

CADTH COMMON DRUG REVIEW

Patient Input

USTEKINUMAB (Stelara)

(Janssen Inc.)

Indication: Ulcerative colitis

CADTH received patient input from:

Crohn's and Colitis Canada

Gastrointestinal Society

September 03, 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	Ustekinumab (Stelara) for Ulcerative Colitis (UC)
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	████████████████████

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 information summarized in this section was compiled from a variety of sources. Information was drawn from Crohn's and Colitis Canada (CCC) published reports, including the 2018 "Impact of Inflammatory Bowel Disease (IBD) Report" , the *Patient and Health Care Provider Input: Non-Medical Biosimilar Switch Policy for Patients with Inflammatory Bowel Disease* report and informational brochures found on the organization's website. Statistical evidence was taken from a CCC 2011 national online survey that explored the physical, social and economic impacts of living with IBD. Over 430 people across the country participated in this survey. A series of interviews and surveys (September 2019) including 13 patients (cross-Canada) managing their ulcerative colitis with Stelara also provided key patient perspective. Also, a library of video vignettes detailing IBD patient perspectives can be found online on our website at:

<http://www.crohnsandcolitis.ca/site/c.dtJRL9NUJmL4H/b.9013439/apps/s/content.asp?ct=14676853>.

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?

UC is a disabling, life-long gastrointestinal condition that primarily affects working-age Canadians. UC 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.

There is an increased risk of colon cancer with UC. During times of active disease, patients spend a lot of time in the bathroom. Even during times of remission, people with UC 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 surveyee 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 UC 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, "UC 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.

Many patients interviewed considered frequency and urgency of bowel movements to be the main aspect of UC that is most important to control. One patient offered that “the simple ability to live life” was the main aspect to potential treatment.

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 ulcerative colitis, however, when compared with other Western countries, there are fewer treatment options available for people with ulcerative colitis.

Most patients do not report experiencing side effects in taking aminosalicylates. 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, acne 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.

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 washroom. Furthermore, none of those surveyed achieved and maintained remission indefinitely.

Participants from surveys shared their treatment experience including the journey toward remission. Many have experienced multiple medications, some over many years. Side effects were varied and in some cases lead to the discontinuation of particular treatment (e.g. pancreatitis; seizures; liver damage).

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?

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 IBD patients if their flares are brought into remission, or indeed managed.

Patients, families and caregivers, when choosing biologic therapies, face trade-offs between infusion and injection - the convenience of taking the treatment at home (self-injection) compared to infusions in a clinical setting with the benefit of close health practitioner monitoring/potential for medical intervention but which requires an appointment and missed school or work. This trade-off may be preferable to patients who prioritize saving time due to the inconvenience of traveling to a clinic for infusion.

Prior to starting treatment with Stelara, many patients reported severe illness and were looking for anything to treat their debilitating symptoms. “[Before starting Stelara] I was looking for something to help – UC for me was absolutely crippling. I was looking for something, anything, to make it stop. I gladly travel to (large city, away from home) to get the treatment. I accept the inconvenience of the distance because of the benefits – gladly.”

Patients were looking for relief and for any improvement in quality of life, including the ability to leave their home for work or social activity. “I went from weeks and months where I didn’t leave my apartment other than to go to medical appointments, no sleep (and depression from sleep deprivation) to going out with my girlfriend and having an actual life only weeks into the Stelara treatment.”

Others were still looking for improved symptom control, but also a more manageable treatment routine. “Previously, I had to take a full day off work to receive my treatment, now time is actually saved with Stelara and they mail it directly to my house and deliver in the evening when they know I will be there to accept it. Was a little frightened of giving myself the needle, but gladly accept it.”

Another patient sums up her experience similarly: “Before starting Stelara, I was looking for a treatment that would take away my symptoms and let me live a normal life. I wanted my urgency to be gone and frequency to be reduced, my blood loss to be gone as well as bad stomach pain. I also wanted to be able to eat without having to immediately run to the washroom. Stelara took away all these symptoms and provided me with a normal and great quality of life! I am able to work, go to school, eat, hang out with friends, and leave the house all day without any issues. Even though I have to go to a clinic once a month because I can’t inject myself, it is absolutely worth it!”

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.

Patients interviewed and surveyed indicated that coverage was generally split between private coverage and compassionate coverage by the pharmaceutical company (BioAdvance). One was part of an ongoing study and another paid out of pocket.

All patients reported to be pleasantly surprised with the ease of use of the medication, though some reported that they paid for nurses at their local clinic to administer the needle. Many were used to taking a full day off work to attend an infusion clinic and were very pleased to be able to avoid this inconvenience, though all indicated that anything that improved their symptoms would be tolerated.

A sample of comments include:

“It is super-convenient and far fewer side-effects. That said, when Remicade was actually working, it was way better in controlling the UC symptoms.”

“I don’t need to go to the infusion centre to get it! I can travel and have unlimited freedom. Very nice nursing staff showed me how to give it to myself. This is a life-changer.”

“Stelara took away all of my symptoms of ulcerative colitis and provided me with the ability to live a normal life and do what I want to do. Of course I have ups and downs but overall it has benefited me so much.”

Patients had poignant and personal stories of “getting their life back” after positive experience with Stelara. These include:

“I work in retail – you can’t just disappear and go to the bathroom all the time. You need to be able to plan (breaks). Now I can plan my day and be a full contributor to the team. I have a sense of security in knowing that I don’t have to worry about going to the bathroom all the time.”

“Any day that I can go out is a good day. I’d love to get to the point where I don’t have to plan bathroom stops just in case. The impact has been the difference between not really living and living.”

“I am able to work, visit family, socialize with my friends and tell people about my life and how well I am doing. I can speak about the things going on in my life because I am able to do them, have new anything, and my illness kept me from succeeding or doing anything. Every aspect of my life has been experiences, and growth as a person versus before using Stelara where I was trapped, and couldn’t do anything. My life has been impacted in a positive way with Stelara, mentally, physically, psychologically, emotionally. I am happy now and can be honest with people instead of putting on a fake smile to hide my illness and symptoms.”

“Treatment for me is mainly about the frequency of going to the bathroom. Before Stelara, I had a lot of blood when I went to the bathroom and it was really, really painful. What people don’t understand is that the pain lasts beyond the bathroom visit. Stelara treatment means the pain is managed and I can plan my day.”

“My expectations in terms of my health are low. I accept the fact that things are never going to be great and I forget what “normal” is. Still have occasional days where I’m not feeling well, but this treatment allows me to live my life productively.”

“I just thought it could help joint pain but it doesn't. But for UC best thing I had in my life.”

“A few weeks before Stelara, I went to see a surgeon for consultation on a colostomy. He said that he'd bump someone to get me in right away because my illness was so severe. I didn't need the surgery after all. I am very happy.”

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.

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 Janssen (Stelara's manufacturer) did provide a briefing to explain the mechanism of action, clinical data, how the product is different from other biologic treatments for UC, and details covered in Stelara'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 ustekinumab to their UC patients to ask for anonymous patient testimonials regarding their experience with the treatment.

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: August 28, 2019

Patient Input Template for CADTH CDR and pCODR Programs

Name of the Drug and Indication	Stelara® (ustekinumab) for ulcerative colitis
Name of the Patient Group	Gastrointestinal Society
Author of the Submission	██████████
Name of the Primary Contact for This Submission	██████████
Email	██████████
Telephone Number	██████████

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.

As the Canadian leader in providing trusted, evidence-based information on all areas of the gastrointestinal tract, the GI (Gastrointestinal) Society is committed to improving the lives of people with GI and liver conditions, supporting research, advocating for appropriate patient access to health care, and promoting gastrointestinal and liver health.

Canadian health care professionals request more than 600,000 of our BadGut® Basics patient information pamphlets each year, and tens of thousands of Canadians benefit from our important quarterly publication, the *Inside Tract*® | *Du coeur au ventre*^{MD} newsletter.

Our free BadGut® Lectures from coast to coast cover various digestive conditions for patients, caregivers, and other interested individuals. We also have dynamic websites in English (www.badgut.org) and French (www.mauxdeventre.org), which has had more than 4,500,000 *unique visitors* in the past 12 months. Organized on a number of topics, GI Society support group meetings offer a wealth of information for those newly diagnosed with a gastrointestinal disorder, as well as those who have lived with a condition for years.

Our highly trained staff and volunteers offer additional patient resources, including responding to information requests and participating in community initiatives. Staff and advisors work closely with health care professionals, other patient groups, and governments at all levels on behalf of GI patients. The GI Society, along with its sister charity, the Canadian Society of Intestinal Research (founded in 1976), has supported a number of significant clinical, basic, and epidemiological GI research.

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.*

This information was obtained primarily through two questionnaires: 1. completed by 133 Canadians (English: 105 and French: 28) with inflammatory bowel disease (IBD), including ulcerative colitis (or their

caregivers or family members). 2. completed by 432 Canadians with IBD, including 180 with ulcerative colitis.

We also had contact with patients affected by IBD through one-to-one conversations at our BadGut® Lectures; a patient roundtable, and recent phone/email/social media interactions with individuals who have Crohn's disease and ulcerative colitis; and stories submitted over time by patients.

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?

Ulcerative colitis is an inflammatory bowel disease (IBD) that can arise at any age, commonly occurring in young people. There is an increased risk for those who have a family member with the condition. Currently, Canada has among the highest prevalence and incidence yet reported in the world, with approximately 120,000 diagnosed individuals. Patients are at a slightly increased risk for colorectal cancer after having ulcerative colitis for about 10-15 years.

The most frequent symptom is diarrhea, often accompanied by cramping abdominal pain. Rectal bleeding, in varying amounts, is common. Low red blood cell count (anemia) can result if diarrhea and blood loss are severe.

Some patients have extra-intestinal manifestations, including fever, inflammation of the eyes or joints (arthritis), ulcers of the mouth or skin, tender and inflamed nodules on the shins, and numerous other conditions. Anxiety and stress are major factors.

Ulcerative colitis often has a profound effect on an individual's life – physically, emotionally, and socially, both at home and at school or in the workplace. It is particularly difficult for children and young adults since it often affects a person's sense of self.

More than anything, patients have told us that sustained remission/treatment response is more important than relieving any one symptom of ulcerative colitis. As a chronic disease, it is never just one flare that dominates the impact of the disease, but the constant concern that there will be future flares, possibly worse than the last, and at unpredictable times, which can disastrously disrupt patients' lives.

In our survey, IBD patients shared similar reports regarding the impact that their disease has on all aspects of their day-to-day lives:

- "I am constantly aware of where a bathroom is and always prepared for the urge to go. My activities are limited for the fear of not being able to find a washroom."
- "My most important concern is the overall fatigue I feel. I am also always very worried when I see blood in the stool. Having to watch my diet is something I never had to do before - seems like I cannot eat much anymore."
- "It makes it difficult to leave my house, play with my son, work, etc. when I am in a flare. When I'm not in an active flare I live in constant fear of when the next flare will occur"
- "It limits my social life; I stay in the house more than I did before. Very tired and weak. Lost 30 lbs, not as strong. Affects overall quality of life. Fatigue limits what I can do in a day."
- "My energy levels have decreased and I get fatigued much more easily, the fear of pain, bleeding, incontinence is horrible. The worst part is fearing the next big flare that will prevent me from being a mom to my 18 month old."

It's one thing to read a list of common symptoms or data on how IBD affects patients, but it is the individual stories of these patients, as summarized above, which astound us and motivate us to support patients' need for more diversity in effective treatments. In addition, treatments should improve quality of life, not cause more symptoms, pain, frustration, or hardship.

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).

The treatment of ulcerative colitis is multi-faceted; it includes managing the symptoms and consequences of the disease along with therapies targeted to reduce the underlying inflammation. Typically, a patient starts on one type of treatment and, if there is inadequate response, then switches to another type.

5-ASA helps to settle acute inflammation and, for some patients, keeps the inflammation inactive when taken on a long-term basis (maintenance). To reduce inflammation in moderate to severe cases of ulcerative colitis, corticosteroids can help. For topical relief in the colon, corticosteroids are available in rectal formulations. These are inconvenient therapies that make it difficult for patients to keep a normal routine. Also, if a patient has significant diarrhea, then the rectal medications may be difficult to hold in place for sufficient time to be effective. Immunosuppressive agents reduce dependence on steroids and help patients who have steroid-resistant disease, but it could take up to six months or more of therapy to see results.

Biologics treat ulcerative colitis when older medications fail to relieve symptoms. However, there are a variety of mechanisms through which they work.

While there are many options available, patients still have a lot of difficulty obtaining remission or adequate symptom relief. In one of our surveys, we asked patients if the currently available medications are adequate to control their disease. Only 28% of those with ulcerative colitis thought that the available medications are adequate. Conversely, 54% found them to be only somewhat adequate and 18% not adequate. Patients are still suffering, and they need new and effective options to achieve mucosal healing and reduce the debilitating symptoms of ulcerative colitis.

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?

Patients affected by ulcerative colitis need access to medications that work. Inadequate access to medication results in preventable patient suffering (e.g., continual, debilitating disease symptoms; secondary illnesses such as depression and anxiety disorders; and loss of family/social interactions). It also leads to unnecessary usage of healthcare resources (e.g., hospital stays, surgeries, diagnostic procedures, other medications) and a ripple effect of financial burden on the government and taxpayers (e.g., through inability to work, long-term disability claims, biologic-related debt, and even bankruptcy).

We know that biologics are effective at treating ulcerative colitis; these medications have revolutionized treatment for inflammatory conditions. In our survey, 63% of respondents reported symptom reduction on a biologic and 23% reported confirmed remission. Many of these individuals had been suffering for years trying to find a treatment that works.

When the ulcerative colitis patient receives the right medication at the right time and for the right duration – as determined between physician and patient – these individuals can live full, rewarding lives as productive, valuable citizens who participate in the workforce and community. However, since patients respond differently to various medications, and in some cases stop responding to medications after using them for some time, it is important to have a variety of options available.

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?

Patients have seen remarkable – sometimes miracle-like – results from biologics when other treatments failed. But not everyone responds to currently available treatments, including biologics. Even if they do, risk remains that one day the treatment will no longer work for them. More options are essential. Biologics come with a number of potential side effects and risk factors, and physicians only prescribe them when they believe these powerful medications are a patient’s best hope of controlling ulcerative colitis.

Stelara®, administered by subcutaneous injection, is a monoclonal antibody that targets the p40 subunit of interleukin-23 and interleukin-12 to prevent their binding to receptors on T-cells and natural-killer cells. This is a different pathway from other biologics and could reach patients for whom existing medications have not worked, or have stopped working.

Stelara® has already been approved for use in patients with Crohn's disease, another type of inflammatory bowel disease, for whom this medication has been very effective.

With moderate to severe ulcerative colitis, Stelara® has the potential to improve the health and quality of life of many individuals currently suffering from ineffective treatments that put an unnecessary burden on them. When other medications don't work well or specific patients cannot tolerate them, Stelara® could be an extremely valuable next step in getting a patient's symptoms under control and inducing and sustaining remission. As one patient currently on Stelara® stated, "It is always good to have hope that there is another option out there for treatment. It is scary when you are running out of options and when whatever you are on is not working."

Each case of ulcerative colitis is unique in that the physician is treating an individual patient, potentially with co-morbidities and influences. What works for one person does not necessarily work for another. Choice among effective treatment options is essential for patients.

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.*

8. Anything Else?

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

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

No.

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

No.

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

Company	Check Appropriate Dollar Range			
	\$0 to 5,000	\$5,001 to 10,000	\$10,001 to 50,000	In Excess of \$50,000
Janssen Inc. 2018				✓
Janssen Inc. 2017				✓

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: Gail Attara

Position: Chief Executive Officer

Patient Group: Gastrointestinal Society

Date: 2019-08-30