PRIOR AUTHORIZATION POLICY

POLICY: Cystic Fibrosis – Pulmozyme® (dornase alfa inhalation solution – Genentech, Inc.)

DATE REVIEWED: 05/20/2020

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.[1] 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.[2]

Disease Overview

CF is an autosomal recessive disease of epithelial chloride transport estimated to affect approximately 30,000 individuals in the US.[2] Dysfunction in the cystic fibrosis transmembrane conductance regulator (CFTR) protein decreases chloride and water transport across mucus-producing cells, leading to viscous sputum.[3,4] The retained secretions allow development of chronic bronchial infection.[4] Subsequent massive neutrophil infiltration causes tissue destruction, as well as release of nucleic acids and cytosol matrix, which further contribute to mucus hyper-viscosity.[5] Pulmozyme cleaves to the extracellular DNA present in the mucus, thereby decreasing sputum viscosity.[1,4]

Guidelines

Guidelines from the CF Foundation (2007, updated in 2013) address the chronic use of medications for management of lung health in CF patients aged 6 years and older.[5,6] These guidelines recommend Pulmozyme use for CF patients regardless of disease severity to improve lung function and reduce exacerbations. Separate guidelines have addressed Pulmozyme use in younger patients.[7,8] Although efficacy data are lacking in patients under 5 years of age, safety and tolerability have been established in patients as young as 3 months.[1,8] CF Foundation guidelines for infants under 2 years of age (2009) and children between 2 and 5 years of age (2016) support Pulmozyme use in these populations based on individual circumstances.[7,8]

POLICY STATEMENT

Prior authorization is recommended for prescription benefit coverage of Pulmozyme. Because of the specialized skills required for evaluation and diagnosis of patients treated with Pulmozyme, approval requires Pulmozyme to be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals are provided for 3 years unless otherwise noted below.

Automation: None

RECOMMENDED AUTHORIZATION CRITERIA

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

FDA-Approved Indications

1. **Cystic Fibrosis.** Approve Pulmozyme for 3 years if Pulmozyme is prescribed by or in consultation with a pulmonologist or a physician who specializes in the treatment of cystic fibrosis.

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. Rationale for non-coverage for these specific conditions is provided below. (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.

REFERENCES

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