A taxonomy of the impact of precision medicine in heath technology assessment

Professor Matthew Taylor [1]

[1] **York Health Economics Consortium, York, United Kingdom**

INTRODUCTION

Precision medicine refers to the tailoring of preventative or treatment strategies to subgroups of patients or subcategories of disease, which differ according to baseline disease risk, prognosis or likely treatment response.

Healthcare systems are increasingly using precision medicine to ensure that patients receive the most appropriate treatment. Whilst the benefits of precision medicine are widely discussed, there is less discussion around the circumstances where precision medicine is most likely to reap benefits, and even less discussion around the potential downsides of precision medicine.

This poster outlines the potential benefits and challenges that may arise from the increased use of precision medicine in healthcare, as well as some recommendations for decision makers.

METHODS

A hypothetical case study was developed that with two available active treatments and one 'no treatment' option. It was assumed that Treatment A was more expensive than Treatment B. It was also assumed that each treatment option was mutually exclusive (e.g. each patient gets only one opportunity for treatment) and that sequencing of treatments would not be possible.

- Treatment A (with an effectiveness rate of 90%)
- Treatment B (effectiveness = 60%)
- No treatment (effectiveness = 0%)

It was assumed that 'effective' treatment would lead to the same outcomes, no matter how that outcome was achieved. Likewise, it was assumed that the outcome for unsuccessful treatment would be the same no matter which treatment had been used.

Different scenarios are run to reflect different types of 'overlap' between treatments, where the individual patients who would benefit from Treatment B are:

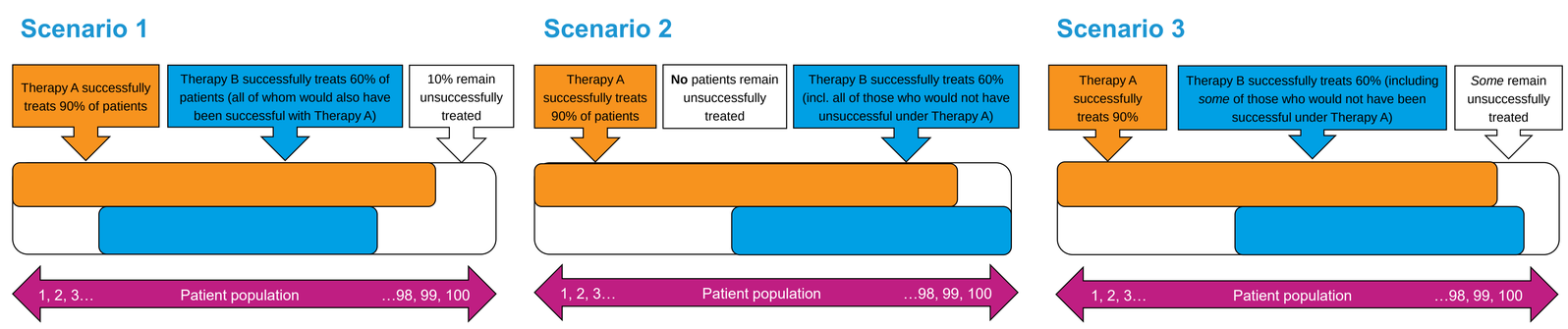
(i) Entirely 'within' the 90% that would also benefit from Treatment A
(ii) Covering the 10% who would not benefit from Treatment A but also including some would benefit from Treatment A
(iii) An 'overlapping' case in-between.

The scenarios are shown in Figure 1.

For each scenario, two options were assessed: 'With precision medicine' (i.e. the decision maker can identify which patients will benefit from each treatment) and 'no precision medicine'. A description is then provided around the likely impact on several different outcomes, including:

- Effectiveness of the policy (health gains)
- Total cost spending
- Data requirements
- Assessment of uncertainty
- Cost effectiveness and value for money

Figure 1: Scenarios



RESULTS AND RECOMMENDATIONS

Maximising health

Without precision medicine: If the aim were to maximise the health of the indicated population then a policy maker would generally prefer to recommend Treatment A, since this option has the highest level of success.
With precision medicine: A policy maker might now use precision medicine to identify which patients will benefit from each treatment.
Impact: In Scenario 1, this will not impact on population health, since the maximum effectiveness (90%) was already reached by approving Treatment A. In Scenarios 2 and 3, however, precision medicine would increase population health.

Measuring the cost impact

Without precision medicine: Depending on the specific cost-effectiveness ratios, a policy maker would need to choose *either* Treatment A or Treatment B (or 'no treatment') for all patients. This could have substantial cost implications if Treatment A was the cost-effective option.
With precision medicine: With the use of precision medicine, policy makers would be able to tailor decisions to each person. The cheaper option (Treatment B) could be prescribed to 60% of patients, with Treatment A only being used for the specific populations that would benefit from Treatment A but not Treatment B. Those people who would not benefit from either option could remain untreated.
Impact: In all scenarios, precision medicine would be likely to reduce overall costs.

Data requirements

Without precision medicine: In the absence of precision medicine, decision makers would reply on clinical evidence comparing Treatment A with Treatment B and also with no treatment. This could be based on RCTs and/or a meta-analysis.
With precision medicine: Data requirements would be very complex. Standard head-to-head trials would not be appropriate, because precision medicine would create specific sub-populations (i.e. those who will be likely to benefit from each treatment).
Impact: Precision medicine would substantially impact on the data requirements for a health technology assessment.

Assessment of uncertainty

Without precision medicine: Decision makers would use standard methods for assessing uncertainty (e.g. one-way sensitivity analysis and probabilistic sensitivity analysis) to compare the outcomes associated with each treatment option.
With precision medicine: Precision medicine would be likely to reduce the uncertainty around treatment effect (since each treatment would only be given to those most likely to benefit from it). However, sample sizes from trials would be likely to be far smaller, due to specific need for subgroup analysis (see 'data requirements', above).
Impact: Some aspects of uncertainty would be greatly improved (i.e. treatment effectiveness). However, it would be more difficult to run alternative scenarios in a model, because standard head-to-head data would not be available for each treatment in comparable populations.

Cost effectiveness and value for money

Without precision medicine: Policy makers will recommend one single treatment option which would, presumably, be a cost-effective option.
With precision medicine: Precision medicine would allow for more efficient allocation of resources, since only those patients who will benefit would receive treatment. The 'number needed to treat' to achieve each additional successful outcome would be reduced, improving the value for money.
Impact: In the short-term, value for money would be improved. However, this assumes that the prices for each treatment do not change. If prices were increased (as a result of each treatment being used more efficiently), the value for money may not improve.

CONCLUSION

Precision medicine may have many benefits, but its impact will be complex. It is likely that the overall health of an indicated population will increase, but there will be challenges with evidence generation and assessment of uncertainty. The impacts are likely to differ for aspects such as the type of disease (acute vs chronic; fatal vs non-fatal) and the accuracy of companion diagnostic.

CONTACT US

matthew.taylor@york.ac.uk | +44 1904 324884 | www.yhec.co.uk | York Health Economics Consortium

Providing Consultancy & Research in Health Economics