Common Drug Review
Patient Group Input Submissions

Ivacaftor (Kalydeco) for Cystic Fibrosis (CFTR Gating Mutations)

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.

CADTH received patient group input for this review on or before July 23, 2014

CADTH posts all patient input submissions to the Common Drug Review received on or after February 1, 2014 for which permission has been given by the submitter. This includes patient input received from individual patients and caregivers as part of that pilot project.

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.

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Cystic Fibrosis Canada

1. General Information

Name of the drug CADTH is reviewing and indication(s) of interest	Kalydeco (ivacaftor) / Cystic Fibrosis (*CFTR* gating mutations)
Name of the patient group	Cystic Fibrosis Canada
Name of the primary contact for this submission:	[redacted]
Position or title with patient group	[redacted]
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Permission is granted to post this submission	Yes

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 $150 million in leading research and care.

1.2 Conflict of Interest Declarations

For the 2013/14 financial year, CF Canada received financial contributions from Abbott, Gilead, Hoffman-La Roche, Merck, Novartis, Vertex and Rx&D. Contributions from pharmaceutical companies accounted for about one and a half percent of the organization's gross revenue in 2013/14.

2. Condition and Current Therapy Information

2.1 Information Gathering

CF Canada reached out to CF patients and their families with the assistance of CF clinics and through the use of social media. CF Canada's national patient data registry was also a credible, reliable and authoritative source of information.

2.2 Impact of Condition on Patients

CF is an inherited genetic disorder primarily affecting the lungs and digestive systems. Of the 4,000 Canadians living with CF, fifteen patients have one of the following gating mutations: *G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P, G1349D* and *G970R*. It is estimated that one in every 3,600 children born in Canada has CF. The disease causes the body to produce thick, sticky mucus. CF patients have difficulty with digesting fats and proteins, and are deficient in vitamins due to lack of pancreatic enzymes. CF causes difficulty in clearing secretions from the lungs, therefore leading to persistent infections, 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 43 CF patients who died in 2012, half were under 32 years old. There is no cure.

Two adults living with CF have shared how the disease impacts them:

"I have always struggled to maintain my lung function and in this past year that struggle has become more difficult as my lung function dropped approximately 15% despite antibiotic and drug therapies. Though I try my best not to let this affect my emotional state, I have to admit that it is a bit scary. I am no longer able to have anything close to a normal day." - Female CF patient, 44 years old.

"Having CF definitely makes everything more difficult. There are days when I can hardly get out of bed. I hate disappointing my children. I want to see them grow up and see their children grow up too." - Female CF patient, 35 years old.

2.3 Patients' Experiences With Current Therapy

Each day, most CF patients take pancreatic enzymes, multi-vitamins and nutritional supplements to maintain normal growth. CF patients work tirelessly every day to improve the clearance of secretions from their lungs. This is done by performing airway clearance techniques at least twice a day for about 30-45 minutes per session. Inhaled medications are used to open the airways while inhaled antibiotic treatments are used to control infections. The total time spent on maintaining lung health is well over two hours each day. When a patient cannot keep up with the amount of secretions or the degree of infection in the airways, a hospital stay of at least two weeks is required. Eventually the ongoing infections destroy the lungs. Lung transplantation may help people with end-stage cystic fibrosis regain health; however following a transplant, the extended median life expectancy is only 34 months.

Two patients have shared their experiences with current therapy, including the potential for lung transplantation:

"I am nineteen years old and a full time college student. I have cystic fibrosis and was diagnosed with this disease when I was three years old. I have learned to live with the adverse effects of my condition. In recent past, it has taken up tremendous efforts to keep up with the strict regiments that are necessary in order to prevent my condition from taking over my time and my life. My condition is catching up to me and sadly, it has been limiting my physical abilities." - Female CF patient, 19 years old.

"My lung function currently is somewhat stable at 30% of what would normally be expected. Over the past couple of decades, my health declined to the point that I have undergone preliminary tests for lung transplantation. Being given the opportunity to take Kalydeco could mean the ability to prolong or even postpone the necessity for lung transplantation." - Male CF patient, 45 years old.

FACT:
In 2012, CF patients spent a cumulative total of almost 20,000 days in hospital, attended over 15,000 clinic visits, and underwent 787 courses of home IV therapy. (2012 Canadian CF Registry Annual Report)

2.4 Impact on Caregivers

Whether as a parent, spouse, grandparent, child, sibling or friend, being a caregiver for a CF patient can have significant emotional, psychological, physical and financial impacts. Caregivers have shared their stories on how the disease has impacted them and their families:

"Our twin boys who are 9 years old were diagnosed at birth with cystic fibrosis. They both have more than 14 hours of treatments per week (inhaled hypertonic saline, airway clearance, and inhaled antibiotics) to maintain an optimal level of health. They have to wake up early in the morning to complete their treatments before going to school. In recent years, they had to be admitted to hospital for pulmonary exacerbations. This disease is a burden on the family. We have three children in total. We are struggling to cope" – Parents of 9 year old twin boys with CF.

"I am a single father raising two young daughters, one 10 and the other 9. We have completely changed everything in our lives from employment to social activities to accommodate this dreadful illness. As a parent we all want what is best for our children. My daughter knows all about the fatal illness that inherited her body and she knows that her life is probably going to be shorter than her friends and sister. She also knows there is a pill out there that can give her hope for a normal, longer life." – Father of an 11 year old CF patient.

"My 11 year old daughter spends in excess of 26 hours a week trying to stay healthy. The fight against CF is all encompassing for the family. It requires giving up 2 to 7 hours every day for her therapies. The physical therapies take a toll on my and my wife's bodies. We both have repetitive strain injuries and arthritis in our hands, wrists and shoulder. This commitment requires scheduling all meals and everyone's activities around her therapies. We restrict our social activities to prevent passing on colds and flus. Each day that a control for cystic fibrosis is not available to her is a day that her lungs are deteriorating. All the treatments that she has access to only try to mitigate her existing health problems, none address the root cause. Without the availability of drugs that fixes the basic defect in cystic fibrosis, our daughter and others like her will lose their valiant fight as they pass away while gasping for air." – Father of a 13 year old CF patient.

3. Information About the Drug Being Reviewed

3.1 Information Gathering

See 2.1.

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

A number of people living with CF and their family have described their expectations of how Kalydeco can help improve their health and quality of life. Based on their understanding of this drug, they expect improved lung function, weight gain and in many cases, avoiding the need for lung transplantation.

"We believe that our nine year old twin sons could benefit from Kalydeco because it will help maintain and possibly improve their lung function, therefore decreasing their hours of treatments. This drug can help them gain weight so they could avoid gastrostomy tube feedings. Receiving their nutrition like other children do will make them feel more normal." – Parents of 9 year old twin boys with CF.

"I notice a lack of lung capacity as I age. My older sister died of CF 5 years ago at the age of 36. She left behind a loving husband and young children. She had the G551D mutation that could have been treated by Kalydeco. I saw through her life that good care won't stop the progression of the disease." – Female CF patient, 31 years old.

"With Kalydeco, I will be able to look forward to a longer life. My family and I have taken care of me the best way possible, so that one day when I meet someone who I would like to spend the rest of my life and have my own family, I will not have to leave them behind because I couldn't get the help I needed when it counted." – Female CF patient, 21 years old.

"Every time I get a cold, I worry about whether I may develop an infection for which there may be no antibiotic. Up until we heard about Kalydeco, I really didn't know what my future holds. If I have the opportunity to take this drug, I will have the opportunity to grow up like a normal healthy person."– Female CF patient, 19 years old.

"My daughter was diagnosed with this fatal disease when she was 7 years old and she has been a trooper all the way – through all the tests, treatments and visits to the hospital. Now that she is a young adult, she is trying really hard to make a life for herself. She would really appreciate a chance to live a normal life and be much healthier." – Mother of a 21 year old CF patient.

"My lung function is decreasing and I have more frequent lung infections. I would like to be able to do activities like other people. I would like to not get so short of breath. I'm tired of not feeling well. I want to live a longer, better life." – Male CF, 23 years old.

"CF greatly affects my energy due to decreased lung function. Living with CF, I truly understand the effects of the disease more that any medical professional could. I know for a fact any medicine capable of thinning the debilitating mucus in my lungs would keep me alive or at least improve my quality of life." – Male CF patient, 21 years old.

Those who have been on Kalydeco either through clinical trials or private insurance have reported improvements in lung function and weight gain. The improvements in health have also led to better quality of life and ability to function normally.

"Since my son and daughter have been on Kalydeco, there has been a significant difference in their lives. Before they were on this drug, they were regularly in hospital for treatment for lung infections requiring intravenous intervention. They had to take more medication daily and the inhaled medication alone took two hours per day. Now with Kalydeco, they are healthy and their lives are more stable and predictable. My son is able to play like a normal child. He could not keep up with his friends before he started taking Kalydeco. My daughter can now do sleepovers without extra equipment and demands on others. Imagine thinking your child's diagnosis is to live not much more than your current age. Now with Kalydeco, normal life expectancy is possible and expected!" – mother of a 12 year old girl and 10 year old boy with CF; on Kalydeco through private insurance.

"Prior to Kalydeco our daughter was often within or fell below the 50th percentile for weight and would often gain very little weight with each clinic visit, but as soon as she started Kalydeco in May of 2013 she gained approximately 10 pounds in 7 months. She has also always had fat absorption issues even with enzymes being pancreatic insufficient and she would often have stomach pain and many very soft bowel movements; however, within a day or two on Kalydeco the stomach pain stopped and her bowel movements became normal. It was amazing we could not believe how quick it happened. When she started Kalydeco she seemed to have even more energy, didn't get as tired so easily with physical activities and she felt better overall. Lots of people even commented that her color was much improved. She has always had very good pulmonary lung function, but in the months she was on Kalydeco her FVC increased from 4-6%; FEV1 increased 6-9% and FEF25-75 increased 18%. The IWK Clinic also repeated a sweat test in July 2013 that had originally been done when she was diagnosed at 17 months old, which resulted in a dramatic decrease from 90% then, to 21%. This had assured us that the drug was doing what it was intended to do. In December 2013, she took her last pill and was one of the first people to ever go off the drug, since we no longer had private drug coverage, and within a few days we started noticing the stomach pain and loose stools were back. Over the past 8 months since she has been off Kalydeco she has had to start on a blocker (Apo-Omeprazole) a couple of times a day and we have had to increase the number of her enzymes required and the prescription strength has also increased from Creon 10's to Creon 25's. We are still struggling with stomach pain and she continues to experience loose stools, which will affect her weight gain. She is now only 8 years old and probably cannot articulate very well what this drug did for her, but she will tell you that it made her feel better and her tummy didn't hurt anymore." - Parents of an 8 year old girl with CF who was on Kalydeco for about seven months through private insurance.

"Our daughter is 16 and our son is 12, both with CF. She used to have a heavy chest cough; he had a throat cough. Since being on Kalydeco, both no longer cough. They both even went through the worst winter of their lives without antibiotics since being on this drug. Regarding the positive impact this has been for us, it's tough to put into words. Kalydeco has not only improved the lives of my two teenage children, but also the lives of their entire support system. Also, there are now fewer people who will end up on the lung transplant waitlist, meaning those on it will get new lungs sooner. We want to demonstrate how Kalydeco has helped improve their health by sharing the data in the accompanying charts." - Parents of a 16 year old girl and a 12 year old boy with CF; on Kalydeco through private insurance.

Twelve Year Old Male With CF on Kalydeco (Started Kalydeco on May 29, 2013)			
Date	**Weight (kg)**	**Lung Capacity (%)**	**Sweat Chloride (mmol/L)**
May 2, 2013 (before Kalydeco)	39.6	73	
May 29, 2013 (baseline)			90
June 25, 2013			32
August 30, 2013	42.8	99	
November 22, 2013	44.4	83	
February 28, 2014	47.4	103	
May 30, 2014	48.9	111	23

Sixteen Year Old Girl With CF on Kalydeco (Started Kalydeco on May 29, 2013)			
Date	**Weight (kg)**	**Lung Capacity (%)**	**Sweat Chloride (mmol/L)**
May 2, 2013 (before Kalydeco)	60.0	66	
May 29, 2013 (baseline)			89
June 25, 2013			22
August 30, 2013	62.0	84	
November 22, 2013	63.2	84	
February 28, 2014	64.4	84	
May 30, 2014	63.3	72 (had a cold)	22

"My 15 year old and 12 year old sons have been on Kalydeco for a week and a half now. One of their two genetic mutations is *S549N*. It has had a positive effect on their frame of mind, knowing this could be a real game changer for their future. At present, both their therapies include the use of the PEP mask to help extract the extra think mucus from their lungs. They nebulize and breathe in a 7% saline solution to help thin down the mucus to make it easier to cough up. These are done twice a day. They regularly take antibiotics to help fight harmful infections. My oldest son must take nose spray to keep the polyps in his nose from getting inflamed. We look forward to the coming weeks where we will see the full impact Kalydeco can be to them." - Mother of a 15 year old boy and 12 year old boy with CF; on Kalydeco through private insurance (dated May 23, 2014).

"Before Kalydeco, my weight was a constant issue to simply maintain; I had a chronic cough and constantly cleared my throat; my lung function floated between 31 and 3%; my appetite was nil; my energy level remained consistently low, and I needed frequent rest and naps; physical endurance has been forever low for most of my life. Only seven weeks after starting Kalydeco, I have gained more than five pounds; my cough halted overnight, lung function rose 5% in thirty days, my appetite is immense, I have energy and endurance I've never had before!" – Male CF patient, 45 years old; on Kalydeco through a compassionate program.

4. Additional Information

Since being approved for use in Canada, over twenty individuals have access to Kalydeco through their private insurance. As of the date of this submission, the drug has been approved for reimbursement by the public drug programs in Ontario and Alberta for people with CF ages six and over with the *G551D* mutation.