

CADTH COMMON DRUG REVIEW

Patient Input

TOFACITINIB (Xeljanz)

(Pfizer Canada Inc.)

Indication:

CADTH received patient input from:

Gastrointestinal Society

July 11, 2018

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Name of the Drug and Indication	Xeljanz® (tofacitinib) for ulcerative colitis
Name of the Patient Group	Gastrointestinal Society
Author of the Submission	██████████
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1. About Your Patient Group

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^{MC}* 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). 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

This information was obtained primarily through a questionnaire completed by 133 Canadians (English: 105 and French: 28) with IBD (or their caregivers or family members). As well, we had contact with patients affected by IBD through one-to-one conversations at our BadGut® Lectures; a patient roundtable, our IBD support group participants, and 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

Inflammatory bowel disease (IBD) is a term that primarily refers to two diseases of the intestines: Crohn's disease and ulcerative colitis. These both involve inflammation of the digestive tract, which is limited to the inner mucosa of the colon in ulcerative colitis, but can include any area of the GI tract and can extend through the entire thickness of the bowel wall in Crohn's disease.

Ulcerative colitis 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 the highest prevalence and incidence yet reported in the world, with

approximately 104,000 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

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. These are large-molecule medications that must be administered intravenously. While they are often very effective, patients must either take injections or attend infusion clinics to take their medication, which makes these medications more difficult to use.

5. Improved Outcomes

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 health care 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).

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.

Xeljanz® is a novel product that consists of a small-molecule Janus kinase (JAK) inhibitor that patients can take orally. Since this medication uses a different pathway and administration method, and works on different cells in the body, it offers an alternative for patients who wish to try another easy-to-use medication before moving to a biologic medication.

6. Experience With Drug Under Review

This product offers a new step in the treatment algorithm. Biologic medications are extremely effective for individuals with ulcerative colitis. However, these medications are very costly and they are only available via injection or infusion. Xeljanz®, a JAK inhibitor, is different from biologics, and could be tried in lieu of proceeding to a biologic. Biologics are large molecules, whereas JAK inhibitors are small molecules and interrupt the signalling pathway from inside the cell involved in the inflammatory pathway. Oral medications greatly improve ease of adherence for patients to a treatment regimen, who can simply take a pill at home rather than make regular trips out to an infusion clinic, or to self-inject at home. It also helps those individuals who have needle aversion or aichmophobia.

Appendix: Patient Group Conflict of Interest Declaration

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
Pfizer Canada Inc. 2018			✓	
Pfizer Canada 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

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