Modelling approaches for histology-independent cancer drugs to inform NICE appraisals: a systematic review and decision-framework

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Plain English summary

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In May 2017, the US Food and Drug Administration granted the first approval for a cancer treatment based on a common biomarker rather than the location in the body at which the tumour originated (the tumour site); that is, a site-agnostic or 'histology-independent' indication was granted. Research from the National Institute for Health and Care Excellence suggests that there are approximately 20 technologies currently in development for histology-independent indications. The first marketing authorisation was granted in Europe in 2019.

Histology-independent treatments have the potential to have important effects in patient populations for whom there are currently limited or no available treatment options. However, it is also important to ensure that the use of these treatments in the NHS is supported by systematic and robust assessments of clinical evidence (i.e. how well the medicine or treatment works) and economic evidence (i.e. the medicine's value for money). These assessments are undertaken by the National Institute for Health and Care Excellence, usually for treatments targeting specific tumours sites. However, a histology-independent marketing authorisation would probably include many tumour sites and it is not possible for the National Institute for Health and Care Excellence to conduct a separate assessment for each tumour site for which the treatment could be beneficial. As a result, the National Institute for Health and Care Excellence needs to consider how these products can be appropriately assessed without creating unnecessary delays in patient access.

This research explores the extent to which the National Institute for Health and Care Excellence's existing approaches for assessing clinical and economic value can be applied to histology-independent indications, and any changes that might be required. We explore the nature and amount of evidence that is typically available at the point of initial marketing authorisation and develop recommendations to establish the evidence and analyses required to help inform the National Institute for Health and Care Excellence's decisions. We use case studies to highlight possible challenges and to explore ways that these challenges might be addressed. This research will help to inform future National Institute for Health and Care Excellence policy on how to appraise cancer drugs with histology-independent indications. It will also inform the development of guidance for those developing these treatments to help their understanding of the clinical effectiveness and cost-effectiveness assessments that will be required to inform the National Institute for Health and Care Excellence's appraisals.

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This report

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