur without the use of Jublia.

Patient Group Responses

3. Onychomycosis, commonly known as toenail fungus, is a fungal infection that gets in through cracks in nails or cuts in skin and is estimated to account for up to 50% of all nail problems. Patients experience pain, changes in colour of the nail or a thickening of the nail. Because toes are often warm and damp, fungus grows well there. Left untreated, it can spread to other toenails, skin, or even fingernails.
   - "I have had toenail fungus for about 15 years. My symptoms include thick, discoloured, brittle nails. I completely avoid wearing any type of sandal...keep nails always covered to hide them."

   Infected nails are usually thicker than normal, could be warped or oddly shaped and can break easily. Nails with fungus might look yellow. Sometimes a white dot shows up on the nail and then gets bigger. When fungus builds up under the nail, it can loosen and even separate the nail from the bed. The fungus can also spread to the skin around the nail."
   - "I have had a toenail fungus in my left great toe for 3 years, at first it didn't bother me but as it got thicker it started to cause pain and pressure which made it difficult for me to wear certain shoes. It was also unsightly and I did not feel comfortable wearing sandals in the summer or to bare my feet at yoga class."

   Proper detection, diagnosis and early treatment is key to prevent long-term damage to the nail. Onychomycosis can be misdiagnosed for other conditions, including nail psoriasis. Patients with diabetes, athlete's foot, or a weak immune system, who smoke, or whose family members have it, are also at a higher risk. Like many patients living with a visible skin condition, onychomycosis has many psychological and social impacts. Toenail fungus infections can be emotionally distressing and have a negative effect on someone's quality of life. Many patients are self-conscious about the appearance of their nails and report that they stop activities such as yoga or swimming so that others will not see their toenails. From our survey, 66% reported being self-conscious of their appearance and 55% reported embarrassment.
   - "It has been one year since my nails turned colour and thickened. Embarrassed to walk in sandals in public."
   - "Had yellow, thick, crumbling nail with thick debris underneath. Did not impact physically. Definitely mentally though. Very self conscious. No open toe footwear or barefoot."
   - "Self conscious and worried about spreading it to family."

Patients Question 6, Patient Groups Question 10:
What drugs or other treatments has the patient used, either now or in the past, to treat the medical condition or disease which the drug under review would be used for?

Patient Responses

1. N/A
2. The jury is out on the efficacy of Jublia, however it seems to work for me. I am also treating the issue with Vicks vapor rub (suggested by my doctor).

Patient Group Responses

1. Common treatments for onychomycosis include topical treatments, oral treatments and physical treatments. Some patients discussed the use of alternative therapies such as Vick's VapoRub, oil of oregano, olive leaf, apple cider vinegar, coconut oil, tea tree oil, grapefruit seed extract. One patient even mentioned soaking her feet in water diluted with listerine and/or bleach. None of these treatments were reported as the cure for onychomycosis.

   Topical treatments are applied directly to the affected nails such as Penlac which is a nail lacquer. The common side effects of topicals is usually redness and skin irritation around the toenail. Topical treatments such as Jublia can provide an advantage in the treatment of people with diabetes with onychomycosis due to their lack of systemic absorption and drug interactions (Joseph W. Onychomycosis in the patient with diabetes. Diabetic Microvascular Complications Today. 2005(Jul/Aug):25-27).

   Oral treatments are often prescribed for severe fungal nail infections that affect the nail root or matrix, and can be toxic to the liver. Other common side effects include headaches, skin rashes and digestive issues. These drugs are Lamisil and Sporanox.

   Physical treatments include laser treatment and removal of the infected nail. Both of these options are expensive and usually need to be used in conjunction with other treatment to actually treat the fungal infection.

   From our survey, approximately 1/3 had tried topicals (other than Jublia), 1/3 tried natural health products (or alternative therapies) and 1/3 had tried laser treatment. There was not a question in the survey asking the reason for this therapy choice but it could be depending on whether or not the patient visited a dermatologist or a podiatrist. All of them reported that nothing was effective and that the laser treatment was very expensive.

Patients Question 7, Patient Groups Question 11:
If the patient has tried the drug under review, please tell us about the effects they experienced.

Patient Responses

1. No.
2. Toe nail fungus does not recur.

Patient Group Responses

1. Eight of the nine respondents to our survey have tried Jublia to treat their toenail fungus infections. The patients who responded to our survey stated that they accessed Jublia through private insurance (25%) or through their health care professional (75%) and paid for the treatment themselves. Only 1 of the 8 reported redness around the nail as a side effect and the others stated that they did not experience any side effects. In terms of effectiveness, respondents reported some success using Jublia, sometimes in conjunction with other therapies:
   - "It actually completely got rid of the fungus, there is no sign of fungus at all."
   - "Resolve of the fungal infection in conjunction with laser treatments."
   - "Nail was clearing."

   The good news is that all 8 respondents with experience with Jublia stated that there no symptoms that Jublia did NOT manage as well as previous treatments they had tried, except for the thickening of the nail.
   - "It's managed the discolouration so far but the thickening of the nail still kind of persists. Hoping it goes away with time.'

   In terms of how easy this drug is in comparison to previous therapies, the six respondents to this question stated that it was easier or the same to use. All respondents also stated that Jublia should be covered by provincial formularies for the indication of onychomycosis:
   - "If oral treatments are covered then the topical should be too. Indicated if less than 60% nail involvement so if treated early no need for more harmful oral medications."
   - "Yes! Or at least have a special authorization option for people with liver function issues."
   - "Why is it not medical problem and as a diabetic could become a bigger problem as I am unable to cut my nails."

Patients Question 8, Patient Groups Question 12:
How can patients benefit from using the drug under review?

Patient Responses

1. Possible cure.
2. It appears to prevent the recurrence of toe nail fungus.

Patient Group Responses

1. "Onychomycosis is a risk factor for ulceration and subsequent amputation in patients with diabetic foot disease[i, ii]. Diabetic complications, such as peripheral neuropathy is a strong risk factor for onychomycosis which in turn leads to a higher rate of secondary infections. The broken skin allows a portal of entry to bacteria which can lead to infections, such as cellulitis or osteomyelitis that may lead to gangrene and amputation[iii, iv].

   i. Canadian Diabetes Association (CDA). 2013 Clinical Practice Guidelines for the Prevention and Management of Diabetes in Canada. Can J Diabetes. 2013;37(S1)227. Available from: http://guidelines.diabetes.ca/App_Themes/CDACPG/resources/cpg_2013_full_en.pdf.

   ii. International Diabetes Federation (IDF). Clinical Practice Recommendations on the Diabetic Foot 2017. 2017. Available from: www.idf.org/e-library/guidelines/119-idf-clinical-practice-recommendations-on-diabetic-foot-2017.html.

   iii. Gupta AK, Humke S. The prevalence and management of onychomycosis in diabetic patients. Eur J Dermatol. 2000;10(5)84.

   iv. Botros M, Kuhnke J, Embil J, Goettl K, Morin C, Parson L, et al. Best practice recommendations for the prevention and management of diabetic foot ulcers. In: Foundations of Best Practice for Skin and Wound Management. A supplement of Wound Care Canada; 2017. 68 p. Available from: www.woundscanada.ca/docman/public/health-care-professional/bpr-workshop/895-wc-bpr-prevention-and-management-of-diabetic-foot-ulcers-1573r1e-final/file

   Access to a treatment like Jublia could help these patients as well as others.

Patients Question 9, Patient Groups Question 13:
Are there any additional factors you would like PharmaCare to consider during its review of this drug?

Patient Responses

1. N/A
2. None.

Patient Group Responses

1. Patients told us that the outcomes that should be considered when evaluating new therapies. Our survey respondents indicated that they are looking for the following improvements:
   - Quick results so that I am as good as new in the end.
   - A permanent cure.
   - To have healthy thin, nails.
   - Manage it if not cure it.
   - I expect the discolouration to go away and for my nail to look a normal colour and texture.

   From the on-line discussion board, others stated that they would like less time on treatment (it usually takes 12-18 months to completely heal) and no recurrence of the onychomycosis (which is common.) Jublia could help patients with these expectations.

Appendix A
Number of Eligible Questionnaires

In order for a response to be eligible for inclusion in this report, the respondent

- must meet the eligibility criteria in question one of the questionnaire;
- must answer all mandatory questions in the personal information and conflict of interest sections of the questionnaire;
- must answer at least one drug-related question; and
- must, if responding on behalf of a patient group:
  - identify the patient group they are representing; and
  - ensure that the patient group has registered with the Ministry of Health to provide input.

Patients

Two patients provided input to the Ministry for its review. These both met the criteria listed above for inclusion in this report.

Caregivers

No caregivers provided input to the Ministry for its review.

Patient Groups

One patient group provided input to the Ministry for its review. This met the criteria listed above for inclusion in this report.

Patient Input for Antithrombotics for PCI Review 3632
for patients who have undergone percutaneous coronary intervention
from Patients, Caregivers and Patient Groups

Prepared for

Members of the Drug Benefit Council (DBC)

DBC Meeting

Meeting held: TBD

Number of Eligible Questionnaires

Patients (3), Caregivers (0), Patient Groups (0)

Source

Your Voice website: questionnaires posted from December 19, 2018 to midnight on January 16, 2019.

Conflict of Interest Declarations

Patient Responses

The patients who completed a questionnaire reported no potential conflicts of interest.

The PharmaCare Drug Information Page

Patient Responses

2 of the 3 patients who completed a questionnaire reported they had read the PharmaCare Drug Information page.

Responses to Drug-related Questions

Patient Question 5:
Describe how having undergone PCI or how having atrial fibrillation and having undergone PCI affects your day-to- day life.

Patient Responses
1. Heart issues continue to severely affect daily living through loss of mobility and stamina.
2. [No response provided.]
3. [No response provided.]

Patient Question 6:
Have you ever used any of the following drugs? (Select as many as apply)
- **a. Clopidogrel (PLAVIX, generics)**
- **b. Prasugrel (EFFIENT)**
- **c. Ticagrelor (BRILINTA)**
- **d. Apixaban (ELIQUIS)**
- **e. Dabigatran (PRADAXA, generics)**
- **f. Rivaroxaban (XARELTO)**

Patient Responses
1. Clopidogrel (PLAVIX, generics)
2. Apixaban (ELIQUIS)
3. Clopidogrel (PLAVIX, generics), Ticagrelor (BRILINTA)

Patient Question 7:
Tell us about your experience with these drugs. Was the treatment successful or unsuccessful, and why? How long did you remain on each medication? If you switched to a different medication, why?

Patient Responses
1. Treatment was successful—as far as it was able to be. I was on plavix for about two years and it delayed the need for further intervention.

2. I've been told I will need to stay on Eliquis for the rest of my life.
3. Brilinta was a success, without issues and helped with prevention of re-stenosis, unlike Plavix which I was placed on with one prior coronary stenting which blocked within 2 months. Would have liked to have stayed on Brilinta longer then the one year under special authority. Reapplication was denied. Once I was removed form taking it (no coverage) 2 weeks later I experienced a ocular stroke which took part of my lower sight in one eye. Now taking Plavix regular with out any issues.

Patient Question 8:
What antithrombotic drugs or other treatments have you used, either now or in the past, to manage your atrial fibrillation or as a treatment after PCI? What was your experience?

Patient Responses

1. Plavix. It worked and was well tolerated.
2. [No response provided.]
3. [No response provided.]

Patient Question 9:
Has PharmaCare coverage of certain drugs, or coverage limits (e.g., limits to the duration of coverage or the maximum dosage) affected your choice of treatment regimen? If so, how?

Patient Responses

1. Lack of Pharmacare coverage definitely affects my drug treatment as often new drugs are not covered and are too expensive for my financial abilities. As long as they work no BC resident should be denied a drug due to finances. Health is a right.
2. [No response provided.]
3. Yes. Unable to continue with Brilinta due to limited duration and not allowed (no coverage by PharmaCare) to be back on Plavix. PharmaCare would not cover the medications. I suffered a, ocular stroke. After the Ocular stroke, was put back on Plavix as Brilinta was still denied.

Patient Question 10:
Are there any additional factors you would like PharmaCare to consider during its review of the existing criteria, limitations, and indications for these drugs?

Patient Responses

1. Make these drugs fully covered and available for as long as doctors consider them useful. Do not transfer the costs of not having these drugs to other areas as patients suffer negative outcomes.
2. [No response provided.]
3. A review needs to be done on PharmaCare policies regarding [their] God like authority. If your family doctor as well your Specialist both prescribe a drug, request special authority and have explained the need PharmaCare must comply and cover the drug. [e]Specially for the common drugs. Diabetic drug should be as well all covered. I now have to balance my groceries and medications as Lantus and Humalog are not fully covered.

Appendix A
Number of Eligible Questionnaires

In order for a response to be eligible for inclusion in this report, the respondent

- must meet the eligibility criteria in question one of the questionnaire;
- must answer all mandatory questions in the personal information and conflict of interest sections of the questionnaire;
- must answer at least one drug-related question; and
- must, if responding on behalf of a patient group:
  - identify the patient group they are representing; and
  - ensure that the patient group has registered with the Ministry of Health to provide input.

Patients

Three patients provided input to the Ministry for its review. These met the criteria listed above for inclusion in this report.

Caregivers

No caregivers provided input to the Ministry for its review.

Patient Groups

No patient groups provided input to the Ministry for its review.