CADTH

# CADTH Reimbursement Review
# Patient Input Template

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| **Name of the Drug and Indication** | **Selumetinib (Koselugo)** |
| **Name of the Patient Group** | **Tumour Foundation of BC** |
| **Author of the Submission** | **Desiree Sher** |

## 1. About Your Patient Group

*Describe the purpose of your organization. Include a link to your website.*

The Tumour Foundation of BC is a registered charitable organization. www.tumourfoundation.ca

The Tumour Foundation of BC has been providing essential information and support services for individuals with neurofibromatosis and their families for 38 years. The mission of the Tumour Foundation of BC is to improve the lives of individuals with NF. Our vision is optimize the health and well-being for all British Columbians affected by NF. Funded primarily by individual donations, fundraising events and a provincial grant, the organization offers a range of programs and services which include: a consultative virtual medical clinic, one-to-one support, community events, educational scholarships, vital resource publications, and an annual symposium that attracts specialists and attendees from around the world.

Neurofibromatosis (NF) encompasses a set of three distinct genetic disorders (NF Type1, NF Type 2, and Schwannomatosis) that share the manifestation of uncontrollable tumour growth.

In NF1, which is the most common form of NF, tumours develop along nerves throughout the body, and can affect the development of non-nervous tissues such as bones and skin. NF1 can cause additional complications such as disfigurement, bone deformities, learning disabilities, and cancer. NF2 is characterized by the development of benign tumours on the nerve that carries sound and balance information from the inner ear to the brain. These tumours affect both ears, often leading to partial or complete hearing loss. People with NF2 may also develop other types of benign brain or spinal tumours. Finally, Schwannomatosis causes the development of benign tumours — called schwannomas — usually on spinal and peripheral nerves. These tumours develop when Schwann cells, which form the insulating cover around nerve fibres, grow abnormally.

NF1 is considered a rare genetic disorder with an incidence of one in 2,500 to 3,000 births. (NF2 has an incidence rate of 1 in 25,000 and the rate for Schwannomatosis is 1 in 40,000). However, rare is a relative term – there are more than 1700 people in BC, over 12,000 in Canada and two million worldwide affected with this disorder. NF is more common than cystic fibrosis, Duchenne's muscular dystrophy and Huntington's disease *combined*. Knowledge of NF within the community and the medical profession, however, falls well below that of less common disorders. As a result, the quality of healthcare available to adult NF patients in BC is severely lacking, highly inconsistent and dependent on the engagement of referring family doctors. There are no NF specialists serving adult patients in BC.

## 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 staff at the Tumour Foundation of BC sent out invitations to individuals with NF1, and NF1 and plexiform neurofibromas, to share experiences living with the disorder. Invitations were sent via the organization's email newsletter and posted on various social media platforms. We sought the community's opinion not only about their experience of living with neurofibromatosis but also on the value of having selumetinib (Koselugo) approved for use in Canada. Participants were invited to join a focus group and/or complete an online health experience survey.

Patients and caregivers affected by NF1 participated in a Zoom focus group in November 2022. The data submitted also reflects the input of 25 individuals who participated in the online health experience survey in November 2022. The respondents included adult patients with NF1 (32%), adult patients with NF1 and a plexiform neurofibroma (24%), and caregivers of a patient with NF1 (44%). 64% of the respondents identified as women and 32% identified as men. Age of the individuals who completed the survey and participated in the focus group ranged from 18 to over 65 and resided in communities across British Columbia.

We have included patients' and caregivers' quotes to ensure that the voice of those affected by neurofibromatosis are captured beyond numerical representation. A report sharing all patient comments is also available for review.

## 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?*

In response to the survey and focus group questions on how NF1 impacts quality of life, five key themes were identified: 1) NF1 limits daily living and social activities: 2) Patients with NF1 experience moderate to severe chronic pain: 3) NF1 can result in a dependency on caregivers into adulthood: 4) Families experience financial stress as a result of the NF1 diagnosis: and, 5) Living with NF1, and limited treatment options, negatively impacts the emotional well-being of patients and families.

The quotes that follow from individuals affected by NF1 throughout British Columbia highlight the impact of the disease that goes well beyond the physical symptoms of tumour manifestation.

## NF1 Limits Daily Living and Social Activities

*"**NF1 and a plexiform neurofibroma impacts all of life-** continued severe impacts to bones: hip dislocation again; increased scoliosis; life-changing mobility issues already, so fear of being bed ridden; dependent on others already for everyday tasks, so fear of not being able to do anything for oneself; and **utter decline in physical and mental health**."*

*"I have **many symptoms with a plexiform tumour** on my ankle (which has been waiting for surgery in Vancouver for years now). **Shaking and tremors** in my hands, Migraines, **Temporal lobe seizures** (focal impaired) causing emotion swings, memory loss and odd behaviours, **Major anxiety and sleep problems."***

*"Being the only one in the family [NF1] affects me greatly. Lately there is **major weakness in my legs** and **bad back pain** near the bottom of my spine."*

*"Our son has an optical glioma so he has **limited vision**. So, for him **leaving the house is an issue**. He's supposed to use an awareness cane so that people can tell that he can't necessarily see their vehicle coming. He won't use it, he's a 20 year old young man so, when he leaves the house, **there are safety issues** certainly for crossing streets."*

*"NF has **impaired his ability to have more friends**, his ability to study and advance more quickly in education."*

*"**Tumours are not the only effect** NF1 has on me. I also have **tibial dysplasia** of my right leg, which bothers me every day. I also have **vascular problems** because of NF1. I also had a kidney removed because of **renal artery stenosis.** This kidney resulted in **malignant hypertension**."*

*"NF impacts us tremendously. My son was just **in hospital for five months** with continuous hip dislocation."*

*"I worry about it [plexiform tumour] growing and **affecting my son's mobility**. **That is my biggest concern**."*

*"He has an **extreme fear of "standing out" in a crowd**, so won't use his awareness cane."*

*"He used to walk without aid and **now he has to use crutches, walker and wheelchair**. **The doctor does not know** when or how to lift these restrictions and have asked us to decide for ourselves…"*

*"He spends so much of his life focused on what he can't do and on how **NF limits him (poor fine motor skills, learning disabilities** affected his high school experience and negatively affected his perspective on higher education)."*

*"**NF has given my son more challenges and hardship than any 17 year old should ever have** to endure **both physically and mentally,** and the most unfortunate thing is, I am **not sure it is going to be any easier** as time goes on. In fact, he has some serious bone issues with some serious decisions ahead."*

*"There is a **leg length discrepancy**, there's **scoliosis** as well, and it's all in where the tumours are and **it's all impacting** those bone issues and we are continuing to have appointments."*

*"After our son had two major surgeries to correct **scoliosis caused by NF tumours,** and a year in recovery where he had to be extremely careful and **limit his physical activities** (**he wasn't allowed to go out on the playground**, etc.), he told us that experience made him very aware of the potential affects of NF in the future and how it could **shorten his life**."*

***"Our son was bed-ridden, barely speaking and could not move or do anything for himself,** so we could not leave him on his own."*

## Patients with NF1 Experience Moderate to Severe Chronic Pain

96% of survey respondents live with chronic pain which was rated 5 or greater on the 0-10 pain scale.

*"**the pain feels likes I am being stabbed**"*

*" **the pain is 8/10 on bad days**…"*

***"NF1 disease is often overlooked** as it not always presents visually but internally **there is a lot of pain.**"*

***"People don't seem to understand** the impact of NF1. **They question your pain** as you may look healthy."*

*"Gabapentin was the only thing offered for pain."*

*"...would just like to feel normal .. **be pain free** ..."*

## NF1 Can Result in a Dependency on Caregivers into Adulthood

*"**My daughter has suffered 47 years and we are 74** years old and still need to take care of her."*

*"**I am 45 years old**…**my parents are still involved a lot to help me**, like doctors, dentist, hair dressing, nail cutting appointments because I don't understand what the doctor or dentist convey to me. I can not cook because I am only one handed and I don't see well."*

*"He cannot fix a meal for himself, cannot bring a bowl of soup to the table for example. **We help with dressing, showering, etc**. We are his carers. **We do not leave him at home by himself** for long; I have had his brother miss school to sit in with him."*

*"**My daughter is 40 now but as far as I'm concerned I still am a caregiver** in that she couldn't possibly support this treatment herself."*

*"… I am saddened when I see my son's face drop as we start conversations about how he will have to learn how to start to manage his own care as he becomes independent now that he's an adult….we witness his efforts to make appointments and organize what should be his absolute prime years of independence and care-free fun times so that instead of that he's setting up appointments for tests and check-ups that **no one else cares to help him with if we do not."***

*"**If our son didn't have NF I would probably retire early**, being in a position to do so, and enjoy travel and hobbies that I will put off for many, many years or not get to do due to a deep need to provide for him in case his health deteriorates and **he can't take care of himself** in what should be for him his productive middle years."*

*"If our son were a typical child, he would likely be living independently with friends, in 3rd year university, thinking about career plans. My husband and I would be thinking about retirement in a year or two, possibly relocating to a smaller town with less expensive real estate. Instead [our son] is at home with us, taking a single course in his first year at a local college. We have to consider the **possibility of him facing life-threatening health challenges and needing our financial help** in the future, and needing to stay in a large centre with access to good medical care."*

*"One of my daughters may **not be able to navigate her future needs without my help**. As a mom, this is very scary to me because she may miss screenings, forget about annual check-ups, or not follow-up on problems quickly enough. If I am incapacitated, she will not be as effective in dealing with these medical appointments and as a result, she may be at **heightened risk for cancer, depression, anxiety, mortality**."*

## Families Experience Financial Stress As a Result of the NF1 Diagnosis

92% of patients with NF1 incur expenses related to the care of their NF (such as prescription or non-prescription drugs, medical equipment, physiotherapy, counselling, or travel for medical care). 40% of respondents indicated they completely fund their own medical expenses without any public or private benefits.

*"I would say I probably **spent fifty thousand dollars over my daughter's care**. Had I not been able to financially help her find treatment early on**,** I don't think she would have survived."*

*"I researched everything I could find on NF hoping there would be a way to treat the condition. We grew very frustrated with the medical system and found the **only way to seek any kind of treatment that would give some hope was by going to private clinics.**"*

*"We have spent money over the course of our NF journey. **Two trips to the US for care** make up the bulk of that expenditure but it seemed the only way to be able to **obtain access to doctors with experience** and willingness to help. It has affected us because these are o**ur savings that are being spent** and we make lifestyle choices and changes because of it. It is frustrating to have to spend one's savings in this manner and makes me angry that our **medical system hasn't supported treatments for NF1.**"*

*"There are **no options for complex plexiform neurofibromas**. Many with **NF cannot afford to self-fund expensive drug therapies** if those become available."*

*"Without my financial help she would not have had the emotional strength or financial means to obtain the treatments that have improved her physical appearance. Again, the main feeling of this is frustration at our **medical systems lack of support for NF patients. "***

*"I get angry that we live in an immensely rich country in the most medically advanced time in human history, we spend billions on legitimate health concerns that have far less impacts on people's lives, and can't find the resources to adequately care for citizens of this country... I don't get paid to assist my son when my province drops the ball on funding adequate case management. We were told we **can't claim tax deductions for caring for someone who is disabled because he's not disabled enough**."*

***"Because I may be gone when our son is middle-aged, I cannot take that chance and need to build up a nest egg to leave to him**…. And in so doing **I am sacrificing quality of life for myself** now. My stress and work load affect my happiness in my family life but I feel very strongly compelled to carry on doing what I can for a future I cannot know and may not see myself."*

*"Non-stop juggling and constantly going - I **used up all my holidays from work, got two weeks sick leave from the GP,** then I had to return to work along with sharing shifts at hospital with husband. Everything was on hold - had to cancel husband's eye surgery date. He had been waiting for almost two years."*

*"When we **went to the US for our care** it was disruptive and tiring, but we had a very experienced surgeon who has since retired: Always had to plan for a successful trip, **taking time off work**, missing school and having to catch up, **money for expenses**."*

**Living with NF1, and Limited Treatment Options, Negatively Impacts the Emotional Well-being of Patients and Families**

*"It's just **too much to face every day**."*

*"Depression from **NF1 led to a suicide plan** and alcoholism."*

*"It was dreadful and devastating for all when our son was in hospital for months. **We were consumed by stress, anxiety, anger and exhaustion** (and if I am honest, I am still all those things at random because there is a huge amount to consider and put in place which **takes all my time and money**)."*

*"I live with anxiety, depression, isolation, complete dependency, debilitating physical and mental health, the anger, no peace of mind, **no joy or quality of life**."*

*"**NF is a very scary illness**. Effects cannot be obvious to others. They therefore do not understand your struggles."*

*"**We do not have a clinical coordinator** and because of that we feel this has r**esulted in numerous errors and increased stress** in our family."*

*"This is not a 'cosmetic' condition. It really **affects our mental health, relationships, etc., and it can be debilitating.** The mental issues may be combatted as often patients feel like they have no hope for this progressive disease."*

*"**NF has taken away normalcy**."*

*"I think the unknown has always been a challenge for me. Because you don't know everybody is so different. It's so random and **there's no there's no rhyme or reason to NF**. There's no sort of like timeline of what to expect. I worry about a lot of things just **because it is so unpredictable and random** it's hard not to let your head go there and then you kind of worry you know how are they going to look after themselves?"*

*"I live with **constant worry and anxiety.**"*

*"**I live in constant fear** that I will miss the date to call a specialist to make a follow-up appointment and that will result in a delay in seeing the correct specialist or getting the correct diagnostic test. I fear that will result in a drastic negative outcome for our son's health. I am aware of the **potential for NF tumours to sometimes change and become cancerous**. The NF community is small, and it feels like I am always hearing of the death of someone we met at a previous NF symposium, or of a former Tumour Foundation board member."*

*"I worry terribly that **he may have a very difficult end time in his life** as we don't know what the ever-growing tumours may bring for pain or disability including motor function, vision loss, mental impairment, breathing or swallowing difficulties…"*

*"**I live in fear** of one of the effects of NF's potential for creating serious consequences for our son's life. **Case management for a person with a complex medical condition is a tremendous burden** that I am not equipped to handle, particularly as I struggle with symptoms of ADHD."*

*"I've been in therapy and that has helped me somewhat to cope but I still sometimes have **waves of panic** related to these worries and feeling of uncertainty and **my son's NF has definitely affected my enjoyment of my own life**."*

*"**NF1 is a source of great sadness for me and creates a lot of anxiety**. I sometimes have bad dreams about him being in pain or being lonely, or him just being sad and I worry in my waking life quite often..."*

*"I get **dizzy spells and vertigo from the pressure of keeping everything organized** and in order. A huge amount of my time is, and has always been, spent researching, trying to understand, asking, emailing, organizing, liaising, deciding, planning, making provisions, filling in forms….and **not enough time for breaks and enjoying life**."*

*"NF is a monkey on your back that will never get off. There is **constant anxiety of what is coming next**, will there be someone willing to help, will I be able to continue to financially help**, what if my daughter gives up hope?** There **is no sense of well being**, it is a constant concern."*

## 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).*

46% of patients with NF1 and plexiform neurofibromas who responded to the survey were never presented with a treatment option**.** 32% of individuals with NF1 have never had treatment options presented. Of those who have received treatment only 17% experienced minimal improvement in their symptoms.

"*Our **doctor has never suggested anything** to help neurofibromatosis patients."*

*"When the **plexiform neurofibromas** were diagnosed, we were told **surgery likely wasn't an option** because they are close to the spine, but at the time**, no other options were offered**. I was stunned that we were being told about a **potentially serious problem and being offered zero solutions.**"*

*"There were **no answers** and **only failed procedures and operations** (6 failed ones in total) until the last operation (#7) before the bone would stay in."*

*"**No one is checking on his NF**, nobody's checking if an intervention should be happening now... It's all on us the parents or him to say there is a problem. **No one is ordering regular scans or mapping the plexiform tumour** or whatever that's on us to manage. And it seems off to me because if somebody had cancer you know there would oncologists that would be sort of tracking all the time."*

*"My son has a **plexiform tumour the size of a dinner plate on his back and nobody ever wants to look at it** unless we go, 'hey, check this out'. It's bizarre to me."*

*"**Made my own edibles…no other options...**"*

## 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?*

Improvements in new therapies that patients and caregivers would like to see are treatments, which would: 1) improve quality of life: 2) decrease pain: 3) increase functionality: and, 4) reduce the number of health care visits.

***"Anything that would improve patients'** lives physically, mentally and socially."*

*"Koselugo is the only approved drug treatment in Canada for NF1 in children. There have been many studies that have shown **drastic reduction in tumour size** resulting in **better quality of life**. Patients are able to **re-enter the workforce**, **pain drastically reduced, mobility increased**, no longer requiring surgery when surgery was the only other option."*

*"If **tumours were shrunken**...it would mean **less visits to health care practitioners. There would be proper control over the disease** and not intervening with archaic management of the disease (surgeries, off label chemo), which are costly and burdensome to the Canadian health care system."*

*"…**an oral treatment option would assist in reducing the number visits to a multitude of health care practitioners**. For example, in 6 months we are visiting: (1) Ophthalmologist (2) Oncologist (3) NF specialist (4) Neurologist (5) Occupational therapists (6) Speech Therapists (7) Physiotherapists (8) Psychologists (7) Cardiologist (8) CT scans/MRIs (9) Spinal Surgeon (10) Neurosurgeon (11) Oral Surgeon (12) Special Orthodontist at Rehab Hospital."*

*"There is always the fear of the unknown and the hope of new research/discoveries and that someday I will see **treatments that can improve the quality of life** and if not in my lifetime hopefully for future generations."*

## 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? If applicable, please provide the sequencing of therapies that patients would have used prior to and after in relation to the new drug under review. Please also include a summary statement of the key values that are important to patients and caregivers with respect to the drug under review.*

Not one of the individuals who participated in the survey or the focus group has been offered selumetinib as a treatment option for their plexiform neurofibromas. However, 100% of individuals indicated that they would consider taking selumetinib if given the opportunity to access it.

One caregiver in the focus group shared that they were aware of the benefits of selumetinib and had attempted to access it for their child who lives with multiple plexiform neurofibromas. However, they were informed they had to try a less effective drug first.

*"A neurologist was ready to do the paperwork for us to trial selumetinib. But then she said that the government had a change of heart and or change in process and said that we had to jump over and try trametinib first. They said that the* ***government wasn't going to fund selumetinib because that they didn't want to fork out all that money. I was in shock.*** *Exhausted and defeated, that* ***I am constantly at the mercy of others for help.****"*

***"There aren't any choices.*** *So, let's* ***pay for the one thing that is an option."***

*"…we're not asking for the Cadillac of drugs, we're* ***asking for a drug****."*

*"…****shouldn't have to suffer*** *for [a drug]."*

*"… to hear there is a potentially very effective solution is truly* ***the only positive news we have heard in nearly 20 years****."*

## 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.*

There are no comments to add to 7. Companion Diagnostic Test, as BC residents with NF1 and plexiform neurofibromas are not accessing selumetinib at the time of this submission.

## 8. Anything Else?

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

*"When there is no other solution, the **hope and possibility of success must trump the possibility of side effects.**"*

*"**We need treatment options** for these patients now."*

*"…**NF1 disease is often overlooked** as it not always presents visually but **internally there is a lot of pain and mental issues** encountered. …**patients feel like they have no hope for this progressive disease."***

*"Having Koselgo accessible to Canadian NF1 children with the government support would assist with **giving those hope where hope was not previously possible."***

*"**My son's NF has made my outlook bleaker**, my own relationship with the world around me poorer, and I am forever heartbroken for him."*

***"Canada should be at the forefront of providing effective new drugs to those with no other treatment options**, rather than not providing and having NF1 patients continue to cycle in and out of the health care system only managing symptoms. This would be **a waste of money and time on the health care system to continue managing symptoms rather than effectively treating with Koselgo**."*

*"It is very important to understand that there are many faces of NF. Each individual has their own unique story and is living with the condition daily that affects many different layers. **Approving this drug will change lives** today, tomorrow and for future generations. Looking at the NF population as a group that has gone through a lot and continues to suffer. **The time has come to make the decision in favour of moving ahead to publicly fund the drug."***

***"Patient care in our NF population has been affected for too long with no treatment solution of any sort**. Personally, my son is only 17 years of age - this drug would give him a fighting chance."*

*"**I was disappointed that the GP** who I had been seeing for many years **would not take me back** [after his leave] and made excuses. He was well aware of my condition and when was seeing me took interest and knew how to recommend/refer to the appropriate specialists and for radiology follow-up. **Finding a new GP was difficult..."***

*"It is **far less cost to the Canadian taxpayer to prevent the disfiguring and disabling symptoms** than dealing with the **resulting loss of employment, emotional toll on entire families** of the affected patients, **hospitalizations and loss of quality of life**. This is the first proven drug to slow and reduce tumours leading to better health outcomes rather than doing nothing and waiting for the patient to be disfigured, disabled and require extensive medical supports."*

*"It is very unfortunate when a medical professional tells a patient that he/she has a cosmetic disorder, not fully understanding what the individual goes through and will continue to. It comes down to the training in medical schools and how much time and investment a GP wants to spend on a patient diagnosed with NF. Many times its **due to the knowledge gap of fully understanding the condition**."*

*"For anyone, **having a child changes their life. But having a child with a rare disease changes it further**."*

***"Knowing there is a potential drug available to treat one of the effects of NF that my son lives with is a huge weight off**."*

*"To hear that there is **a possibility the drug may not be funded is mindboggling**; how could this hope be offered, then for families without the means, for that hope to be dashed is simply cruel."*

*"…in Canada there could be there could be thousands of people who should access this drug because **taking it now will prevent,** from the government's perspective, **further expensive problems down the line,** from the patient's perspective you know life issues..."*

*"The **lack of care shown to sufferers of NF** by a massive industry of health care in this province and this country is appalling. It's shameful and enrages me when my tax bill comes along and I read political messages received around election time telling me all the efforts being made and money being spent on making sure '**no Canadian gets left behind' and 'health care for everyone** regardless of where they live."*

*"Every occurrence of NF is individual, which means **a diagnosis for your child means spending a life feeling like you're standing with your child on the edge of a precipice with your toes hanging just off the edge**. What you say out loud to your friends, family and to your child is,* 'Lots of people with NF go through life almost completely unaffected'. *But your inner voice says, '*And **some die of cancer in their 20s and 30s**. And some are crippled or **terribly disfigured by tumour growth'.** *My emotional and mental health have been negatively affected; **I live with anxiety and depression**, and I carry a constant concern for our son's future."*

*"…if the government is just interested in watching the bottom line this is kind of like putting on your seat belt instead of waiting for the car to crash.. if it's just about money, **it's cheaper to prevent a problem than to try to fix it***

## Appendix: Patient Group Conflict of Interest Declaration

To maintain the objectivity and credibility of the CADTH reimbursement review process, 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.

   None

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.

   None

3. List any companies or organizations that have provided your group with financial payment over the past 2 years AND who may have direct or indirect interest in the drug under review.

### Table 1: Financial Disclosures

| Company | $0 to 5,000 | $5,001 to 10,000 | $10,001 to 50,000 | In Excess of $50,000 |
|---|---|---|---|---|
| Alexion | X | | | |
| | $4,991 in support of educational initiative; 2022 NF symposium | | | |

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:** Desiree Sher
**Position:** Executive Director
**Patient Group:** Tumour Foundation of BC
**Date**: November 16, 2022