Assessing the likelihood of receiving a positive recommendation without a PAS or CAA, through NICE's STA process

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Introduction: The National Institute for Health and Care Excellence (NICE), England's primary HTA body, is unlikely to approve drugs that have a high cost and do not offer a significant benefit over existing treatments for the NHS.

One way to improve a drug's value proposition is through the use of a Patient Access Scheme (PAS). A PAS, as defined by NICE, is a scheme proposed by a company that is a member of the 2019 Voluntary Scheme for Branded Medicines Pricing and Access (VPAS) and agreed with NHS England. The broad intent is to promote patient access to a technology where NICE's assessment of value, on the current evidence base, is unlikely to support the list price. PAS has been a part of England's formal pricing and reimbursement landscape since it was codified in the 2009 Pharmaceutical Price Regulation Scheme. There are two types of PAS: Simple and Complex. A Simple PAS can be either a fixed pricing agreement that is lower than the list price of the treatment, or a percentage discount from the list price. Complex schemes include outcomes-based dose caps, rebates and upfront free stock – essentially anything beyond a straightforward confidential price discount. NICE and NHS England's preferred option is the Simple PAS because these incur a lower administrative burden. Simple discounts have been the most frequently employed option for companies since 2009.

Commercial Access Agreements (CAA) are also included in this study. A CAA between a company and NHS England supports use of a technology for which at least 1 indication is currently, or has been, considered as part of the Cancer Drugs Fund.

Whilst there is no doubt that the existence of these schemes increases patient access to higher cost drugs, NHS England's preference for reductions to list prices rather than more innovative risk-share agreements, may have a longer-term impact on England's commercial attractiveness as a primary launch market for new drugs.

Objectives: This study investigates the likelihood of receiving a positive recommendation through NICE's Single Technology Appraisal (STA) process, whilst at the same time filing a PAS or a CAA. The objective of this study is to provide a detailed breakdown of the proportion of recommendations which were made with either a PAS or a CAA, via NICE's STA process.

Methods: Twenty years of NICE's publicly available technology appraisal data was reviewed in March 2019. All Multiple Technology Appraisals (MTAs) were removed from this study. MTAs are currently less frequently used by NICE. Of the 419 remaining STAs, 42 appraisals were withdrawn in the event where a company did not complete an evidence submission. This left 377 completed STAs in total. 313 STA recommendations were positive, the remaining 64 technologies that were not recommended were discarded from this study. The study analysed the 313 individual STAs to distinguish the type of recommendation that each was awarded and to assess whether a recommendation was made for a sub-group (an optimised recommendation).

Where individual STAs included recommendations for products with several indications, this study considers each indication as a separate STA.

NICE's data on technology appraisals that used either a PAS or CAA was also analysed. After establishing the type of recommendation of each topic included in the study, the research then sought to establish whether the recommendation was made on the basis of the inclusion of any of the types of PAS or CAA previously outlined (see table). Data was analysed to calculate the proportion of STAs that were recommended with an attached PAS, either in full or with modifications.

Results: The research found that 83.06% of the STAs in this study were granted a positive recommendation (313/377).

Of the 313 positive recommendations, 177 (56.57%) were approved with either a PAS or a CAA attached. 112/177 were with a recommendation for the full licensed population and 64/177 were with an optimised recommendation. One drug in this study was granted an 'only in research' recommendation with a Simple PAS attached to it.

The most common type of PAS was a Simple discount, 130 (41.54%) of all positively recommended appraisals. 81 (25.88%) of these were with a recommendation in line with the patient population at market authorisation, 48 (15.34%) were optimised, and 1 (0.32%) was for only in research. The large number of optimised recommendations with a PAS confirmed the need for further study in this area. A second piece of research was subsequently conducted on this matter.

Finally, 36.07% of all appraisals, including those not recommended, achieved a positive recommendation without a PAS or a CAA. The majority of these (90), had a recommendation for the full licensed population, 42 had an optimised recommendation, and 4 received an 'only in research' recommendation.

Discussion: NICE stated in 2019 that 82% of their technology appraisals were 'positive'. This is certainly positive for patients. However, for pharmaceutical companies, the high likelihood of needing a PAS to get a drug onto the NHS contributes to the dwindling incentives of launching in England. That disincentive can be added to a list including fees for submission to NICE and continuing uncertainty over Brexit. The issue, therefore, is not just that companies make less profit, but a less attractive English market may also result in delayed patient access to new medicines.

The Patient Access Scheme Liaison Unit (PASLU) looks at the proposal made by the company to see if a scheme would work in the NHS. PASLU has stated that the average PAS discount ranges between 55-60% of the list price. This discount will last until, and perhaps beyond, when the guidance for a particular drug is reviewed. NICE states that a review provides a useful opportunity to assess how the PAS or CAA is operating and consider whether it would be appropriate to make any changes to simplify and improve its operation. This is particularly appropriate for an outcomes-based PAS. NICE technology appraisal guidance includes a date at which NICE will consider whether to review the guidance. Timing on reviews vary, but may take up to three years. In which instance, a company may face considerably weaker commercial gain than anticipated for those three years and, likely, beyond.

On the other hand, the alternative would be for companies to postpone launching in England whilst further trial data is collected. PAS and CAA support patient access whilst data is often immature, and allows further evidence generation in a real-world setting.

Recommendation Type	Number	% with positive outcome	% of total appraised
All Only in Research			
Only in Research	4	1.28	1.06
Only in Research - PAS (Simple Discount)	1	0.32	0.27
All Optimised			
Optimised	42	13.42	11.14
PAS (Complex - Free Stock)	4	1.28	1.06
PAS (Complex - Time Cap)	1	0.32	0.27
PAS (Simple Discount)	48	15.34	12.73
(CDF) - CAA	8	2.56	2.13
CAA (PAS Withdrawn)	3	0.96	0.80
All Recommended			
Recommended	90	28.75	23.87
PAS (Complex - Dose Cap)	3	0.96	0.80
PAS (Complex - Free Stock)	3	0.96	0.80
PAS (Complex - Response Scheme)	1	0.32	0.27
PAS (Complex - Single Fixed Price)	1	0.32	0.27
PAS (Complex Discount)	1	0.32	0.27
PAS (Discounted Price - this indication only)	1	0.32	0.27
PAS (Simple Discount)	77	24.60	20.42
(CDF) - CAA	17	5.43	4.51
(CDF) - PAS (Simple Discount)	4	1.28	1.06
CAA (PAS Withdrawn)	4	1.28	1.06
Total	313	100	83.06

Conclusion: This study has shown that over half of positively appraised medicines in NICE's STA pathway are recommended with an accompanying PAS or CAA. This figure suggests that PAS' are becoming less of an exception and increasingly the rule. If the average price discount for PAS is in the range of 55-60%, this represents a large saving for the NHS, but a heavy cost for manufacturers.

Further work would be beneficial, to establish whether the English market is becoming less attractive because of the strong possibility of needing a PAS in order to gain market access. Such research could inform NHS England and NICE as to the sustainability of the current system in ensuring patients in England have access to future treatments. A European wide study of pricing discounts would also be useful to see how England compares to its counterparts.

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