Poster ID #121129

Investigating the correlation between UK-based clinical trial sites and NICE decision making

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Objective

- Understand if UK-based clinical trial sites lead to a higher likelihood of a positive NICE reimbursement decision

Conclusions

- Although causation was not established, **UK-based late phase clinical trial sites have a positive correlation to NICE decision making** as part of a wider robust submission
- Across all therapeutic areas, the rate of positive NICE decision is higher on average when UK-based clinical trial sites are included (p=0.01)
- **UK clinical trial sites were shown to have a consistently positive influence** in AIML analysis relative to a number of confounding factors

Plain language summary

Why did we perform this research?

To understand if the inclusion of UK-based clinical trial sites supports a new medicine receiving a positive access decision

How did we perform this research?

We used a statistical and machine learning approach, utilising both publicly available and IQVIA owned data

What did we discover?

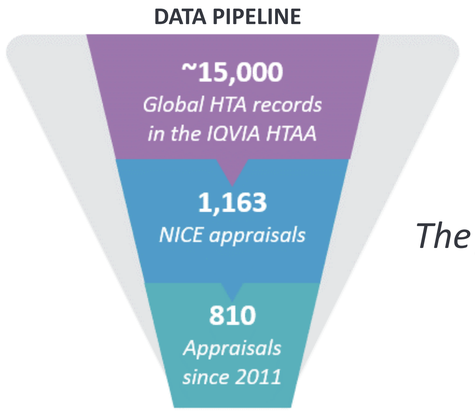
We identified a correlation between UK-based clinical trial sites and positive access decisions, but note that we cannot use this research to establish causation

Introduction

- **Many factors influence NICE HTA assessments**, such as product efficacy, disease area unmet need, and clinical trial design
- **Clinical trial location is not traditionally considered a main driving factor**, but has implications for patient access to medical innovation
- To understand the value of UK based clinical trial sites, this analysis aimed to investigate if the inclusion of UK sites could have an impact on securing a positive NICE HTA decision

Methods

- **A dataset of NICE decisions was built to include ~30 influencing factors** including measures of efficacy, safety, unmet need, and pivotal trial design
- The appraisals were analysed descriptively, and then **using AIML to account for the impact of confounding factors**
- Due to class imbalance, large feature sets, missing data, and collinearly correlated variables, AIML was chosen over traditional regression modelling

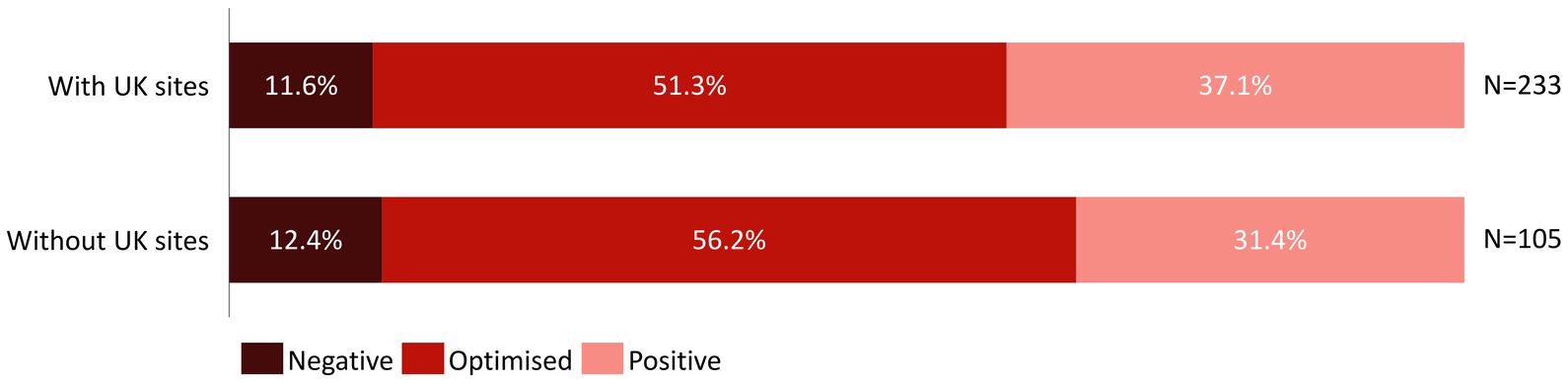


The final analysis dataset included over 800 NICE appraisals

Analysis

Descriptive statistical analysis shows a small but significant advantage for appraisals with at least one UK trial site (see Figure 1). However, this does not account for confounding factors or imply causality.

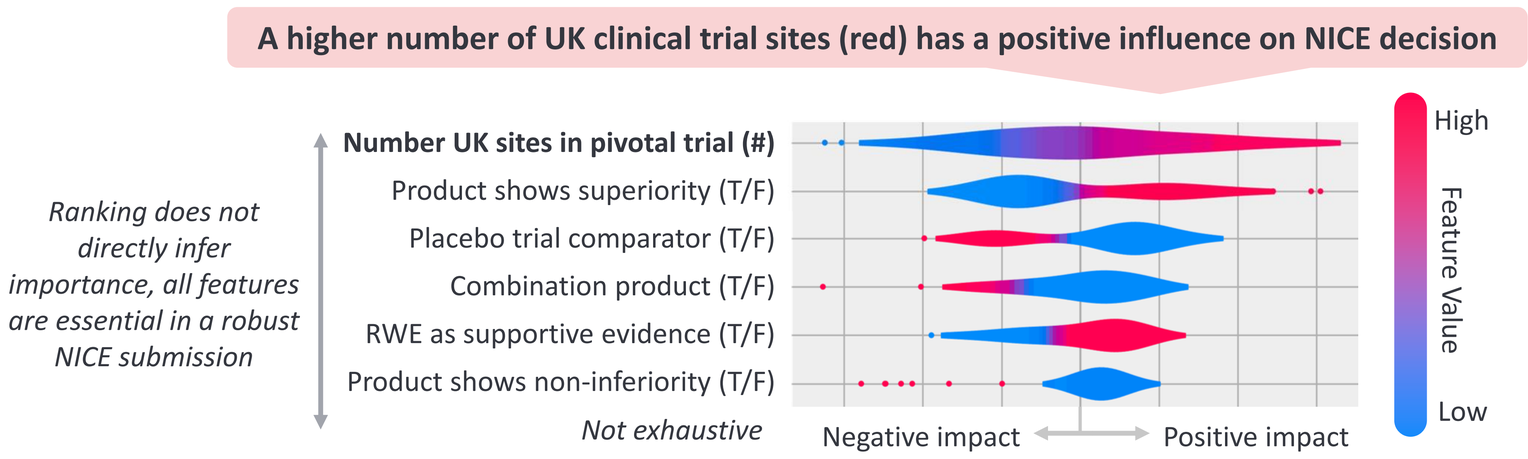
Figure 1. Descriptive analysis of correlation between UK trial sites and NICE decision (N=338)



Optimised defined as any recommendation in a smaller group of patients than originally stated in the marketing authorisation. Cancer Drug Fund reimbursement classed as optimised or positive as appropriate

A machine learning model was built to analyse the impact of UK clinical trial sites on NICE decision relative to other confounding factors, showing a consistent positive influence. Analysis below shows that the **higher the number of UK sites in a pivotal trial, the higher the positive influence on the prediction of NICE decision.**

Figure 2. Analysis of feature impact in the AIML model for positive NICE decision (N=381)



Colour scale for numerical features (#) corresponds to a continuous value, with binary features (T/F) represented as TRUE (red) or FALSE (blue). Each dot is a record in the data, colour noting value of the feature. Position on X-axis: Positive should be interpreted as having a positive influence on the prediction[1]

Limitations & Discussion

- Limitations of the research include inability to infer causality, reliance on correct reporting of clinical trial locations in the data sources, and the relatively small number of historical NICE appraisals for analysis
- Although descriptive analysis shows a **small and consistent correlation between UK-based clinical trial sites and NICE decision**, it does not identify it as a driving factor
- AIML showed **the small but consistently positive influence of UK site inclusion on the prediction of positive NICE decision relative to a wide range of confounding factors**

Poster presented at ISPOR EUROPE 2022
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Disclosures

IQVIA received fees for the research presented in this poster. At the time of the research H. Taylor, C. Banks, V. Ohlmeyer and N. Hogan were employees of Takeda UK Ltd. H. Taylor, V. Ohlmeyer and N.Hogan own Takeda stock.

Abbreviations

AIML: Artificial Intelligence & Machine Learning, **HCP**: Health Care Professional, **HTA**: Health Technology Assessment, **NICE**: National Institute of Care Excellence, **SHAP:** Shapley Additive Explanations

Sources

(1) https://arxiv.org/pdf/1705.07874.pdf