WHAT METHODS ARE APPLIED IN ASSESSESSING THE CLINICAL AND COST-EFFECTIVENESS OF HEALTH TECHNOLOGIES BASED ON THE USE OF SURROGATE OUTCOMES?

A COMPARISON OF HTA REPORTS ACROSS INTERNATIONAL AGENCIES

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Background

Surrogate endpoints are increasingly relied upon during evaluations for coverage decisions. Previous experience has shown that underexplored surrogate endpoints can lead to harmful policy decisions [1], so appropriate validation of surrogate endpoints in HTA has been advocated in recent years [2].

A previous review of HTA guidance from international agencies has shown a recent trend to acknowledge and explore the uncertainty associated with surrogate endpoints; however, the level of detail in recommended approaches varied greatly [3].

Objectives

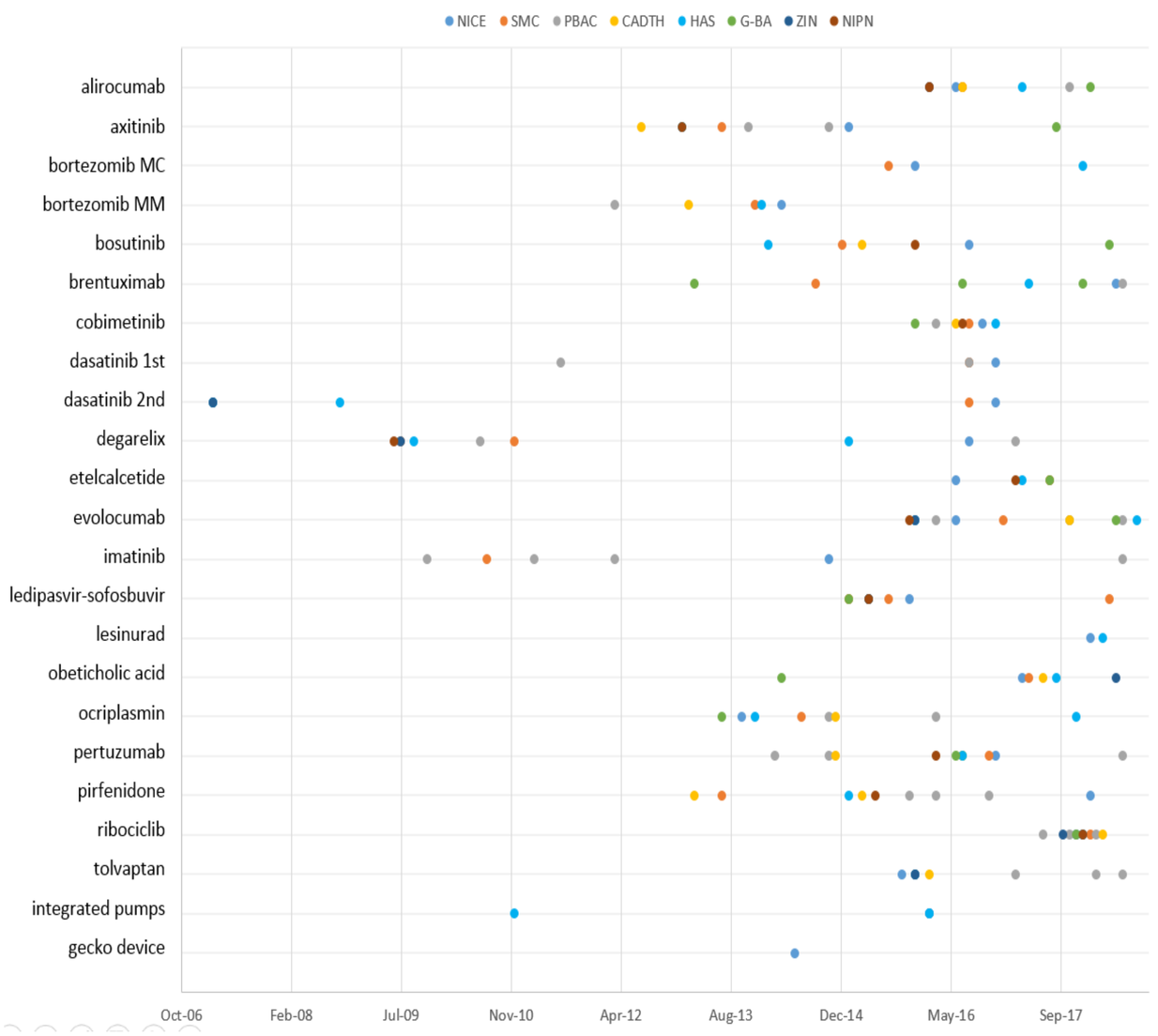
Building on two previous reviews in this field [3,4], we conducted this study with two objectives:

(i) to map the range of methodological approaches (evidence, association, quantification of relationship) adopted in HTA practice to handle the use of surrogate endpoints across international agencies;

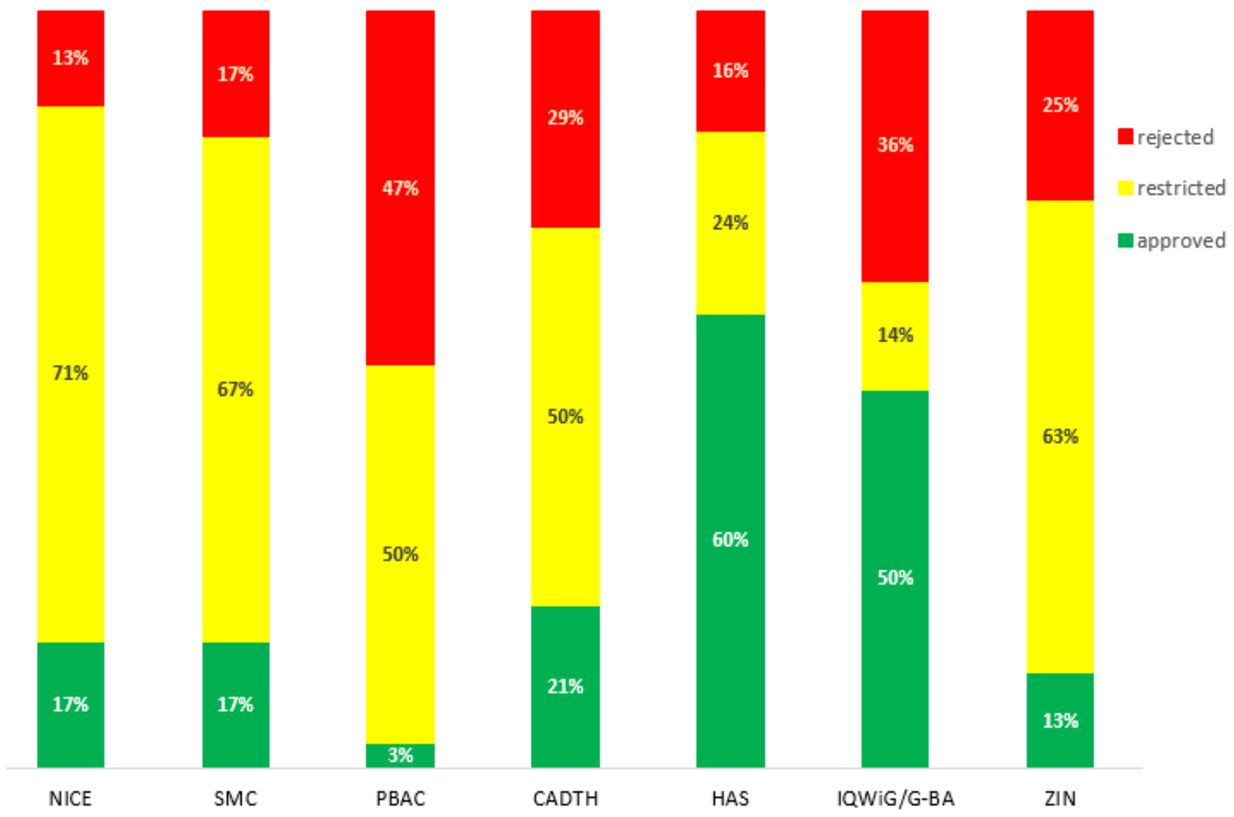
(ii) to assess how the use of surrogate endpoints influences the coverage or reimbursement decisions on health technologies across HTA agencies.

Methods – We applied a two-step approach to the selection and inclusion of HTA reports in this study. First, we identified health technologies and HTA reports that involved the use of surrogate endpoints by examining National Institute for Health and Clinical Excellence (NICE) health technology evaluations undertaken between May 2013 and June 2018. Second, we identified HTA evaluation for the same health technology and clinical indication across selected HTA agencies (HIS/ SMC/ HAS / PBAC/ MSAC/ CADTH/ IQWiG/ G-BA/ ZiN/ NIPN). We extracted data from HTA reports on how surrogate outcomes were considered and validated in the context of the assessment of both clinical and cost-effectiveness. In particular, we focused on the consideration of their acceptability, justification, validation and method of incorporation in the cost-effectiveness model.

Availability and timeline of included reports



Policy decisions on health technologies by HTA agency



Results – We identified a total of 125 evaluations on 23 technologies across 8 HTA agencies. The most frequent surrogate endpoint considered was progression-free survival (7(30%) of technologies). Other endpoints were cytogenetic response (4 (17%)), and LDL-C levels (2(9%)). The application of surrogate validation methods is generally limited despite available guidance. The acceptability of the same surrogate endpoint varies across agencies, with IQWIG taking the stricter approach.

The reliance on surrogate endpoints generally increased decision uncertainty across international HTA agencies, often leading to a restrictive or rejected reimbursement decision. Further data on the HTA agencies specifically how handle evidence on surrogate endpoints in the context of medical devices is needed.

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