



August 31, 2015

Robyn Seely, Pharm.D
Director, Utah Medicaid DUR
Utah Department of Health
Division of Medicaid and Health Financing
P.O. Box 143106
Salt Lake City, UT 84114-3106

Dear Ms. Seely,

On behalf of patients and families with cystic fibrosis (CF), we write to recommend that Utah Medicaid classify lumacaftor/ivacaftor (Orkambi™) and ivacaftor (Kalydeco®) on the preferred drug list (PDL) for all cystic fibrosis patients who meet the appropriate respective label criteria per the Food and Drug Administration's (FDA) approval. For people with the appropriate mutations, modulating therapies like Kalydeco and Orkambi target the underlying cause of cystic fibrosis rather than addressing the symptoms and clinical manifestations. This can in turn prevent permanent, irreversible lung damage that currently characterizes the disease.

Cystic fibrosis is caused by genetic mutations that result in the malfunction of a protein known as the cystic fibrosis transmembrane conductance regulator (*CFTR*). Decreased *CFTR* function causes irreversible damage and the associated symptoms of cystic fibrosis and leads to early death, usually by respiratory failure. As the world's leader in the search for a cure for CF and an organization dedicated to ensuring access to high quality, specialized CF care, the Cystic Fibrosis Foundation accredits 115 care centers, including the Intermountain CF Care Center in Utah, and 60 affiliate programs nationally that provide multidisciplinary, patient-centered care in accordance with systematically reviewed, data-driven, clinical practice guidelines. Treatment options for this rare, life-threatening disease are extremely limited.

Orkambi is the only FDA-approved medication that improves the function of *CFTR* for individuals with the *F508del* mutation while Kalydeco acts similarly for individuals with a number of mutations (including G551D, G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P, G1349D and R117H). Restricted access to life-saving therapies could result in severe and avoidable health consequences for CF patients. People with cystic fibrosis have a fundamental medical need for increased *CFTR* protein function. Symptomatic therapies such as inhaled antibiotics and hypertonic saline are intended to aid in clearing mucus that obstructs the patient's airways, but they do not increase *CFTR* protein function and therefore do not address the underlying cause of cystic fibrosis. Even when these other therapies are partially successful in clearing mucus from the airways, CF patients still require timely access to an appropriate *CFTR* modulator.

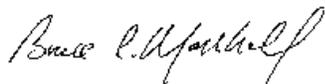
Modulating therapies for CF patients have been shown to immediately improve airway surface liquid properties, reduce airway obstruction, and improve deficiencies in non-respiratory organ systems. Evidence show significant improvements in lung function (FEV₁) as well as trends indicating reductions in the rate of pulmonary exacerbations, increased body mass index

(BMI), and improvement in patient-reported respiratory outcomes (CFQ-R). The totality of efficacy evidence is indicative of overall benefit.

Initiating treatment with modulators in patients with the indicated *CFTR* gene mutation earlier in the disease progression process helps to ensure patients have the greatest potential for overall lifetime benefit. Appropriate treatment has the potential to slow the progressive decline in health and prevent permanent, irreversible organ damage (lung, pancreas, etc.) characteristic of cystic fibrosis. It is not medically reasonable or responsible to withhold an effective treatment until the patient suffers an irreversible decline in health and loss of lung function.

Per each drug's respective FDA label, the CF Foundation recommends the Utah Medicaid program make Orkambi available to all CF patients age 12 and older with two copies of the *F508del* mutation; and make Kalydeco available to individuals with on-label mutations aged 2 and older. Please contact Jackie Erdo, Public Policy Manager, at jerdo@cff.org or 301-841-2628 with any questions.

Sincerely,



Bruce C. Marshall, M.D.
Senior Vice President of Clinical Affairs



Mary Dwight
Senior Vice President for Policy &
CF Community Affairs