Early Scientific Advice from Regulators and HTA: A NICE Perspective

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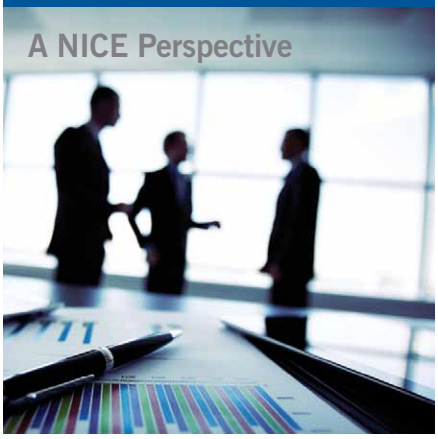
KEY POINTS . . .

The National Institute for Health Care Excellence (NICE) was the first agency to establish health technology assessment (HTA) scientific advice service.

Today, companies have an option of obtaining national HTA advice, parallel advice with HTA agencies and EMA, joint advice with HTA agencies, and in some countries - national parallel advice with regulatory agencies. Now NICE SA offers a discounted service for SMEs.

NICE works closely with different agencies to identify synergies in our approaches to offering advice. We participate in a number of initiatives to make scientific advice compatible with the regulatory and market changes.



The following article is based on a presentation given during the Second Plenary Session, "Early Engagement Between Manufacturers, HTA Assessors, and Regulators: Learning From the Past to Guide the Future," at the ISPOR 16th Annual European Congress, 2-6 November 2013, Dublin, Ireland). The content has been updated to reflect the figures as of September 2014.

Introduction

The National Institute for Health Care Excellence (NICE) was the first agency to establish health technology assessment (HTA) scientific advice and therefore is, fair to say, the most experienced in the field. NICE began offering standard advice in 2009 and over the last few years has also engaged in parallel advice with regulatory agencies and other HTA agencies as well as developed educational offerings for pharmaceutical and med-tech companies.

How well did NICE do to date? There has been positive feedback from NICE's clients, but the real measure of its success is repeat business. NICE has worked with 33 companies; almost half came back for more projects and advice, and one-third of business comes from the top three clients (Fig. 1).

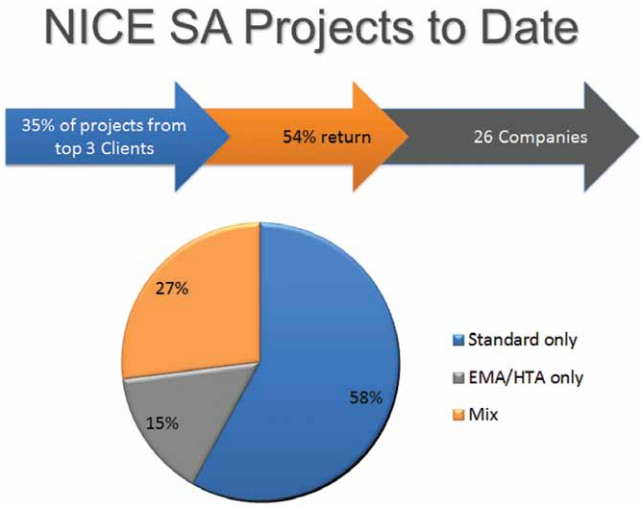
Of the companies who approached NICE for scientific advice over 60% came for the written standard advice, 18% took advantage of different processes offered at NICE and with HTA/EMA colleagues and 18% of companies came for parallel advice with HTA and regulatory agencies only.

The reason for which NICE originally set up scientific advice was to help product developers generate evidence for the future technology appraisal. The aim was to help companies present robust evidence to the committees, decrease delay around decision making and uncertainty, and in the long term, speed-up access of new treatments to patients. As enthusiasm for scientific advice increased, it turned out to offer a bit more variety than initially anticipated.

A few things should be noted when looking back at the first 77 scientific advice requests NICE had received (Fig. 2). To date, only two products that received scientific advice from NICE have gone through a technology appraisal and one received a positive recommendation while the other product was not recommended for reimbursement.

About one-third of products were discontinued and about 50% of products are still either in trials or obtaining regulatory authorization. NICE is looking forward to those products still coming to technology appraisals in the future and learning from this experience.

Figure 1. Breakdown of type of advice NICE clients received.



Potential Value of Scientific Advice and NICE Experiences

What value does NICE scientific advice have for companies besides going through a future technology appraisal (Fig. 3)? Awareness of payer's perspective could be impacting company's strategic plans, helping the company optimize their development process and generating relevant evidence package for enabling faster market access regardless of whether the product ends up going through the NICE evaluation/appraisal in the future.

Figure 2. Audit of the first 77 NICE Scientific Advice Requests.

Analysis of the first 77 SA requests

Projects up to April 2012

Outcome	Completed Advice (60)	Withdrawn Projects (17)
TA completed*	**2**	-
TA terminated	1	-
Development ongoing	28 (43%)	3
MA application submitted (EU)	3 (5%)	-
MA granted (EU)	**3 (5%)**	-
MA not granted (EU)	3 (5%)	
Development terminated	**18 (30%)**	**4**
No information	2	10

*one positive, one negative recommendation Analysis date: September 2014

Speaking from experience, NICE has identified some key issues regarding companies seeking standard advice. When coming to NICE it is important to think carefully of what the company wants to ask and be ready to provide their perspective. As with most situations, the better the questions asked, the better answers are provided. Contacting NICE early is good practice and it is also important for companies to plan in scientific advice early in their processes to inline it with regulatory advice and prior to the start of pivotal trials. The ideal scenario is to receive scientific advice when there is still an opportunity to amend development programs. Finally, collaboration between the regulatory team and the health outcomes team in the company is of paramount importance to ensure successful market access. This collaboration starts early on and the decision to come to scientific advice should be made jointly.

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For those seeking parallel EMA/HTA advice, the number of questions and the number of agencies present at the meeting is an important decision due to time constraints. The company has only four hours to explore all the issues and to get their answers. The participants should enable the meeting for a productive dialogue by having a manageable number of questions and opinions.

The most frequently question asked is how the variety of opinions received from different agencies is dealt with. Experience to date shows that although there is more synergy in the views rather than differences there is still room for improvement. A number of initiatives are currently taking place to develop a more harmonized approach to scientific advice among HTA agencies and the regulators.

Program Expansion and Future of Scientific Advice

NICE's aim is not to reach the capacity of regulatory agencies in terms of offering scientific advice, but to grow HTA scientific advice at different levels. NICE will be offering more advice to companies developing devices and diagnostics, providing a more affordable service for SMEs, expanding educational offers, and participating in research and international collaborations. Joint advice with the British regulator, Medicines and Healthcare Products Regulatory Agency (MHRA), has not been taken up by companies but this service has been re-launched to support the newly established Early Access to Medicines Scheme in the UK and we already started a few projects this year. The developers can obtain MHRA-NICE joint advice earlier than they would consider coming to the EMA. This process will include a face to face meeting with the Company in London and two reports are produced by each agency. An addition to this process is an optional input from Clinical Practice Research Datalink (CPRD). Specific questions could be addressed by CPRD in this parallel process as well as in NICE standard advice.

NICE is working hard on maximizing the usefulness of the scientific advice process for manufacturers. We work with different agencies to identify synergies in our approaches to offering advice: NICE participates in a number of initiatives to make scientific advice compatible with the regulatory and market changes. As a result, NICE wants to see the uptake of HTA scientific advice across Pharma, MedTech and BioTech sectors and establish better and closer collaboration with the regulators. Our ultimate aim is to help patients to get faster access to treatments and to help companies develop new products more efficiently. ■

Figure 3. Potential Value of HTA Scientific Advice.