



Leveraging Machine Learning for Predicting Health Technology Assessment Outcomes

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Background

- Heterogeneity in Health Technology Assessment (HTA) outcomes across different settings contributes to disparities in patient access to innovative medicines.
- Variations in HTA decisions can arise from differences in assessment methodologies, evidence interpretation, and contextual factors among agencies.
- Despite the critical impact of these decisions, there is limited research on the predictive ability of pre-submission data to forecast HTA outcomes across various agencies and settings.

Objectives

- Evaluate Predictive Modelling Approaches:** Assess the effectiveness of different machine learning models in predicting HTA outcomes using pre-submission data.
- Identify Influential Features:** Determine which variables have the greatest impact on HTA decisions across different agencies.
- Enhance Understanding of HTA Decision-Making:** Provide insights into the factors influencing HTA outcomes to inform future submissions and policy-making.

Results

The **XGBoost** model achieved the highest performance, with an accuracy of **91%**, F1-Score of **0.91**, and ROC-AUC of **0.95**. The **Random Forest** model closely followed with an accuracy of **90%**, F1-Score of **0.90**, and ROC-AUC of **0.96**. Logistic Regression and Decision Tree models showed lower performance.

Model	Accuracy	F1-Score	ROC-AUC
Logistic Regression	86%	0.87	0.92
Decision Tree	87%	0.87	0.88
Random Forest	90%	0.90	0.96
XGBoost	91%	0.91	0.95

Superior Performance of Ensemble Models:

- Handling Complex Interactions:** **XGBoost** and **Random Forest** outperformed simpler models due to their ability to capture non-linear relationships and handle complex interactions while reducing overfitting.
- Robustness to Multicollinearity:** These models are less sensitive to multicollinearity, which complements the steps taken to address it.
- Feature Importance:** Provides insights into which variables are most influential after addressing multicollinearity.

Key Features Influencing HTA Outcomes:

- Type of Economic Analysis:**
 - 'Cost minimisation'** (VIF 3.47) analyses significantly increased the likelihood of approval. Indicating that submissions focusing on cost-saving measures are favored.
- Trial Blinding Type:**
 - 'Open label or unblinded'** (VIF 3.43) trials were influential in predicting outcomes. Suggests that transparency in trial design may influence HTA decisions.
- ICER Submitted Band:**
 - 'Not Reported'** ICERs had a high VIF (6.35) and were associated with less favorable outcomes.
 - Higher ICER bands **'£75k+'** (VIF 2.45) negatively influenced approval likelihood, emphasising the importance of cost-effectiveness in HTA evaluations.
- Agency-Specific Effects**
 - The specific HTA agency (e.g., NICE, SMC) had VIFs around 2, indicating that the specific HTA agency plays a role in the outcome.
- Therapeutic Areas:** Certain areas like 'Oncology' (VIF 6.56) and 'Pulmonology' (VIF 1.43) were significant predictors, possibly due to high unmet medical needs or the availability of innovative treatments in these areas.

Conclusion & Key Takeaways

- The study demonstrates that pre-submission data can effectively predict HTA outcomes using when multicollinearity is addressed, with **XGBoost** being the most effective model. Ensemble Methods outperform simpler models by capturing complex patterns.
- Key factors influencing HTA decisions** include the type of economic analysis, trial design, cost-effectiveness data, and the specific HTA agency.
- Insights from this type of analysis can guide **submission strategies** for manufacturers, while **policy-makers** can use these findings to reflect on assessment processes and address disparities.

Note: This poster presents a preliminary and exploratory study into predictive modeling for HTA outcomes. The findings offer valuable insights but should be interpreted within the context of the study's limitations.

Methods

1. Data Collection:

- Data on drug characteristics, clinical evidence, economic evidence, disease characteristics, and firm characteristics were extracted for 560 HTA decisions from 2009 to 2024 using HTA-Hive's database.
- The analysis focused on five established HTA agencies conducting cost-effectiveness analyses: **NICE** (England), **CADTH** (Canada), **SMC** (Scotland), **INESSS** (Quebec, Canada), **TLV** (Sweden).

2. Data Preprocessing:

- Variable Selection:** Excluded 'Manufacturer' and 'Diseases' variables due to high dimensionality and to prevent overfitting. Focused on variables with significant impact and manageable levels of multicollinearity.
- Target Variable:** Binarised 'HTA Outcome' into '**Approved**' (including 'Listed with Criteria, LWC' and 'Listed, L') and '**Not Approved**' ('Do Not List, DNL') for simplified modelling.

3. Feature Engineering:

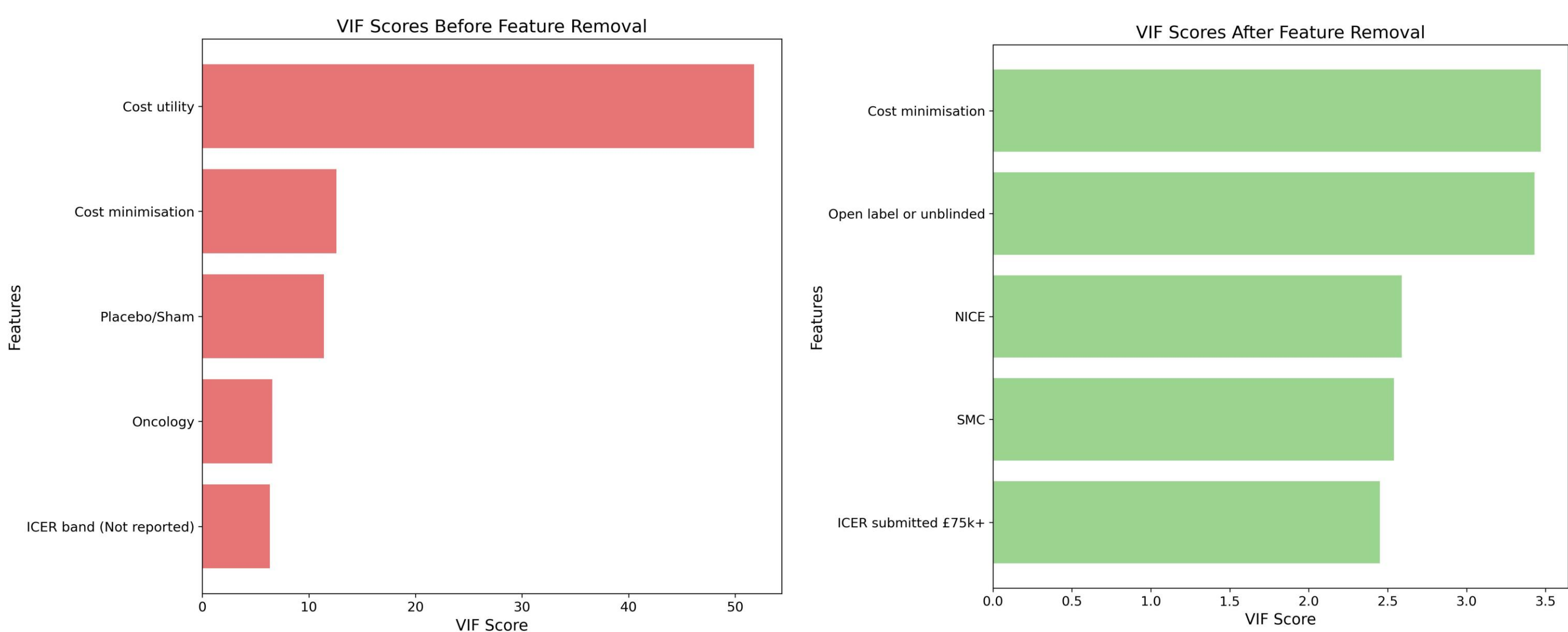
- Encoding Categorical Variables:** Applied one-hot encoding to transform categorical variables into numerical format suitable for modelling.
- ICER Submitted Band:** Categorised ICERs into bands (£0–15k, £15–30k, £30–45k, £45–60k, £60–75k, £75k+), including 'dominant', 'not reported', and 'confidential'. All ICER values converted to **GBP** for consistency.
- Feature Removal:** Removed features with **VIF > 5** to reduce redundancy and improve model stability.

4. Addressing Class Imbalance:

- Utilised **Synthetic Minority Over-sampling Technique (SMOTE)** to balance the dataset and enhance model training.

5. Model Development:

- Models Evaluated: **Logistic Regression, Decision Tree, Random Forest, XGBoost**.
- Training and Testing:** Split data into training and testing sets (80/20 split). Performed **cross-validation** and **hyperparameter tuning** to optimise model performance and prevent overfitting.



Future Research

- Expanded datasets may enhance predictive power of models:** Larger datasets will enable exploration of variable sets with greater dimensionality including firm characteristics and disease.
- Quantifying and comparing clinical benefit across disease remains a challenge:** There is a need for standardised metrics to compare the clinical efficacy across settings.
- Policy variations:** Differences in how agencies interpret evidence and apply additional criteria suggest that models could be further refined to account for these nuances.
- Broader Application:** While preliminary results provide insights on England, Scotland, Sweden, and Canada, further research exploring determinants of HTA outcome in other settings would be of interest.
- Enhancing Model Transparency:** Address the 'black box' nature of machine learning models to increase trust among decision-makers. Using interpretable models or explainability techniques like **SHAP values** to elucidate feature impacts.