Template for Submitting Patient Group Input to the Common Drug Review at CADTH

Section 1 — General Information

Name of the drug CADTH is reviewing and indication(s) of interest		Tofacitinib/Xeljanz
Name of the patient group		Canadian Arthritis Patient Alliance
Name of the primary contact for this submission:		Laurie Proulx
	Position or title with patient group	Steering Committee Member
	Email	laurieproulx@arthritispatient.ca
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Name of author (if different)		
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	Telephone	
	Address	Virtual
	Website	www.arthritispatient.ca
Permission is granted to post this submission		X□ Yes □ No

CADTH will post this patient input submission on its website if permission is granted. See CDR Update — Issue 99 for details.

1.1 Submitting Organization

Please provide an overview of the organization that is making the submission, including the purpose or aim(s) of the organization and an outline of the type of membership.

CAPA is a grass-roots, patient-driven, independent, national education and advocacy organization with members and supporters across Canada. It creates links between Canadians with arthritis, assists them to become more effective advocates and seeks to improve the quality of life of all people living with the disease. CAPA believes the first expert on arthritis is the individual who has the disease as theirs is a unique perspective. We assist members to become advocates not only for themselves but all people with arthritis. CAPA welcomes all Canadians with arthritis and those who support CAPA's goals to become members.

1.2 Conflict of Interest Declarations

CADTH requires that all participants in the CDR process disclose any conflicts of interest to ensure that the objectivity and credibility of the CDR process is maintained. Patient groups must declare any potential conflicts of interest that may influence or have the appearance of influencing the information submitted. This information is requested for transparency — a declaration of conflict of interest does not negate or preclude the use of the patient input.

(Examples of conflicts of interest include, but are not limited to, financial support from the pharmaceutical industry [e.g., educational or research grants, honorariums, gifts, and salary], as well as affiliations or personal or commercial relationships with drug manufacturers or other interest groups.) The names of all manufacturers providing funding should be listed, not just the manufacturer of the drug under review.

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

Our operations depend on support from many sources that include organizations, industry, government and individuals who believe in what we do. Sources of grants and support received by CAPA in the last year include: AbbVie, Amgen Canada, Arthritis Alliance of Canada, The Arthritis Society, Canadian Rheumatology Association, Janssen, Novartis, Ontario Rheumatology Association, and UCB Pharma. Additionally, CAPA has also received support in the past from: Canadian Institutes for Health Research, Hoffman-La Roche, Pfizer Canada, Rx&D, Schering Canada, Scleroderma Society, and STA Communications.

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

No conflict

Section 2 — Condition and Current Therapy Information

In each of the following sections, guidance or examples are provided to help identify the type of information that CADTH, CDEC, and participating drug plans will find most helpful in understanding the needs and preferences of the majority of patients. Objective, experiential information that is representative of the majority of the patient group is preferred. There is no need for patient groups to submit published information, as CADTH's CDR review team and CDEC have access to current scientific literature through the manufacturer's submission and a rigorous, independent literature search. However, relevant unpublished studies may be submitted in addition to the completed template.

2.1 Information Gathering

Please briefly identify how the information to complete Section 2 was obtained. Was it obtained, for example, through personal experience, focus groups, one-to-one conversations with a number of patients using the current therapy, printed sources, etc.?

The information was obtained through personal experiences of the Board of The Canadian Arthritis Patient Alliance in living with inflammatory arthritis, in addition to many years of interfacing with our membership.

2.2 Impact of Condition on Patients

What are the condition-related symptoms and problems that impact the patients' day-to-day life and quality of life? Examples of the type of information that could be included are:

- What aspects of this condition are more important to control than others?
- How does this condition affect day-to-day life?
- Are there activities that the patients are unable to do as a result of the condition?

Rheumatoid Arthritis (RA) is a serious, disabling auto-immune disease that affects every aspect of a patients' day-to-day life. Patients are typically diagnosed when they are between the ages of 25 and 50. It affects three times more women than men and 1 in 100 Canadians are affected by RA or roughly 300,000 Canadians. There is currently no cure for RA – once a person develops RA, they live with it for the remainder of their life.

The disease is characterized by inflammation in the joints that destroys the lining of the joint and ultimately the surrounding bone resulting in the need for a total joint replacement. Once damage occurs, it is not reversible and can cause significant pain and disability. It is well documented that RA is a systemic disease and can be accompanied by fatigue and numerous co-morbidities, such as cardiovascular disease, Osteoporosis and lung disease.

RA is a challenging disease to manage and physicians and patients often have to try different drugs to find something that works well. In addition, a patient's immune system can adapt to a drug making it necessary to switch to another treatment when one becomes ineffective. As a result, patients require many medication options as treatment response is not possible to predict and changes over time.

For those whose RA is not well controlled, day to day activities, such as participating in post-secondary education, becoming and staying employed, taking care of oneself, walking, cooking, grocery shopping, house work, being in a relationship, getting married, having and caring for children and social activities become impossible to do. It is vital that inflammation be controlled early so that patients can continue to be productive members of society. We can imagine that the economic benefits to society of keeping people living with RA in the work force and as productive members are greater than those required of the healthcare system if patients do not receive treatments for their disease.

2.3 Patients' Experiences With Current Therapy

How well are patients managing their condition with currently available treatments?
Examples of the types of information that might be included are:

- What therapy are patients using for this condition?
- How effective is the current therapy in controlling the common aspects of this condition?
- Are there adverse effects that are more difficult to tolerate than others?
- Are there hardships in accessing current therapy?
- Are there needs, experienced by some or many patients, which are not being met by current therapy? What are these needs?

A number of medications are currently available for patients living with rheumatoid arthritis, such as disease modifying anti-rheumatic drugs (such as methotrexate) and biologics. This is welcome for patients as we were much more limited in terms of medication options prior to the advent of biologics (which first started coming out in Canada in 2000). As stated in the previous section, managing rheumatoid arthritis can be challenging as there is often a long period of trial and error in order for a given patient to find a medication that works for them. Even when an effective medication is found, a patient's immune system can adapt to a drug making it necessary to switch to another medication when one becomes ineffective. As a result, patients require a number of medication options (including Xeljanz) in order to manage their disease effectively over their life.

Xeljanz is a new molecular target (Janus Kinase (JAK) pathway) and is the first new pathway to be targeted in many years. The availability of a variety of treatment targets is important to patients. Even with the medication options available today, there are patients with RA who do not respond to current therapy. In addition, RA is a lifelong disease and patients continue to need innovative drugs and a variety of treatment options in order to manage their disease effectively throughout their lives.

With the advent of biologics for the treatment of RA, so has the need for either infusions or injections. This means that some patients have vein scarring and scar tissue from numerous infusions and injections. In the most extreme case, a patient would have been giving themselves injections or receiving infusions for 14 years – a reality faced by many patients living with RA. Therefore, the ability to take an oral medication provides patients with a viable option when faced with vein scarring and scar tissue.

Patients may also face scheduling issues for infusions and need to take time off work or find someone to deal with family commitments (e.g. babysitting young children). An oral medication eliminates these issues for the patient. Another benefit is that it makes travel easier for the patient. Currently, infusions need to be scheduled around travel dates (or vice versa).

All disease-modifying anti-rheumatic drugs (DMARD's) and biologics suppress the patient's immune system. Biologics suppress the immune system to a greater extent than the oral DMARD's. Infections are always a concern for patients with RA as even a common cold can quickly turn into a nasty infection, such as pneumonia. The use of Xeljanz provides patients with the option of taking a medication which suppresses the immune system to a lesser extent thereby lowering a patient's risk of serious infection.

2.4 Impact on Caregivers

What challenges do caregivers face in caring for patients with this condition? What impact do treatments have on the caregivers' daily routine or lifestyle? Are there challenges in dealing with adverse effects related to the current therapy?

A patient's spouse and family may need to help patients with RA with day to day activities depending on the extent of disability or if the patient's disease is not well controlled. Spouses or family members

(e.g. children, siblings) may need to take additional time off work in order to assist the patient in activities of daily living. Additional costs may need to be expended in order to assist in caring for the patient if the spouse or family member is unable to do so (e.g. homecare, attendant care). This places strain on relationships and imposes additional financial hardships on the entire family.

In some situations, a caregiver may give the patient their injection or need to take over family responsibilities while the patient is receiving their infusion. The ability to take an oral medication would give the control back to the patient and their family.

Section 3 — Information about the Drug Being Reviewed

In this section, guidance or examples are provided to help identify the type of information that CDR, CDEC, and participating drug plans will find most helpful in understanding the needs and preferences of the majority of patients. Objective, experiential information that is representative of most in the patient group is preferred. There is no need for patient groups to submit published information, as CDR and CDEC have access to current scientific literature through the manufacturer's submission and a rigorous, independent literature search. However, relevant unpublished studies may be submitted in addition to the completed template.

3.1 Information Gathering

Please briefly identify how the information to complete Section 3 was obtained. Was it obtained, for example, through personal experience, focus groups, one-to-one conversations with a number of patients using current therapy, printed sources, etc.?

The information was obtained through personal experiences of the Board of The Canadian Arthritis Patient Alliance in living with inflammatory arthritis, in addition to many years of interfacing with our membership.

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

a) *Based on no experience using the drug:*

- Is it expected that the lives of patients will be improved by this new drug, and how?
- Is there a particular gap or unmet patient need in current therapy that this drug will help alleviate?
- Would patients be willing to experience serious adverse effects with the new therapy if they experienced other benefits from the drug?
- How much improvement in the condition would be considered adequate? What other benefits might this drug have — for example, fewer hospital visits or less time off work?

To reiterate the points made in section 2:

- ***Patients require a number of medication options (including Xeljanz) in order to manage their disease effectively over their life;***

- ***Vein scarring and scar tissue is a significant issue for patients who need to self-inject or receive their medications via infusion, and an oral medication would eliminate these issues for patients;***
- ***The availability of another oral medication would reduce the amount of time spent by patients and their families on injections and infusions allowing them increased independence, and decreased time spent 'as a patient';***
- ***Xeljanz targets a different molecular pathway than currently available DMARDs and it is conceivable that targeting this molecular pathway will provide significant relief of their RA symptoms; and***
- ***Xeljanz suppresses a patient's immune system to a lesser extent which is beneficial to patients and reduces healthcare costs overall.***

b) *Based on patients' experiences with the new drug as part of a clinical trial or through a manufacturer's compassionate supply:*
   - What positive and negative effects does the new drug have on the condition?
   - Which symptoms does the new drug manage better than the existing therapy and which ones does it manage less effectively?
   - Does the new drug cause adverse effects?
   - Which adverse effects are acceptable and which ones are not?
   - Is the new drug easier to use?
   - How is the new drug expected to change a patient's long-term health and well-being?

n/a – drug only recently approved in Canada (April 2014) so limited patient experience

Section 4 — Additional Information

Please provide any additional information that would be helpful to CADTH, CDEC, and participating drug plans. This could include suggestions for improving the patient input process, indicating whether the questions are clear, etc.

It is important to reiterate the debilitating, often aggressive nature of RA, along with the need for new medication options for patients. Currently there is no way to predict which patients will respond to which medications optimally, so a person with RA may undergo various periods of medication changes and subsequent trial and error with medications over their lifetime. Providing as many options to these patients as possible is extremely important.

With the introduction of cheaper disease modifying anti-rheumatic drugs, there is concern that patients may be forced to switch to a less expensive medication even when their disease is well controlled. This practice is extremely detrimental to the patient given they may not respond to the cheaper medication as well. It often takes an inordinate amount of time for patients to find the right combination of medications that effectively controls their disease. Drug plan managers and sponsors should be aware that when a patient's disease becomes unstable, this means more healthcare dollars are spent and a greater burden on the overall healthcare system.

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The Canadian Arthritis Patient Alliance finds it unconscionable that CADTH would put out a call for patient input on a drug that is currently in a queue with no time frame for review by CDEC. Patients living with severe, disabling inflammatory arthritis who have exhausted all their options for treatment are waiting for new therapies with the hope that they will regain some measure of quality of life. CADTH should not be asking for input from patients on potential new treatment options until the option is actually going to be considered by the review committee.