EXPLORING ECONOMIC VALUE IN TREATING *PSEUDOMONAS AERUGINOSA* INFECTIONS IN CYSTIC FIBROSIS – EARLY MODELLING TO INFORM PRODUCT DEVELOPMENT

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INTRODUCTION

With an increasing number of patients with Cystic Fibrosis (CF) over the age of 18 years of age, treatment of chronic infection is commonplace. *Pseudomonas aeruginosa* (*P. aeruginosa*) is the predominant bacterial pathogen, associated with more aggressive decline in pulmonary function in the CF lung. Daily maintenance therapy is an important part of suppression of chronic infection and comprises mainly nebulised or inhaled antibiotics. There remains significant unmet need in suppressing chronic *P. aeruginosa* infections, in particular managing the side effects of long term antibiotic use, addressing antimicrobial resistance, and in reducing exacerbations.

The novel approach under review has several mechanisms of action in relation to *P. aeruginosa,* firstly interfering with quorum sensing (QS) between *P. aeruginosa* cells, thereby preventing formation of biofilms by these cells and secondly it is bactericidal by virtue of its ability to bind to cysteine residues in bacterial enzymes. In essence it acts as a resistance breaker and potentiator for antibiotic use. The question was whether even if successful, could it be assessed as cost effective.

OBJECTIVES

To develop an economic model to explore the relative contribution of potential mechanisms driving the value of a new technology to manage *Pseudomonas aeruginosa* infections in Cystic Fibrosis (CF).

METHODS

Following a literature review to identify published models in CF, a previously published model (Tappenden et al, 2013) was replicated and adapted to explore a variety of clinically plausible mechanisms by which a new technology could impact upon health-related quality of life, resource use and cost. These included: drug acquisition costs, reduction in inhaled aminoglycoside use, exacerbation risk, exacerbation level (major/minor), exacerbation costs, exacerbation disutility, mortality risk, CF progression and HRQL. The model estimated costs from the perspective of the NHS and health outcomes using quality-adjusted life-years (QALYS) in line with UK guidelines for economic evaluation and was developed probabilistically to account for parameter uncertainty. The new technology was defined as an adjunct to standard of care treatment (in this case inhaled aminoglycosides).

RESULTS

Model results indicated that the cost-effectiveness of the new technology in CF was highly sensitive to its price with large shifts in incremental cost-effectiveness observed across relatively small changes in the daily acquisition cost. The potential reduction in adjunctive inhaled aminoglycoside therapy was the next most important driver of cost-effectiveness. The third most important driver of cost effectiveness was reduction in risk of exacerbations.

FIGURE 1.

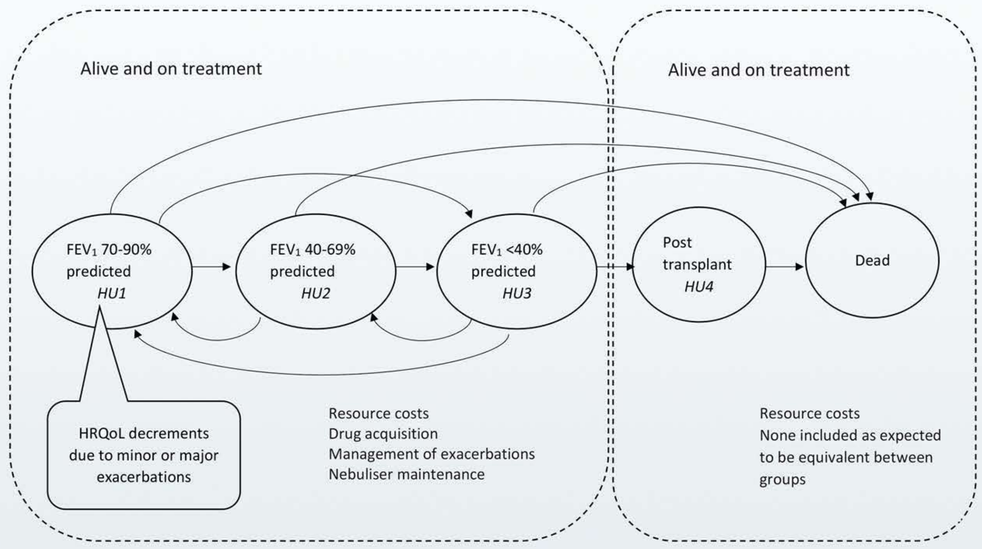


FIGURE 2.

Deterministic results						
	Life years	Discounted life years	QALYs	Discounted QALYs	Costs	Discounted costs
Tobramycin	17.9	12.6	13.572	9.532	£ 145,471	£ 103,788
New treatment	17.9	12.6	13.597	9.551	£ 146,224	£ 104,325

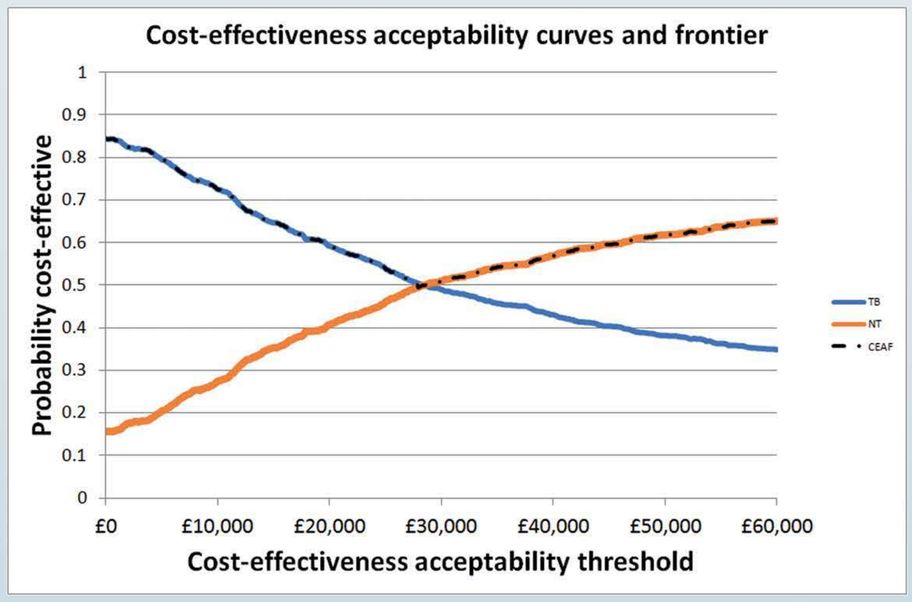
	Incremental Costs	Incremental QALYs	ICER
Tobramycin	0.00	0.000	0.00
New treatment	536.78	0.018	29,194

Probabilistic results						
	Life years	Discounted life years	QALYs	Discounted QALYs	Costs	Discounted costs
Tobramycin	18.0	12.5	13.59	9.49	£ 145,764	£ 103,438
New treatment	18.0	12.5	13.62	9.50	£ 146,522	£ 103,974

	Incremental Costs	Incremental QALYs	ICER
Tobramycin	0.00	0.000	-
New treatment	£ 536.47	0.019	27,833

Probability cost-effective at £20k	Probability cost-effective at 30k
59%	49%
41%	51%

FIGURE 3.



CONCLUSION

The development of an early model allows for a detailed understanding of the mechanisms which drive the potential value of a new technology. This can be used to focus not only technology development but also the design of studies for evidence collection to best capture the value. The wider benefits of reduced inhaled aminoglycoside use, e.g. reductions in resistance and treatment-related side-effects, are an area of uncertainty and warrant further research.