

predictNMB: An R package to estimate if or when a clinical prediction model is worthwhile

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Summary

Clinical prediction models are frequently developed for identifying patients at risk of adverse health events, and possibly guiding the use of treatment, but are often not validated or implemented in clinical practice (Hendriksen et al., 2013; Steyerberg et al., 2013). This could be due to several factors including poor performance or the lack of an effective intervention that can be implemented in response to prediction of high risk. The predictNMB R package performs simulations to evaluate the use of hypothetical clinical prediction models (with a binary outcome) to help inform development and implementation decisions, and estimate potential impacts in terms of costs and health outcomes. This package allows the user the flexibility to adjust simulation inputs regarding the prediction model's performance, its target population, the costs of the event being predicted, and the effectiveness of interventions that the model is being used to recommend. More details about the package, including guides and a detailed example are available on the package site.

Statement of need

Clinical decision support systems are often used to classify patients into high- or low-risk groups and to recommend treatment assignment (Steyerberg et al., 2013). These systems can only perform as well as the underlying model(s) informing decision support recommendations, the treatments being recommended, and the implementation of the system within clinical settings. Often, the cost-effectiveness of these systems is not known until they are developed, implemented, and evaluated (Reilly & Evans, 2006; White et al., 2023). The predictNMB R package aims to avoid this delay by facilitating early estimation of the cost-effectiveness of these systems. We expect most users to be either: 1) those involved in health service decision making regarding investment in development or implementation of clinical decision support systems, or 2) clinical prediction model developers, who may be deciding whether to invest efforts into clinical prediction model development or validation. Characteristics of the user's given patient population are incorporated using Monte Carlo simulation to estimate the expected cost-effectiveness of a given system (under an assumption of ideal implementation and complete adherence to recommendations) to provide guidance on cost-effectiveness before prediction models are developed or implemented. For example, by evaluating this simulated decision support system and finding that a clinical prediction model would only be effective (better than a treat-all or treat-none approach) at an unrealistically high level of model performance, users would then have opportunity to reduce research waste by avoiding model development, implementation, and evaluation in a clinical setting. Similar to a statistical power analysis, predictNMB allows users to estimate how well their model would need to perform, and its



expected benefit, if implemented to offer a treatment recommendation. It may be found that a given decision support system may only be likely to improve care when the available treatment is of a certain level of effectiveness or when the prevalence of the condition is relatively low or high, and this may better guide the user regarding which treatment the system should be recommending, or for which patients.

Features

predictNMB simulates well-calibrated prediction models using logistic regression and incorporates a range of inbuilt cutpoint selection methods, including a treat-all (cutpoint=0) and treat-none (cutpoint=1) method, and two methods that aim to maximize the Net Monetary Benefit (NMB): 'cost-minimizing' (Wynants et al., 2019) and 'value-optimizing' (Parsons et al., 2023). It also allows the user to specify any other function for cutpoint selection. Evaluation of the models in terms of the NMB requires the user to pass information regarding the costs associated with each of four possible classifications. A helper function is provided to make this process easier by taking arguments in terms of treatment effectiveness and outcome costs, along with their uncertainty (see creating NMB functions vignette for more details).

The simulations are stored as one of two types of objects, depending on whether a single scenario was used for simulation or if a range of values were screened over several simulation scenarios. Plotting and summarizing methods for these objects are exported to easily visualize and evaluate the results of the simulation study (see summarising results vignette for more details).

A detailed example of a pressure injury model using inputs from the literature is included as a vignette. Applying predictNMB for this use case indicates that, when using realistic values for the intervention and prevalence of pressure injuries and their costs, the clinical prediction model may be useful when the model is particularly well-performing (area under the receiver operator characteristic curve > 0.8) and when the event rate for pressure injuries is lower (event rate of 0.05). When the event rate was higher, the treat-all strategy was preferred to any of the model-guided approaches or treating none. This suggests that model development and implementation efforts should target patient populations where the event rate of pressure injuries is lower than 0.05.

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