CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

July 11, 2018

Colleen McLachlan
Division of Health Care Financing and Policy
1100 E. William Street, Suite 101
Carson City, Nevada 89701

Dear Ms. McLachlan and Members of the Drug Use Review Board:

On behalf of people in Nevada living with cystic fibrosis (CF), we write to urge Nevada Medicaid to cover tezacaftor/ivacaftor (Symdeko™) for all CF 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] We also commend Nevada Medicaid for making ivacaftor (Kalydeco®) available to eligible CF patients and ask that you continue to provide coverage for this therapy per the FDA label.

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 2 in Nevada, 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]

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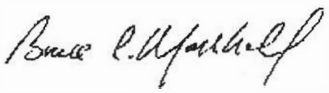
For those with eligible residual function mutations, evidence shows significant improvements in FEV_1 and CFQ-R as well as improvements in BMI and a reduction in pulmonary exacerbations.[3] This therapy provides a therapeutic alternative for some individuals with residual function mutations currently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

Policy Recommendations

The CF Foundation recommends Nevada 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 also ask that Nevada Medicaid continues to cover ivacaftor for eligible individuals per the FDA label.

We stand ready to answer any questions about CFTR modulators or other CF treatments. We would be happy to connect you with local CF experts to further discuss this important issue. Please contact Jackie Erdo, MPH, Manager of Policy and Advocacy, at jerdo@cff.org or 301-841-2628.

Sincerely,



Bruce C. Marshall, MD
Senior Vice President of Clinical Affairs

Lisa Feng, DrPH
Senior Director of Policy and Advocacy

[1]Eligible mutations include: E56K, R117C, A455E, S945L, R1070W, P67L, E193K, F508del (two copies), S977F, F1074L, R74W, L206W, D579G, F1052V, D1152H, D110E, R347H, 711+3A→G, K1060T, D1270N, D110H, R352Q, E831X, A1067T, 2789+5G→A, 3272-26A→G, 3849+10kbC→T

[2]Taylor-Cousar, Jennifer L., et al. "Tezacaftor–Ivacaftor in Patients with Cystic Fibrosis Homozygous for Phe508del." *New England Journal of Medicine* 377.21 (2017): 2013-2023.

[3]Rowe, Steven M., et al. "Tezacaftor–Ivacaftor in Residual-Function Heterozygotes with Cystic Fibrosis." *New England Journal of Medicine* 377.21 (2017): 2024-2035.

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