Common Drug Review
Patient Group Input Submissions

levofloxacin (Quinsair) for **Cystic fibrosis with chronic pulmonary Pseudomonas aeruginosa infections**

Patient group input submissions were received from the following patient groups. Those with permission to post are included in this document.
Cystic Fibrosis Canada — permission granted to post.
Patient Family Advisory Board, Cystic Fibrosis Program, St. Michael's Hospital, — permission granted to post.

CADTH received patient group input for this review on or before June 21, 2016

The views expressed in each submission are those of the submitting organization or individual; not necessarily the views of CADTH or of other organizations.

While CADTH formats the patient input submissions for posting, it does not edit the content of the submissions.

CADTH does use reasonable care to prevent disclosure of personal information in posted material; however, it is ultimately the submitter's responsibility to ensure no personal information is included in the submission. The name of the submitting patient group and all conflict of interest information are included in the posted patient group submission; however, the name of the author, including the name of an individual patient or caregiver submitting the patient input, are not posted.

Cystic Fibrosis Canada

Section 1 — General Information

Name of the drug CADTH is reviewing and indication(s) of interest		Levofloxacin - Cystic fibrosis with chronic pulmonary Pseudomonas aeruginosa infections
Name of the patient group		Cystic Fibrosis Canada
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Position or title with patient group		[redacted]
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1.1 Submitting Organization

Cystic Fibrosis Canada (CF Canada) is a charitable non-profit corporation with a mission to help people with cystic fibrosis (CF). CF Canada funds research towards the goal of a cure or control for CF, supports high quality CF care, and promotes public awareness of CF. Since its establishment, CF Canada has invested more than $226 million in leading research, innovation and care.

1.2 Conflict of Interest Declarations

During the 2015/16 financial year, CF Canada received financial contributions from the following pharmaceutical companies: Abbot Laboratories, BPG Pharma, Gilead, Hoffmann-LaRoche, Insmed, Mereck, Mylan, Novartis, Prometic, PTC, Roche, Rx&D and Vertex. Less than 2% of CF Canada's funding comes from pharmaceutical companies.

Section 2 — Condition and Current Therapy Information

2.1 Information Gathering

Information was gathered through a survey of patient members of CF Canada's Adult CF Advisory Committee.

2.2 Impact of Condition on Patients

CF is the most common fatal genetic disorder in Canada. It is estimated that one in every 3,600 children born in Canada has CF. There are currently over 4,000 people with CF in Canada. The disease affects all exocrine glands of the body and results in the production of thick, sticky mucus amongst other clinical symptoms. The principal clinical impacts are on the lungs and digestive systems. Eighty-five percent of CF patients in Canada lack pancreatic enzymes and have difficulty digesting fats and proteins. Fat and nutrient absorption are also impacted and patients struggle to maintain a healthy weight and are typically deficient in vitamins.

The most significant clinical impact however is in the lungs. Patients have difficulty clearing secretions from the lungs, which in combination with aberrant inflammation leads to persistent infections with cycles of inflammation that are ineffective in clearing infections and lead to progressive scarring of the airways and a decline in lung function. This eventually leads to respiratory failure which is the main cause of death in CF.
Of the 40 CF patients in Canada who died in 2013, half were under 35 years old. There is no cure.

A demanding treatment routine combined with regular visits to specialized CF clinics, acute infections and episodic flare-ups called exacerbations that lead to frequent hospitalizations have a significant impact on day-to-day quality of life affecting life decisions including education, career, travel, relationships, and family planning.

Adults living with CF explain how the disease impacts their day-to-day life below.

"If you are not familiar with cystic fibrosis, I encourage you to try this experiment for a few hours. Using a simple drinking straw, breath only through that straw and not through your nose. ONLY the straw! Go about your day just breathing through that straw. This will give you a small glimpse what it is to have CF." **50 year old female**

"CF affects my daily life in every way possible. There is not a waking hour that passes in which I do not have to consciously think about my health condition. I must take pills, insulin, breathing treatments, physio, and exercise – all to maintain a quality of life that is still lower than that of an average person." **27 year old female**

"Cystic Fibrosis does not define me, but the weight of the disease is quite a burden. Most people my age are in college, living life to the fullest and here I am, at home, unable to work and contribute to society as I have most of my life. I take an average of 40 pills every day, just to live. Chronic lung exacerbations are exhausting, life gets put on hold once again and you find yourself in the hospital for 2 weeks, dreaming of the day you get discharged and are not in such distress just trying to breathe. As I've gotten older, the amount of exacerbations I have had has dramatically increased; I seem to be on a schedule of at least 3 or 4 [hospital] admissions per year…Each lasting a minimum of 2 weeks. During that time, I'm secluded from my family, my friends and my life. It's a very lonely time." **23 year old female**

"Cystic Fibrosis has a major impact on my day to day. Each day I take upwards of 45-50 pills for antibiotics, immunosuppressant from the lung transplant I had due to a decline in my lung function, enzymes, depression medication from PTSD, vitamins and minerals. On top of that I also have an insulin pump as a result of the CF related diabetes I was diagnosed with post-transplant. I also have had a very hard time maintaining my physical activity during this entire process." **38 year old male**

"I am a wife, a mother of young children, a healthcare professional, and a cystic fibrosis patient. I balance many important roles and responsibilities throughout my day. Every day my top priority is ensuring that I do everything I can to stay on top of my CF so that I can indeed maintain my ability to complete all of these roles and stay alive to be there for my children, my husband, and all the important people in my life. The older I get, the more complicated and lengthy my daily CF regime becomes. I spend roughly 3 hours every day doing inhaled therapies and chest physiotherapy. I currently cycle through two different inhaled maintenance antibiotic medications (4 weeks on Cayston, 4 weeks on Tobi podhaler)." **30 year old female**

"There are financial effects of having cystic fibrosis. I have not been able to work steadily for the last 10 years and have suffered economically in both the amount of income I can earn and the amount I pay in medical bills and related expenses." **38 year old male**

2.3 Patients' Experiences With Current Therapy

Individuals with CF spend hours each day managing their disease, and antibiotics are a critical part of this routine.

Adults living with CF explain their experiences with current therapies below.

"Treating CF is an intricate puzzle. In order to treat one problem, other problems often arise based on side effects. CF patients must adhere to strict schedules as far as doing inhaled therapies, taking all the right medication and trying to stay physically active so our bodies don't deteriorate any faster than they already do. As someone who has spent many years on IV antibiotics, I know the feeling of struggle. I am on IV antibiotics more than I'm not." **23 year old female**

"I need IV antibiotics at least 6 times per year lately because my maintenance antibiotics aren't doing the job well enough. IV antibiotics that my body is tolerating less and less. Recently, I am struggling with the realization that I am running out of feasible options to treat my chronic lung infections. It is a terrifying and helpless place to be." **30 year old female**

"I have to take Ventolin, Tobi Podhalers, and Symbicort on a daily basis as well as azithromycin. These are taken when I am well. If I develop a cold or infection other antibiotics need to be added such as Cipro. This is for my breathing only. Meds for my other symptoms are also very extensive." **50 year old female**

"I use oral antibiotics, and IV antibiotics when needed. I cannot take TOBI nebulized because it makes me sick. I should be taking Tobi Podhaler and then Cayston in a month to month rotation, but I have not gotten back on track with that since my last hospitalization last month (we stop all inhaled antibiotics when I get admitted or put on home IVs). I am also on an oral anti-fungal, and have been for the last five years." **27 year old female**

"I currently take oral antibiotics. After the lung transplant I no longer have need for physio, oxygen or inhalation therapy. Prior to transplant I spent 3 years on oxygen, and did physio and TOBI Podhaler inhalation." **38 year old male**

Section 3 — Information about the Drug Being Reviewed

3.1 Information Gathering

Information was gathered through a survey of patient members of CF Canada's Adult CF Advisory Committee.

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

Patients in Canada do not have experience with the new drug. They are hoping, however, that the new drug will provide an additional option for an inhaled antibiotic that will address the needs that are the result of resistance and intolerance of existing options for inhaled antibiotics.

Adults living with CF explain their expectations of the new drug below.

"The idea of having access to more inhaled antibiotics is exciting. As a CF patient I'm on some kind of antibiotic almost constantly which increases my risk of eventually becoming resistant to that antibiotic; the problem with this is it gives doctors less options to play around with to my keep my symptoms under control. In CF, the more options we have the better, it's a hard disease to pinpoint treatment for as it seems many of us respond differently. This knowledge of a new medication makes me excited, it makes me feel stronger knowing that maybe someday it will help me down the road when I'm in a position I need more options." **23 year old female**

"Gaining access to a new antibiotic option would truly mean the world to me. It could mean an extra defence against my chronic lung infections, potentially needing less courses of IV antibiotics that are so hard on my body and mind, a chance to slow the burden of antibiotic resistance to existing therapies, and peace of mind that I am not indeed running out of options. Furthermore, having an antibiotic delivered via high-efficiency nebulizer means less time is spent doing medications and more time to dedicate to my growing children." **30 year old female**

"Any effective treatment to help us manage our day is a huge benefit. We already spend our lives with treatments. If only something could be created to clear our lungs for a full day, now that would be amazing! Anything close to that would also be a blessing." **50 year old female**

Adults living with CF explain the benefit of a drug that is administered through a high-efficiency, portable nebulizer below.

"Ease of use and integration into daily life. These types of nebulizers save so much time (it's difficult to sit through 35 minutes of neb inhalation when you know it could take 3 minutes or less). Also portability would allow me to take this to work and do my mid-day session at my desk, seeing as the device is so small and quiet!" **27 year old female**

"Small, portable nebulizers that can be more portable and provide a faster delivery of the med is always beneficial in giving time for the things CF of patients need to be doing. Living normal healthy lives. My first portable nebulizer was the size of a cereal box, weighed 5 lbs and was louder than some car mufflers. Treatments also could take up to 45 mins – 1 hour." **38 year old male**

"A small, portable nebulizer would be incredible. Carrying my compressor around with me when I go on overnight trips is such a hassle, they are awkward and heavy to carry." **23 year old female**

Patient Family Advisory Board (PFAB), Cystic Fibrosis Program, St. Michael's Hospital, Toronto

Section 1 — General Information

Name of the drug CADTH is reviewing and indication(s) of interest		Quinsair (inhaled Levofloxacin)
Name of the patient group		Patient Family Advisory Board, Cystic Fibrosis Program, St. Michael's Hospital, Toronto.
Name of the primary contact for this submission:		[redacted]
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1.1 Submitting Organization

Our PFAB acts as an advisory resource to the St. Michael's cystic fibrosis care team. Our objectives are to: work collaboratively with the CF team, actively participate in the development of new programs, review recommendations, and provide input with the ultimate goal of shaping a service system (the clinic) that is patient/family centred, efficient and cost effective.

In addition, PFAB promotes improved relationships between patients, families and staff. This partnership allows PFAB and the CF care team to foster a partnering relationship where concerns are listened to and addressed. This results in more effective planning to ensure that services really meet the needs of CF patients.

PFAB membership consists of adult cystic fibrosis patients and family members.

1.2 Conflict of Interest Declarations

a) *We have the following declaration(s) of conflict of interest in respect of corporate members and joint working, sponsorship, or funding arrangements:* **NONE**

b) *We have the following declaration(s) of conflict of interest in respect of those playing a significant role in compiling this submission:* **NONE**

Section 2 — Condition and Current Therapy Information

2.1 Information Gathering

Personal experiences

2.2 Impact of Condition on Patients

Cystic fibrosis is a debilitating, multi-system disease that affects primarily, but not only, the lungs and digestive system. Most people with CF are also pancreatic insufficient. Symptoms include: chronic cough with thick, coloured sputum, pain (chest, stomach, headache, joints), shortness of breath, chest heaviness/tightness, hemoptysis, chronic sinusitis, digestive upset (acid reflux, intestinal cramps, bowel blockages), and cystic fibrosis related diabetes. The chronic lung disease is the most severe and life-shortening aspect of CF. Half of people with CF do not live beyond their 40's and most often, lung failure due to chronic infection is the cause of their death.

The day-to-day life of a CF patient is a gruelling, multi-hour regiment to try to reduce the bacteria-load in sputum (ie exacerbations of CF leading to chronic lung infections). Many forms of medication (inhaled antibiotics, inhaled bronchial dilators and anti-inflammatory medication, inhaled mucolytic), assorted pills (antibiotics, digestive enzymes, vitamins, probiotics, antacids) plus chest physiotherapy are the typical components of daily treatments. Additionally, IV antibiotics (sometimes frequent and long courses) are required to treat lung exacerbations.

Many adults with CF are too ill to work and are forced into being on disability. The severe lung disease and cycle of chronic lung infections makes many tasks difficult at times (or impossible for some), such as, working, household chores, walking, running/exercise, playing with children/reading aloud, and showering, just to highlight a few.

2.3 Patients' Experiences With Current Therapy

As outlined above, therapies include:

Inhaled – antibiotics, mucolytic, bronchial dilators, anti-inflammatory
Oral – antibiotics, vitamins, antacids, probiotics, digestive enzymes, steroids
IV – antibiotics
Injection – insulin for diabetes
Chest physio therapy

Adverse effects can include: drug allergies to required medications, side effects from medications (ototoxicity, neurotoxicity, side effects from steroids (prednisone)), antibiotic resistance to current antibiotics due to over-, but necessary, exposure to treat lung infections.

Hardships include: access to drugs not covered by the Ontario Drug Plan (financial hardship), lifestyle restrictions due to the time/energy required to do daily treatments.

The most difficult hardships are:

(1) Antibiotic resistance: We are constantly fighting lung infections, and therefore constantly requiring antibiotics, however the efficacy of antibiotics is diminished after repeat exposure. We are running out of antibiotics to turn to, and as our lungs are irreversibly damaged and scarred after so much infection, this leads to a downward spiral of progressive lung disease, and ultimately, lung failure.

It feels like new drugs to treat *Pseudomonas*, as well as *Cepacia*, are not being created, and this is a huge need.

(2) Access to drugs: There are many CF drugs which are covered under the Ontario Drug Plan, but also many which are not. CF drugs are incredibly expensive and a person who does not have private insurance will find access to some new CF drugs completely out of reach. Without access, life expectancy is dramatically affected.

2.4 Impact on Caregivers

The impact on caregivers affects many levels: emotional stress seeing a loved one chronically ill, financial stress if loved one is dependent on them, and physical stress if caregivers have to do a substantial part of household duties, in addition to their regular employment. Caregivers may also feel added stress with respect to their workplaces – there can be significant pressure to keep a job that provides family benefits if a loved one is unable to work full-time, as well as stress created when time off is required to attend to medical appointments with their loved one. When the patient's health and quality of life improves, so does that of the caregiver.

Section 3 — Information about the Drug Being Reviewed

3.1 Information Gathering

Personal experiences

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

a) Based on no experience using the drug:

The hope and expectation with Quinsair would be:

A new form of antibiotic to target *Pseudomonas* and hopefully be more effective than antibiotics available today (ie drug resistance issue), resulting in being less sick, less often, and requiring fewer hospital admissions. This would deliver better quality of life, improving daily life and being able to be a more contributing member of society, and less time off work (for those who can work).

Additionally, the healthier we can be, for as long as possible, would postpone or maybe even remove the need for a double-lung transplant when our lung disease gets to this desperate, end stage. Beyond the obvious emotional drain CF has on the patient and family, there is a corresponding financial ripple effect, not only to the family, but also the healthcare system. The healthier a CF patient = less time in hospital/being sick = less cost on the healthcare system and less pressure to already strained resources.

The capacity for "serious side effects" is subjective, and depends on the definition of "serious." Irreversible damage to other systems would not likely be something a patient would risk. A side effect that is "inconvenient" and reversible would be another matter. It would depend on the patients' individual status – if desperate, a patient might be willing to risk more if all other options have been exhausted.

b) Based on patients' experiences with the new drug as part of a clinical trial or through a manufacturer's compassionate supply: **N/A**