### Pulmozyme

**Description**

**Pulmozyme (dornase alfa)**

**Background**
Cystic fibrosis is caused by defects in the cystic fibrosis gene, which codes for a protein transmembrane conductance regulator (CFTR) that functions as a chloride channel and is regulated by cyclic adenosine monophosphate (cAMP). Mutations in the CFTR gene result in abnormalities of cAMP-regulated chloride transport across epithelial cells on mucosal surfaces.

Six classes of defects resulting from CFTR mutations have been described with an autosomal recessive inheritance pattern. Most mutation carriers are asymptomatic and there is some variability in clinical phenotype in persons homozygous for the different mutations.

Dornase alfa is a highly purified solution of recombinant human deoxyribonuclease I (rhDNase), an enzyme which selectively cleaves DNA. The enzyme hydrolyzes the DNA present in sputum/mucus of patients with cystic fibrosis and reduces viscosity, thereby improving clearance of secretions.

**Regulatory Status**
FDA-approved indication: Pulmozyme is a recombinant DNase enzyme indicated in conjunction with standard therapies for the management of cystic fibrosis (CF) patients to improve pulmonary function.
Off-label Uses:
The use of Pulmozyme should be considered for all CF patients who may experience potential benefit in pulmonary function or who may be at risk of respiratory tract infection (2-4).

Related policies
Kalydeco, Orkambi, Symdeko

Policy
This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Pulmozyme may be considered medically necessary for the treatment of cystic fibrosis.

Pulmozyme may be considered investigational for all other indications.

Prior-Approval Requirements

Diagnosis
Patient must have the following:

1. Cystic Fibrosis (CF)

Prior – Approval Renewal Requirements

Diagnosis
Patient must have the following:

1. Cystic Fibrosis (CF)

Policy Guidelines

Pre - PA Allowance
None

Prior - Approval Limits
Duration 12 months
### Prior – Approval Renewal Limits

**Duration**
12 months

#### Rationale

**Summary**

Daily administration of Pulmozyme (dornase alfa) Inhalation Solution in conjunction with standard therapies is indicated in the management of cystic fibrosis patients to improve pulmonary function (2).

Prior authorization is required to ensure the safe, clinically appropriate and cost effective use of Pulmozyme (dornase alfa) while maintaining optimal therapeutic outcomes.

#### References


#### Policy History

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| September 2011  | Criteria modified to delete requirement for FVC >40%, based on manufacturer’s package labeling: *
Pulmozyme (dornase alfa) Inhalation Solution has also been evaluated in a second randomized, placebo-controlled study in clinically stable patients with baseline FVC <40% of predicted. Pulmozyme did not significantly reduce the risk of developing a respiratory tract infection requiring parenteral antibiotics. |
| September 2012  | Annual editorial and reference update                                   |
| March 2013      | Annual editorial review                                                 |
| March 2014      | Annual review                                                           |
| March 2015      | Annual criteria review and reference update                              |
This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 15, 2019 and is effective on April 1, 2019.