PRIOR AUTHORIZATION POLICY

POLICY: Cystic Fibrosis – Pulmozyme Prior Authorization Policy
- Pulmozyme® (dornase alfa inhalation solution – Genentech/Roche)

REVIEW DATE: 05/07/2025

OVERVIEW
Pulmozyme, a recombinant human deoxyribonuclease I, is indicated in conjunction with standard therapies for the management of patients with **cystic fibrosis** to improve pulmonary function.[1]

Guidelines
According to Patient Registry data compiled by the Cystic Fibrosis Foundation (2023), Pulmozyme is used by the vast majority of patients with cystic fibrosis.[2] Guidelines from the Cystic Fibrosis Foundation (2007, updated in 2013) address the chronic use of medications for management of lung health in cystic fibrosis patients ≥ 6 years of age.[3,4] These guidelines recommend Pulmozyme use for patients with cystic fibrosis regardless of disease severity to improve lung function and reduce exacerbations. Separate guidelines have addressed Pulmozyme use in younger patients.[5,6] Although efficacy data are lacking in patients < 5 years of age, safety and tolerability have been established in patients as young as 3 months.[1,6] Cystic Fibrosis Foundation guidelines for infants < 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.[5,6]

POLICY STATEMENT
Prior Authorization is recommended for prescription benefit coverage of Pulmozyme. All approvals are provided for the duration noted below. 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, approval requires Pulmozyme to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None

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

FDA-Approved Indication

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

Coverage of Pulmozyme is not recommended in the following situations:

1. **Asthma.** Mucus hypersecretion may be mediated by a variety of causes, including inflammation, irritation, stimulation, or mucus-producing tumors.[7] However, efficacy of Pulmozyme is not established for conditions other than cystic fibrosis.[10] In a pilot study of patients with severe acute asthma (n = 50), there was no significant difference in forced expiratory volume in 1 second with Pulmozyme use vs. placebo.[8,10]

3. **Bronchiectasis, Idiopathic.** A multicenter, double-blind, randomized, placebo-controlled 24-week trial (n = 349) examined the effect of Pulmozyme vs. placebo in patients with idiopathic bronchiectasis (i.e., bronchiectasis not related to cystic fibrosis).[9] 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. Another small double-blind, placebo-controlled, Phase II trial (n = 14) examined Pulmozyme in adults with non-cystic fibrosis bronchiectasis. Pulmozyme failed to show any significant change in any of the outcome variables or in sputum transportability.[10,11]

4. 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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