

www.theibta.org

We are an alliance of brain tumour support, advocacy and information groups around the world, including brain tumour patients and caregivers, researchers, scientists, clinicians and allied health professionals who work in the field.

greater collaboration ► greater knowledge ► greater hope



Report on NICE Citizens Council meeting

Departing from the threshold

November 27 – 29, 2008

A submission in response to this report by the International Brain Tumour Alliance (IBTA) (Submitted 24 April 2009)

The International Brain Tumour Alliance (IBTA) welcomes the opportunity to respond to the recent Report from the NICE Citizens Council on “Departing from the threshold”.

The IBTA is an alliance of support, advocacy and information groups for brain tumour patients and caregivers in different countries. We are a not-for-profit organisation registered in England and Wales where we have one of our international offices.

We wish to commend the NICE Citizens Council for its helpful response to the question it was asked by NICE to consider: “In what circumstances should NICE recommend interventions where the cost per QALY is above the threshold range of £20-30,000?”

We also support the Citizens Council decision that “*It is clear...that the great majority of us do not think that a view based solely on formulaic considerations of health economics is a satisfactory basis on which to make recommendations about the use of drug or other interventions by the NHS. Judgements also need to take account of other factors.*”

This endorses what we have been saying in the brain tumour community for some time – that far greater flexibility is crucial in the appraisal of promising new therapies so that patients benefit by obtaining access in a timely and consistent manner based primarily on clinical efficacy and need. Therapies for rare and less common cancers like brain tumours don’t easily fit into the regulatory or health technology appraisal systems so we are also grateful to the Citizens Council for examining the issue of the QALY ceiling.

The following comments reflect a perspective from the brain tumour patient and caregiver community on some of the conclusions reached by the Citizens Council.

Chair: Mr Denis Strangman, 10 Carrodus Street, Fraser, ACT 2615. Australia. Tel: (61) (2) 62583912. Email: chair@theibta.org **Co-Director:** Mrs Kathy Oliver, PO Box 244, Tadworth, Surrey KT20 5WQ, United Kingdom. Tel: (44) (0) 1737 813872, Fax: (44) (0) 1737 812712, Mobile: (44) (0) 777 571 2569. Email: kathy@theibta.org

The IBTA is a not-for-profit, limited liability company incorporated in England and Wales, Company Number: 6031485. Registered address: c/o Roxburghe House, 273-287 Regent Street, London W1B 2AD, UK. Address for correspondence: Co-Director, IBTA, PO Box 244, Tadworth, Surrey, KT20 5WQ, United Kingdom.

We note that of the list of 15 “various possible circumstances” which would justify a departure upwards in value from the existing QALY threshold, nine of the circumstances are applicable to people with brain tumours.

“the illness is rare”

Brain tumours are considered a rare disease; primary malignant brain tumours affect 3.7 males per 100,000 per annum and 2.6 females per 100,000 per annum.¹

“the patients are children”

Brain tumours strike irrespective of age, sex or geographic location, affecting all groups of people with equal ferocity. Mortality figures relating to childhood brain tumours in several developed countries, for example, indicate that paediatric brain tumours have now overtaken childhood leukaemias as the number one cause of childhood cancer death.^{2, 3, 4}

“the illness under consideration is extremely severe”

Because of their location in the body, brain tumours strike at the very core of who a person is, affecting cognition, behaviour and physical ability. Deficits suffered by brain tumour patients combine the very worst aspects of serious neurological conditions with the very worst aspects of cancer. These can include blindness, paralysis, serious mental impairment, epilepsy, inappropriate behaviour, loss of speech and hearing. Most patients die within a short time.

“there are no alternative therapies available”

Because so little is known about the causes of brain tumours, prevention is impossible and screening is unrealistic. As far as we are currently aware, lifestyle changes are irrelevant to brain tumours. So by and large, the only hope available to these patients lies in the development of and access to promising new treatments. Because research and development in the brain tumour field are woefully underfunded, and many pharmaceutical companies are not interested in small-population diseases, there are extremely limited treatment options for brain tumour patients.

“the intervention will have a major impact on the patient’s family”

“the treatment is life extending”

“the condition being tackled is time-limited”

Despite the appalling prognosis (patients diagnosed with a malignant brain tumour have a life expectancy of 8 to 12 months) there are some patients for whom the chemotherapy, temozolomide, for example, has enabled them to return to work for a time and be fully contributing members of society. Temozolomide was originally rejected for NHS funding by NICE as being over the QALY threshold despite it being the standard of care in most other developed countries of the world. A re-appraisal of the therapy, prompted by concerned patients, medical specialists and industry, resulted in subsequent approval by NICE. Access to temozolomide (which ironically was developed in the United Kingdom and yet people in the UK were denied it) has provided patients with additional and meaningful time with their families and has, for many, generally resulted in far better quality of life for the patient himself.

“Life extending”, of course, is a very subjective term and concept. For someone with a prognosis of 8 to 12 months, even a few additional weeks of life could be highly meaningful and productive.

“the intervention would prevent more harm in the future”

Sadly, there is still no cure for a primary malignant brain tumour so we cannot yet talk of any treatments in question as being life-saving (one of the special circumstances mentioned by the Citizens Council). However, one of the goals of current treatment approaches is to endeavour to develop interventions which might at least allow brain tumours to be treated as a chronic disease. So “preventing more harm in the future” might well apply to the quest for a solution to this challenge. Cutting edge therapies which aim to do this (such as anti-angiogenesis therapies for brain tumours or anti-proliferation and anti-migration therapies) don’t come cheap and will inevitably require a departure from the QALY threshold.

“the intervention will encourage more scientific and technical innovation”

It is crucial not to forget about the incremental benefits of providing promising therapies to patients, even though the therapies are above the QALY threshold. By allowing patients access, not only can a wider group of people benefit from the intervention but more is then learned about the intervention itself from its wider application. Relative successes such as the temozolomide story – a therapy which is now used in combination with other treatments for brain tumour patients – have encouraged researchers to be more innovative with their approaches. In an atmosphere of restrictive access to clinically efficacious therapies, what incentives are there for the development of better treatments?

The “greatest treatment breakthrough in the history of brain tumours”; the “most amazing therapy in half a century”; the “silver bullet”; the “cure”: these are all meaningless if, after all the years of research, investment, development, clinical trials and licensing approvals, the therapies just don’t get to the patients.

In conclusion, we ask Government, the pharmaceutical industry and Primary Care Trusts to fully appreciate the devastation and heartache wreaked on patients, their families and caregivers following a brain tumour diagnosis. We believe that compromise is the way forward. Departure from the QALY threshold; a pharmaceutical pricing structure that is more flexible; and a consistent approach across health authorities regarding processes for access will all help patients attain more equitable treatment.

Submitted 24 April 2009

See next page for Notes.

Report on NICE Citizens Council meeting

Departing from the threshold

November 27 – 29, 2008

A submission in response to this report by the International Brain Tumour Alliance (IBTA)

(Submitted 24 April 2009)

Notes:

1. *World-wide incidence of primary malignant and non-malignant brain tumours*, Society for Neuro-Oncology (SNO) abstract EP12, 2007 Carol Kruchko (1), Jennifer Propp (2), Kate Schellinger (2) and Bridget McCarthy (2); 1. CBTRUS, Hinsdale, IL, USA; 2. Division of Epidemiology, University of Illinois, Chicago, Illinois, USA.

2. USA statistics: Surveillance, Epidemiology, and End Results (SEER) Program (www.seer.cancer.gov), SEER*Stat Database: Mortality – All COD, Aggregated with State, Total U.S. (1969-2005), National Cancer Institute, DCCPS, Surveillance Research Program, Cancer Statistics Branch, released April 2008, Underlying mortality data provided by NCHS [www.cdc.gov/nchs]

3. Canada: Canadian Cancer Society/National Cancer Institute of Canada: Canadian Cancer Statistics 2008, Toronto, Canada, 2008

4. UK: Mortality Statistics, Deaths Registered in 2007. DR 07. Review of the National Statistician on Deaths in England and Wales, 2007.