RATIONALE FOR INCLUSION IN PA PROGRAM

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 (1).

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 (1).

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 (2).

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 (2).

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).

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

1. Cohen-Cymberknoh M, Shoseyov D, Kerem E. Managing cystic fibrosis. Strategies that increase life expectancy and improve quality of life. Am J Respir Crit Care Med 2011;183: 1463-1471.
2. Pulmozyme [package insert]. South San Francisco, CA: Genentech, Inc.; February 2024..
3. Infant Care Guidelines: Cystic Fibrosis F, Borowitz D, Robinson KA, et al. Cystic Fibrosis Foundation evidence-based guidelines for management of infants with cystic fibrosis. The Journal of pediatrics. 2009;155(6 Suppl):S73-93.
4. Preschool Guidelines: 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).