

## **Cancer Research UK submission to the House of Commons Health Select Committee inquiry into the National Institute for Health and Clinical Excellence**

---

**March 2007**

### **1. Summary**

- 1.1. Cancer Research UK welcomes this timely inquiry and the opportunity to be involved in this debate. Cancer Research UK believes that the current approach to NICE and NHS access to anti-cancer treatments requires urgent review.
- 1.2. Cancer Research UK would be very pleased to provide oral evidence to this inquiry as it progresses.
- 1.3. This inquiry is particularly important for cancer. There are an increasing number of potential new anti-cancer treatments coming through the research pipeline. Combined with increasing public pressure for their provision by the NHS, this means that if cancer patients are not to be disadvantaged the framework within which NICE operates needs to be better defined.
- 1.4. It is of paramount importance to note however, that it is the combined effect of the way in which NICE operates alongside the Pharmaceutical Price Regulation Scheme (PPRS) that particularly disadvantages cancer patients in the UK.
- 1.5. It is vital that the quality of NICE appraisals is of a consistently high standard. To this end, reform is needed to address:
  - The use of appropriate expertise on appraisal committees;
  - The consistency and transparency with which appraisals consider quality of life measures;
  - Cost considerations, such as whether calculated cost to patients covers solely the cost of the drug or includes the total cost of treatment;
  - The extent to which appraisals take account of indirect treatment costs and savings;
  - The transparency of the appeal process, and whether this is conducted by an appropriately representative body;
- 1.6. Much has been made of the role that NICE could play in the future development and availability of treatments in the NHS. We would like to see NICE, the Government and the pharmaceutical and biotechnology industries working closely together with independent expert organisations, such as Cancer Research UK, to further develop these emerging ideas.
- 1.7. We note that the previous Health Select Committee inquiry into NICE in 2003 produced a number of laudable recommendations for how NICE might improve involvement, transparency and external perception of the organisation. We note that while a number of these recommendations have been taken forward, others have not. We would welcome, as part of this inquiry, a review of all these recommendations within the current climate.

## 2. Background

- 2.1. As the major non-commercial cancer research funder in the UK, and as a charity directly supported by one in ten people in the UK, Cancer Research UK is a unique and important stakeholder in this debate. Our focus, as always, is in achieving the best possible health outcomes for patients with all forms of cancer.
- 2.2. We fully understand and support the role that NICE plays in providing guidance to clinicians and the NHS both on clinical effectiveness, and on whether a new medicine constitutes value for money for the NHS. The number of innovative new anti-cancer therapies being approved puts pressure on NICE and on the NHS to make these therapies available to patients.
- 2.3. Our rapidly increasing level of understanding of how cancer develops has led to a new era of development of new anti-cancer therapies. A new generation of cancer therapies, targeted at the specific abnormalities found in cancer, are already becoming available. There are currently over 1,000 anti-cancer therapies in development. The whole pharmaceutical industry is investing heavily in oncology.
- 2.4. It is recognised that many of the newer treatments are being priced at levels much higher than historical averages. This, coupled with growing cancer incidence and expected lower levels of funding for the NHS, looks set to place strain on the NHS budget. This strain will also increase through future best practice using combinations of the newer, more expensive treatments.
- 2.5. We fear that the current NICE and PPRS processes work in conjunction to systematically undermine the adoption of new cancer treatments in the NHS. In areas where there are few or no competitor treatments the profit-cap system of PPRS actively encourages treatments to be priced as high as possible. These therapies will generally be those in areas of unmet medical need, such as cancer. Conversely in those addressing diseases for which a wealth of treatments options exists, such as cardiovascular disease, the PPRS encourages companies to set prices at suitably competitive levels. This problem is exacerbated by the current NICE process, which judges treatments according to the price set by manufacturers. The result is that expensive cancer treatments in areas of highest unmet medical need are therefore unlikely to receive NICE approval.
- 2.6. Furthermore, clinical development of the majority of new drugs is conducted in the end stage of the disease. This is due both to the often high toxicity of new cancer therapies, and the nature of disease, which means that proven treatments are used in the first stages of disease to prolong life, with newer-unproven treatments only resorted to when all other options fail. However, when setting the price of these treatments, manufacturers are aware that further research in an active clinical setting is likely to uncover suitable populations of patients at an earlier stage of their disease. This will naturally be a much larger market, and one where value for money is higher. Under the current PPRS, price negotiations with the Department of Health only ever result in a decrease in price. The manufacturers will therefore initially set as high a price as possible for their new therapy. When NICE then come to appraise such a treatment, they will necessarily find it too expensive in the population for which data are available, and in which marketing authorisation has been gained.

- 2.7. We are keen that solutions are found to these problems and we want to work with Government and the pharmaceutical industry to do this. The UK is a world-leader in the discovery and development of new anti-cancer treatments, but is perceived as being backward in the use of these new medicines. This is surely an untenable situation.
- 2.8. Our response to the specific areas raised by the Committee and the future role of NICE in the pricing and development of new treatments are below.

### **3. Why NICE's decisions are increasingly being challenged**

- 3.1. As exciting new treatments are approved by regulatory agencies for use, it is inevitable that patients will expect access to them. We have already seen individual campaigns to lobby NICE and the Department of Health for specific anti-cancer medicines to be funded by the NHS. We expect that an increasing number of NICE decisions will be afforded a high media profile, particularly when the decision is negative.
- 3.2. This confusion is not helped by uncertainty among the health community (both professionals and patients) around the method by which NICE appraisal committees calculate whether a drug is cost effective. This is particularly true for the application of the 'willingness to pay' threshold, which is generally accepted to be around £30,000 per QALY<sup>1</sup>. Transparent public discussion around the threshold figure and its appropriateness is important and required.
- 3.3. The public should know where the £30,000 figure came from and why it is set at that level. We urge the Committee to recommend that Government enter a debate with stakeholders about what threshold is appropriate for a country such as the UK and for the NHS in the future.

### **4. Whether public confidence in the Institute is waning, and if so why**

- 4.1. Recent media coverage on NICE and its decisions suggests that public confidence in the Institute is not high. We believe that much of this is due to a misunderstanding of NICE's role in the process of making NHS treatments available to patients.
- 4.2. As a UK funder, we also consider it central to our role to ensure that the new and more effective treatments, all of which result from research, are accessible to cancer patients in the whole of the UK. There is a fear that the public's response to negative NICE decisions may develop into a lack of faith that medical research and medical research charities' advances are delivered to patients.
- 4.3. Indeed, the public may question the value of donating to cancer research if the resulting discoveries are then deemed too expensive for UK NHS patients to receive. Were this situation to worsen, it would obviously be very serious not only for medical research charities but also for the whole of biomedical research with its associated economic benefits in the UK.

---

<sup>1</sup> Cost per quality adjusted life year.

## **5. NICE's evaluation process, and whether any particular groups are disadvantaged by the process**

- 5.1. A greater number of more expensive anti-cancer treatments are gaining UK marketing approval. Combined with the increasing public scrutiny of NICE's decisions, it is increasingly important for Government to assess the evidence for the cost-effectiveness threshold and how it is determined by NICE.
- 5.2. We welcome recent moves by NICE to improve the transparency of its processes, but believe that more should be done. This is particularly important in terms of the weight and consideration given to **quality of life** evidence considered in NICE appraisals, especially where data from clinical trials are lacking at the time of appraisal.
- 5.3. We also believe that there are circumstances where it is appropriate that exceptions to NICE's cost-effectiveness rules should be made. Examples of this include **orphan indications** for which there is a therapeutic void. It is important that the Government considers how flexibility may be built into the current process to address this need.
- 5.4. We welcome recent moves by NICE to actively pursue disinvestment in older, potentially poorly effective treatments, to free up resources for new treatments in the NHS. However, NICE must be adequately resourced to undertake these tasks.
- 5.5. It is currently unclear how dates for future review of NICE guidance and appraisals are set. It is important that review is not set for a somewhat arbitrary future date but should be undertaken when the specified additional information becomes available.

## **6. The speed of publishing guidance**

- 6.1. While the NICE approval process must be deliberative and consultative it must not unduly delay patients getting access to the best treatments. In the case of some new anti-cancer therapies, it is clear that beneficial treatments are not getting to patients as swiftly as they ought.
- 6.2. We welcome the introduction of NICE's Single Technology Appraisal process, which has been shown to be faster and effective. However, it is important that the existence of such a process does not disadvantage drugs being appraised by NICE not selected for this process.
- 6.3. It is also important that drugs are referred to NICE in a timely manner, and at an appropriate time in their development. For new treatments this will need, at least, to be brought in line with the time of successful application for Marketing Authorisation.

## **7. The implementation of NICE guidance, both technology appraisals and clinical guidelines**

- 7.1. It is widely recognised that, despite a positive NICE appraisal, situations still exist where PCTs do not make an approved drug quickly available to their patients. The "postcode lottery" remains real.

- 7.2. It is important to recognise that while the current legislation states that NICE approved treatments should be made available within three months, PCTs are required to find funding for these treatments from their existing budgets. It may be that a separate ring-fenced budget is required to ensure new treatments approved within a financial year are made available to patients.
- 7.3. While the recommendations of NICE clinical guidelines are necessarily not mandatory we would like to see more emphasis on consideration of these recommendations at the local level. We would like to see it made a condition of the Healthcare Commission 'Annual Health Check' that PCTs record how recommendations of emerging NICE guidelines have been considered and incorporated into future decisions.

## **8. NICE's role in encouraging future research activity**

- 8.1. Currently, when a negative announcement is made by NICE on a particular drug, there is no mandatory next step in terms of further research. We need, collectively, to be able to turn negative NICE decisions into positive action.
- 8.2. We believe that where a need for further research is identified by NICE, Government, the research community and industry should commit collectively to ensuring that appropriate further research is conducted to identify possible specific applications for the drug. In the case of cancer treatments, Cancer Research UK would be more than willing to facilitate this research.
- 8.3. We endorse the need highlighted in the Cooksey Review of UK Health Research Funding to identify resource to support NICE's research recommendations. We believe that a separate funding stream for NICE dedicated to taking these research recommendations forward would be the most appropriate solution.
- 8.4. There is also a need to establish formal arrangements between NICE, the NHS and the commercial sector to ensure that the output of research can be fed more systematically back in to the NICE review process and inform future recommendations.

## **9. NICE's role in setting drug prices for the NHS**

- 9.1. Fundamental to the issue of access to medicines is the price set for them and Government's role in influencing those prices. We welcome the recent report from the Office of Fair Trading (OFT) recommending future reform of the Pharmaceutical Price Regulation Scheme. It is important that the difference between the cost of production and price of treatments is appreciated and carefully considered.
- 9.2. The current pricing mechanism disproportionately disadvantages new cancer treatments, because they often address an unmet need and often carry a higher price than other treatments. The conclusion of the OFT that there are several medicines for which the cost to the NHS significantly 'outweighs their benefit to patients' and proposals that the current 'profit-cap- and price-cut' scheme be replaced by a value-based pricing scheme clearly opens up an opportunity for the involvement of NICE. We would welcome further discussion about the role of NICE in future drug pricing.

- 9.3. We would fully endorse the introduction of **conditional approvals** by NICE. We consider it completely appropriate that NICE have a role in establishing the price the NHS is prepared to pay for particular treatments. The appraisal process would thus look at how effective NICE considers the drug to be, in comparison to other treatment options available. Then, rather than simply rejecting a new treatment based on a price set by the manufacturer, NICE should make recommendations on what an acceptable price would be for the treatment until new data is available that might justify a higher price.
- 9.4. We believe that a new system with a greater role for NICE would not disadvantage manufacturers as it would include flexibility for future changes in drug prices as more data become available.

## **10. NICE's role in the drug development pathway**

- 10.1. We believe that positive action is needed to address current disincentives to development and innovation in the UK. A culture of cautiousness in adopting new technologies in the NHS and limitations set by the necessity for new technologies to be approved for use in the NHS act to restrict or delay access by patients to appropriate treatments.
- 10.2. We support recent discussion on how NICE can be integrated earlier in the process of drug development. However, we are concerned that these proposals are unlikely to work in practice. The UK constitutes only 3% of the global market for pharmaceuticals and is slow in adopting new treatments. It is highly unlikely therefore that companies would reconfigure their global clinical trials strategy around UK considerations.
- 10.3. Notwithstanding this, we do believe that companies should be encouraged to involve NICE in the process of drug development. A dialogue between NICE and companies, similar to that which already takes place with the Food and Drug Administration in the USA and the EMEA in Europe prior to finalising the design of clinical trials, would be very helpful.
- 10.4. A more open and permissive approach to the pharmaceutical and biotechnology industry to foster closer working relationships and intelligence sharing is likely to have long-term benefits in this area. It is important that changes to the drug development process are established with the involvement of all major funders of research, including charities, across the UK.

**We hope you find these comments useful. Cancer Research UK would be delighted to provide oral evidence to this inquiry.**