CYSTIC FIBROSIS FOUNDATION®

July 21, 2023

Dr. Lisa Ghotbi
Chief, Pharmacy Benefits Division
Department of Health Care Services
MS 4604 P. O. Box 997413
Sacramento, CA 95899-7413

Dear Dr. Ghotbi,

On behalf of the more than 2,500 people with cystic fibrosis (CF) in California, the CF Foundation extends our sincerest appreciation for the Department of Health Care Services' (DHCS) inclusion of numerous therapies used for treatment of cystic fibrosis on the *Medi-Cal Rx Extended Duration Prior Authorization List*, including all four FDA approved CFTR modulators and multiple inhaled antibiotics, pancreatic enzymes, mucus thinners, and anti-reflux medications. The Foundation also applauds the inclusion of other classes of drugs that are used by people with CF such as bronchodilators, asthma medications, and immunosuppressive medications. The ability to obtain multi-year authorizations for these products will lighten the significant administrative burden patients and care teams face and help ensure people with CF maintain uninterrupted access to their life-saving medications, many of which are taken for their entire life.

After reviewing the list of medications, we identified additional therapies that are frequently used by people with CF and adding them to the extended duration prior authorization (PA) list could help ensure patients have continued access to the most effective and appropriate CF treatments.

About Cystic Fibrosis
Cystic fibrosis is a life-threatening genetic disease that affects nearly 40,000 children and adults in the United States. CF causes the body to produce thick, sticky mucus that clogs the lungs and digestive system, which can lead to life-threatening infections. Cystic fibrosis is both serious and progressive; lung damage caused by infection is irreversible and can have a lasting impact on length and quality of life. As a complex, multi-system disease, CF requires an intensive treatment regimen including multiple medications. For people with CF, it is not uncommon to take seven therapies every day and as many as twenty.[1] While these therapies are helping people with CF live longer, healthier lives, patients often encounter administrative barriers to accessing them. Limiting the need for frequent, repeated authorizations for these medications will create better access to therapies for people with CF and allow providers to direct their valuable time and resources towards direct patient care instead of paperwork.

Inhaled Antibiotics
Inhaled antibiotics are used to improve respiratory symptoms in people with cystic fibrosis who colonize bacteria in their lungs; many of these lung infections are associated with increased morbidity and

[1] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC2680350/

mortality.[2] Infections can last for many years, resulting in many people with CF needing take inhaled antibiotics as an ongoing part of their maintenance treatment regimen. Use of CF specific antibiotics has been shown to decrease certain bacteria in sputum and improve lung function and quality of life.[3,4,5]

We appreciate the inclusion of Tobramycin inhalation solutions (Kitabis®, Bethkis®) and inhalation powder (TOBI® Podhaler) on the current extended duration prior authorization list. However, people with CF need access to different antibiotics depending on the bacteria they culture. Additionally, even for a given bacteria, people sometimes need to alternate between different antibiotics to maintain effectiveness. For example, both aztreonam lysine for inhalation (Cayston®) and azithromycin (Zithromax®) are indicated for the treatment of *Pseudomonas aeruginosa*, a bacterium often found in the lungs of people with CF. Similarly, amikacin liposome inhalation suspension (Arikayce®) has been approved for the treatment of different lung disease caused by *Mycobacterium avium complex* (MAC), a type of nontuberculous mycobacteria (NTM).

To ensure people with CF have continuous access to the inhaled antibiotics they need to treat their infections, the CF Foundation requests the inclusion of aztreonam lysine for inhalation, azithromycin, and amikacin liposome inhalation suspension on the *Medi-Cal Rx Extended Duration Prior Authorization List*.

Hypertonic Saline

Mucus thinners, such as mucolytics, improve lung function and reduce infection rates by thinning the accumulated mucus. One such mucolytic is hypertonic saline (Hypersal®, sodium chloride for inhalation, PulmoSal®, NebuSal®, generic hypertonic saline), which is highly concentrated sterile salt water inhaled by people with CF to help clear mucus from the lungs. Mucociliary clearance is an essential component of CF care and CF pulmonary guidelines recommend the use of hypertonic saline in individuals ages 6 and up.[6] Specifically, treatment with mucolytic products—including inhaled hypertonic saline solution—is shown to help clear mucus from the lungs, resulting in fewer lung infections, improved lung function, and better quality of life for people with CF.[7]

The Foundation appreciates that Medi-Cal Rx included both mannitol (Bronchitol®) and dornase alfa (Pulmozyme®), two other mucus thinners used by people with CF, on the list of medications eligible for extended duration prior authorization. Similarly, we request the inclusion of hypertonic saline in multiple concentrations (3%, 3.5%, and 7%) due to its wide usage by and benefits for people with the disease.

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[2] Emerson J, Rosenfeld M, McNamara S, Ramsey B, Gibson RL. Pseudomonas aeruginosa and other predictors of mortality and morbidity in young children with cystic fibrosis. Pediatr Pulmonol 2002;34:91-100.

[3] Retsch-Bogart GZ, Quittner AL, Gibson RL, Oermann CM, McCoy KS, Montgomery AB, Cooper PJ. Efficacy and safety of inhaled aztreonam lysine for airway *Pseudomonas* in cystic fibrosis. *Chest* 2009;135:1223-32.

[4] Ramsey BW, Pepe MS, Quan JM, Otto KL, Montgomery AB, Williams-Warren J, Vasiljev KM, Borowitz D, Bowman CM, Marshall BC*, et al.* Intermittent administration of inhaled tobramycin in patients with cystic fibrosis. Cystic Fibrosis Inhaled Tobramycin Study Group. *N Engl J Med* 1999;340:23-30.

[5] Quittner AL, Buu A. Effects of tobramycin solution for inhalation on global ratings of quality of life in patients with cystic fibrosis and *Pseudomonas aeruginosa* infection. *Pediatr Pulmonol* 2002;33:269-276.

[6] Mogayzel, Peter, Jr., Naureckas, Edward, et al. Cystic Fibrosis Pulmonary Guidelines. American Journal of Respiratory and Critical Care Medicine, Vol. 187, 2013.

[7] ibid

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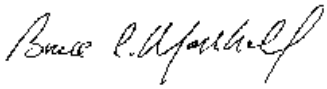
We appreciate the Department's consideration of the needs of people with CF as they continue to develop these policies for Medi-Cal Rx. We stand ready to answer any questions about the cystic fibrosis care regimen; please contact Amanda Attiya, State Policy Specialist, at aattiya@cff.org or (240) 482-2879. We would be happy to connect you with local CF experts to discuss this further.

Sincerely,

Mary B. Dwight
Chief Policy & Advocacy Officer
Senior Vice President of Policy & Advocacy



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