

CADTH COMMON DRUG REVIEW

Patient Input

latanoprostene bunod (Vyzulta)

(Bausch & Lomb Incorporated, a subsidiary of Valeant Canada LP)

Indication: Open-angle glaucoma or ocular hypertension

CADTH received patient input from:

Canadian Council of the Blind (CCB), Canadian National Institute for the Blind (CNIB), The Foundation Fighting Blindness (FFB)

December 12, 2018

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	Vyzulta (Latanoprostene bunod ophthalmic solution, 0.024%)
Name of the Patient Group	Canadian Council of the Blind (CCB) Canadian National Institute for the Blind (CNIB) The Foundation Fighting Blindness (FFB)
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.

The Canadian Council of the Blind (CCB) was founded in 1944 by blind war veterans and graduates from schools of the blind. All officers and directors are blind or visually impaired, which gives a unique sensitivity to the needs of the blind community. The CCB is a registered charity pursuant to the provisions of the Income Tax Act (Canada); charity number is: 11921 8899 RR0001. The CCB has over 70 chapters across Canada, and with over 1,500 members, is the largest membership-based organization for the blind. **The purpose of the CCB is to give people with vision loss a distinctive and unique perspective before governments.** The CCB deals with the ongoing effects of vision loss by encouraging active living and rehabilitation through peer support and social and recreational activities. CCB promotes measures to conserve sight, create a close relationship with the sighted community and provide employment opportunities. For the 21st century, the CCB is committed to an integrated proactive health approach for early detection to improve the quality of life for all Canadians. <http://ccbnational.net/fresco/>

The Canadian National Institute for the Blind (CNIB) is committed to creating an inclusive, accessible, barrier-free society that provides the tools blind or partially sighted Canadians require to live safe, fulfilling and independent lives. CNIB believes in making communities

accessible, caring and inclusive. We believe that people living with vision loss should have no limitations placed on their ability to succeed and we work hand-in-hand with Canadians who are blind or partially sighted to advocate for a barrier-free society. As Canada's main provider of post-vision loss rehabilitation therapy, CNIB ensures its clients are able to receive the support they need throughout their journey through vision loss. Whether it be safety and mobility training, assistance with remaining gainfully employed, or gaining access to alternative formats of published works, CNIB operates across Canada providing these services to the best of the organization's ability and funded almost entirely by charitable donations received from the public. www.cnib.ca

The Foundation Fighting Blindness is Canada's leading charitable funder of sight-saving research. Our Charitable Registration Number is: 11912 9369 RR0001. **The mission of the Foundation Fighting Blindness is to lead the fight against blindness by advancing retinal disease research, education and public awareness.** We work with Canadian families affected by retinal diseases and with vision scientists at hospitals and universities across Canada. Over the past 43 years, the Foundation has contributed over \$32 million to sight-saving research. We have a rigorous process of peer review, and the systems and processes in place to support and monitor complex research projects. We do not charge membership fees and consider our community of various stakeholders (donors, educational event participants, researchers, etc.) to be our general members. www.ffb.ca

Together we are co-signatories on the Canadian Patient Charter for Vision Care (<http://www.cnib.ca/en/get-involved/join-an-event/Vision-Health-Month/Documents/CHARTER-12x18-ENG.pdf>), which illustrates our commitment to ensuring that patients have access to the highest standard of vision care across Canada. We do not recommend specific treatments because we believe that these decisions are between the patient and her/his doctor. We advocate for the best care.

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 CCB, CNIB, and FFB recently submitted feedback for CADTH's draft assessment of MIGS, titled "Optimal Use of Minimally Invasive Glaucoma Surgery: A Health Technology Assessment." Since that feedback was not designed to focus on MIGS specifically, but rather to shed light on the experiences of glaucoma patients across Canada, we believe it is relevant to the review of new treatments for glaucoma—whether that be Vyzulta or any other drug or device—and would like to offer that information here again. Our hope is that it can help guide CADTH's decision-making along patient-centered lines, and that it can better foreground the importance of patient experiences in relation to the evolving treatment landscape. Below is the submitted feedback for the MIGS report, provided in full:

1. DISEASE OVERVIEW

Affecting over 400,000 Canadians, glaucoma is a disease of the optic nerve and the leading cause of blindness in North America. Several disease-types have been identified, but in all forms of glaucoma the eye's drainage canals are blocked, leading to a build-up of aqueous humor fluid and an increase in intraocular pressure (IOP) that can eventually damage the optic nerve, the pathway for carrying visual information to the brain. Though glaucoma is typically conceptualized as distinct from "retinal diseases," it is the retinal cells responsible for processing visual information and sending it along the optic nerve—called "retinal ganglion cells" (RGCs)—that are first damaged; as such, glaucoma is a disease that directly affects the retina. While there is no cure for glaucoma, early detection and treatment can avert damage to RGCs and, as a result, prevent loss of vision.

The current standard of care for glaucoma is largely split between, on the one hand, drug therapies in the form of eyes drops and pills, and on the other, surgical approaches encompassing laser surgery and trabeculectomy. The focus across all treatment types is on lowering the build-up of fluid in the eye and the resulting IOP.

Minimally invasive glaucoma surgery (MIGS) refers to a group of devices and procedures that have emerged more recently, distinguished by their novel use of small cuts or micro-incisions, usually through the cornea, that minimize trauma to surrounding tissue and decrease, in some cases, the occurrence of side-effects. As identified in CADTH's Environmental Scan, MIGS approaches are only covered by provincial health insurance in Alberta and Quebec, but surgeons in other parts of Canada are beginning to use MIGS as a replacement for the standard of care, and in some cases as a paid "upgrade." The resulting implementation is a mishmash of heterogeneous criteria, practices, and payment models, leaving little in the way of clarity or direction for Canadian patients.

2. SURVEY AND SUBMISSION OVERVIEW

CADTH's draft assessment of MIGS, titled "Optimal Use of Minimally Invasive Glaucoma Surgery: A Health Technology Assessment," makes clear that a lack of formal criteria is in some ways inevitable in the context of health technology innovation. But when referring to stakeholder input in the Environmental Scan, the draft also suggests that "the more widespread use of MIGS has crossed over from the early-innovation stage to one in which the lack of criteria for allocation of MIGS threatens to be arbitrary and poorly organized, and hence an unacceptable form of differential treatment" (109, 3367-70).

To address the ad hoc nature of contemporary MIGS practices, to ascertain the best criteria for specialists, and to ensure that MIGS devices and procedures are implemented in the most equitable and effective way possible, it is essential to know as much as we can about the relevant patient group: in this case, Canadians living with glaucoma. To aid in this process and to support

CADTH's assessment, the Foundation Fighting Blindness posted and disseminated an online, 30-question "burden of illness" survey on July 20, 2018. Designed to collect data on the physical, psychological, financial, and other burdens associated with the disease, the survey collected 244 responses, providing a range of insights into the experiences of patients across Canada.

The data show that the survey respondents belong to a diverse patient population, with a significant majority of the surveyed group located in Ontario (73%).¹ The remainder specified being located in British Columbia (12%), Alberta (5%), Quebec (3%), Newfoundland (2%), Nova Scotia (2%), Saskatchewan (2%), and Manitoba (1%). The average year of birth provided by respondents was 1950, with a median of 1948, and the average year provided for a glaucoma diagnosis was 2000, with a median of 2007. Most patients indicated having glaucoma in "both eyes" (76%), while a minority indicated having the disease in "one eye" (18%), and the remainder selected "other." Patients rated the **"severity of vision loss resulting from your glaucoma"** on a scale from 1 to 10: most indicated 1 for "no vision loss" (24%), followed by ratings of 2 (17%), 3 (14%), 4 (12%), and 8 (7%). The average of these ratings is 3.88.

Using this information and data from other responses, the Canadian Council for the Blind, the CNIB Group, and the Foundation Fighting Blindness (hereafter "we") performed a preliminary review to determine if there is anything of value in relation to CADTH's draft HQA assessment of MIGS, focusing in particular on section 5 ("Patient Preferences and Experience Review"). Our review made it clear that, especially in the context of an overall paucity of literature on the subject, the data does offer relevant insights that are not currently represented in the draft assessment, and that these insights can aid stakeholders and policymakers in determining the best course of action for MIGS. The feedback offered in the following sections, oriented around the notion of "gaps," therefore responds to a question posed by CADTH on its stakeholder feedback page: **"Are there any inaccuracies in the report, or is any relevant information missing?"**

3. GAPS IN UNDERSTANDING THE BURDEN OF GLAUCOMA

In the "Patient Preferences and Experience Review" of CADTH's assessment, it is immediately clear that little in the way of high-quality research exists that effectively evaluates the toll that glaucoma takes on the Canadians who live with it. The literature search performed in this section begins with 7,133 citations, narrows to 67 full-text articles, and ends with 15 studies that meet CADTH's inclusion criteria. Once these studies are subjected to a quality appraisal, however, very few remain reliable: only one study is viewed as "credible," two as "trustworthy," and three as "transferable."

This shortage of credible research points to potential gaps in our understanding of the reality of glaucoma for Canadian patients, one of which involves a gap in our awareness of the burden of

¹ All survey percentages are rounded to the nearest whole number.

the disease on patients. The CADTH draft assessment does a commendable job retrieving information from these studies that sheds some light on the daily challenges of glaucoma patients, but this deficiency presents a considerable hurdle vis-à-vis the potential implementation of MIGS in Canada.

The draft assessment details how some glaucoma patients perceive changes to their vision as a “symptom of normal aging” (97, 2859), leading to them coping by “restructuring how they engaged with everyday tasks” (97, 2864) instead of seeking medical support. This is a valuable insight, demonstrating the need to counter the notion that the disease is “normal” or “inevitable,” rather than one that is manageable if detected before irreversible damage is done to the optic nerve.

There is a chance, however, that this point could be interpreted out of context to suggest that the disease does not present a psychological burden, that, being considered natural or unavoidable, it is forgotten. The majority of our survey respondents answered the question **“How serious do you consider your glaucoma to be?”** by indicating “very serious” (31%), implying the opposite, that the disease does not go unnoticed and is not forgotten. Other responses to this question ranged from “fairly serious” (29%), “not very serious” (25%), and “not at all serious” (5%). The remainder indicated “other,” with some providing insights that imply a spectrum of experiences and of severity; one patient wrote “I realize that glaucoma is a serious condition, but my experience has been pretty benign, all things considered,” while another wrote “It is serious; have had multiple laser surgeries, a trabeculectomy, and a trabeculectomy correction with a donor cornea patch.”

Most of the surveyed patients responded to the question **“How often do you think about your glaucoma?”** by indicating “very often (at least once a day)” (37%). Other responses included “rarely or never (less than once a month)” (21%), “occasionally (at least once a month)” (17%), and “often (at least once a week)” (16%). Many of the patients who answered “other” connected their tendency to think about the disease often to the frequency of their eye drops; one patient wrote “Basically twice daily when taking my eye drops,” another answered “I would assume every day because I put drops in my eye every night and my eye appearance has changed so I see it each day,” and one participant replied with “At least twice a day when putting drops in (3 different drops, 2/day).” As with their perspectives on the severity of the disease, the frequency with which many respondents think about their glaucoma alludes to a significant psychological burden; in this case, one that is connected, for many, to the often-daily routine of eye drops.

CADTH’s draft assessment does gesture towards the psychological toll of glaucoma, largely through the lens of its recurrent association with blindness: “This association between glaucoma and blindness belies a common perception of eye conditions as being either common or normal minor problems [...] or as those that cause complete sight loss. Glaucoma, not falling into the category of minor vision issues, was instead conceptualized as blindness” (98, 2922-26). This is an association that is repeated in various ways in responses to our survey, with the word “blindness” appearing eight times and the word “blind” appearing five, though in distinct contexts.

The CADTH draft links this association to a common fear that patients experience: “Across studies, patients articulated a fear of blindness” (99, 2943). This is also echoed in our survey data, though with a different inflection: a large percentage of patients (34%) selected “fear knowing the condition is getting worse” as a response to the question **“Have you experienced any other barriers to taking medications or receiving treatment for your glaucoma?”** This supports the notion of fear in relation to blindness, here framed as fear of the condition worsening, but it also foregrounds that fear as a potential barrier to taking medication or receiving treatment. Other barriers selected by respondents included “length of travel time” (14%), “wait time to see specialist is too long” (10%), “cost of transportation” (7%), “unavailability of someone to take me in” (6%), and “did not know how important it was” (4%). A large portion of patients indicated “other” in their response (48%), with a diverse range of barriers expressed, including “uncertainty of exact diagnosis, i.e. type of glaucoma,” “Appointment time with ophthalmologists is too short when one is facing possible blindness,” and “My only wish is that there were more medication options available. I developed an allergy to my first medication and I am allergic to sulfa so I feel my options are limited.” Many respondents also referenced a lack of any barriers in their open-ended responses.

The CADTH draft does include a robust section on “the challenges patients face with eye drops, their primary treatment for glaucoma” (99, 2972), but a nuanced exploration of other significant barriers—framed as just that, barriers—does not appear in the draft’s “Patient Preferences and Experience Review” section—meaning, of course, that it may not exist meaningfully in current research, since CADTH is surveying relevant literature. As already suggested, this points towards a gap in our understanding of the burden of glaucoma, a gap that could very well impede a serious assessment of MIGS in the Canadian landscape, since an understanding of the multifaceted barriers to treating the disease would ideally be a guiding factor.

A general lack of detailed information in the CADTH draft on the daily challenges associated with living with glaucoma heightens the sense of a gap in our understanding of glaucoma’s burden on patients. Responding to the question **“What are the daily challenges you face living with glaucoma?”** our survey respondents selected a wide range of challenges, with many selecting multiple. These included “no daily challenges” (40%), “difficulty reading” (40%), “frequent visits to the eye doctor” (37%), “not able to drive” (26%), “depression” (15%), “difficulty cooking” (11%), and “general mobility” (10%). Many of those who selected “other” (29%) provided insights that illustrate how complex their daily challenges are, including “Need enlarged monitor with computer,” “have hard time seeing if dishes I was are clean, vacuuming, wash floors - close-up work,” “problems with depth perception, tripping,” “Difficulty walking through busy public areas, people bumping into you,” “Regular Medication + Interventions,” and “Anxiety.”

Responses to the question **“Are there activities that you find particularly difficult or can no longer do?”** demonstrated just as much complexity. Again, many patients selected multiple responses, including “no activities I find difficult or can no longer do” (50%), “reading” (34%),

“driving” (29%), “travelling” (17%), “housework” (10%), and “cooking” (6%). Open-ended responses flagged as “other” (21%) included “Sports,” “Can no longer repair small, intricate equipment,” “Threading a needle, sewing more difficult,” “Writing, sewing, gardening,” and “No longer can play tennis, which I played from age 9.” The responses show how pervasive glaucoma is for many patients, affecting not just what many would consider indispensable activities, such as driving, but also the smaller and more personal intricacies of daily life, such as sewing, being physically active, and repairing equipment.

4. GAPS IN UNDERSTANDING PERCEPTIONS OF GLAUCOMA TREATMENTS

If an overall lack of credible research into the burden of glaucoma and its daily complexities demonstrates a gap in our understanding of the disease, it may be that a lack of information on patient awareness of the treatment landscape demonstrates a different kind of gap. This is an important gap, since the ways patients perceive treatments play a crucial role in the design of health systems and health policy, especially if those systems and policies embrace informed consent as a critical factor.

Surveying existing literature, the CADTH draft assessment does highlight the experience of treatments in several ways, mostly focusing on the experience of eye drops—“patients wished they could take less drops less often and wanted to explore alternatives to their current treatments” (103, 3142-43)—as well as differing views on filtration surgeries. The draft also does an admirable job outlining nuances in the patient-provider relationship, particularly in its critique of a concept of compliance that places blame on patients, marking those who do not comply with treatment regimens “as deviant, and their behavior as something to be corrected” (102, 3097). But at the same time, detailed information on how patients comprehend treatments beyond those they are receiving is largely missing; again, this likely signifies a gap in the literature surveyed, which could very well reflect a related gap in our overall understanding of glaucoma, this time associated with how patients experience and conceptualize glaucoma treatments.

Most of the patients we surveyed were aware of what kind of treatment they receive. When asked to **“specify the type of treatment you receive or medication you take for your glaucoma,”** only a small group selected “not sure what type” (1%). Unsurprisingly, the majority selected “drug therapy (eye drops or pills)” (47%), while the remaining responses included “don’t receive treatments or take medication” (6%), “laser surgery” (6%), “conventional surgery” (5%), and “MIGS” (1%). Many of the open-ended responses to “other” (34%) included a list of the treatments that have been received over time.

While many respondents were aware of treatments they receive, the majority indicated that they have never been made aware of treatments that could function as alternatives: most replied “no” (68%) to the question **“Have you been made aware of any treatment/medication options that could function as an alternative to the treatments or medications you are receiving now?”** The remainder indicated “yes” (23%) or “other” (9%). Open-ended responses alongside “other”

encompassed extremes such as “I am made aware of all treatments and surgeries” and “Am having an operation on August 20 but am not clear as to the purpose” When asked to specify **“which treatment or medications were you made aware of?”** respondents selected from the following options, with some selecting multiple: “haven’t been made aware of any treatments or medication” (42%), “laser surgery” (29%), “drug therapy (eye drops or pills)” (23%), “other” (21%), “conventional surgery” (14%), and “MIGS” (6%).

When asked **“Would you be willing to switch to a different treatment or medication if a more effective one was offered?”** the majority of patients replied “yes” (71%), followed by “other” (15%), “don’t receive treatments or take medication” (7%), and “no” (6%). Many who responded “other” provided comments that show a high degree of trust in their specialist or physician, including “whatever is recommended,” “not sure, I trust my doctor,” “as per doctor’s instructions,” and “Only if my Ophthalmologist was in agreement.” These comments underscore the vital role ophthalmologists and physicians play in the way patients relate to the treatment landscape.

The survey asked patients to rate their level of comfort in relation to four main treatment categories: drug therapy, laser surgery, conventional surgery, and MIGS. When asked to **“Please indicate how comfortable you are with the idea of receiving drug therapy (eye drops or pills) as a treatment for your glaucoma?”** respondents selected from a standard scale comprising “not comfortable” (1%), “not very comfortable” (4%), “fairly comfortable” (25%), “very comfortable” (58%), and “other” (12%). In relation to **laser surgery**, respondents indicated “not comfortable” (7%), “not very comfortable” (10%), “fairly comfortable” (37%), “very comfortable” (28%), and “other” (18%). By comparison, responses to **conventional surgery** were more evenly distributed: “not comfortable” (19%), “not very comfortable” (22%), “fairly comfortable” (30%), “very comfortable” (15%), and “other” (14%). And in relation to **MIGS**, patients selected “not comfortable” (11%), “not very comfortable” (24%), “fairly comfortable” (33%), “very comfortable” (16%), and “other” (15%).

The CADTH draft assessment foregrounds the patient experience of current treatments, particularly eye drops (the literature is likely stronger in this area), but what is missing in the draft points towards a lack of understanding in how patients view and experience other treatments, or more broadly the environment of existing and emerging treatments. This is a particularly relevant subject within the MIGS conversation, seeing as patients will approach MIGS analogously to how they approach any health innovation—that is, as a new reference point that must be factored into an already-complex web of health services, procedures, and schedules. How they relate to that existing framework is of course important, but understanding how they relate to information outside that framework is important as well.

5. GAPS IN UNDERSTANDING THE EXPERIENCE OF GLAUCOMA

Material on how glaucoma patients understand and relate to their disease appears to be more robust than on other subjects, and is described comprehensively in the draft assessment. In particular, the emphasis on the “indirect” nature of glaucoma aligns with many of the open-ended responses to our survey focused on the scheduling and busywork of managing the disease, or on fear in relation to its potential to worsen. The draft frames glaucoma as an “asymptomatic condition,” one that “is experienced by patients as the disruption in their lives by eye drops, as interactions with health care providers, and ideas and worries about blindness” (102, 3102-04). This is a kind of gap, certainly, though one between the patient and the direct experience of intraocular pressure, which for many is managed through eye drops and, as a result, not experienced as a pathology. This differs markedly from the more direct experiences of patients with inherited retinal diseases such as retinitis pigmentosa, where the disease is symptomatic and experienced in a very perceptible way.

The draft also highlights the invisible nature of glaucoma very clearly, describing a lack of familiarity with the disease, its asymptomatic tendency, and the fact that non-patients are unable to “see” vision loss since they cannot experience it themselves—three forms of invisibility. And again, the perception of the disease as an inevitable component of aging emerges: “perhaps because it is common amongst older people, its association with aging seemed to contribute to the perception that [it] is just part of normal aging” (98, 2910-11).

The disease is invisible in another way as well, which is at least partially covered by the idea that glaucoma is “unfamiliar, unknown, and as such not within their view (invisible)” (989, 2894). When asked the question **“Do you remember what type of glaucoma you were originally diagnosed with?”** over half of the group we surveyed responded with “don’t remember” (52%), while the remainder selected disease-types from a provided list. The draft assessment does indicate that unfamiliarity with the disease can continue post-diagnosis, but this response underlines this particular notion of invisibility: that most glaucoma patients, at least from those surveyed, are not aware of the form of glaucoma they have. In other words, it is invisible to them. For these patients, this is a central aspect of their experience of the disease, and one we should work to understand more fully.

6. CONCLUSION

It should be reiterated that by focusing on gaps in our understanding of glaucoma—gaps in how we understand the burden of the disease, how patients relate to treatments, and how patients experience the disease—this feedback is not insinuating that there are deficiencies in CADTH’s draft analysis of MIGS. Rather, missing information in the draft likely exposes the gaps as they exist elsewhere, largely in the available research on the subject and, relatedly, in our own perceptions and misunderstandings of glaucoma. The focus of this submission, rather, has been to discuss these gaps and to work towards filling some of them with our own patient survey data. And our overarching goal is to contribute meaningfully to the discussion of the potential implementation of MIGS—in particular, to guide that discussion along lines that are patient-

centered, that focus on optimal and equitable outcomes, and that recognize the value of the perspectives of glaucoma patients.

We look forward to continuing to work with CADTH to support Canadians living with glaucoma, and to advance our collective understanding of the optimal use of MIGS.

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?

Please see our response to question #2, above.

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).

Please see our response to question #2, above.

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?

Please see our response to question #2, above.

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?

Please see our response to question #2, above.

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.
-

NA

8. Anything Else?

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

The committee, it is anticipated would be aware of this drug with more similar ones on the way therefore patients need to have options in their treatment process.

Please see our response to question #2, above.

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.

Surveys were made available to patients as mentioned earlier in the submission by three organizations – Foundation Fighting Blindness (FFB), CNIB and the Canadian Council of the Blind (CCB). Analysis of the responses was completed by the FFB.

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: Organization contacts:

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Main Contact: Louise Gillis

Position: National President

Patient Group: Canadian Council of the Blind

Date: Nov. 29th 2018