



15 August 2008

Mr Clifford Middleton
National Institute for Health and Clinical Excellence
MidCity Place
71 High Holborn
London
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Dear Mr Middleton,

Re: Citizens Council Report on “Quality Adjusted Life Years (QALYs) and the severity of illness”

The organisations listed at the end of this letter wish to submit the following comments with regard to the above report by NICE’s Citizens Council.

First, we welcome the opportunity to comment on this report and are pleased to see that the topic is being evaluated.

Second, as patient groups we are very mindful of the devastation which a serious illness can wreak, not just on the patient but on the family and caregivers as well. While it is extremely difficult to arrive at an objective, fair and comprehensive definition of severity – as the Citizens Council rightly acknowledges in its report – brain tumours provide one of the best examples of a severe illness because of the nature of the disease.

Brain tumours strike at the very core of who a person is. Depending on where they are located in the brain, they can cause significant physical deficits such as hemiparesis, difficulty with swallowing, loss of balance, facial weakness, difficulty with fine motor skills, vision deterioration and a substantial decrease in verbal acuity. There can also be devastating changes in cognition, behaviour and personality, as well as chronic seizures. The social effects of a brain tumour can include loss of one’s driving license, stigma at work and the collapse of relationships.

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But the most “severe” aspect of a brain tumour is its relentlessly life-limiting nature. The prognosis for people diagnosed with a malignant glioma, the most common type of primary brain tumour, is extremely poor.

Brain tumours are also severe in that they are no respecters of age (affecting tiny babies as well as young adults and older people), socio-economic background or geography.

Brain tumours are one of the most severe of all diseases.

Their causes are generally unknown, so there can be no prevention strategies for brain tumours. Screening is unrealistic and there are no known lifestyle changes that can prevent a brain tumour.

Currently, the only hope brain tumour patients have lies in access to promising, new therapies which, although they may not save life, can extend survival with a good quality of life. It is for this reason that our particular group of patients relies so heavily, and hopefully, on NICE’s vital decisions concerning access to treatments.

We feel that severity of disease should most certainly be taken into account when NICE evaluates new therapies.

We agree wholeheartedly with the Citizens Council, though, that severity should not be included as an integral part in the calculation of the QALY because, as the Council points out, the QALY is already a very complex measure which many people - especially patients - find difficult to accept as being a realistic, fair and sharp-enough tool on which to base such crucial decisions as access on the NHS to groundbreaking medical treatments.

We also agree with the Citizens Council that severity should definitely be taken into account alongside considerations of clinical effectiveness and cost evidence. We place clinical efficacy first because, in our opinion, this is the most important aspect of assessing whether or not a new therapy should be made available to patients.

It seems to us that severity is inextricably wound up with issues surrounding health related quality of life (HRQOL) and that more studies should be done in this area so as to provide a better framework in which to evaluate the provision (or not) of NHS-funded therapies.

In the brain tumour arena, health related quality of life issues are gaining prominence. A study of HRQOL was included in the landmark clinical trial combining radiotherapy with temozolomide chemotherapy (now known as the “concomitant” or “Stupp” therapy – see Taphoorn, MJ et al, “Health-related quality of life in patients with glioblastoma: a randomised controlled trial”, *Lancet Oncology*, 2005 Dec; 6(12):937-44).

A Wyke et al (*British Journal of Healthcare Management* 2008 Vol 14 No 3) wrote in an article “What is ‘quality of life’ for patients?” that “Failure to measure QOL adequately can result in an underestimation of the cost-effectiveness of a health technology.

The EuroQol EQ-5D questionnaire referred to in the Citizens Council report – presumably devised by health economists, researchers, social scientists and psychologists (we are unsure if any patients or their representatives were involved) – does need reviewing and updating as the Council suggests. It surely would not be an impossible task to develop a questionnaire which better addresses the impact of disease severity on a patient’s quality of life.

For example, the QALYity Project, comprised of representatives from patient organisations, academics, policymakers and other experts, has highlighted in useful depth some “unique definitions” for quality of life which they have categorised into three domains:

- (1) access to, and excellence of, treatment and care.
- (2) physical wellbeing as a result of care
- (3) general outlook on life as a result of care

The project ensured that their domains and the definitions within them could apply as measurements of QOL across many disease areas (and not just cancer, for example).

It’s interesting to note that in a patient survey about these definitions (there are 31 of them), the one which “consistently drew a high vote from all sub-categories of patients, regardless of medical condition or background, as the most important priority [was]: ‘Getting the correct treatment, support or care.’”

One patient involved in this project said: “I believe you should be able to have the best quality of life, and treatment to make that possible – even if you only have a short time to live. You should have the right to pain relief, and the best care to the end of your life, and not be told it is a waste of money when there is no cure.”

We agree with Rachel Rowson of Cancerbackup that the QALY is a “blunt instrument” and Peter Mansell of the National Patient Safety Agency that the QALY “masks individual differences”.

We would further add that currently the QALY also does not take into consideration the issue of unmet need for people with severe diseases like brain tumours; the realistic total cost to the NHS of delivering a therapy; the rareness or uncommonness of a disease; the impact of a disease on family and caregivers (which has its own economic ramifications within society at large) or the true value to society of a human life – even though that life may be tragically cut short.

The QALY also doesn't take into account the financial burden to the state which can be significantly lessened when a caregiver is involved, nor the contributions which are made to society by patients who access clinically effective life-extending therapies and become well enough to return to work, if only for a while.

As Professor Paul Dolan (Imperial College) and Richard Edlin (Leeds University) pointed out in their presentation to the Citizens Council, the allocation of resources could not only be influenced by the severity of a disease, but also “the age of those experiencing it”.

A very interesting study – which albeit refers to the allocation of research funding rather than the allocation of money to purchase treatments for use on the NHS – looks at years of life lost from cancer as a measure of burden. The author, Professor N G Burnet (Department of Oncology, University of Cambridge) points out that “Years of life lost (YLL) summed over each individual dying after a diagnosis of cancer represents a population-based mortality indicator of the impact of that disease on society.”

To use brain tumours as an example of this measure – which could perhaps also be taken as a measure of “severity” – Professor Burnett's report states that: “Brain tumour patients, however, suffer more than three times as much loss of life [than patients with prostate cancer], with an AYLL [“average years of life lost”] of just over 20 years...brain and CNS have the highest AYLL of all 17 tumour sites [reported on in the study]...” (N G Burnet et al, Years of Life Lost (YLL) from cancer is an important measure of population burden – and should be considered when allocating research funds, British Journal of Cancer 2005, 92, 241-245)

Of course, there are also many patients who live with extremely severe illnesses which are not life-threatening, condemning them to a lifetime of suffering from the effects of their diseases. This is also devastating.

Additionally, the level of severity in an illness may vary during the different phases of a disease.

It's interesting to note in a recent report from the European Observatory on Health Systems and Policies entitled “Ensuring value for money in health care – the role of health technology assessment in the European Union (Observatory Study Series No. 11, 2008) that France's and Germany's health technology assessment agencies include the nature and severity of disease in their assessment criteria.

We hope that the Citizens Council Report on “Quality Adjusted Life Years (QALYs) and the severity of illness” does not ultimately go the way of their study on the “Rule of Rescue”. We note with concern that NICE eventually rejected the “Rule of Rescue” (as defined by the Citizens Council) in its recently released Social Values Judgement.

“NICE recognises that when it is making its decisions it should consider the needs of present and future patients of the NHS who are anonymous and who do not have people to argue their case. NICE considers that the principles provided in this document are appropriate to resolve the tension between the needs of an individual patient and the needs of other users of the NHS, present and future. Therefore, the Institute has not adopted an additional ‘rule of rescue’.

(NICE, Social Value Judgements, Second Edition, July 2008)

The Rule of Rescue appears to have been rejected by NICE despite the fact that the majority of its Citizens Council members and a clear majority of respondents in the public consultation for the Rule of Rescue Report opposed its rejection.

An overwhelming majority (over 90%) believed that NICE should consider “if the intervention was required to avoid immediate loss of life, or if there was a good chance of an increased life expectancy, or whether the intervention will result in a significant improvement in quality of life.” (Page 3, NICE Citizens Council, Rule of Rescue Report, Patient and Public Comments, June 2006).

Finally, we would agree with the Citizens Council statement: “NICE must recognise that, for some people, or under some circumstances, length of life is by itself of value, whatever its quality. Surviving to see something important happen, for example. Severity judgements are currently dominated by a medical model of disease. We would like to see NICE take more account of social factors.” (NICE’s Citizens Council report on “Quality Adjusted Life Years (QALYs) and the severity of illness”, pages 13 and 14)

The tools of one’s trade are vitally important – whether you are a plumber, a lawyer, an entertainer or an agency tasked with evaluating medical treatments for use on the NHS. It seems crucial that health technology assessment organisations like NICE, whose role is to make, literally, life and death decisions, should have the very best tools with which to do so.

We sincerely hope that NICE will adopt the Citizens Council recommendations about severity of illness. We hope that NICE will progress the Council’s report by ensuring that their tools for making decisions about access to therapies are more firmly rooted in social value judgements.

The Citizens Council point out that "...severity is a difficult concept. One way of viewing it is as 'how far away you are from your final destination, perfect health'". For some people, such as those on a brain tumour journey, the question is not how far they are from perfect health, but how close they are to achieving the best possible quality of life while coping with their disease.

We hope that the distance to the best possible quality of life for such patients becomes shorter and shorter with the incorporation by NICE of more flexible and relevant approaches in health technology appraisals.

Yours sincerely,

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