August 8, 2018

Iowa Medicaid Enterprise Attn: Pharmacy Services 100 Army Post Road Des Moines, Iowa 50315

Dear Members of the Pharmaceutical and Therapeutics Committee:

On behalf of people in Iowa living with cystic fibrosis (CF), we write to urge Iowa Medicaid to include tezacaftor/ivacaftor (Symdeko™) on the preferred drug list (PDL) for all cystic fibrosis patients age 12 years and older who have two copies of the *F508del* mutation or at least one mutation in *CFTR* gene that is responsive to tezacaftor/ivacaftor per the Food and Drug Administration's (FDA) approved label.[1] For those with an eligible mutation, tezacaftor/ivacaftor targets the underlying cause of cystic fibrosis rather than addressing the symptoms and clinical manifestations. This can in turn prevent the permanent, irreversible lung damage that currently characterizes the disease.

About the Cystic Fibrosis & the CF Foundation

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 123 care centers, including 3 in Iowa, and 55 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 limited.

About Tezacaftor/Ivacaftor

Tezacaftor/ivacaftor is an FDA-approved therapy that improves the function of the CFTR protein for individuals with specific mutations in the *CFTR* gene. People with cystic fibrosis have a fundamental medical need for increased CFTR protein function. This therapy presents an opportunity to preserve health and lung function in eligible individuals with CF by slowing the progression of the disease and preventing costly hospitalizations, declining health status, deteriorating quality of life, and premature death.

For those with two copies of the *F508del* mutation, evidence shows improvement in lung function (FEV_1), body mass index (BMI), and patient-reported respiratory outcomes (CFQ-R) as well as a reduction in pulmonary exacerbations.[2] This therapy presents a therapeutic alternative for those patients with two copies of the *F508del* mutation who are not able to take lumacaftor/ivacaftor (Orkambi®) due to adverse side effects such as chest-tightness or drug-drug interactions.[2] In particular, tezacaftor/ivacaftor decreases the likelihood of adverse events and the need for strict monitoring while on therapy for those with FEV_1 <40% who are more likely to experience adverse side effects on lumacaftor/ivacaftor.[2]

For those with eligible residual function mutations, evidence shows significant improvements in FEV_1 and CFQ-R as well as improvements in BMI and a reduction in pulmonary exacerbations.[3] This therapy provides a therapeutic alternative for some individuals with residual function mutations currently eligible for ivacaftor (Kalydeco®).[3]

Policy Recommendations

The CF Foundation recommends Iowa Medicaid make tezacaftor/ivacaftor available to all eligible CF patients per the FDA label when the patient's treating physician determines it is medically necessary and appropriate to begin therapy.

We stand ready to answer any questions about tezacaftor/ivacaftor or other CF treatments. We would be happy to connect you with local CF experts to further discuss this important issue.

Sincerely,