

CADTH COMMON DRUG REVIEW

Patient Input

Monoferic (Iron (III) Isomaltoside 1000)
Pharmacosmos A/S

Indication : iron deficiency anemia

CADTH received patient input from:
Crohn's and Colitis Canada
Kidney Foundation of Canada

July 18, 2019

Disclaimer: The views expressed in each submission are those of the submitting organization or individual; not necessarily the views of CADTH or of other organizations.

CADTH does not edit the content of the submissions.

CADTH does use reasonable care to prevent disclosure of personal information in posted material; however, it is ultimately the submitter's responsibility to ensure no personal information is included in the submission. The name of the submitting patient group and all conflict of interest information are included in the posted patient group submission; however, the name of the author, including the name of an individual patient or caregiver submitting the patient input, are not posted.

Patient Input Template for CADTH CDR and pCODR Programs

Name of the Drug and Indication	Monoferric (Iron Isomaltoside 1000 for injection)
Name of the Patient Group	Crohn's and Colitis Canada
Author of the Submission	██████████
Name of the Primary Contact for This Submission	██████████
Email	██
Telephone Number	416-920-5035 ext. 229

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.

www.crohnsandcolitis.ca

Crohn's and Colitis Canada is the only national, volunteer-based charity focused on finding the cures for Crohn's disease and ulcerative colitis, the two main forms of inflammatory bowel disease (IBD), and improving the lives of children and adults affected by these diseases.

Crohn's and Colitis Canada is one of the top health charity funders of Crohn's and colitis research in the world, investing over \$122 million in research since our founding in 1974. The organization also delivers on its promise through patient programs, advocacy and awareness. We help improve the quality of lives today by:

- sharing accurate and reliable information on treatments, research and issues related to life with Crohn's and colitis through website, print materials, webinars and live events;
- increasing public washroom access through the go-here.ca decal and free mobile app;
- raising awareness about these Canadian diseases with bilingual public advertising campaign via TV, print, radio and digital carriers;
- offering kids with Crohn's or colitis camp experience; and
- providing an online peer support program to newly diagnosed people.

Crohn's and Colitis Canada is comprised of approximately 65,000 supporters including volunteers, donors or individuals interested in engaging with the organization. There is no paid membership. Crohn's and Colitis Canada is governed by a national volunteer Board of Directors. The organization has a network of volunteer-led Chapters in 45 communities across the country, offering information, events, fundraising opportunities and encouragement. There are thousands of volunteers from coast-to-coast supporting Crohn's and Colitis Canada's mission.

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.

The Monoferric patient testimonial data was gathered in Canada in June and July of 2019. Crohn's and Colitis Canada asked gastroenterologists to ask patients of theirs who had used Monoferric for anonymous testimonials regarding their experience with Monoferric.

Anonymous survey data, collected regularly since 2015, also informed more general responses regarding disease symptoms and impacts of the disease on quality of life.

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?

IBD is a disabling, life-long gastrointestinal condition that primarily affects working-age Canadians. IBD symptoms include bloody diarrhea, bloating, abdominal pain and fatigue. Most patients experience the isolation, anxiety, and debilitating frequent and urgent bowel movements. This was elucidated in a Crohn's and Colitis Canada 2011 survey where 73% of respondents affected by IBD said they experienced between five to 20 or more bowel movements a day.

IBD patients' disease, particularly when in a flare, can cause frequent and constant bloody diarrhea and malabsorption of nutrients, vitamins, and minerals due to intestinal malfunction. This malfunction can occur when the small or large bowel wall is compromised due to ulceration. Blood loss due to gastrointestinal bleeding (from intestinal ulcers) and malabsorption of iron from nutritional sources can both cause anemia. These are two common occurrences for IBD patients and therefore iron deficiency or anemia is also quite common. IBD patients experiencing these symptoms are often prescribed oral iron supplements or in serious cases, particularly when intestinal malabsorption of iron occurs or iron deficiency symptoms need to be relieved quickly, iron infusion is prescribed.

There is an increased risk of colon cancer with Crohn's disease or ulcerative colitis. During times of active disease, patients spend a lot of time in the bathroom. Even during times of remission, people with IBD feel that they can't be too far away from the bathroom. Blood in the stool and abdominal pain were noted as important aspects of the disease, however bathroom access dominated concerns since it changed people's lifestyle. As one interviewee stated, "when you have to go to the washroom 20 times a day, it impacts everything you do." Another says, "When the disease takes control of your body, you feel very tired. When my large bowel is affected, I get bloody diarrhea quick and practically live in the bathroom. It plays havoc with my head, I can't sleep and I get headaches and other problems as a result."

People living with IBD must limit their activities. The disease makes it challenging to work. "You simply can't lead a normal life of working and going to the office." For others, "Crohn's disease

hampered my ability to earn a living.” Because of the stigma associated with these diseases, it is difficult for an individual to disclose their condition.

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).

Canadians have one of the highest rates of prevalence of Crohn’s disease and ulcerative colitis, however, when compared with other Western countries, there are fewer treatment options available for people with moderate to severe forms of Crohn’s or colitis. Once diagnosed, patients are often prescribed first line treatments that include anti-inflammatory class of drugs (5-ASA, mesalamine) together with corticosteroids used to control flares. For those who are unresponsive or develop a moderate to severe form of IBD, second line treatments usually consist of immune-modulators/immunosuppressants (azathiopurine), sometimes together with corticosteroids and biologics. These classes of medication work to reduce inflammation by suppressing the immune system.

These drugs often work well for those experiencing mild to moderate levels of Crohn’s disease or colitis, but often fail in maintaining remission for those experiencing severe forms. For some patients, these treatments keep their condition in remission for long periods of time at early stages of their disease, and for others using aminosaliclates or immunosuppressants the treatments did not change their symptoms and overall condition.

Most patients do not report experiencing side effects in taking aminosaliclates. Some patients report liver problems arise from taking immunosuppressants (azathiopurine). The majority of patients do report numerous side effects from steroid use. Most common cited effects included mood swings (easily angered or high anxiety), moon face, and weight gain. One interviewee mentioned that Predisone use, with 16 pills a day, made him feel better by 60% but never ended bloody stools. It also led to the development of cataracts in both eyes. The negative impact of steroid use over the long term, including increased mortality and morbidity, is well documented in scientific literature.

For the patients who provided these testimonials, initially these treatments would help to relieve some symptoms but it did not control their symptoms, including the constant and urgent use of the washrooms. Furthermore, none of those interviewed achieved and maintained remission indefinitely.

People experiencing severe forms of Crohn’s or colitis may be prescribed anti-TNF biologics that inhibit immune system molecules involved in the inflammatory pathway.

One interviewee stated that because of the protocol from his private insurer, he had to go through first line available therapies before he could get access to the biologic drug that worked

for him. It took him three years to go through this process also taking a toll on his mental well-being and disease progression.

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?

When considering iron supplement treatments, particularly in the context of IBD patients and the chronic and debilitating nature of their disease, evaluating its impact on helping a patient resume a normal quality of life is paramount. As the common symptoms of iron deficiency (weakness, fatigue, shortness of breath, and poor concentration) compound with other common IBD symptoms (debilitating urgency and frequency of diarrhea, abdominal pain, fatigue, and nausea) a patient's quality of life can be highly compromised.

Patients seek any treatments that can mitigate these symptoms to protect a patient's ability to work productively, attend school and social events, and even basic daily necessities like leaving the house to run errands or have the energy to maintain a household or raise children. Quality of life could be greatly improved in iron deficient IBD patients if their iron levels are stabilized. Although in itself this treatment cannot mitigate all IBD symptoms, this treatment can reduce negative symptoms of the disease that other IBD treatments cannot.

Expediency of reducing symptoms is particularly important for patients who face significant consequences from missing school or work. So, iron infusions add high value to values who require immediate iron deficiency symptom relief. Monoferric's rapid impact compared to other spaced out iron infusion treatments or oral tablets would be highly preferable for this cohort of patient.

Patients, families and caregivers, when choosing iron supplementation therapies, face trade-offs between slower response (oral tablets) but convenience of taking the treatment at home compared to iron infusions in a clinical setting which requires an appointment and potentially missed school or work. This trade-off may be preferable to patients who prioritize immediate results over the inconvenience of traveling to a clinic for infusion.

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? If applicable, please provide the sequencing of therapies that patients would have used prior to and after in relation to the new drug under review. Please also include a summary statement of the key values that are important to patients and caregivers with respect to the drug under review.

Two IBD patients who had received Monoferric infusions provided testimonials to the following questions:

1. How did you like/dislike Monoferric therapy, compared to previously trialed therapies for anemia.

Patient 1: "I found the Monoferric amazing - my body responded to the treatment very well, could not believe how quickly my energy levels returned and held with only one infusion."

Patient 2: "I liked that it was a one-time treatment instead of the previous every 6 to 8 week infusion."

2. What were some of the benefits of Monoferric?

Patient 1: "Single dose - quick infusion time - excellent results."

Patient 2: "Benefit is that it is quick and easy and the effects were noticed within a few days."

3. What were some of the disadvantages of Monoferric?

Patient 1: "Cost, I was lucky enough that benefits covered."

Patient 2: "It is expensive for anyone who does not have coverage. There are not that many clinics that provide it, and if they do it is during work hours, therefore I had to miss work for it."

4. How has receiving Monoferric impacted your life/your family/your caregiver?

Patient 1: "It has allowed me to get back to my daily activity much quicker than in the past and I don't have to worry about going back every 3 weeks to be infused."

Patient 2: "I feel much better than I did before. I still am fatigued, however I can see a big difference compared to how bad it was before."

5. Did you experience any side effects with Monoferric? If so, how did it compare to previously trialed therapies?

Patient 1: "No side effects, I have not experienced any side effects with any treatment."

Patient 2: “Yes, I seemed to have some sort of reaction to it when the infusion first started. My body began to feel like it was burning, my face and ears turned red and I had heart palpitations. The nurse quickly stopped the treatment, gave me benadryl and started the infusion again at a lower speed.”

7. Companion Diagnostic Test

If the drug in review has a companion diagnostic, please comment. Companion diagnostics are laboratory tests that provide information essential for the safe and effective use of particular therapeutic drugs. They work by detecting specific biomarkers that predict more favourable responses to certain drugs. In practice, companion diagnostics can identify patients who are likely to benefit or experience harms from particular therapies, or monitor clinical responses to optimally guide treatment adjustments.

What are patient and caregiver experiences with the biomarker testing (companion diagnostic) associated with regarding the drug under review?

Consider:

- **Access to testing:** for example, proximity to testing facility, availability of appointment.
 - **Testing:** for example, how was the test done? Did testing delay the treatment from beginning? Were there any adverse effects associated with testing?
 - **Cost of testing:** Who paid for testing? If the cost was out of pocket, what was the impact of having to pay? Were there travel costs involved?
 - **How patients and caregivers feel about testing:** for example, understanding why the test happened, coping with anxiety while waiting for the test result, uncertainty about making a decision given the test result.
-

Regular bloodwork to evaluate hemoglobin levels may be request by the prescribing physician to ensure that iron infusions are effectively treating iron deficiency. Blood testing is accessible (limited travel cost to clinics in urban areas, however, rural areas may have limited access to clinics) and covered by provincial health insurance plans. Patients are caregivers are unlikely to have anxiety or uncertainty associated with the test result – the results will likely correspond with the continuation or alleviation of iron deficiency symptoms the patient will experience in the days following infusion.

8. Anything Else?

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

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.

Crohn's and Colitis Canada did not receive any direct help in writing this submission. However, a Medical Science Liaison from Pfizer (Monoferric's manufacturer) did provide a briefing to explain the mechanism of action, the intended use for IBD patients, the reasons why an IBD patient may have iron deficiency, how the product is different from oral iron tablets and other iron infusion treatments, and details covered in Monoferric's product monograph.

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.

Crohn's and Colitis Canada solicited the help of Canadian gastroenterologists who had prescribed Monoferric to their IBD patients to ask for anonymous patient testimonials regarding their experience with the treatment. One gastroenterologist agreed and provided the feedback of two IBD patients, as detailed in this submission.

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
Pfizer Canada Inc.				X
Janssen				X
AbbVie				X
Merck				X
Takeda				X
Roche		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: Mina Mawani
 Position: President & CEO
 Patient Group: Crohn's and Colitis Canada
 Date: July 17, 2019

Patient Input Template for CADTH CDR and pCODR Programs

Name of the Drug and Indication	Monoferric (Iron (III) Isomaltoside 1000), Iron deficiency anemia.
Name of the Patient Group	The Kidney Foundation of Canada
Author of the Submission	██████████
Name of the Primary Contact for This Submission	██████████
Email	████████████████████
Telephone Number	416-566-1145

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.

The Kidney Foundation of Canada is the national volunteer organization committed to eliminating the burden of kidney disease through:

- Funding and stimulating innovative research for better treatments and a cure;
- Providing education and support to prevent kidney disease in those at risk and empower those with kidney disease to optimize their health status;
- Advocating for improved access to high quality healthcare;
- Increasing public awareness and commitment to advancing kidney health and organ donation.

For more information, please visit: www.kidney.ca

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.

Patient input was collected in June 2019 by the Kidney Foundation of Canada. A self-administered questionnaire to people across Canada was open for two weeks and available in both English and French. The survey was directed at people living with Chronic Kidney Disease and their caregivers and

inquired about respondents' lived experience with chronic kidney disease and medications and expectations for new drug therapies in Canada. The survey posed a number of questions specifically about the drug under review, Monoferric (Iron (III) Isomaltoside 1000). Awareness about the surveys was generated through the Kidney Foundation of Canada's social media channels (Twitter and Facebook). It was also promoted on the Kidney Foundation of Canada's website.

A total of 47 people responded to the survey. Of the respondents that answered the question, 24 (85.7%) respondents identified as being a person living with chronic kidney disease and 4 (14.3%) identified as being a caregiver for a person with chronic kidney disease (n=28). Of the 15 people that responded to the questions about age and time since diagnosis, 1 was aged 25-39, 3 were aged 40-54, 8 were 55-69 and 3 were aged over 70. There were 2 respondents who reported having lived with chronic kidney disease for 3-5 years, 5 for 6 to 10 years, 1 for 11 to 20 years and 7 more than 20 years with the disease.

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?

Kidney disease describes a variety of disease and disorders that affect the kidneys. Most disease of the kidney attack the nephrons and damage their ability to eliminate wastes and excess fluids. Often, kidney disease is associated with other medical conditions such as diabetes, high blood pressure and heart disease.

Chronic kidney disease (CKD) is the presence of kidney damage, or a decreased level of kidney function, for a period of three months or more. Kidney disease can range from mild to severe and in some cases, lead to kidney failure (sometimes referred to as end-stage kidney disease, or ESKD). There are usually no specific symptoms of kidney disease until the damage is severe. When the kidneys fail, wastes accumulate in the body and dialysis treatments or a kidney transplant are needed to survive.

Dialysis is the most common treatment for kidney failure, with kidney transplant being another option. There are two types of dialysis: peritoneal dialysis and hemodialysis. Canadians with kidney failure and their families face significant out-of-pocket costs. This burden is further compounded by the loss of income that is often associated with starting dialysis. It is important to note that poverty is a determinant of health. This means that patients and their families that live in poverty may not be able to achieve optimal management of their medical issues.

In the early stages of chronic kidney disease, self-management strategies such as lifestyle changes; engaging in regular physical activity, maintaining a healthy body weight, stopping smoking and reducing sodium, managing other medical conditions and medications may slow or stop damage to the kidneys.

Most people with moderate-to-severe (Stage 4-5) kidney disease develop anemia. Anemia is a condition commonly caused by chronic kidney disease which is a reduction in the number of red blood cells in the blood. Patients may have a reduction in red blood cells for several reasons: from eating less iron-rich food, by losing small amounts of blood from frequent blood tests, from blood lost in the hemodialysis tubing after each session and due to the effects of chronic disease. Over time, this can lead to anemia. The kidneys make a hormone called **EPO (erythropoietin)** when the body senses a reduction in red blood cells. EPO tells the bones to make more red blood cells. As kidney disease progresses, most people with moderate to severe kidney disease are not able to make enough EPO in response to low red

blood cells. When anemia is mild, there may not be any negative effects on the body. However, as anemia becomes more severe, it may lead to low energy, tiredness, shortness of breath and sometimes, increased sensitivity to cold negatively impacting the quality of life for patients with CKD.

Most survey respondents who participated in the survey spoke negatively of their experience with CKD. Respondents talked about their constant tiredness. They said, “very tired at times, but can’t sleep well either”, itchy skin, tiredness”, “constantly cold, and tired but unable so (sic) sleep are the biggest challenges”, and “energy level low...except when I drain pause the machine to go to the gym for an hour...can’t remember when I had continuous sleep...dry skin patches...need a few naps a day”.

Most respondents also spoke about the impact CKD has on their day-to-day lives, their inability to complete chores without being tired, participating in activities they previously enjoyed such as exercise and missing out on events with their families. Caregivers also spoke about lost time and experiences and the increased level of stress they experience as a result of their family member’s condition.

When asked if there are any particular challenges living with both CKD and anemia, responses were similar to CKD and respondents reported fatigue, shortness of breath and cold extremities. Home patients described the challenges of going to the hospital to receive iron infusions.

Some quotes that demonstrate these challenges are found below:

“Symptoms have blended into each other, the anemia compounds the issues that are presenting from esrd [end stage renal disease](sic). I am taking iron and injections to attempt to compensate for the deficiencies but still encounter the symptoms to varying degrees. Its a good day bad day process at this point.”

“Hard to balance work life and home life.”

“When hemoglobin and iron are low, I have weakness in my legs.”

“I have issues with anemia occasionally and have to go to the unit to get iron infusions. This comes with its own issues trying to get in is troubling seems to be more difficult to fit home patients in to get our iron then the regular in center patients.”

“basically no social life...either no energy or social life like I had before. Can’t remember the last time I had a continuous sleep.”

Many people living with CKD have a number of other conditions. Of the respondents to this question, 79% reported living with high blood pressure, 79% reported high potassium levels, 82% reported having a low hemoglobin and 75% reported having low iron levels.

Below are some quotes from this survey that further illustrate the degree and extent to which chronic kidney disease affects daily living and the quality of life of those with the disease:

“Having Kidney Disease impacts my energy level and prevents me from doing some of the activities such as biking or walking which I had previously enjoyed doing.”

“Still working but dealing with fatigue, loss of breath, itchy skin, lack of sleep, joint pain, cramps.”

“Often tired, have aneurysms in my hands, hands and feet swell and can be painful.”

“I have extreme drops in blood pressure during treatment which makes me miserable for the remainder of the day.”

“I spend about 5 hours a day getting home hemo dialysis, followed by several trips per month to the dialysis unit for supplies, information and check ups. I then have medical several appointments per month for peripheral issues caused be either CKF (sic) or dialysis. My skin itches badly, I tend to bleed easily due to having skin like paper. Physical tiredness is normal w/o (sic) iron infusions and ironically, I can't seem to sleep at night.”

“I am a home dialysis patient (3 years on Home dialysis) and 2 on in center dialysis. My quality of life greatly improved since I started Home Dialysis however, still affects my daily life. After treatment I am unable to sleep and spend a lot of nights awake just thinking about how I could get to sleep. My blood pressure in some instances is low during treatment and this feels awful. Overall it helps make enable me to live a "normal" life tho (sic) it certainly comes with its share of cons.”

“I'm depressed always have anxiety.”

“hubby is on dialysis, since 2018 he does not sleep well, very itchy, unable to do the same chores as before .for me the caregiver it has put a lot of stress on me, from grocery shopping, work around the house to doctor appts (sic).”

“Insomnia lack of energy, stamina unable to do all the physical things I would like to do.”

“Caregiver: My life as youngest son has been one of lost time and experiences due to my fathers illness. I have spent much time at hospitals, clinics, and Dr appointments with my father through all stages including kidney transplant and deterioration of the new kidney to once again needing dialysis.”

“Takes longer to do things , like garden cooking, crafts, have very little sleep, my skin is itchy always scratching it, Have stage 5 of kidney disease.”

“Living with chronic kidney disease has decreased my ability to do many of the daily activities of my normally active life. I get tired easily. I find I need to sleep during the day many times and miss out on events my family attends. I have a general sense of sadness.”

“quality of life has gone down because everything revolves around dialysis I do chores like laundry, grocery shopping etc. but after doing only one of these I feel exhausted.”

“Quality of life has gotten worse because everything revolves around dialysis I feel exhausted after grocery shopping or laundry etc.”

“I feel like I am wasting my life because I had not planned to spend my retirement this way at all... It's hard to accept.”

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).

44% of respondents said they have had dietary changes (increasing iron rich food) either currently or in the past, while 7% did not know. The majority (73%) of respondents have taken a medication for anemia. The medications that had been taken or are currently being taken at the time of survey completion included vitamin or mineral supplements (B12, folic acid, multivitamin formulated for kidney disease) (70%), iron tablets (44%), intravenous iron (65%), intramuscular iron (9%), erythropoietin (70%), blood transfusions (26%), other (4.3%).

There were 25 respondents who answered how satisfied they were with the medication/combination of medications currently being taking for anemia, 13 were “very satisfied” (2) or “satisfied” (11), 4 were neither satisfied nor satisfied and 2 responded that they were “very unsatisfied”. 5 indicated that the question was not applicable to them as they were not currently taking medication(s) for anemia.

Below are some direct quotes to describe what patients like and dislike about their current therapy:

“It takes such a long time to get my hemoglobin up.”

“On dialysis it’s easy to take meds (sic) through IV.”

“I like that I feel, even just for a short time, more like my normal self. I have energy to join [n] family activities I dislike that I have to go to the hospital, sit and wait, usually til (sic) way past appoint time, and that the entire 1.5 he process takes well over 3 or more hours. I am quite capable of doing this at home while on dialysis.”

“Iron supplements make me feel very sick/nauseated.”

“... seems to be helping to keep my hemoglobin good.”

“Not sure I notice much of a difference in symptoms, as I have gone on and off the pills a few times and I don’t feel any different.”

“The costs are astronomical. I don’t know how patients without a job make ends meet.”

When asked about what factors were “very important” or “important” in choosing chronic kidney disease medications in general, the following factors were identified as “very important” or “important” by the majority of respondents: tiredness, interference with sleep, foot edema, effect on mood, interference with other medications, changes in appetite, cost and length of time on the medication.

Additionally, patients when asked what else was important to them when choosing a kidney disease medication said “That it works”, safety and side effects. One respondent further offered, *“The most important factor in choosing a kidney disease medication is whether it will interfere with the possibility of*

getting a kidney transplant. Other important factors are the long term effects the medication will have on other organs such as the liver or the heart.”

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?

When asked about their expectations for new CKD therapies, respondents said the following:

“Hoping that new medications improve so that less invasive measures are needed with less side effects.”

“Need less treatments.”

“Help relive some of the effects of kidney disease on the body.”

“I hope they will help us to feel better and give us some better quality of life.”

“need some more energy. tired of being tired...don't even walk my dog anymore. Lucky my son still lives with me. 10 years and still; awaiting (sic) on a transplant.”

“Improve energy, support more activity and daily movement.”

“I would try other medications or therapies as they become available or would take part in a clinical trial.”

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.

Unable to respond to this question.

7. Companion Diagnostic Test

If the drug in review has a companion diagnostic, please comment. Companion diagnostics are laboratory tests that provide information essential for the safe and effective use of particular therapeutic drugs. They work by detecting specific biomarkers that predict more favourable responses to certain drugs. In practice, companion diagnostics can identify patients who are likely to benefit or experience harms from particular therapies, or monitor clinical responses to optimally guide treatment adjustments.

What are patient and caregiver experiences with the biomarker testing (companion diagnostic) associated with regarding the drug under review?

Consider:

- Access to testing: for example, proximity to testing facility, availability of appointment.

- Testing: for example, how was the test done? Did testing delay the treatment from beginning? Were there any adverse effects associated with testing?
- Cost of testing: Who paid for testing? If the cost was out of pocket, what was the impact of having to pay? Were there travel costs involved?
- How patients and caregivers feel about testing: for example, understanding why the test happened, coping with anxiety while waiting for the test result, uncertainty about making a decision given the test result.

This question is not applicable to this submission.

8. Anything Else?

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

Canadians living with kidney failure face significant financial challenges as a result of dialysis treatment. Starting dialysis often results in a decrease of income at the same time that out-of-pocket costs increase, such as those for transportation to treatment and medication. Government coverage and financial support for people on dialysis varies, resulting in inequalities across jurisdictions. Those living with kidney failure tend to be part of a low-income and high-cost population. In order to address the financial burden for people with kidney disease and to minimize disparities in accessing medications for people with kidney disease, mechanisms need to be developed to offset costs equitably across jurisdictions.

Those living with chronic kidney disease experience additional health and financial challenges. Many would benefit from effective, affordable treatments that they can access equitably and in a timely manner that may help people to achieve better health outcomes and improve their quality of life.

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.

There was no assistance from outside the Kidney Foundation of Canada to complete this submission.

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.

There was no external assistance with data collection or analysis used for this submission.

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
Alexion Pharma Canada Corp		X		
Amgen Canada Inc.			X	
Astellas Pharma Canada Inc.			X	
AstraZeneca Canada Inc.			X	
Boehringer Ingelheim (Canada)	X			
Fresenius Medical Care Canada Inc		X		
Horizon Pharma Inc.				X
Jamp Pharma Corporation	X			
Janssen Pharmaceutical Companies				X
Otsuka Canada Pharmaceutical Inc.				X
Pharmascience	X			
Sanofi		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: Lydia Lauder
Position: National Director, Programs and Public Policy
Patient Group: The Kidney Foundation of Canada
Date: 08 July 2019