Common Drug Review
Patient Group Input Submissions

Ivacaftor (Kalydeco) for Cystic Fibrosis, R117H CFTR gating mutation

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.

As reported in *CDR Update Issue 99*, CADTH posts all patient input submissions 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.

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

1. General Information

Name of the drug CADTH is reviewing and indication(s) of interest	Kalydeco (ivacaftor) / Cystic Fibrosis (*R117H mutation on CFTR gene*)
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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Website	www.cysticfibrosis.ca
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 2014/15 financial year, CF Canada received financial contributions from Mylan, Gilead, Hoffman-La Roche, Merck, Insmed, Vertex and Rx&D. Contributions from pharmaceutical companies accounted for less than 2 percent of the organization's gross revenue in 2014/15.

Section 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, 55 patients ages 18 and above have the R117H mutation. 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 a

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 40 CF patients who died in 2013, half were under 35 years old. There is no cure.

A demanding routine combined with regular visits to the specialized CF clinics, infection and hospitalization 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 have shared how the disease impacts them:

"When you have a life-threatening illness, you can let fear overtake your life or you overcome fear and fight back with every bone in your body. The hospital stays, the hours of treatments and everything else that goes along with managing cystic fibrosis can take a toll on your emotional stamina." – Male CF patient, 27 years old

"I have experienced many health crises related to cystic fibrosis leaving me with no other option but to consider a double-lung transplant. In 2011 my lung function reached an all-time low sitting at 26 percent and my family and I were faced with the difficult reality of having to make a decision. At this point I was so exhausted I couldn't even perform basic tasks." – Female CF patient, 27 years old

"My biggest challenge with cystic fibrosis has been the insecurity of what the disease holds for my future." – Female CF patient, 42 years old

"I lost three friends in three months, while they waited for a lung transplant. It's not right to bury your friends all under the age of 25. I've been to more funerals than weddings in my life." - Female CF patient, 23 years old

"I struggled to keep up with work and uni, and had to spend up to 2 hours a day on exhausting, never ending, treatments. For 20 years I had about 3 hospital admissions a year. This meant I had over 60 hospital admissions, equaling more than 3 years of my life in hospital." - Female CF patient, 29 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.

"Her day starts with a ventolin puffer, 25 minutes of physiotherapy and then a mask that lasts for 30 minutes. Throughout the day she must have her enzymes to digest when she consumes food. At 4:30 p.m. she does breathing exercises that lasts 30 minutes to relieve sputum from her chest and once again follows the same procedure that she dealt with in the morning which lasts 55 minutes. During the evening hours we will hook her up to a feeding machine that goes through the night. Once we wake up

this procedure starts over. At least 1-2 times a year [she] gets pseudomonas and requires two additional masks (that take 35 minutes) into this already hectic schedule" Father of a 7 year old CF patient

"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. I saw through her life that good care won't stop the progression of the disease." – Female CF patient, 31 years old.

FACT: In 2013, CF patients spent a cumulative total of almost 25,000 days in hospital, attended over 16,500 clinic visits, and underwent 676 courses of home IV therapy. (2013 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:

"It is devastating to have two children with a life threatening disease. It is difficult to imagine and breaks my heart to think of when my children fully grasp the information about cystic fibrosis in the way we do. They are used to the treatments from young ages, but the disease still affects their ability to have a normal childhood – shortened time with friends, early mornings to fit in treatment before school and even with daily therapy to manage the disease we still have hospitalizations which are very disruptive to the whole family. When one of them is hospitalized we have to find childcare for our other children, my husband has to take time off work, and we have to put everyone's life on hold."

"When two of my children were first diagnosed, the doctor told me I'd never go back to work again. It is a full-time job keeping my children healthy. From helping with their physio to clear mucus, frequent CF clinic visits, hospital stays, and on top of that ensuring our third child does not feel left out as a healthy child." - Mother of a 8 year old girl and a 3 year old boy with CF

"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 fix 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.

Section 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.

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.

a) Based on no experience using the drug:

"My 8 year old daughter, who was unable to compete in gymnastics due to time away from practice during hospital stays, told me she wished for a drug she could take which would mean she didn't have to take all the medications she currently does. Not only will access to Kalydeco mean a longer, healthier life, it gives them a chance to live a fuller life in terms of university, finding their dream job and starting a family – all without CF looming over them constantly." - Mother of an 8 year old girl and a 3 year old boy with CF

"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.

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

"My kids have not shown changes, but I did not expect it. Excellent health, CT scans of lungs come back normal. I look at this as more preventive for us." – Mother of 6 year old and 3 year old boys with CF

"[I have] been on it 3 months. No more night wheezing, hardly any throat clearing, no more days where I feel like crap. My family has noticed I don't clear my throat or cough as much. BUT, I can still tell I have CF - still a little bit of a cough and mucus production. Hoping I will benefit from a corrector as well!"– Female CF patient, on Kalydeco in the US

"Started Kalydeco 4 months ago and it's been wonderful. Hardly any coughing, my PFT went up, no side effects, and my energy level is way up! I feel wonderful and very blessed!"– Female CF patient, 36 years old, on Kalydeco in the US

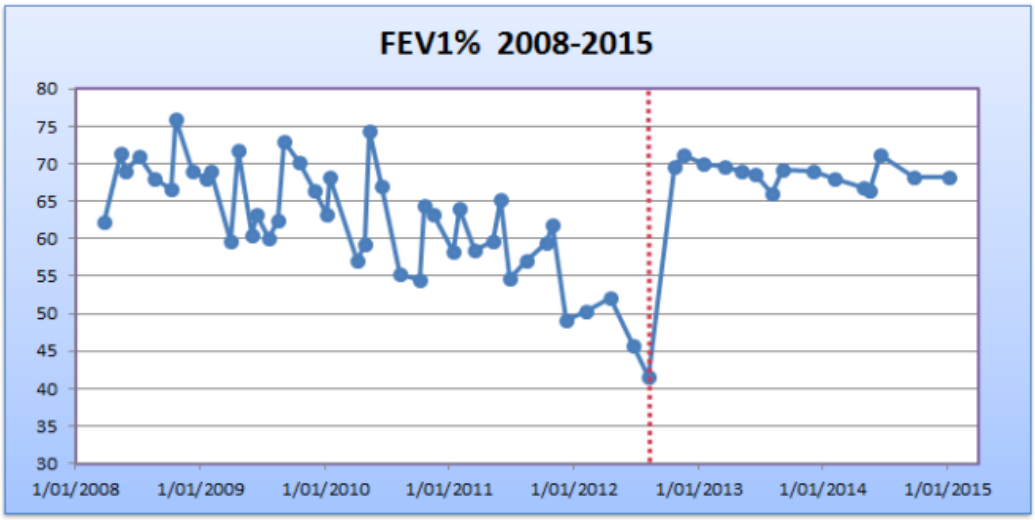
"You can get sick on Kalydeco just as anyone else can. My daughter has been on Kalydeco since October 2013 and got the flu in January. She had to be hospitalized 3 weeks.....but Kalydeco has given her her life back"– Mother of 18 year old girl with CF

"[Our son] recently made the decision to return to swimming lessons. With Kalydeco he has a lot more energy and stamina and with that his confidence has increased enough to give things a go. He's not coughing in the pool and he's not puffed out at all, he is simply swimming like a pro." - Mother of 8 year old boy with CF on Kalydeco

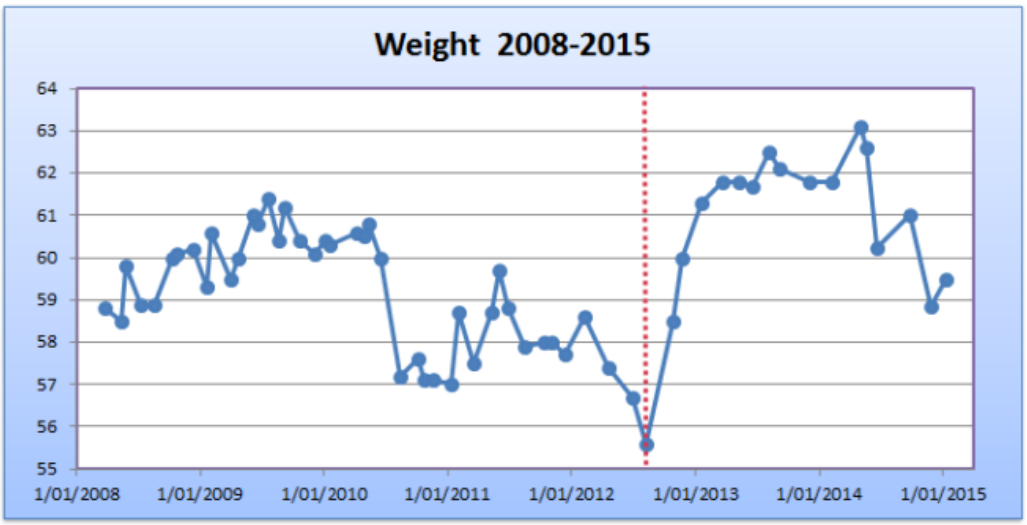
"We are so grateful for Kalydeco. She tried very hard since 2012 to put weight on which she never could. Last December she was down to 49kg. Since starting Kalydeco her weight has gone up 6kg to 55kg, which is amazing. Her PFT is the best in years, going from 73FEV1 8 weeks ago to 94FEV1. Her sweat test went from 115 to 43. So all excellent results - I am so pleased" – Mother of 17-year old girl with CF, with access to Kalydeco in Australia

A 29 year old female CF patient shared her FEV1 and weight before and after access to Kalydeco through compassionate use in Australia:

"This is my very exciting lung function graph showing how stable I have been for the first time in my life. The small drop in 2013 was from my South America trip and the slightly lower numbers in 2014 were most likely affected by my inability to exercise and also to blow lung function tests properly. The peak in June 2014 was following IVs."



"My weight dropped with the vein issue but I have plateaued now"



Section 4 — Additional Information

Since being approved for use in Canada, individuals ages 6 and above with the G551D mutation have access to Kalydeco through public drug plans in Ontario, Alberta, Saskatchewan, Nova Scotia, Manitoba, New Brunswick, Yukon and British Columbia. As of the date of this submission, the drug has received a positive recommendation by the Common Drug Review for use in 9 additional gating mutations for individuals ages 6 and above, and is currently subject to the Pan-Canadian Pharmaceutical Alliance negotiations.