



Common Drug Review *Patient Group Input Submissions*

ustekinumab (Stelara) for Crohn's disease.

Patient group input submissions were received from the following patient groups. Those with permission to post are included in this document.

Crohn's and Colitis Canada — permission granted to post.

GI (Gastrointestinal) Society — permission granted to post.

CADTH received patient group input for this review on or before October 3, 2016.

CADTH posts all patient input submissions to the Common Drug Review received on or after February 1, 2014 for which permission has been given by the submitter. This includes patient input received from individual patients and caregivers as part of that pilot project.

The views expressed in each submission are those of the submitting organization or individual; not necessarily the views of CADTH or of other organizations. While CADTH formats the patient input submissions for posting, it 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.

Crohn's and Colitis Canada

Section 1 — General Information

Name of the drug CADTH is reviewing and indication(s) of interest	<u>Ustekinumab (Stelara)</u> for Crohn's Disease
Name of the patient group	Crohn's and Colitis Canada
Name of the primary contact for this submission:	[REDACTED]
Position or title with patient group	[REDACTED]
Email	[REDACTED]
Telephone number(s)	[REDACTED]
Patient group's contact information:	
Email	[REDACTED]
Telephone	416-920-5035 [REDACTED]
Address	600-60 St.Clair Avenue East, Toronto, Ontario M4T 1N5
Website	www.crohnsandcolitis.ca

1.1 Submitting Organization

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 \$100 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.

1.2 Conflict of Interest Declarations

a) *We have the following declaration(s) of conflict of interest in respect of corporate members and joint working, sponsorship, or funding arrangements:*

In the fiscal year 2014-2015, Crohn's and Colitis Canada received less than 11% of its total revenue from pharmaceutical companies. The non-restricted grants are used to sponsor patient education events, research and medical conferences, educational brochures, youth camps and post-secondary scholarships for inflammatory bowel disease (IBD) patients. The majority of Crohn's and Colitis Canada's funding comes from individual donors contributing to fundraising events including the annual Gutsy Walk.

b) *We have the following declaration(s) of conflict of interest in respect of those playing a significant role in compiling this submission:*

None of the pharmaceutical companies have played a role in contributing to this submission. This patient input submission was developed and prepared solely by the staff at Crohn's and Colitis Canada. With input from our patient community.

Section 2 — Condition and Current Therapy Information

2.1 Information Gathering

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 2012 "Impact of Inflammatory Bowel Disease (IBD) 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. 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>.

Additionally, CCC management representatives recently attended a focus group involving Canadian patients who are taking ustekinumab to treat their Crohn's disease.

2.2 Impact of Condition on Patients

Crohn's disease is a disabling, life-long gastrointestinal condition that primarily affects working-age Canadians. Crohn's and ulcerative colitis, together, are twice as common as multiple sclerosis or Parkinson's disease and are about as common as Type 1 diabetes or epilepsy. Sadly, Canada has a rate that ranks highest in the world. New evidence suggests that these diseases are now escalating in children at an alarming rate, especially those under 10 years old. Over the past 15 years, the number of children with Crohn's and colitis has increased by more than 40%. With an expected 10,200 new diagnoses every year, in addition to the nearly quarter of a million Canadian living with Crohn's and colitis, these diseases are becoming increasingly prevalent in Canada.

Overall, Canadians have more reasons to be concerned about Crohn's and colitis than anyone else in the world. With one in every 150 Canadians being diagnosed with Crohn's disease and ulcerative colitis - the two most common forms of Inflammatory Bowel Disease (IBD) - these conditions are becoming "Canada's diseases". Families new to Canada, predominantly those of South Asian descent, are developing Crohn's and colitis for the first time – often within the first generation. The burden that Crohn's disease and ulcerative colitis place on individuals and the healthcare system is significant and will continue to grow as the number of people diagnosed increases.

Crohn's disease symptoms include bloody diarrhea, bloating, abdominal pain and fatigue. The majority of Crohn's sufferers indicate that frequent and urgent bowel movements are the most frustrating symptom they experience. This corresponds with results based on 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. Some expressed concern about the increased risk of colon cancer with longstanding Crohn's disease. During times of active disease (flare ups), 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 severely 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 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 Crohn's disease 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.

According to a Crohn's and Colitis Canada's 2012 publication, *The Impact of IBD Report*, 43% of employed people with Crohn's and colitis took time off work per year, and each employed person with either colitis or Crohn's disease took 7.2 days off per year due to their chronic condition. The report also highlights that people with colitis or Crohn's disease are more likely to have lower labour participation rates than the general population, ranging from three to 13% less employment. Caregiver work absences in Canada are estimated to cost \$7 million per year for parents of paediatric IBD cases, plus \$86 million per year for severely ill people with IBD.

When patients are not receiving effective treatments, they must limit their activities such as going out to dinners, movies and concerts, doing physical activities and using the public transportation system. Thirty-four per cent of survey respondents frequently missed out on playing sports, 22% missed school trips, 20% skipped family vacations, 40% avoided parties and 22% did not attend special events, which includes, graduations or family weddings. One interviewee missed the first few months of her newborn son's life because she was hospitalized because of her disease. This is time lost that she will never gain back.

2.3 Patients' Experiences With Current Therapy

Canadians have one of the highest rates of prevalence of Crohn's disease, however, when compared with other Western countries, there are fewer treatment options available for people with moderate to severe forms of Crohn's. 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, but often fail in maintaining remission for those experiencing severe forms. For some interviewees, these treatments kept their condition in remission for long periods of time at early stages of their disease, and

for others using aminosalicylates or immunosuppressants the treatments did not change their symptoms and overall condition.

Most did not report experiencing side effects in taking the aminosalicylates. One interviewee reported liver problems arose from taking immunosuppressants (azathiopurine). The majority of those interviewed did report numerous side effects from steroid use. Most common cited effects included mood swings (easily angered), 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 those interviewed, 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 remission. 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.

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

Ustekinumab is a monoclonal antibody antagonist of human interleukin-12 and interleukin-23, molecules that are believed to play a role in the autoimmunity that leads to diseases such as Crohn's. What makes ustekinumab unique is that it is a therapeutic option which targets a different inflammatory pathway than currently available biologics used to treat moderate-to-severe Crohn's disease. The best candidates are vulnerable patients, particularly those with multiple indications which can be treated by ustekinumab for inducing a clinical response and remission in adult patients who at least have moderate to severe Crohn's disease. The most likely candidates are ones who have also already been treated with anti-tumor necrosis factor (TNF)-alpha therapy and were either unresponsive or intolerant to it.

People living with Crohn's require access to a wide variety of therapies and treatments since not all respond well to current options while others may eventually lose response due to antibody formation.

Accordingly, medical experts and patients demand a variety of biologics that target different mechanisms. Patients with Crohn's disease represent a very sick and vulnerable population. With a severe and progressive form of the disease, and limited therapies, it is ever more important that new treatments that work differently are made available to be able to personalize treatments. Patients should work with their doctor to get access to the right treatment at the right time.

Based on the Impact of IBD Report, more than half of Crohn's disease patients have received surgery. Some patients can opt for additional surgeries however, surgery does not necessarily improve the quality of life, and in the case of Crohn's disease, surgery is often just a temporary fix. Post-surgery complications include soiling, poor pouch function, pouchitis and sexual dysfunction. Unfortunately, many females who undergo surgery are at an increased risk of losing fertility. Surgery is unpleasant and is often the last resort, because of limited drug treatments available through public programs. One male interviewee stated that "proposing surgery as a viable treatment option is inhumane and not fair. Surgery should be considered an option of last resort. It is a shame that there is nothing else to take." Ustekinumab provides another treatment option for patients with Crohn's disease who have failed on

currently available biologics, or as another first-line biologic therapy for those with high-risk profile or other inflammatory diseases.

In addition, there are several other patient subgroups for which ustekinumab may prove useful: Patients who have suffered a serious infection due to their treatment (biologic or conventional therapy); Patients who have lost response to biologics; Patients looking for long-term response to avoid the stress of switching treatments; Patients that have other inflammatory diseases in addition to Crohn's disease; Patients that have undergone surgery (one or multiple); Patients who aren't eligible for anti-TNFs (Remicade or Humira) because of an infection risk or are too afraid to try.

2.4 Impact on Caregivers

For caregivers affected by Crohn's disease, caring comes with challenges. Absences from work, high costs of care, fatigue and stress can take a toll on the caregiver's mental health and physical well-being. Caregivers often act as advocates for their loved ones and take on the burden of care, including financial out-of-pocket costs associated with managing these diseases. The overall cost of caregiving for people living with severe forms of Crohn's and colitis is estimated to be at \$86 million annually.

With increasing numbers of children being diagnosed with IBD, parents play an important caregiver role. Based on the Impact of IBD Report, the caregiver costs for parents of children living with Crohn's and colitis totalled \$7 million for the estimated 5,900 children with IBD in Canada in 2012.

Section 3 — Information about the Drug Being Reviewed

3.1 Information Gathering

Information was drawn from the Crohn's and Colitis Canada (CCC) published reports, including the 2012 "Impact of Inflammatory Bowel Disease (IBD) Report" and informational brochures found on the organization's website. Crohn's and Colitis Canada management staff attended a focus group and gathered qualitative data from a dozen patients who have experience with ustekinumab to treat their Crohn's disease.

3.2 What Are the Expectations for the New Drug or What Experiences Have Patients Had With the New Drug?

Ustekinumab offers people with severe forms of Crohn's disease a new treatment option, to help them properly manage their disease well. According to patients in the aforementioned focus group, it is another effective therapy before invasive surgery. It was mentioned that the self-injection form of ustekinumab also reduces treatment burden compared to treatment by 3-hour-long infusion in a hospital or clinic.

For maintenance therapy, it is expected that with new and proven treatments, many people with Crohn's disease will be able to have a 'normalized' life and these diseases will become a minor inconvenience. Remission is a significant achievement since individuals no longer need to plan their activities and lives around sudden washroom visits. People can focus on work and build their career paths. Young students will be able to focus on school and socialize with other children. And for many women wishing to have children, the introduction of a new targeted biologic treatments will avoid surgery – a treatment option that decreases fertility rates. Research has demonstrated that flare-ups can also lead to a decreased rate of fertility.

With advancements of new medications, there is reduced need for surgery. In the case of Crohn's disease, surgical rates have been falling as well as the rates of hospitalization. The Impact of IBD Report attributes the drop in surgeries to the increased availability of biologics and immunosuppressants.

Patients worry about the potential cost of the drug and the possibility that they may not be able to afford treatment without adequate private or public insurance coverage. Patients currently on the clinical trial wonder if there will be infusion clinics closer to their homes and expressed concern about coverage on public drug programs and whether they will be grandfathered into these programs, as they are unable to financially support the costs to continue on these life-changing treatments.

Another interviewee who works in the construction industry shared his concern about wanting public coverage of the drug. "In the construction business when you have a flare up, you get tired and work becomes harder to do, staying home is not an option. In this work environment I don't get understanding or empathy when I want to go the bathroom. I want this drug to get approved because for people like me there is no coverage from work. You have to look at the drug from the benefit it provides rather than the costs because when you are at 100% you don't need to worry about being sick, feeling tired and wondering about who is going to take care of your kids."

In this focus group, 12 patients spoke of their experiences with ustekinumab as treatment. One man in his 30s spoke of his extremely compromised lifestyle while being house-bound for 2 months with Crohn's disease and perianal abscesses. After failing on other treatments, including other biologics, the patient was resigned to undergo surgery to remove his rectum in hopes of somewhat improving his quality of life. He shared his fears of experiencing social stigma from the effects of irreversible surgery, such as the social complications of an ostomy bag. However, since his surgery was scheduled for several months later, his colorectal surgeon suggested treatment in the meantime with ustekinumab, hoping at this point to attempt all forms of treatment before having her patient subjected to invasive and life-altering surgery. The ustekinumab treatment worked, and the patient spoke very highly of his experience and how grateful he was that this drug allowed him to avoid an unwanted surgery.

A young woman, 29 years old, spoke of the dual benefit of ustekinumab to dually treat her plaque psoriasis and Crohn's disease. She had previously been on Remicade for Crohn's disease and needed to take other medications for her psoriasis. She had many concerns about the drug interactions of her combination therapy. She spoke of how ustekinumab's effective treatment of both diseases successfully eliminated the need for invasive and concurrent medications and greatly improved her quality of life.

GI (Gastrointestinal) Society

Section 1 — General Information

Name of the drug CADTH is reviewing and indication(s) of interest	Stelara® (ustekinumab) for Crohn’s disease
Name of the patient group	GI (Gastrointestinal) Society
Name of the primary contact for this submission:	██████████
Position or title with patient group	██████████
Email	██████████
Telephone number(s)	██████████
Patient group’s contact information:	
Email	info@badgut.org
Telephone	604-873-4876
Address	231-3665 Kingsway, Vancouver, BC V5R 5W2
Website	www.badgut.org

1.1 Submitting Organization

Our mission: 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 550,000 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 or liver condition, as well as those who have lived with an illness 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. In addition, we occasionally hold continuing education events for pharmacists, nurses, dietitians, and physicians. The GI Society, along with its sister charity, the Canadian Society of Intestinal Research (CSIR – founded in 1976), has supported a number of significant clinical, basic, and epidemiological research projects in the field of gastroenterology/hepatology.

1.2 Conflict of Interest Declarations

a) *We have the following declaration(s) of conflict of interest in respect of corporate members and joint working, sponsorship, or funding arrangements:*

The GI Society receives financial contributions from several pharmaceutical companies in addition to other corporations, governments, foundations, service clubs, and individuals in support of our independent charitable work for Canadians affected by GI/liver conditions. Supporters have no input into the editorial content of our resource material, which is approved by the GI Society's Medical Advisory Council (made up of GI/liver health experts only). Other pharmaceutical companies from which we have received support of any kind, such as charitable donations or grants, sponsorships, subscriptions to the *Inside Tract*[®] newsletter, etc. in the last two years include AbbVie Corporation, Actavis/Allergan, AstraZeneca Canada Inc., Innovative Medicines Canada, Ferring Inc., Gilead Sciences Canada Inc., GlaxoSmithKline Inc., Hoffman-La Roche Limited, Janssen Canada, Johnson & Johnson, LifeScan, Merck Canada Inc., Pfizer Canada Inc./Hospira, Shire Canada Inc., and Takeda Canada Inc.

b) *We have the following declaration(s) of conflict of interest in respect of those playing a significant role in compiling this submission:*

None. The GI Society has solely prepared this submission entirely independently of any outside groups or individuals.

Section 2 — Condition and Current Therapy Information

2.1 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, and recent phone/email/social media interactions with individuals who have Crohn's disease and ulcerative colitis; and stories submitted over time by patients.

2.2 Impact of Condition on Patients

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.

Crohn's disease can arise at any age, commonly occurring in young people, such that about 10% of newly diagnosed cases each year are in children. 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 129,000 diagnosed individuals. Patients are at a slightly increased risk for colorectal cancer after having Crohn's disease for about 10-15 years.

The most frequent symptom is diarrhea, often accompanied by cramping abdominal pain. Rectal bleeding, in varying amounts, occurs in most patients with inflammation in the colon, where blood is obvious within and covering the stool. Low red blood cell count (anemia) can result if diarrhea and blood loss are severe. Individuals with Crohn's disease can also experience weight loss, along with delayed development in children with the disease.

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.

Crohn's disease 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 Crohn's disease. 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.

One woman with severe active Crohn's disease described life with the disease as hell, "Every day was a struggle. I went to the washroom 23 times a day or more and got up every hour of the night with night sweats and severe cramping. My Crohn's disease made me so ill that I required numerous surgeries. Once, my surgeon even said I was lucky to end up in emergency surgery as within another week I might not have survived."

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.

2.3 Patients' Experiences With Current Therapy

The treatment of Crohn's disease 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 Crohn's disease, 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.

Monoclonal antibodies (biologics) treat Crohn's disease when older medications fail to relieve symptoms. 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.

80% of the patients who responded to this part of our survey believe there is a gap in currently available treatments. Of these patients, more than 80% felt that safer and more effective medication options are especially lacking.

Some problems they described with current treatments include the following:

- “On the prednisone I have developed the "moon" face, I face challenges with emotions. I experience depression off and on. I am really, really, really tired of using enemas & suppositories!”
- “I get many side effects when taking prednisone.”
- “With my current treatment, I often fail to get to the bathroom on time.” [Stop and imagine for one minute how much anxiety and mess this would cause!]

Patients know that biologics are expensive medications, but they believe that government decision-makers should consider the tremendous costs associated with the disease when they do not have appropriate treatment, as these costs are much more burdensome to Canadian taxpayers.

“Before biologics, none of the treatments worked for me,” said one patient. “I lived without remission of my Crohn's disease for ten years.”

Patients affected by Crohn's disease need access to medications that work. Inadequate access to biologic 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 Crohn's disease 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.

2.4 Impact on Caregivers

When treatment does not provide effective relief, patients require more frequent use of hospital resources, increasing the public health care burden and disempowering individuals. In addition, caregivers may need to devote more resources to a family member suffering from Crohn's disease flares who is unable to complete day-to-day tasks such as errands, cooking, hygiene, etc., because of unresolved disease problems.

Section 3 — Information about the Drug Being Reviewed

3.1 Information Gathering

We gathered information from discussions gastroenterologists and patients, including a patient who has been using Stelara® for more than a year.

3.2 What Are the Expectations for the New Drug or What Experiences Have Patients Had to Date With the New Drug?

For one patient we interviewed, Stelara® is the third biologic she has been on in the last 16 years. After 10 years struggling with severe active Crohn’s disease, her gastroenterologist prescribed Remicade®, which worked for eight years before she lost response to it. A subsequent shift to Humira® did not help. “During the time I was on Humira®, I had two surgeries to remove diseased bowel. After each, the pathology reports came back and I still had active Crohn’s disease.”

For patients who have lost response to other biologics available for Crohn’s disease, Stelara® offers hope, potential for remission, and a chance at a stable life with quality. In the words of the patient, “I’ve only been on this a year and I feel like it’s working. I can continue on. Otherwise, I’d be on the same cycle as I have been in the past with surgeries and having other drugs that do not put me in remission.”

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 Crohn’s difficult, sometimes incapacitating symptoms.

With moderate to severe Crohn’s disease, 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 Crohn’s disease symptoms under control and inducing and sustaining remission. As one patient 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,” said the patient currently on Stelara®.

Each case of disease 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.**

Surgery versus Biologics

Physicians typically don’t recommend surgery for Crohn’s, a systemic disease, because it can affect the entire GI tract and beyond. Crohn’s disease has a high recurrence rate of up to 75%, even after surgical removal of all visible and microscopic disease. However, in some cases, surgery is required, especially when medical management fails and complications arise, such as obstruction, strictures, and fistulae or abscess formation. Yet surgery is **not a cure**; removing any part of the GI tract for Crohn’s disease can lead to other complications, and **the systemic disease remains**.

One patient explained, “Surgery is the last option that I would want to go through. Each time I go through surgery it gets harder because they are removing more and more bowel. You get faced with

other challenges and more problems to manage each time because now your bowels are shorter and shorter”.

As with ulcerative colitis, any loss of colon function results in frequent elimination that is mostly liquid. This means that even after surgery, patients could face troublesome gastrointestinal symptoms – up to 20 bowel movements a day. Moreover, if a physician recommends removal of the colon (colectomy), it may no longer be possible to eliminate waste (have a bowel movement) from the usual anatomic route. The surgeon will remove diseased tissues and then redirect the end of the remaining intestinal tract to the surface of the skin, where the surface opening is called a “stoma” or ostomy. An appliance is attached to collect waste. Finally, if a patient has a surgically-created pouch to hold stool before elimination, it can also become inflamed, a condition called pouchitis, which requires further medical attention.

Many patients will also continue to experience serious extraintestinal manifestations of IBD, such as arthritis of the spine (ankylosing spondylitis), ongoing inflammation or ulcerations of the skin, and many other serious conditions.

98% of patients in our survey said they would rather receive a biologic medication, despite the potential risks and side effects, than get a colectomy. Even if their disease became very severe, they still said they would prefer a biologic medication over colectomy. One patient explained, “I have a strong desire to keep my body intact. The colon serves a myriad of beneficial functions and is self-cleaning.”

Patients welcome a new treatment for the Crohn’s disease and physicians need all the tools available in their medicine bags. Please recommend Stelara® be listed for Crohn’s disease patients.