Dornase Alfa
A Review of Pharmacoeconomic and Quality-of-Life Aspects of its Use in Cystic Fibrosis

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Cystic fibrosis (CF) is a fatal hereditary disease; patients with CF have an average lifespan of 30 years. By cleaving neutrophil-derived DNA, dornase alfa (recombinant human deoxyribonuclease I) decreases the adhesiveness and viscoelasticity of sputum in the infected lungs of patients with CF. As a result, respiratory function is improved in patients with all degrees of disease severity, and the relative risk of pulmonary exacerbations is reduced in patients with mild to moderate disease.

Resource utilisation (days spent in hospital or receiving parenteral antibiotics) in patients with mild to moderate disease is also reduced by dornase alfa, as evidenced by a placebo-controlled trial in >900 patients. Cost savings generated by these reductions in resource use during 24 weeks of dornase alfa therapy offset about 17 to 37.5% of the acquisition cost of the drug, depending on local cost data for various countries. Reductions in resource utilisation with dornase alfa have not been observed in patients with severe disease.

Available cost-effectiveness and cost-utility analyses are not fully published. One analysis estimated that the incremental cost of avoiding one hospitalisation was about $Can 15 000 relative to standard therapy after 1 year of treatment. Informal analysis in the UK suggests a cost per quality-adjusted life-year of £25 000 for dornase alfa.

Some quality-of-life (QOL) domains (mainly cough frequency and chest congestion) have shown modest improvement in patients treated with dornase alfa, mainly those with mild CF. Persuasive evidence of QOL benefit is lacking in those with more severe disease.

Identifying patients most likely to benefit from dornase alfa therapy is essential to maximise clinical and cost benefits. The lack of a demonstrated reduction in resource utilisation in patients with severe CF makes its use more difficult to justify economically in this group than in those with less severe disease. However, in the absence of other treatments for this group, economic considerations must be weighed against clinical benefits.

In conclusion, the acquisition cost of dornase alfa is partially offset by savings gained by reducing resource utilisation in patients with mild to moderate CF, and the drug appears to improve quality of life in some patients, mostly those with less severe disease. However, in the absence of guidance from definitive cost-effectiveness analyses, individual healthcare providers must make their own decisions about how best to provide dornase alfa to patients with CF in a rational and cost-justifiable manner.

Dornase alfa (recombinant human deoxyribonuclease I; rhDNase) is an important adjunct agent in the management of cystic fibrosis (CF). It improves lung function in patients with all degrees of disease severity and reduces pulmonary symptoms in patients with mild to moderate disease (section 2). The high cost has implications for opportunity costs in other areas of health care. This article examines pharmacoeconomic and quality-of-life (QOL) issues surrounding the use of dornase alfa in the management of CF.

1. Economic Aspects of Cystic Fibrosis

1.1 Epidemiology and Pathophysiology

CF is a fatal hereditary disease; individuals with CF have an average lifespan of about 30 years. CF is the most common lethal autosomal recessive