

# Cost-effectiveness of Disease Monitoring Versus Lifelong Eculizumab on aHUS

HTA212

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SCAN ME

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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.

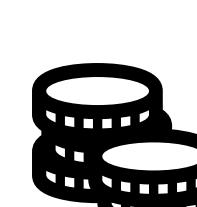
## RESULTS

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

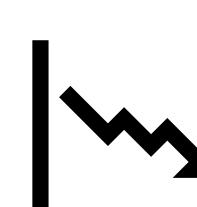
Stopping eculizumab with disease monitoring compared with the lifelong maintenance of eculizumab over an 80-year time horizon led to:



**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.**

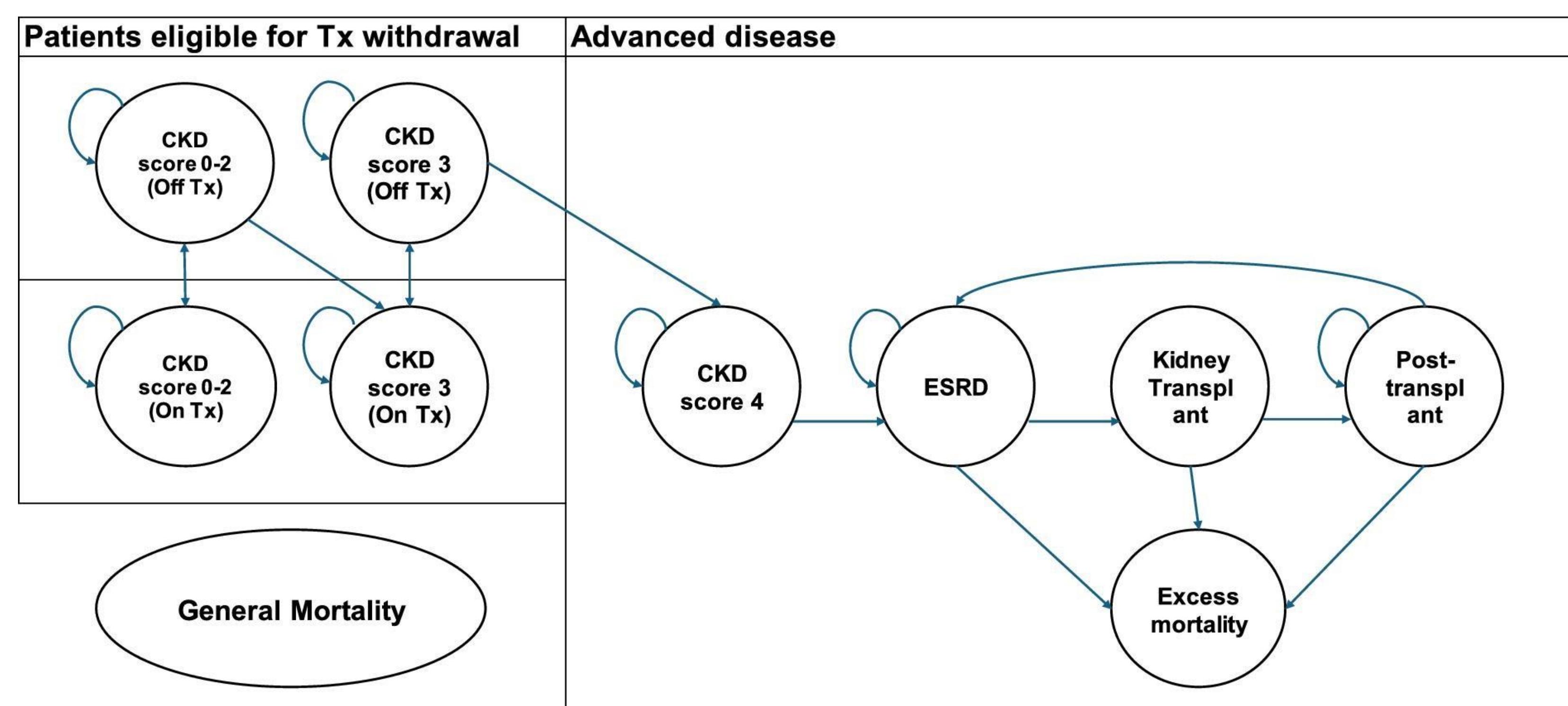


Figure 1: Decision model structure split into off treatment, on treatment, general mortality, and advanced disease. Tx = treatment, CKD = chronic kidney disease, ESRD = end stage renal disease

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.

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## REFERENCES

- Bryant A, Lecouturier J, Orozco-Leal G, et al. Eculizumab withdrawal and monitoring in atypical haemolytic uraemic syndrome (SETS aHUS): a multicentre, open label, prospective, single arm trial. *The Lancet Regional Health–Europe*. 2025 Sep 1;56.
- Orozco-Leal G, Vale L, Oluboyede Y, et al. Cost-effectiveness of lifelong eculizumab versus disease monitoring of aHUS. *Nephrology Dialysis Transplantation*. 2025 Aug 22:gfaf166.

## Additional information

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