

KIDNEY CANCER CAMPAIGN

UPDATE 15TH August 2008

Since publication of the article below in the May 2008 edition of KCUK's INSIGHT magazine, there has clearly been a number of important developments. The NICE appraisal of the 4 new drugs for kidney cancer – Sutent, Nexavar, Torisel and Avastin –reached the stage at which a consultation meeting was held in Manchester on July 9, which KCUK attended. Following this NICE issued on August 7 its Appraisal Consultation Document (ACD) containing its draft recommendations.

Unfortunately these are that none of the 4 drugs are recommended for NHS funding.

Publication of the ACD was greeted by a veritable media storm. There has been huge coverage, both intensive and extensive, across TV, radio and the press. KCUK gave TV interviews on BBC1, for which the item was the lead story on its Breakfast Show and also on SKY television and BBC Midlands Today. Several national newspapers covered the story in major articles on their front pages, including The Daily Telegraph and the Daily Mail; but virtually all the national dailies carried something on it. As did the regional papers and local radio stations, many of which featured interviews with kidney cancer patients in their respective areas. **The tenor adopted by the media seemed overwhelmingly hostile to the NICE position.** At the same time the scale of media attention exceeded our wildest expectations, possibly due to the NICE document coming out as it did in a season of relatively scant news. KCUK has been seeking to take full advantage of both these things.

The next date in the NICE appraisal is August 29, the deadline by which comments on the ACD must be submitted. **WE urge everyone with an interest is this to submit their views on the NICE website:**

<http://www.nice.org.uk/guidance /index.jsp?action=article&o=41473>

THE MORE PEOPLE THAT SUBMIT THE BETTER

At the same time as all this is going on there is a review into the present restrictions placed on co-payments. This review is being undertaken by Professor Richards the Government's cancer tsar. KCUK has made a submission, arguing for some liberalisation whilst not advocating complete laissez-faire. But whatever is to be done about co-payments is very much secondary to KCUK's prime objective of getting the new drugs accepted for NHS funding.

PROGRESS SO FAR to 21st July 2008

THE CAMPAIGN

In recent years new drugs have been developed for use in the treatment of metastatic renal cell carcinoma (mRCC). These are:

- Sutent, the brand name for Sunitinib;
- Nexavar, the brand name for Sorafenib;
- Avastin, the brand name for Bevacizumab
- Torisel, the brand name for Temsorolimus.

Clinicians all over the UK consider that these drugs represent a major advance - perhaps the most important one to date - in the treatment of a condition where other agents do not have a major impact. Despite the drugs

showing great promise for extending patients' lives, they have not so far been approved by the relevant government authorities, as cost-effective and therefore eligible for funding on the NHS.

The purpose of the Kidney Cancer Campaign is to change that.

The Campaign has effectively been in operation since my appointment as a KCUK Trustee early last year. So I am still very much a 'new kid on the block', so to speak.

The Campaign is being conducted along essentially four fronts. These are:

1. **Raising awareness by exposure in the media**
2. **Exerting pressure in political terms**
3. **Considering the possibilities for taking legal action**
4. **Submitting evidence to the cost-effectiveness authorities, which are: the National Institute for Health and Clinical Excellence (NICE) covering England, the Scottish Medicines Consortium (SMC) and the All Wales Medicines Strategy Group (AWMSG).**

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1. **EXPOSURE IN THE MEDIA**

It has not proved all that difficult to engage with the media in furtherance of our cause; and in many ways this has been the most successful part of the Campaign so far. The press in particular has a great hunger for human-interest stories; and such stories abound amongst people struggling with kidney cancer.

To present you with just a few examples. An early article appeared in *The Sunday Times*, featuring two young KCUK members and their experiences with Sutent and young people have featured quite regularly in newspaper stories on kidney cancer.

The Daily Express carried the story of a 24-year-old man in Skegness initially denied NHS funding for Sutent; whilst very recently *The Oxford Times* printed the story of 33-year-old man who has just been turned down by his local Primary Care Trust (PCT). However, it is by no means only the young who get mentioned. *The Daily Mirror* included the moving story of a 66-year-old lady from Budleigh Salterton in Devon initially refused Nexavar by Devon PCT, even though she was seriously ill at the time and subsequently died (at just about the same time as her appeal was upheld).

There have been many other studies in the newspapers, both national and, especially regional or local. Amongst the regional newspapers pride of place must go to *The Northern Echo*, whose Health Editor, Barry Nelson, has been waging a Campaign of his own and who has contributed countless articles on kidney cancer drugs. Several of them have appeared as front-page splashes, including one in which our very own Professor Tim Eisen features. We are most grateful to Barry Nelson for his telling coverage of the issues which may well have had a significant bearing on the decision by a group of twelve PCT's on the North East of England to grant NHS funding to all patients prescribed Sutent by their consultants.

Because there had already been a large number of stories in the newspapers, we began to think we might be running into diminishing marginal returns with further press coverage. For we contacted *The Sunday Times* about a remarkable story of an eminent scientist who had just been awarded an OBE in the Queen's Birthday Honours List but who, more or less at the same time, was turned down by his local PCT for NHS funding of the kidney cancer drug prescribed by his consultant. There was even some suggestion of the patient becoming so unwell as not to be able to attend his investiture at Buckingham Palace. A journalist on *The Sunday Times* wrote an article on this but it was at the last minute cut from the edition because of pressure on space. However, since that time, there have been other articles appearing, including a two page in-depth article in *The Times* concerning a KCUK member in Rugby appealing for Sutent to Warwickshire PCT for Sutent funding.

In addition to newspaper stories, there have been numerous interviews on local radio, at times almost every other week. There have been somewhat fewer appearances on television but what few there have been have been rather notable ones. Perhaps the most notable of all was the appearance of Keith Ditchfield on ITV's *Tonight* programme with Sir Trevor MacDonald. Keith's contribution to this was to explain how a cancer patient could use the Internet to access new treatment therapies. He was of course a TV personality in his own right, having previously been involved in many house 'makeover' programmes. On *Tonight* he did an excellent job in highlighting the importance of new drugs for kidney cancer. It is desperately sad that he has since passed away.

The Campaign has featured on three regional TV programmes as the 'lead' item, once on BBC *Spotlight Southwest* and twice on BBC *Look North*. The *Spotlight Southwest* programme centered round the plight of the lady from Budleigh Salterton; and we were given a full 10 to 12 minutes of prime airtime to develop our case. The two *Look North* programmes were both concerned with the story of the young man in Skegness; and here too we were allocated a generous amount of airtime. But again it is heart-breaking to record that the young man in question has subsequently passed away.

Very recently there was an excellent piece on BBC *Midlands Today* featuring a patient and his consultant in clinic at the Queen Elizabeth Hospital, University of Birmingham. This new story came out at about the same time as the news broke of the Pan-Birmingham Cancer Network declaring that from 1st April 2008, both Sutent and Nexavar are to be routinely funded on the NHS for all patients for whom one or the other drug has been prescribed by a consultant. The Pan-Birmingham network covers a population of 1.6 million, encompassing 1 strategic health authority 5 PCT's and 6 hospital trusts.

It is hoped that all this media publicity is going to have some effect in influencing decisions on NHS funding but of this we cannot be sure. What we can be more certain about is that it is having the effect of raising awareness - amongst officials, doctors, relatives and of course the patients themselves. To do more than this requires some kind of political pressure.

2. POLITICAL PRESSURES

On-line Petition

One of the earliest steps in the Campaign was the setting up on the KCUK website of an e-petition to the Number 10 Downing Street website. We are most grateful for this to Rose Woodward who wrote the petition and got it registered. The petition was opened for a year from 5th February 2007. A grand total of 8,302 people signed it, which is most encouraging. It is acknowledged that this does not compare with the 1.8 million who signed the anti-road pricing petition; and it is less than the 46,750 signatories appended so far to the petition seeking to install Jeremy Clarkson as Prime Minister! Nonetheless it is great that so many people have shown themselves willing to stand with us in asking for the drugs to be NHS-funded.

The petition now having closed, we have received a response from the Prime Minister's Office. The response reads as follows.

Sutent (sunitinib) and Nexavar (sorafenib) are licensed in the UK to treat advanced renal cell carcinoma when standard therapy has not helped to stop the disease or is considered unsuitable for the patient. Therefore, clinicians are able to prescribe them on the NHS. The Department of Health has referred these drugs, along with Avastin (bevacizumab) which is awaiting a licence for this condition, to the National Institute for Health and Clinical Excellence (NICE) for appraisal of their clinical and cost effectiveness. NICE will be publishing a timetable for this work in due course.

NICE is an independent body, which makes decisions on the clinical and cost effectiveness of products based on the latest evidence. It is important not to prejudge NICE's guidance, but the Department of Health recognizes that the Institute's decisions have serious implications for patients and their carers.

NICE's appraisal process includes wide ranging consultation, during which the Institute invites comments from patient groups and individual patients, carers and other members of the public.

Decisions to fund treatments are made by Primary Care Trusts (PCT's) in consultation with the healthcare professionals who are best placed to decide on the most appropriate treatments for their patients. The Department of Health has made it clear to the NHS that it is not acceptable for funding for licensed treatments to be withheld from patients simply because NICE has not published guidance on them. In these circumstances, the Department of Health expects PCT's to take full account of available evidence when reaching funding decisions. This is confirmed in the Department of Health's Good Practice Guide issued to the NHS in December 2006.

The Department has now announced the Cancer Reform Strategy. The Strategy, launched on the 3 December, recognizes that the Department of Health should reduce the period when local decisions are necessary to a minimum by ensuring that NICE issues guidance as soon after the drug is licensed as possible. The Department of Health will achieve this by continuing to work with the Institute to ensure that its fast-track (Single Technology Appraisal) process, launched in November 2005, is used appropriately and works as effectively as possible.

It is also proposed that, as a default position, all new cancer drugs and significant new licensed indications will be referred to NICE, provided that the Institute agrees that there is a sufficient patient population and evidence base on which to carry out an appraisal and that there is not a more appropriate alternative mechanism for appraisal. This will provide greater certainty at an earlier stage about whether NICE guidance will be forthcoming on individual drugs.

There is little if anything in this statement that we did not know before but is good to see clinicians' ability to prescribe the new drugs re-affirmed in print. It is also good to see clarification of the non-acceptability of the patients being denied licensed treatments merely because NICE has not yet issued guidance on them. Otherwise however, it seems that the Prime Minister's Office is rather side-stepping the fundamental issues involved.

(It may be noted that the statement makes reference to only three of the four new drugs developed for the treatment of mRCC. No reference is made to the drug Temsorolimus. But this is understandable because the original petition was couched only in terms of Sutent and Nexavar.)

House of Commons Select Committee on Health

This body of MP's recently undertook an enquiry into the work of NICE. Two out of the five points in the Committee's terms of reference appeared especially relevant to the Campaign: firstly, the evaluation process used by NICE and whether any particular groups are disadvantaged by it; and secondly, the speed at which NICE performs its assessments and publishes its Guidance.

Accordingly, KCUK submitted written evidence. The Committee published its final report on 10 January 2008.

In our submission we argued that, if not by intent, then most certainly in effect, a lack of NHS funding for the new drugs bears spectacularly unfairly upon kidney cancer patients. After all these are patients who may not - for the most regrettable of reasons - constitute much of a charge on NHS funding in other respects. The median age at contracting kidney cancer is 62.4 years. Many people reaching this age will have clocked up 40+ years of National Insurance contributions; and yet they may not be able to look forward to receiving the state pension for the same period of 15 years or more that other people can expect. From a broader perspective the expenditure of large sums of money on new drugs for individual patients does not amount to the great largesse it may seem at first sight.

Organisations like NICE work at a glacially slow pace its assessments to perform. Notice of mRCC multiple assessments was announced by NICE on 25 September last. But the final result will not be given until sometime in January 2009, about 16 months later. There is also sometimes a long wait between drugs getting licensed and then receiving NICE appraisal.

The Health Committee's principal recommendations are as follows. (1) The cost effectiveness of treatment should be judged in terms of the wider social benefits they would bring, including the benefits to carers. (2) There should be closer working with PCT's on implementing guidance. (3) There should be faster assessment of treatment at the time of licensing. KCUK welcomes these recommendations but still feels that the report does not lay sufficient emphasis on reforming certain aspects of NICE assessments, in particular the cost-per-QALY benchmark, something that is explained and discussed further below.

Following the report we were invited to pen an article on all of this for the *National Health Executive* magazine. The resulting piece was then published in the Volume 5 Number 1 issue of the magazine which appeared in February 2008.

Constituency MP's

If a patient is refused funding by a PCT, then he/she is strongly recommended to lobby his/her Member of Parliament, with a view to getting the MP to send a supporting statement to the PCT.

This can often be very helpful. As just one example, the Honourable Member for Dorset West might be forgiven for feeling a certain glow of satisfaction, after all three of his kidney cancer constituents won their appeals for NHS funding within what was a relatively short space of time. So far as I could see, the MP didn't do anything very exceptional, other than asking the PCT some rather awkward-sounding questions!

International Comparisons

The UK may not be the best country in the world in which to combat mRCC. Patients have more chances of gaining free access to the new drugs, if they are resident in North America or in other countries of Western Europe like, for example, Sweden which has a very similar health service to that in the UK. In Sweden there is a body equivalent to NICE; and this Swedish authority has already approved both Sutent and Nexavar as suitable for state funding.

Similar positions obtain in France, the Netherlands, Denmark and in countries like Argentina, Mexico and South Korea. So in this respect the UK compares rather unfavorably not just against countries at a similar stage of development, but also against some less advanced nations. It is clear that there are some wide inequalities between the UK and other countries. Amongst patients there is obviously a swell of anger and concern that the quality of care is markedly better elsewhere and that life expectancies of kidney cancer patients abroad are longer than those of patients who live in the UK and on occasion, UK patients have sought treatment abroad rather than be denied treatment altogether. (A recent newspaper report told of a lecturer at Oxford Brookes University traveling to Denmark to get free treatment with Nexavar. The Danish government was prepared to grant the patient free treatment because his wife is a Dane.)

It is possible that the unfavorable position of the UK in terms of the availability of treatment is also reflected in international comparisons of cancer survival rates. Some statistics recently released appear to indicate that, whilst survival rates are improving everywhere, other countries tend to compare favorably with the UK, in terms of the speed at which they are improving. Of course there may be a whole host of reasons explaining why survival rates vary over time and from country to country but it has more than just crossed our minds that that variation in the speed at which new anti-cancer drugs are being taken up has got something to do with variation in survival rates. This is a general point affecting all forms of cancer, but it seems especially germane to kidney cancer where the improvement in survival rates has been so disappointingly slow.

At various points in the Campaign, attention is being drawn to points like these.

'Post-code Lotteries'

If the UK as a whole is not particularly good place for mRCC patients, there are some places within it that are worse than others. Some PCT's have shown themselves willing to fund Sutent and Nexavar, the two drugs which already have their marketing authorisations from the European Agency for the Evaluation of Medicinal Products (EMA). At the same time other PCT's have steadfastly refused to do so in the absence of a 'cost-effectiveness badge' from NICE or the equivalent bodies in Wales and Scotland. There seems to be something of a 'hawks and doves' situation developing here. It can depend crucially upon exactly where you live, ie in which PCT area, whether or not you are going to get NHS funding. In this context the twelve PCT's in the North East which have decided to fund Sutent for all patients appear as doves, whereas just across the border, the North Yorkshire PCT might be cast as a hawk. A patient living in Richmond, seven miles over the border from one of the North East PCT's which are now funding Sutent for all patients, was refused similar funding by her own PCT, North Yorkshire. And a similar situation has now developed between the health authorities in Birmingham and those in the neighbouring counties of Warwickshire and Worcestershire. This was something well drawn out in the BBC *Midland Today* television programme referred to earlier.

To illustrate the growing absurdity of post-code lotteries and to introduce a note of levity into an otherwise very serious issue, a charming question was raised at the last KCUK Annual Conference held in November. A patient who lives on a boat wished to know where he should moor it in order to maximize his chances of gaining NHS funding. Assuming it is not possible to navigate the canals into Birmingham, the best suggestion is that the patient should sail to the North East and dock somewhere in the Newcastle area—provided he does not mind the cold!!

It would be unutterably foolish of us not to make some political capital out of the post-code lotteries issue. After all, one of the reasons advanced for setting up the present system of cost-effectiveness assessments was precisely to avoid post-code prescribing. In this connection we have been written to by Julia Black who has been monitoring NHS post-code lotteries since the issue first emerged in a big way in relation to the Herceptin drug for breast cancer. Julia tells us that she is setting up a 'Post-code Lotteries' website, not just for kidney and breast cancer but also for all forms of the disease. We would like to co-operate fully in this venture. As Julia says, this is a problem that will not go away. It will only get worse, as more and more new drugs come through and as patients become more knowledgeable and less prepared to wait for something to go through the NICE appraisal process. It might be good if Julia could come up with some kind of league table, ranking PCT's by, say, their relative willingness to fund drugs on the 'exceptional needs' basis. Such a league table could be widely exposed in the media.

Funding Issues

The new drugs can work out rather expensive on a per patient basis, at something over £3,000 per month. Most patients in the UK do not carry private medical insurance; and even if they did it is not always certain that the insurer will cover the (full) cost of drugs not approved as cost-effective by NICE. Many patients simply do not have the financial resources to fund treatment for what, it can now fervently be hoped, will be a considerably longer period of time. Some who have been refused funding by their PCT's have been considering some desperate measures, like selling (or at least re-mortgaging) their homes, or commuting annual pensions into immediate lump sums. But these courses of action can of course affect other members of the patient's family and consequently cannot be entered into lightly.

On top of all this, if a patient chooses to self-fund purchase of the new drug, he/she might then be classed as a private, as opposed to NHS, patient. As a result he/she could be made responsible for the *total* cost of the treatment, not just the drug itself. Given that the total cost might include the costs of blood tests, scans, clinician's fees and even the full cost of any further hospitalisation, it is clear that this is rapidly entering the realms of impossibility for many patients.

There is a lack of clarity over this. A Urological Cancers Workshop was held at the Department of Health on 31st May 2007, an event designed to inform the *Cancer Reform Strategy* being developed under the leadership of Professor Mike Richards, National Cancer Director, aka the Government's 'Cancer Tsar'. It was attended by surgeons and oncologists from across the country and its remit was to produce a vision paper on urological

cancer services and what they should be able to achieve by 2012. At this workshop I raised the issue of what happens if a patient pays for drugs not funded by the NHS, an issue known more generally as 'co-payment'. It was agreed that there should be greater clarity about whether, or when, co-payments are allowed.

At present there are certain 'grey' areas in this, and it seems that much depends on whether the drugs treatment can be regarded as a separate episode to any other treatment (eg further hospitalisation) the patient needs. But at present this is anything but clear and the question needs to be clarified.

There is at least something positive for patients seeking to fund themselves temporarily whilst waiting for a (hopefully successful) verdict from their PCT's Exceptional Needs committee. This is because there is a company, known as *Healthcare at Home*, which can shave £600-800 per month off the cost of private funding, depending on the particular drug prescribed and the dosage required. The company can do this because it can (perfectly legitimately) avoid VAT, doesn't put a mark-up on its pharmacy charge and puts a lower mark-up on the ex-works price of the drug itself when supplying self-funding patients. (Representing the company, two very nice ladies have been to my house to explain all this to me.)

For the majority of patients, purchasing from *Healthcare at Home* may be no real substitute for NHS funding. Nevertheless it may sometimes be useful as an interim arrangement.

3. LEGAL CONSIDERATIONS

Over the past few months KCUK has been considering the possibilities for legal action. To this end it has been taking advice from a Queen's Counsel (QC). In this we have been greatly helped by a KCUK member who is a solicitor and who has personal experience of kidney cancer. Also we have just been offered some further help from another barrister who is also a kidney cancer patient.

The legal possibilities are two-fold. First, there might be a challenge to the whole NICE process of assessment: This was the route chosen by the Alzheimer's Society, after certain new drugs for Alzheimer's disease had been assessed as not cost-effective. However, the Alzheimer's Society's challenge was unsuccessful; and there seem to be many formidable difficulties in achieving a successful outcome along this route.

The second possibility is an individual challenge by Judicial Review (JR) of a PCT's decision to refuse funding on the ground that the refusal was irrational (one which the PCT, acting rationally, could not have reached). This remains the most likely route for legal intervention. Thus KCUK supplied the QC with details of a number of cases in which PCT's had refused funding. These were then examined for similarities with a JR case recently brought against a PCT by a bowel cancer patient, a case which *did* have a successful outcome, the patient winning.

Essential for any JR proceedings would be one of the following 3 scenarios: Type (i) where the PCT was found to have no policy for dealing with cases of patients seeking funding for drugs without a cost-effectiveness badge. Type (ii) where the policy itself was open to attack as being one that no reasonable PCT could adopt. Type (iii) where the PCT failed to apply the policy in a material way. Other things being equal Type (iii) cases might be expected to be the most common, Types (i) and (ii) being comparatively rare.

An initial examination of the kidney cancer cases did not reveal, in legal terms at least, sufficient prima facie evidence as helpful to a JR as that which obtained in the successful bowel cancer case. In any case, before long, all but one of the kidney cancer patients were successful in their appeals for funding - and the remaining case is one that is very complicated.

Pursuing legal action is something that must be done with a great deal of caution. The risks are high, not just because of legal costs but also on account of the possible implications, in class-action terms, of an unfavorable verdict. Losing a court case could do a lot of damage to patients seeking exceptional-needs funding in the future.

However indignant we may feel about PCT's refusing to fund, we can only proceed along a legal route if the case is very strong, in legal terms. However, KCUK will continue to look at the possibilities.

4. SUBMISSIONS TO COST-EFFECTIVENESS AUTHORITIES

KCUK has already submitted evidence on Sutent to the Scottish Medicines Consortium (SMC) and the All Wales Medicines Strategy Group (AWMSG). It did so by completing (religiously) the authorities' templates for submitting evidence and duly met the deadlines imposed. In both cases the outcomes were very disappointing. Neither the SMC nor the AWMSG were prepared to give Sutent their cost-effectiveness badge. The SMC result was especially disappointing because this appraisal was conducted after it became known that the manufacturer of the drug (Pfizer) had reduced its price by 5% *and* made drugs for the first cycle of treatment entirely free of charge for all patients. If the manufacturer had lodged an appeal, then KCUK would have tried to support it. (But, as we understand it, the manufacturer may be planning to reapply afresh for SMC recognition of Sutent).

A submission in respect of all four of the new drugs was made to NICE at the beginning of the year. In addition to matters already discussed in the present paper, the submission covered the following points.

Survival Rates

It should surprise nobody that to patients the most important of all concerns is that of their survival. Until recently patients with mRCC had little or no reason to be hopeful. The disease is highly resistant to chemotherapy and radiation. Treatment with the immunotherapy drug Interferon Alpha does have a modest effect in prolonging survival. Interleukin 2 is not proven to increase survival and has substantially more side effects. The evidence appears to be mounting that the new-targeted drugs do significantly lengthen survival, certainly progression-free survival and probably overall survival too. For example, trial results indicate a median progression-free survival of 11 months for Sutent compared with one of 5 months for Interferon a more-than-doubling effect from the new drug.

Another important point is that significantly more patients appear to derive benefit from the new drug as compared to Interferon. So, in short, the most important facts about the new drugs are that they (a) help more people and (b) help them for longer.

Patients are aware that neither the immunotherapy drugs nor the new-targeted drugs lead to totally curative outcomes. Patients with advanced mRCC realise that one day they may die from the disease (unless they die from some other cause beforehand). They also realise that, in common with other cancer patients in similar circumstances, the drugs treatment administered to delay the growth/spread of tumors generates some adverse side effects which they will have to tolerate. Nonetheless they welcome being treated by these drugs because as the saying goes, 'where there's life, there's hope'. They may hope that provided they can stay alive long enough, some new 'magic bullet' producing curative outcomes may be discovered. Whatever the likelihood of any such discovery, there is absolutely nothing wrong in patients entertaining hopes of it. After all, hope is supposed to be one of the three cardinal virtues.

Side Effects

A further advantage with the new targeted drugs is that they are much more easily tolerated, with the side effects of high-dose Interleukin are such that, in order to put up with them, a patient could do with the constitution of a highly trained Olympic athlete! But the effects of the new-targeted drug are much less difficult to live with and patients are very conscious of this. It is true that there are still some adverse effects, eg Sutent leads to diarrhoea, high blood pressure, hand-foot syndrome and occasional vomiting but a very large majority of patients can tolerate these effects well enough to stay on treatment.

Bearing in mind that many mRCC patients, by reason of age, often present with other medical problems, any reduction in adverse side effects is a most valuable benefit. Judging by posts entered on the forums of KCUK and the James Whale Fund websites, where notes are exchanged on various problems associated with treatment, patients do appear to be getting along with the side effects of a drug like Sutent quite well.

Applying for funding

Another concern lies with the process by which a patient applies to the PCT to be funded as an 'exceptional needs' case. The application process can seem rather formidable. It's like the patient applying for some kind of job - and a pretty exalted one at that! In addition to a reasoned case set out by the patient's consultant, the application might also contain a supporting statement from somebody like the patient's constituency MP. Then the patient might appear before the relevant panel in the PCT that decides on these things to offer some verbal arguments and it is not unknown for a patient to arrive for this armed with a PowerPoint presentation. However, many patients may feel ill-equipped to do all this and may therefore be deterred from entering an application at all.

The Cost per QALY benchmark

Many patients question the appropriateness of deciding upon the economic worth of a new drug on the basis of its cost per Quality Adjusted Life Year, QALY, an upper bound, if not limit, for which has been set at £30,000.

The use of a QALY concept in this context raises in our minds a veritable multitude of questions. Here we content ourselves with just a few. To begin with, why £30,000? Why not £60,000? Or why not £90,000? In an article in *The Times* in January last year the Chairman of NICE admitted the £30,000 figure is pretty arbitrary. It seems to be set more in line with what the NHS can currently afford than with any true assessment of the overall *net benefit* of the new treatment. To get that, one really needs a full-blown cost-benefit analysis. In a cost-effectiveness analysis the accent is, naturally enough, very much on costs. In a cost-benefit analysis the evaluation is more even-handed, asking the question whether, *in total*, the benefits of a new drug treatment are greater than the costs.

Does a QALY represent total benefits? In our view it does not. Consider the case of a patient who on learning that her exceptional-needs case for Sutent funding had been accepted and that, as she saw it, she was now going to have an extra year or two to live, spoke very enthusiastically about her plans. One was struck forcibly by the rigour with which she had thought things through. Of course, having cancer often does have the effect of concentrating the mind wonderfully. But in the mind of this patient, the concept of a Quality Adjusted Life Year has only a very limited meaning. The benefits of remaining alive are much, much more than that. The problem is that the 'much, much more' plays no part in the evaluation at all.

If there is a question over what is left out of the benefits side, there is also a question of what is included on the costs side. The query here concerns the treatment of any taxes paid. The costs of acquiring the new drugs should, in an economic analysis of public expenditure, exclude all taxes. For these are not real resource costs, merely transfer payments, from the purchasers of the drugs to the government. Hence the 17.5% VAT an NHS hospital pharmacy has to pay on purchases of Sutent and Nexavar should not, for the purpose of economic evaluation, enter into measurement of the costs of these new drugs. If taxes are not excluded, then real resource costs are being over-estimated.

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