A Review of Issues Affecting the Efficiency of Decision Making in the NICE Single Technology Appraisal Process

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Background

Escalating demands upon NICE's Single Technology Appraisal (STA) programme mean a 2.5x increase in capacity is required by 2020[1]. Pressure upon regulators to make drugs available earlier in the development cycle presents new challenges to decision-makers; increasingly immature data and tissue-agnostic drugs are adding even greater complexity and volume to NICE's work. Currently, two or more committee meetings and extensive consultation are typically required before a final decision can be made. This increasing strain upon Committee resources is unsustainable, and threatens to compromise the rigour of the STA process.

In 2018, NICE introduced changes aiming to expedite final decisions[2]. Companies are now invited to discuss their submission earlier in the regulatory process, with technical guidance offered by NICE prior to the evidence submission. New powers allow Committee chairs to make a recommendation based on updated commercial considerations following committee meetings, and a new technical engagement stage offers the opportunity to address obstacles and uncertainties earlier, theoretically allowing more focused committee discussion. While this may facilitate cooperation between stakeholders, current barriers to decision-making may only be a symptom of deeper issues in the way companies engage with the appraisal process.

This study aimed to understand causes underlying negative preliminary decisions, and why certain topics require more meetings and resource to reach a final decision through a review of past appraisals.
This information could inform more targeted reform of STA processes to sustainably accommodate inevitable increases in appraisal throughput.

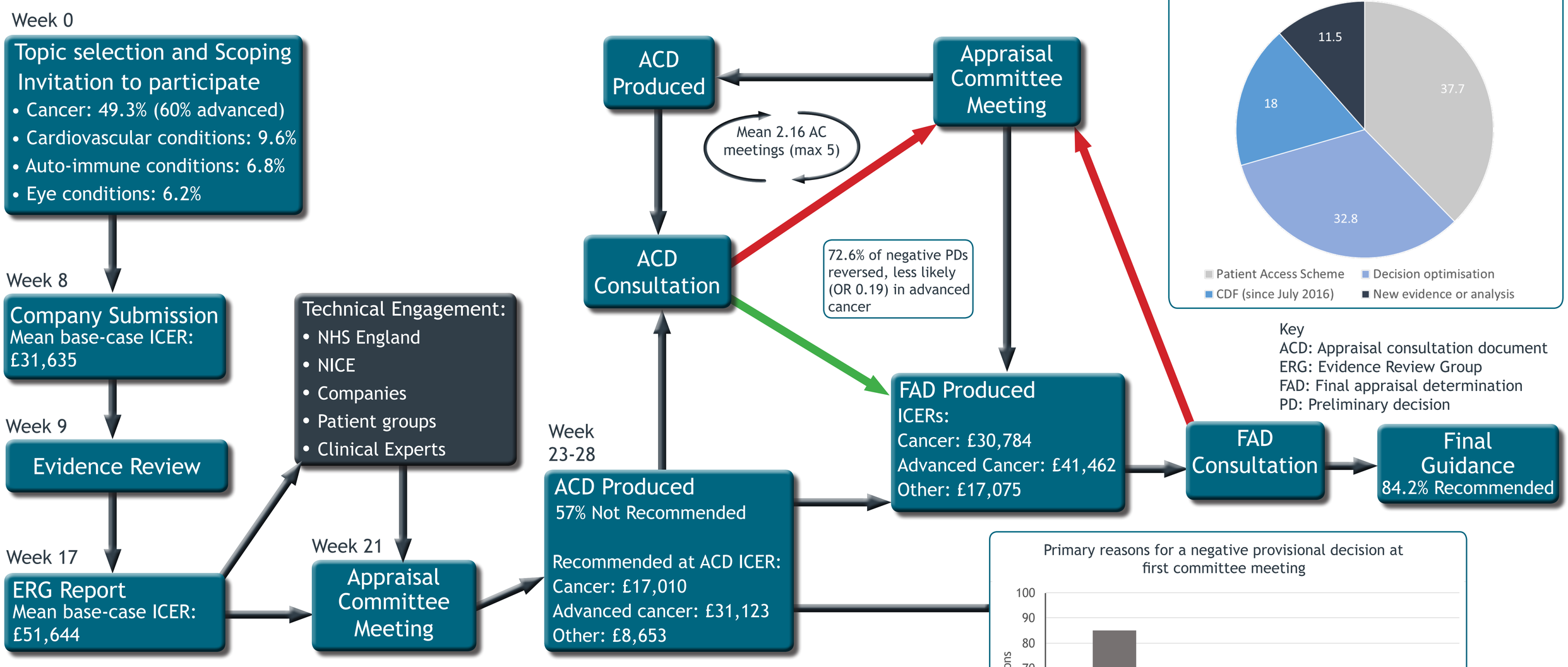
Methods

This study reviewed all Single Technology Appraisal guidance between 01/2010 and 01/2018, excluding updates or re-considerations. An updated review of decisions for cancer drugs up to to 08/2018 was also performed to assess the effect of the new Cancer Drugs Fund.

Data were extracted from 170 STAs, and included disease area, cost-effectiveness estimates, committee decisions and rationale at each stage, number of Appraisal Committee Meetings, Patient Access Schemes, and Cancer Drugs Fund (CDF) considerations were extracted from Committee papers and ERG reports, Appraisal Consultation Documents (ACDs), and Final Appraisal Determinations (FADs).

Descriptive statistics were generated for quantitative variables. Relationships between the characteristics of a technology and the likelihood of positive recommendations were explored using logistic regression analysis, as were factors involved in the reversal of negative decisions.

Overview of the NICE Single Technology Appraisal Processes and Results Summary (n=170)



Discussion

Not unexpectedly, the NICE STA process works most efficiently when appraising technologies with well-characterised efficacy profiles, priced well below NICE's willingness-to-pay threshold. Unfortunately, the greater cost and clinical uncertainty associated with drugs indicated for advanced cancers leads to slower, and more commonly negative decisions.

Cost was the primary factor preventing a decision at the first committee meeting in most appraisals, which delayed the production of final guidance by an average of 142 days. As a strategic player, manufacturers are unlikely to pre-emptively reduce their price to increase the likelihood of an earlier recommendation, or re-focus their submission on a more cost-effective subgroup (i.e. decision optimisation. Moreover, it seems unlikely that the types of issues raised prior to the first ACM can be resolved through earlier consultation alone, particularly given the number of negative decisions based on uncertainty in efficacy and cost data.

As most negative preliminary recommendations due to clinical uncertainty are for cancer drugs, it is likely that the recently reformed CDF (post-2016) will account for a significant proportion of resolvable clinical uncertainty in appraisals. Indeed, since its inception, the CDF has been used as a vehicle for over half of all cancer drug recommendations; in the two years since the relaunch of the CDF, 81.1% of cancer drugs appraised have received a positive recommendation, compared to 63.3% between 2000-2016.

Conclusions

Recent changes to the STA processes may help alleviate delays and inefficient use of the committee for often repetitive cost and commercial discussion.

Given the proportion of technologies requiring further committee meetings for discussion of decision optimisation and alternative commissioning strategies, it is unclear whether the new processes will meaningfully improve the efficiency of the appraisal process.

References: 1. National Institute for Health and Care Excellence. Proposals for increasing capacity within NICE's technology appraisal programme. NICE. 2017.
2. National Institute for Health and Care Excellence. Guide to the processes of technology appraisal. NICE. 2018.