MEDICAL MUTUAL®

Drug Policy

Policy:	Pulmozyme (dornase alfa) inhalation solution	Annual Review Date: 12/21/2023 Last Revised Date: 12/21/2023

OVERVIEW

Pulmozyme, a recombinant human deoxyribonuclease I (rhDNase), is indicated in conjunction with standard therapies for the management of cystic fibrosis (CF) patients to improve pulmonary function. According to Patient Registry data compiled by the Cystic Fibrosis Foundation (2018), Pulmozyme is used by the vast majority of patients with CF and its use continues to rise.

POLICY STATEMENT

This policy involves the use of Pulmozyme. Prior authorization is recommended for pharmacy benefit coverage of Pulmozyme. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Pulmozyme as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Pulmozyme be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

AUTOMATION: When available, claims with specialty providers and ICD-10 codes for Cystic Fibrosis (ICD-10: E84.*) will be used for automation to allow approval of the requested medication.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Pulmozyme is recommended in those who meet the following criteria:

1. <u>Cystic Fibrosis</u>

Criteria. *Patient must meet the following criteria*

A. The medication is prescribed by or in consultation with a pulmonologist or a physician who specializes in the treatment of cystic fibrosis.

Initial Approval/ Extended Approval.

A) *Initial Approval:* 365 days
B) *Extended Approval:* 365 days

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Pulmozyme has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. **Asthma.** Mucus hypersecretion may be mediated by a variety of causes, including inflammation, irritation, stimulation, or mucus-producing tumors.[9] However, efficacy of Pulmozyme is not established for conditions other than CF. In a pilot study of patients with severe acute asthma (n = 50), there was no significant difference in forced expiratory volume in 1 second (FEV_1) with Pulmozyme use vs. placebo.[10]

2. **Bronchiectasis, Idiopathic.** A multicenter, double-blind, randomized, placebo-controlled 24-week trial (n = 349) examined the effect of Pulmozyme vs. placebo on patients with idiopathic bronchiectasis (i.e., bronchiectasis not related to cystic fibrosis).[11] Patients in the Pulmozyme arm experienced worsened lung function and more frequent pulmonary exacerbations vs. placebo. The authors concluded that Pulmozyme should not be used in this population.

3. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

1. Pulmozyme® inhalation solution [prescribing information]. San Francisco, CA: Genentech, Inc; July 2021.
2. Cystic Fibrosis Foundation. Patient registry. Annual data report of the center directors, 2018. Available at: https://www.cff.org/Research/Researcher-Resources/Patient-Registry/2018-Patient-Registry-Annual-Data-Report.pdf. Accessed on May 18, 2020.
3. Rafeeq MM, Murad HAS. Cystic fibrosis: current therapeutic targets and future approaches. *J Transl Med*. 2017;15(1):84.
4. Yang C, Montgomery M. Dornase alfa for cystic fibrosis. *Cochrane Database Syst Rev*. 2018;9:CD001127.

5. Flume PA, O'Sullivan BP, Robinson KA, et al. Cystic Fibrosis Pulmonary Guidelines: Chronic Medications for Maintenance of Lung Health. *Am J Respir Crit Care Med*. 2007;176:957-969.
6. Mogayzel PJ Jr, Naureckas ET, Robinson KA, et al, Pulmonary Clinical Practice Guidelines Committee. Cystic Fibrosis Pulmonary Guidelines: Chronic Medications for Maintenance of Lunch Health. *Am J Respir Crit Care Med*. 2013;187(7):680-689.
7. Borowitz D, Robinson KA, Rosenfeld M, et al, Cystic Fibrosis Foundation. Cystic Fibrosis Foundation evidence-based guidelines for management of infants with cystic fibrosis. *J Pediatr*. 2009;155(6 Suppl):S73-93.
8. Lahiri T, Hempstead SE, Brady C, et al. Clinical practice guidelines from the Cystic Fibrosis Foundation for preschoolers with cystic fibrosis. *Pediatrics*. 2016;137(4):
9. Rubin BK. Aerosol medications for treatment of mucus clearance disorders. *Respiratory Care*. 2015;60(6):825-832.
10. Silverman RA, Foley F, Dalipi R, et al. The use of rhDNase in severely ill, non-intubated adult asthmatics refractory to bronchodilators: a pilot study. *Respir Med*. 2012; 106(8):1096-1102.
11. O'Donnell AE, Barker AF, Ilowite JS, Fick RB. Treatment of idiopathic bronchiectasis with aerosolized recombinant human DNase I. rhDNase Study Group. *Chest*. 1998;113(5):1329-1334.