

Cost-effectiveness of Disease Monitoring Versus Lifelong Eculizumab on aHUS

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INTRODUCTION

Atypical Haemolytic Uraemic syndrome (aHUS) is a rare life-threatening disease that can lead to the formation of blood clots throughout the body, typically affecting the kidneys. **If left untreated this condition can lead to kidney failure.**

Eculizumab is an effective treatment that prevents disease recurrence and progression, improving survival.

OBJECTIVE

The benefits of lifelong eculizumab treatment are uncertain, and lifelong therapy has high costs for healthcare providers.

This economic evaluation applied the results from the Stopping Eculizumab Treatment Safely in aHUS (SETS aHUS) trial [1] and assessed the cost-effectiveness of replacing lifelong eculizumab with a disease monitoring strategy over the long term.[2]

METHOD

De novo Markov model informed by the patient characteristics of the SETs aHUS trial [1]: Adult patients, 51% males, receiving eculizumab with normal renal function, or Chronic Kidney Disease (CKD) stages 1 to 3b.


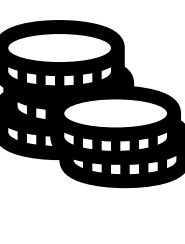

The Markov model focused on the long-term risk of needing to re-start eculizumab, and the risk of kidney disease progression from stopping treatment, specifically towards advanced disease (CKD 4, end stage renal disease; ESRD, and kidney transplant), see Figure 1. The model **also included the impact of lifelong treatment on patient quality of life.**

The risk of re-starting eculizumab, and the risk of kidney disease progression were estimated from trial time-to-event data, and the impact of stopping treatment and re-starting treatment on quality of life was measured using EQ-5D-5L data from the trial.[2] The impact of long-term disease was informed by the published literature.

RESULTS

Health effects were measured in **quality adjusted life years (QALY) and costs in 2022 Pounds Sterling (£).**

Stopping eculizumab with disease monitoring compared with the lifelong maintenance of eculizumab over an 80-year time horizon led to:

-  **Average increase in 0.08 QALYs per patient**
(95% CrI: 0.53 to -0.33 QALYs)
-  **Average cost-savings of -£4,234,196 per patient**
(95% CrI: -£6,403,694 to -£684,495)
-  **Average decrease in survival of 0.0005 years per patient**
(95% CrI -0.0029 to 0)

Stopping eculizumab had a 100% probability of being cost-effective up to a £250,000/QALY willingness to pay threshold and increased average QALYs in 64% of the simulations while still being less costly.

Uncertainty around: risk of restarting eculizumab, the quality-of-life difference from stopping eculizumab, and the cost of eculizumab had the largest impact on cost-effectiveness estimates. However, **results remained robust after testing alternative parameters and assumptions.**

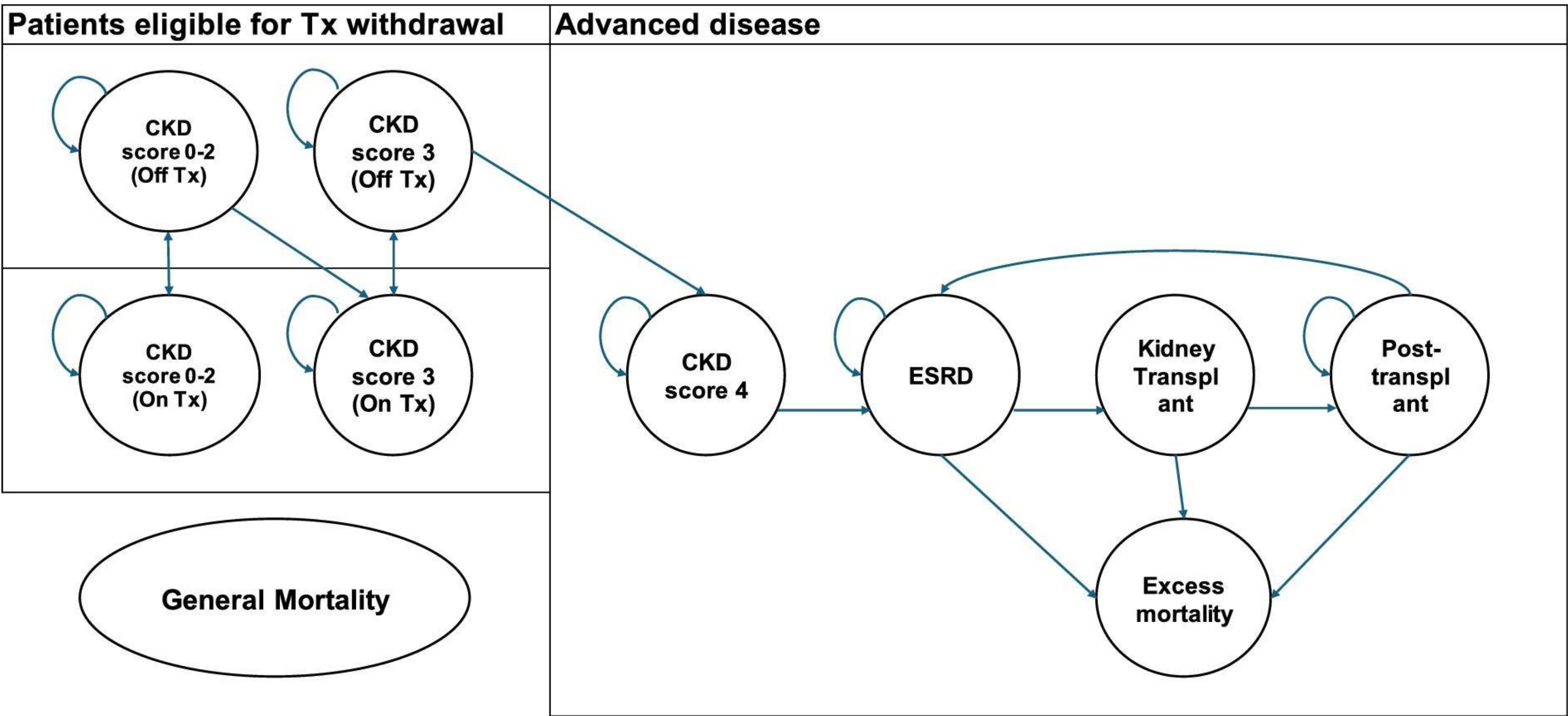


Figure 1: Decision model structure split into off treatment, on treatment, general mortality, and advanced disease. Tx = treatment, CKD = chronic kidney disease, ESRD = end stage renal disease

Intervention	Life Years	QALYs	Costs	Incremental QALYs (95% CrI)	Incremental costs (95% CrI)
Lifelong eculizumab	25.44	21.04	£8,879,821		
Disease monitoring	25.44	21.12	£4,645,625	0.08 (-0.33 to 0.53)	-£4,234,196 (-£6.4m to -0.7m)

Abbreviations: QALY = quality adjusted life year; CrI = credible interval

Table 1: Probabilistic cost-effectiveness results from the decision model. Stopping treatment with disease monitoring is shown dominant (less costly and more effective) compared to lifelong eculizumab.

CONCLUSIONS

Results from our decision model suggested that **stopping eculizumab coupled with disease monitoring was a cost-effective alternative to the lifelong delivery of eculizumab.** Stopping eculizumab with disease monitoring led to **average cost savings of £4.2 million per patient** with a **minimal impact on health-related quality of life** (0.08 QALYs), and a **minimal impact on survival** (-0.0005 LYs, equivalent to 4 hours) over 80 years, see Table 1.

There is potential to use Real World Evidence to generate further evidence over: longer time horizons, alternative treatments (e.g. ravulizumab), and other restart strategies.

REFERENCES

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Additional information

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