CADTH

CADTH COMMON DRUG REVIEW

# Patient Input

**ulipristal acetate (Fibristal)**

(Allergan Inc.)

Indication: Uterine fibroids (signs and symptoms)

CADTH received patient input from:

**CANFib (Canadian Women with Fibroids Incorporated)**

**Women's Health Initiative Network**

May 21, 2019

CADTH

# Patient Input Template for CADTH CDR and pCODR Programs

| | |
|---|---|
| **Name of the Drug and Indication** | **Fibrstal** |
| **Name of the Patient Group** | **CANFib (Canadian Women with Fibroids Incorporated)** |
| **Author of the Submission** | [redacted] |
| **Name of the Primary Contact for This Submission** | [redacted] |
| Email | [redacted] |
| Telephone Number | [redacted] |

## 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.

**CANFib has Registered with CADTH**

## 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.

**Information used for this submission is derived from a combination of a Survey performed in December 2019 along with aggregate data extracted from discussion groups based on Key Word tags and accumulated responses that were subsequently slotted into groups designed to fit survey criteria. A total group of 343 women were involved in the information gathered for CADTH today.**

## 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?

**By the time a Fibroids Patient joins any group, it's not to tell us they are doing great. The growing numbers in this group (now 21K) are a numerical indicator of the growing instances of unacceptable symptoms of Fibroids that patients deal with day to day.**

**Most have adapted to life in black track pants; this helps hide the blood should there be an episode in public. All women agree that when going out they need to know in advance where the public washrooms are (if they intend to go to a Restaurant, Concert, Party or other social venue). 100s per year suggest simply no longer leave the house. They face depression, weakness, pain and isolation.**

**Of the 343 Women addressed in this survey and data collection, 331 outline extreme pain during and/or in-between periods. 343 explain bleeding has come to an excess that has them worrying about their blood levels, 176 have had at least one blood transfusion and 294 miss at least two days of work each month due to the severity of their period symptoms. 6 have explained they have had to replace an office chair, car seat or similar furniture due to blood stains and 44 have had to purchase a new bed / mattress for the same reason.**

**This data is just that. Data. What it outlines is the drastic nature of the Fibroids Patient symptoms and the methodical way these symptoms take over one's life. Every single respondent suggested they had "no quality of life" up to a certain point and most indicated the quality improved with Fibristal use (data supplied in other responses).**

## 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).

**Fibroids has few treatments that are outside the surgical spectrum and patients want to avert that for understandable reasons.**

**Medications in use to address Fibroids symptoms most commonly include Birth Control/Merina IUD, Ibuprofen, Lupron and Fibristal. All past surveys of CANFib members give us the same data. Birth Control often works well for a period of time but has a point where there is no longer an efficiency of symptom control. Women are clear of their concern that hormones were the only medication option for some time. Women commented they are also often in need of a higher level of bleeding control (during periods) than Birth Control methods offer.**

**Of all respondents and participants, three have taken Lupron after opting out of Fibristal due to the side effects. Of the three, one cites ongoing issues more than 8 months after it's use; issues attributed to Lupron by her GYN.**

**All respondents agreed that Ibuprofen is useful from time to time in addition to their chosen therapies for treatment of Fibroids but is not sufficient relief to be their frontline choice.**

**22 women suggested they plan to use Fibristal to Menopause in order to avert surgery completely. All others that use Fibristal, or used Fibristal, responded with a plan to choose (or had chosen) some kind of surgery but wanted plenty of time to make that choice, and good stores of blood (as well as an optimal time to take work leave) to undergo surgery with. 317 of the 343 women in this survey, indicated the foresee surgery in their future but added that 1-2 years was the time frame they felt most comfortable with in making their choices as to which and when as well as taking time to prepare their household and work life for the downtime. 4 Women were unsure of their future choices at all.**

## 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?

**Women in all groups at CANFib agree that, given the limited choice in non surgical treatments, what they would like are more options that are not ingested hormones.**

**During the collection of aggregate data, 96% of all Fibroids Patients agreed that they were willing to live with headaches and a short period of adjustment that may include dizzy spells and confusion if the return result was the discontinuance (or notable reduction) in bleeding.**

**Women look to treatments that allow them to continue their work week without interruption or loss of income, and they seek outcomes that involve as few blood transfusions and other "risky" procedures that could be avoided with medications.**

**As noted above, women also express the need have take time to make the right surgical choices (where those are involved) as well as time to build strength and blood stores. Women responding that use (or used) Fibristal successfully indicated they have enjoyed a life and lifestyle very close to their "day to day" as it was before the onset of their Fibroids symptoms (either immediately or within two months).**

## 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?

**Most patients (80%) had access to Fibristal through insurance. Some paid out of pocket (14%) and the rest used the Patient Drug Compassion Program having applied for that through their GYN.**

**Benefits were not compared to any other drug. Of the 343 women, 315 were satisfied with the results of the drug. 28 Found the side effects difficult during the first month and of those 21 found month two (and onward) acceptable and 7 discontinued use for that reason. One patient discontinued use due to elevated liver enzymes, citing that she wished to continue taking Iron and Pain Killers, thus giving up Fibristal was her choice in the matter.**

**Of the 315 satisfied, 266 described their experience as "Extremely Satisfied" using terms such as Life Saving and Live Changing as the underlying means of their satisfaction.**

## 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.

**N/A**

## 8. Anything Else?

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

**Yes. Because it was mandated that GYNs have a discussion with their Patients (using Fibristal or about to be prescribed it) regarding liver issues, no members were unaware of the year past during which Fibristal came under review.**

**Given this, during this past year, there has lively discussion regarding the topic. Ultimately the discussion has agreement that there is fear of a drug that works being taken away.**

**Women with Fibroids are afraid to go back to the life that was, if it's now under great improvement.**

**The few that opted out of using Fibristal did so with regret that it did not fit with them and some committed to wanting to give it another try in time.**

**Few major pharmaceutical companies in Canada have not been so kind as to grant CANFib funds for carrying out it's mandated activities.**

**No grant in aid requires favorable review nor opinion from the Administration or Leadership of the CANFib and all members are aware of**

**A: any grants in aid and**

**B: the purpose behind surveys and the collection of aggregate data**

**CANFib is committed to supporting choices, both non surgical and surgical (not product or brands). I respectfully leave the safety of all products / brands to those who have the experience to carry out such studies and choices – but will stress that medication options of any approved type (with regard to Fibroids symptoms) are a powerful tool and incredible gift, particularly when they provide a high level of relief to a large percentage of users.**

## 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.

**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 |
| Allergan | | X | | |
| Bayer | | 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: Patricia Lee
Position: Founder and President
Patient Group: CANFib (Canadian Women with Fibroids Incorporated)
Date: April 16, 2019

CADTH

# Patient Input Template for CADTH CDR and pCODR Programs

| | |
|---|---|
| **Name of the Drug and Indication** | **Fibristal – uterine fibroids** |
| **Name of the Patient Group** | **Women's Health Initiative Network** |
| **Author of the Submission** | [redacted] |
| **Name of the Primary Contact for This Submission** | [redacted] |
| Email | [redacted] |
| Telephone Number | [redacted] |

## 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.

Women's Health Initiative Network is a **National Organization** that aims to change the way we educate women about the following quality of life health conditions that are rarely discussed:

Uterine Health – Fibroids
Vaginal Health
Sexual Health
Bladder Health

We are a registered, non-profit charitable organization that exists for the enhancement of women's health to provide awareness, policy change, influence patient engagement and lead consumer research to ultimately empower women.

http://whin.ca

## 2. Information Gathering

CADTH is interested in hearing from a wide range of patients and caregivers in this patient input submission. Describe how you gathered the perspectives: for example, by interviews, focus groups, or survey; personal experience; or a combination of these. Where possible, include **when** the data were gathered; if data were gathered **in Canada** or elsewhere; demographics of the respondents; and **how many** patients, caregivers, and individuals with experience with the drug in review contributed insights. We will use this background to better understand the context of the perspectives shared.

Interviews with physicians and 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?

Uterine fibroids typically strikes women at the busy time of their lives. It may impact fertility, workplace advancement, the ability to care for their children and activities of daily living.

## 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).

Currently women undergo hysterectomy which has associated risks such as bleeding and infection. Since it requires a hospital stay, women are required to take an inordinate amount of time off. They will need to pay for pay for child care and loss of income. They may not have felt that they have completed their families and are forced into having a surgery that renders them infertile.

## 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?

New therapies should be conservative, cost effective, available and beneficial for patients. Fibristal significantly reduces bleeding and in some women fibroid size is decreased. Any treatment that replaces surgery is beneficial. When a woman chooses a treatment she would appreciate a reduction in symptoms fairly immediately without a lot of risk.

## 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?

Patiens have had access through clinical trials, samples at doctor's offices and through private insurance. The benefits are that the patient can have a fairly fast relief of the bulking symptoms associated with fibroids with minimal risk. Liver function tests are checked on a monthly basis. Patients report very few side effects with Fibristal.

## 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.

Patients need to have liver function tests performed monthly. There are laboratories available for patients to have this done usually in every city. The testing does not delay treatment.

The cost of the testing is covered under MSP or insurance.

The patients and doctors that I spoke to do not feel the lab testing required impacts their lives negatively at all.

## 8. Anything Else?

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

Fibristal is a much needed treatment for women suffering with fibroids and a fantastic advancement in women’s health.

CADTH

## 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. No, I did not receive any help from any other organization to complete this submission.

2. Did you receive help from outside your patient group to collect or analyze data used in this submission? If yes, please detail the help and who provided it. No, the information submitted was based on patient information.

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 |
| | | | | |
| | | | | |
| | | | | |

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: Maureen McGrath
Position: Executive Director
Patient Group:Women's Health Initiative Network
Date:June 21, 2019