**Year 12 ATAR Human Biology ATHBY**

**Task 8: Science Inquiry 2021**

**Conditions:**

**Thursday:** Read & annotate printed material provided.

**Friday:** Complete in-class assessment.

Text book and notes permitted.

**Time for task: 40 min**

**Task weighting: 3 % Total Marks:20**

**Introduction** Since identification of the CFTR gene over 25 years ago, gene therapy for cystic fibrosis (CF) has been actively developed. Gene therapy is currently the most advanced form of CF genetic medicine. Since cloning of the CFTR gene in 1989 extensive pre-clinical research led to approximately 27 clinical trials involving about 600 patients being completed.

Recently, a double-blind, placebo-controlled multi-dose trial was carried out to test the effectiveness of liposome vectors in carrying the normal CFTR gene into cystic fibrosis suffers.

Patients 12 years or older with moderate or mild lung disease as a result of cystic fibrosis received 5 ml of nebulized pGM (normal CFTR gene in a liposome vector) or 5 ml 0.9% saline every month for 12 months.

The scientists measured the change in lung function of patients as a relative change of percent predicted forced expiratory volume in 1 second (FEV1). Lung function (FEV1) was measured at each visit before administration of the treatment.

Data from 116 patients (who received nine or more doses) were analysed and the results are presented on the graphs below. Data are expressed as relative percent change from the baseline FEV1.

Error bars show the standard error of the mean.

Graph A All patients receiving treatment

Graph B Patients with more severe reduced lung function at start of treatment (Baseline FEV1 50–70%)

Graph C Patients with less severe reduced lung function at start of treatment (Baseline FEV1 70–90%)





