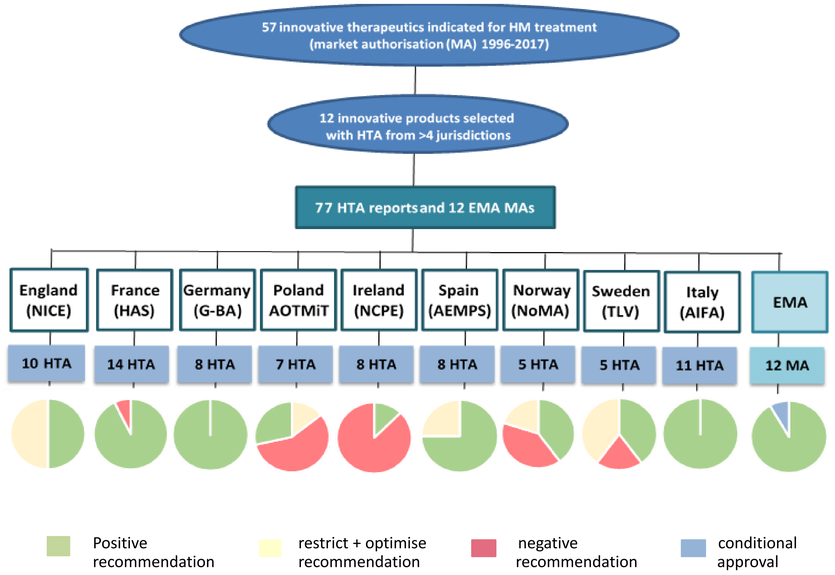
Above and beyond: Assessing the nature and impact of additional considerations above clinical and cost effectiveness by 8 HTA agencies for a rare disease area

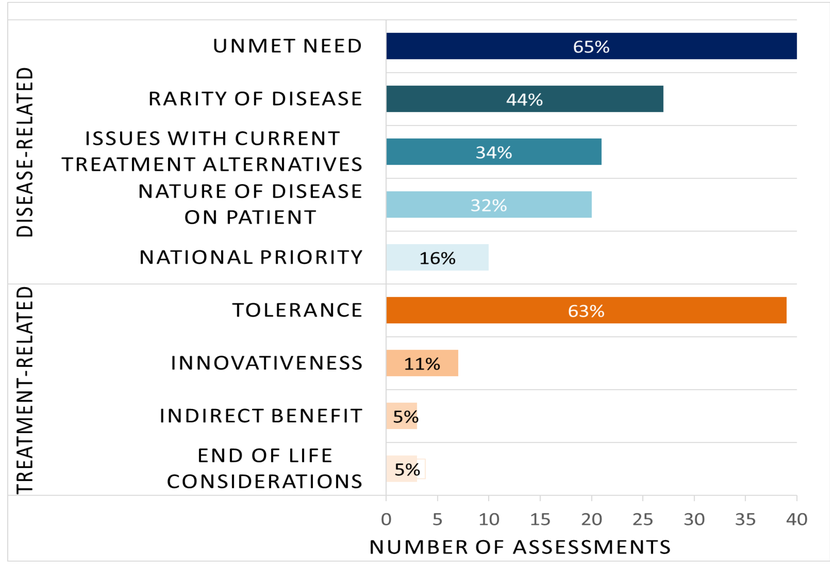
Harrison KL, O'Rourke D, Jonsson P
National Institute for Health and Care Excellence ,UK.

Objective – Making innovative treatments available across the European Union is a complex process due to differing evidence requirements, assessment methods and value judgements by HTA agencies. These extend beyond quantitative therapeutic value and cost-effectiveness with decisions influenced by social, ethical and equity factors which vary across jurisdictions due to differing political, budgetary, social, and values mandates. This is particularly important for drug treatment in rare conditions with novel mechanisms of action or orphan designation, as in haematological malignancies (HM). These are often characterised by high levels of uncertainty and high incremental cost-effectiveness ratios due to difficulties in producing robust evidence in small and heterogenous patient populations, as well as their high prices.

The HARMONY Alliance is an Innovative Medicines Initiative (IMI) public-private partnership project with over 90 organisations from 22 European countries with varying expertise in evidence development strategies to support new treatments and indications. To guide the consortium this study aimed to explore variance and potential influence of additional considerations (AC) on the assessment decision across HTA agencies.

Method – ACs were extracted from 8 national HTA agencies publically available source language reports (n=62/72) for 12 HM innovative drugs. No information was obtainable from AIFA. The identified 170 "other considerations" were themed and classified into categories and sub-categories in line with previous findings from literature.) Most assessments, 81% (50/62, included > one AC (mean 2.74 considerations/report).





Results and conclusion – The inclusion of an additional consideration (rarity of disease, current treatment issues, patient impact, national priority, tolerance, indirect benefit, end of life) significantly increased the likelihood of a positive recommendation as did increasing numbers of additional considerations (Figure 1). Patterns of reporting and considerations varied by mechanism of drug action (Figure 2) and HTA agency (Figure 3). This is likely a consequence of agency-specific value preferences. Inclusion of these considerations in HTA assessment is associated with positive reimbursement recommendations and indicates that given the challenges in producing robust evidence for these rare HM, scientific and social value judgments are an important part of the decision processes for these drugs.

Figure 1. Recommendation outcome by frequency of Additional considerations

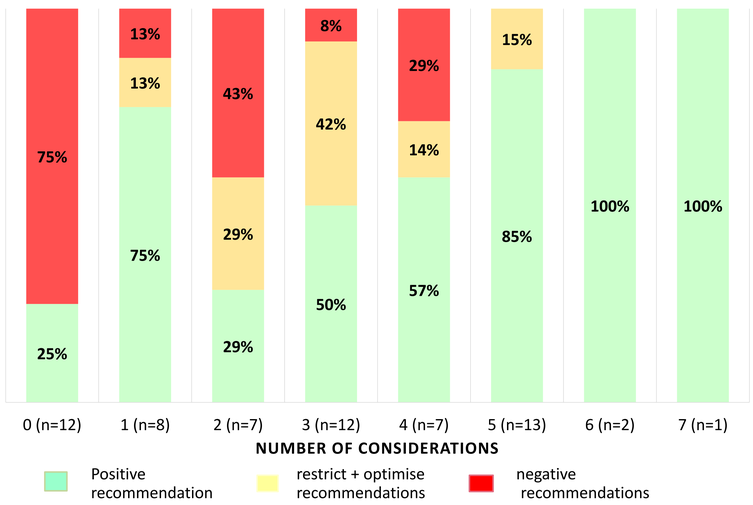


Figure 2. Mechanism of drug action and frequency of additional considerations

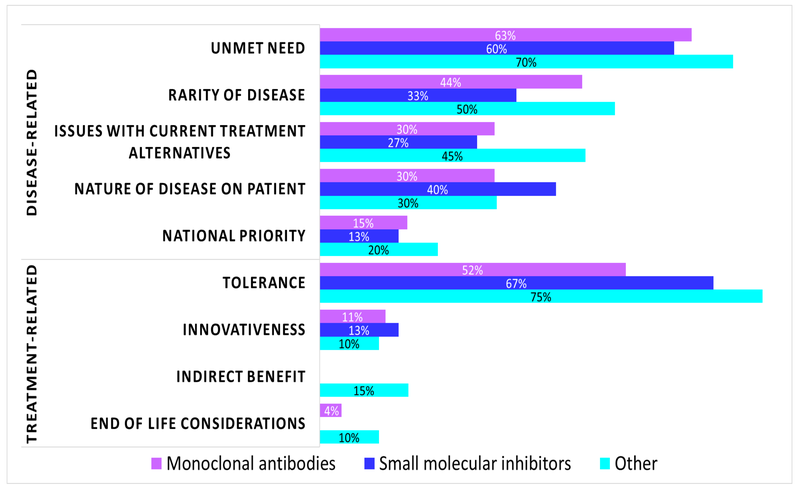
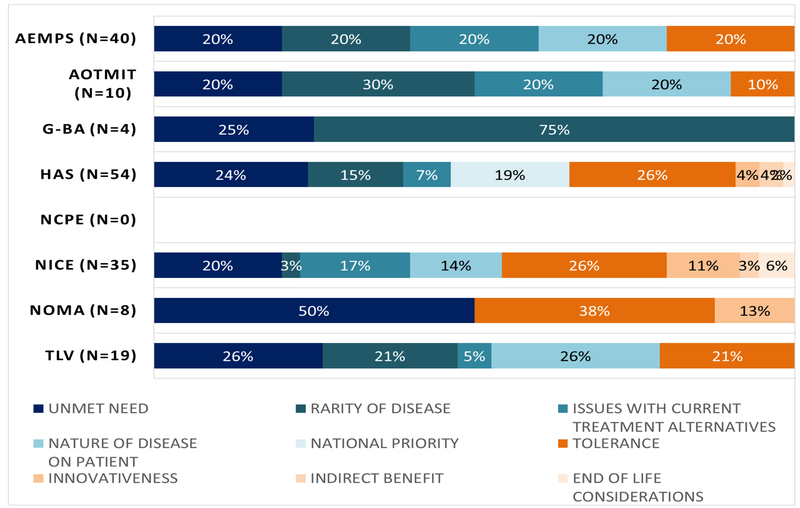


Figure 3. Frequency of Additional considerations in reports by HTA agency



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