A budget impact analysis of a new treatment for adults with homozygous familial hypercholesterolaemia

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Introduction

- Homozygous familial hypercholesterolaemia (HoFH) is a rare and life-threatening disorder caused by an impaired low-density lipoprotein receptor (LDLR) pathway, resulting in significantly elevated plasma levels of low-density lipoprotein cholesterol (LDL-C), leading to premature atherosclerosis.[1]
- Lomitapide was clinically commissioned by the National Health Service (NHS) England for the treatment of adults with HoFH in 2018.[2]
- HoFH patients who do not reach their LDL-C goal* on conventional lipid lowering treatments (LLTs) (e.g., statins, ezetimibe, apheresis, and PCSK9 inhibitors [PCSK9i]) are eligible for additional treatment with lomitapide **(Figure 1).**
- In 2024, NICE recommended evinacumab as a new treatment option for HoFH patients aged ≥12 years; positioned as a replacement for lomitapide.[3]

Figure 1. HoFH treatment pathway in England

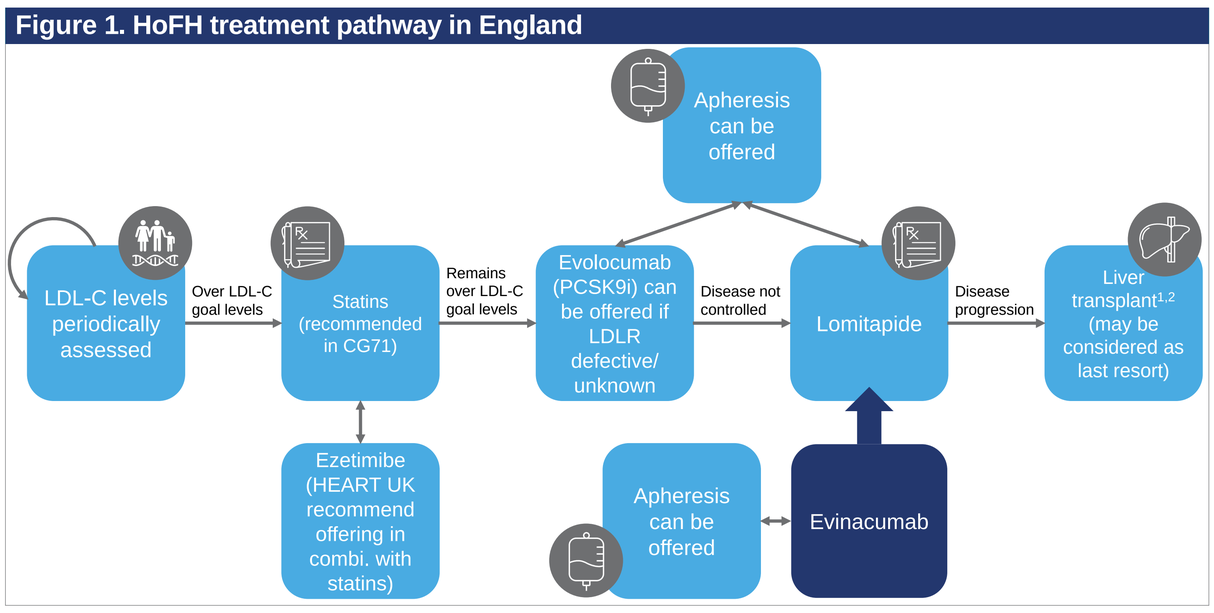


Figure adapted from NICE evinacumab guidance (TA1002), NICE clinical guidelines (CG71), and KOL input.[3,4]
Abbreviations: HoFH, homozygous familial hypercholesteraemia; KOL, key opinion leader; LDL, low-density lipoprotein; LDL-C, low-density lipoprotein cholesterol; LDLR, low-density lipoprotein receptor; NICE, National Institute for Health and Care Excellence; PCSK9i, proprotein convertase subtilisin/kexin type 9 inhibitor; UK, United Kingdom.

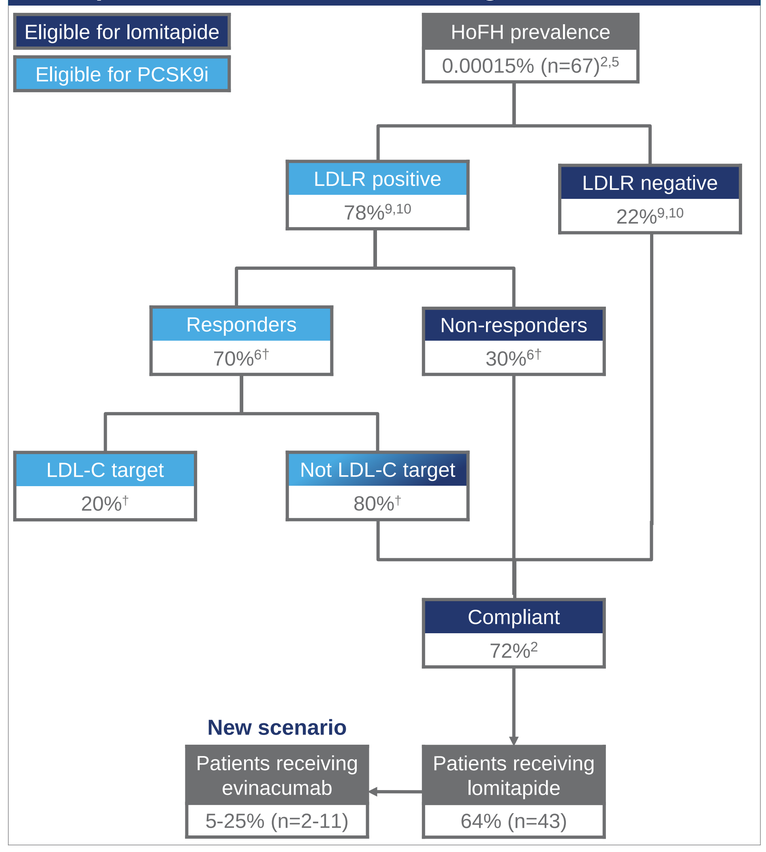
Objective

- To investigate the potential budget impact of introducing evinacumab in adult HoFH patients in England compared to current standard of care (SoC).

Methods

- A budget impact analysis with a 5-year time horizon was conducted from an NHS/Personal Social Services (PSS) perspective.
- The number of patients was estimated from the adult population in England and the estimated prevalence of HoFH (1 per 670,000).[2,5]
- A new scenario with evinacumab estimated the costs if a hypothetical incremental 5% of lomitapide-eligible and -compliant patients received evinacumab each year instead of lomitapide, rising to a 25% market share in year 5 (**Figure 2**).
- Evinacumab dosage was calculated based on an average body weight of 72.7 kg in the ELIPSE trial (4 vials per 28 days).[6]
- Patients receiving lomitapide were assumed to receive one pack per 28 days, based on the average dose of 10-20 mg/day observed in real-world evidence studies and confirmed by a key opinion leader (KOL).[7,8]

Figure 2. Patient flow to show current population receiving lomitapide and new scenario receiving evinacumab



[2]NHS England (2018). [5]Office for National Statistics (2023). [6]Raal et al. (2017). [9]Sánchez-Hernández et al. (2016). [10]Alonso et al. (2016).
[†]Confirmed in interview with KOL with experience in managing HoFH patients in England.
Abbreviations: HoFH, homozygous familial hypercholesterolaemia; LDL-C, low-density lipoprotein cholesterol; LDLR, low-density lipoprotein receptor; PCSK9, proprotein convertase subtilisin/kexin type 9.

Table 1. Model assumptions and inputs

All patients received statins and ezetimibe[4,11]	57% of patients receiving lomitapide also received apheresis[†]
LDLR+ patients who were responsive to treatment received evolocumab	38% of non-lomitapide patients received apheresis[†]
Patients who wouldn't reach their LDL-C goal with PCSK9i would receive lomitapide (**Figure 2**)	All non-lomitapide patients and 75% of lomitapide patients received apheresis biweekly[†]
Lomitapide treatment compliance was 72%[2]	25% of lomitapide patients reduced apheresis to monthly[†]
Costs (2022 £) included disease and drug monitoring, drug acquisition at list price, and infusion and apheresis costs	

[†]Assumption informed by interview with KOL with experience in managing HoFH patients in England.

Annual Budget Impact at Year 5

- £11.539m without evinacumab
- £12.667m with evinacumab

Incremental Budget Impact With Evinacumab

- +£226,243 in year 1 (2024)
- +£1,128,525 in year 5 (2028)

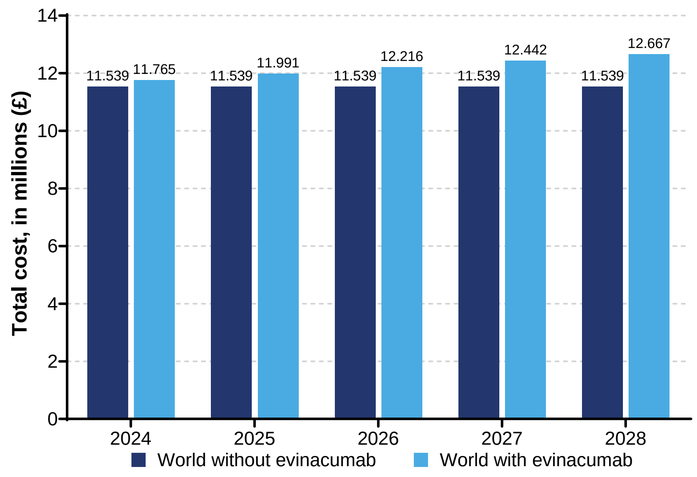
Medical Cost Impact

A 25% market share of evinacumab in lomitapide-eligible patients increases treatment costs by £1.131m, and lowers monitoring costs by £2,802

Results

- Based on reported prevalence rates in England, 67 patients have HoFH, and 43 patients are eligible for and compliant with lomitapide.[2,5]
- The gross budget impact to NHS England to treat patients with HoFH with current SoC (including lomitapide) at baseline was £11,538,953/year at list price (**Figure 3**).
- Introduction of evinacumab as a replacement to lomitapide in 5% of eligible patients per year was estimated to lead to a net budget increase from £226,243 in year 1 to £1,128,525 in year 5 (**Figure 4**).
- In year 5, increased treatment costs contribute £1,131,327 to the budget impact, and monitoring costs reduce the budget impact by £2,802.

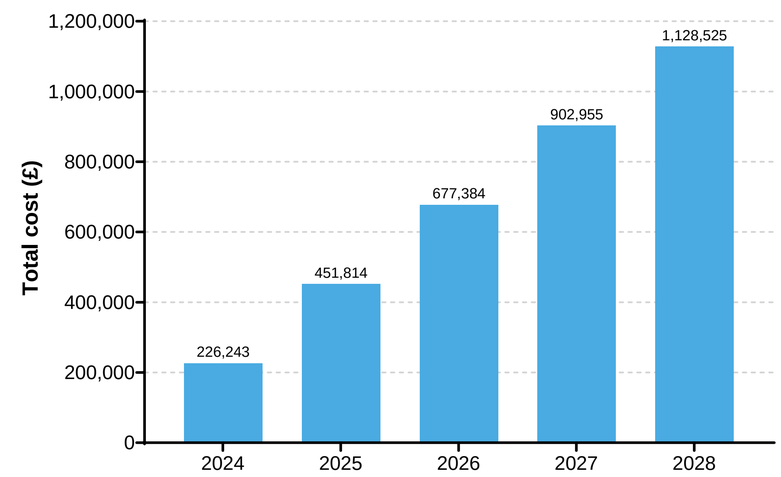
Figure 3. Total annual budget impact of introducing evinacumab, over a 5-year timespan



Limitations

- HoFH is an ultra-rare disease, meaning that model inputs are drawn from a small number of patients in real-world data sources from countries across Europe.
- To mitigate the limitations, a KOL was consulted to ensure that inputs were relevant to HoFH treatment practices in England.
- As net prices in England are confidential, only published list prices were used in this analysis.

Figure 4. Net budget impact of introducing evinacumab (LDLR testing included, patients stay on PCSK9i)



Abbreviations: LDLR, low-density lipoprotein receptor; PCSK9i, proprotein convertase subtilisin/kexin type 9 inhibitor.

References

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Footnotes

*The European Atherosclerosis Society recommends the following LDL-C goals[12]:
- Children and adolescents: <3 mmol/L (<115 mg/dL)
- Adults with no major atherosclerotic cardiovascular disease (ASCVD) risk factors: <1.8 mmol/L (<70 mg/dL)
- Adults with ASCVD/major ASCVD risk factors: <1.4 mmol/L (<55 mg/dL)

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