The role of national agencies in the managed introduction of new drugs for arthritis

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Abstract

The role of the various agencies involved in the managed introduction of new drugs in the United Kingdom is discussed, particularly with regard to the work of the National Institute for Clinical Excellence (NICE). The process by which the National Health Service in the UK identifies key new drug technologies of major clinical, financial and service significance is discussed. This includes the decision making process for selection of products for appraisal by NICE. The appraisal procedure and the impact of NICE guidance for the NHS will, it is hoped, encourage quality in clinical practice. All healthcare systems face budgetary constraints and the introduction of new technologies bring particular challenges. The launch of the new biological agents for the treatment of rheumatoid arthritis, into a speciality where previously relatively inexpensive agents were prescribed, raises questions concerning managed introduction and re-imbursement.

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There are several organisations in the United Kingdom involved in the managed introduction of new drugs, including the National Institute for Clinical Excellence (NICE), the National Prescribing Centre (NPC) and the National Horizon Scanning Centre (NHSC).

The National Prescribing Centre is funded by the United Kingdom Department of Health and was formed in April 1996 to facilitate the promotion of high quality, cost effective prescribing through a coordinated and prioritised programme of activities aimed at supporting all relevant professionals and senior managers working in the National Health Service.

NICE came into being on the 1 April 1999 and has three broad functions:

- 1 To provide guidance to the NHS on the use of selected new and established health technologies.
- 2 To develop clinical guidelines on individual conditions.
- 3 To promote clinical audit.

The stimuli for managed introduction of new drugs

There is increasing emphasis in the United Kingdom on the quality of the health service provided and on patient empowerment.

Various developments have prompted debate on the introduction of new drugs including:

- The breadth and efficiency of research in the pharmaceutical industry, particularly the

development of new technologies for previously untreatable chronic diseases, for example, multiple sclerosis.

- The pressure on NHS resources and the need to deliver value for money.
- The priority to deliver healthcare based on what is proven to work effectively; evidence-based medicine.
- Prioritising the delivery of healthcare locally by identifying the needs of the population—that is, maximising health gain for both public and patient.
- An increasing number of elderly patients.
- The impact of new technologies on service provision.
- The increasing awareness of the public of new technologies.
- The development and introduction of so called lifestyle drugs.
- The priority to deliver high quality health care in an equitable manner across the country.

The role of the various agencies

NATIONAL PRESCRIBING CENTRE AND NATIONAL HORIZON SCANNING CENTRE

A major aspect of the NPC's role is the identification of new drugs that are considered likely to affect service delivery and be of major therapeutic and/or financial significance to the NHS. Products in research are monitored by various means and when a product is thought to be approximately 18 months from the market, consideration is given to providing independent information to key decision makers, for example, budget holders on the role of the product. The selection of these drugs is done by the use of a set of pre-defined criteria.

This work informs two processes:

- 1 Supporting effective planning and local management action. This work operates separately to NICE, but is regarded by the Department of Health as important in alerting the wider NHS to important issues for consideration.

- 2 Informing the selection process by which products are chosen for appraisal by NICE.

The NPC works in conjunction with the National Horizon Scanning Centre (NHSC), a public health academic unit, to cover both medicinal and non-medicinal technologies and alert the Department of Health to new and emerging interventions of likely significance that will require assessment. This may involve providing briefings on selected topics and advising on the need for new or updated guidelines.

Lists of health technologies, possibly two to three years from the market, are submitted by NHSC to the Department of Health on a regular basis for consideration.

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A decision may then be made to refer a technology either:

- 1 to the NHS Research and Development Programme (incorporating the Health Technology Assessment Programme)—if the underlying issue is a lack of knowledge, or
- 2 to NICE—if there is a need for clinical guidance in the use of a particular clinical intervention (an appraisal) or more generally to best clinical practice for a medical condition or patient group (a clinical guideline).

Issues relating to existing health interventions or areas of clinical practice may be identified to the Department of Health via consultation with the wider NHS, professional bodies, patient organisations and by scrutiny of the findings from research in the UK or elsewhere.

Recommendations go forward to health ministers who then decide, after due consultation, on the topics that will be referred to NICE. Towards the end of the process interested parties, including companies manufacturing healthcare products relevant to the interventions being considered, are given the formal opportunity to comment on whether the topics should be referred to NICE.

Selection of technologies for appraisal by NICE

Technologies for appraisal are selected on the basis of one or more of the following criteria:

- is the technology likely to result in a significant health benefit, taken across the NHS as a whole, if given to all patients for whom it is indicated?
- is the technology likely to result in a significant impact on other health related government policies (for example, reduction in health inequalities)?
- is the technology likely to have a significant impact on NHS resources (financial or other) if given to all patients for whom it is indicated?
- is NICE likely to be able to add value by issuing national guidance? For instance in the absence of such guidance is there likely to be a significant controversy over the interpretation of the available evidence on clinical and cost effectiveness?

The appraisal process of NICE

Guidance has been issued that outlines the arrangement for submitting evidence, consulting on draft guidance and instigating appeals in the event of a dispute over NICE's final recommendations.

It is intended that the appraisal process will have no effect on requirements for medical products obtaining a marketing authorisation or on safety requirements for medicinal devices. The process will be formally independent of the processes operated by the UK Medicines Control Agency and the UK Medical Device Agency.

Independent appraisals of the selected technologies are commissioned by NICE from various agencies. Detailed methodology is being developed for assessing the evidence and reaching an overall view on the clinical cost

effectiveness of the intervention. The appraisal will consider some or all of the following points:

- How robust is the assessment?
- What is the estimated impact on quality and (where appropriate) length of life?
- What will be the estimated average health improvement per treatment initiated?
- Is there convincing evidence that the proposed intervention will be cost effective for the NHS?
- Are there any particular subgroups for which the treatment is likely to be more or less clinically cost effective? Should treatment be targeted on the groups who would derive most benefit?
- Should use of the intervention be subject to particular conditions?
- What issues need to be considered in the overall risk/benefit balance for individual patients?
- Total likely impact on NHS resources?
- Are there any associated government funded personal social services costs and savings?
- Should further research be carried out, possibly utilising the national Health Technology Assessment Programme?

The final Appraisal Determination is a public document that can offer guidance in one of four ways:

- 1 Unrestricted use in the NHS for the appropriate indications.
- 2 Restricted use within the NHS to defined categories of patients.
- 3 Use should be restricted to formal clinical trials or evaluations.
- 4 Advise that the technology should not be used in the NHS at the present time.

Additional research can be advised concomitant to any of the above recommendations, where the full potential of an innovative product remains to be defined.

Guidance will need to take account of the clinical needs of patients for the technology, particularly in relation to its relative effectiveness. Consideration needs to be given to the NHS's broad clinical priorities and resources available. However, it is not the intention of NICE to discourage innovation. The organisation can be seen as a conduit for encouraging the practice of high quality evidence-based medicine.

Methods for reimbursing new technologies

Much debate has taken place in the United Kingdom over the last few months concerning the percentage of gross domestic product spent on healthcare. Figures vary depending upon the organisation from whom they are taken, but the UK spent approximately 6.8% (£58 billion) on healthcare in 1998, of which over 80% came from public spending. This compares to an average of 8.6% in Europe.

Ministers remain strong in support of a public health system that encourages equity and is based upon a person's need for health care rather than ability to pay. Various commentators have discussed the advantages and disadvantages of a health system facing the 21st century funded by general taxation rather than patient charges or private or social insurance.

It is hoped that NICE will identify which treatments are less cost effective and thus, free up money for more cost effective treatments. The possibility of increased funding for newer agents regarded as cost effective has not been ruled out. While professionals in the health service will be expected to take account of NICE guidance, decisions on which treatments are to be funded from budgets will still be made locally between hospitals and purchasers.

In the United Kingdom the price of a product is dependent upon the degree of profit made by the manufacturer concerned. Pricing and a judgement of cost effectiveness remains separate to licensing. However, once a product has a licence and is marketed then there is usually no restriction on prescribing of the product by doctors. This system differs to that in Europe where a manufacturer may have to negotiate with a national agency for a certain reimbursement price before the product can be prescribed.

The impending introduction of new biological agents for the treatment of rheumatoid arthritis, for example, infliximab and etaner-

cept should be seen in the context of increasing early introduction of disease modifying drug therapy to improve outcome, albeit with relatively inexpensive agents such as methotrexate and sulfasalazine. Thus the budget currently available to rheumatology specialists for the introduction of the newer agents starts from a low baseline among competing priorities at local level.

As of February 2000 NICE had not issued guidance on the use of the new biological agents and specialists would need to negotiate locally with purchasers. Criteria for defining those patients who would and would not be appropriate for treatment could be defined. Equally important would be a definition of when to stop treatment with a drug either because of lack of efficacy and/or adverse effects.

The long term effects of these agents will be interesting to observe and optimal care may well be achieved by provision through specialist centres. Unnecessary treatment could be minimised by this means.