Patient/carer organisation statement template

Thank you for agreeing to give us your views on the technology and the way it should be used in the NHS.

Patients and patient advocates can provide a unique perspective on the technology, which is not typically available from the published literature.

To help you give your views, we have provided a template. The questions are there as prompts to guide you. You do not have to answer every question. Please do not exceed the 8-page limit.

About you

Your name: Michael Francis – Patient expert

Name of your organisation: Nominated by Bone Cancer Research Trust

Other Issues

Please include here any other issues you would like the Appraisal Committee to consider when appraising this technology.

Since September 2001, I have been a Director of the Guy Francis Bone Cancer Research Fund, set up by my son Guy shortly before his death from Osteosarcoma (OS) in April 2002. Since December 2005, I have also been Chairman of Trustees of the Bone Cancer Research Trust, a national charity devoted to promoting and funding world class research into the causes and treatment of Primary Bone Cancers (PBC), particularly Osteosarcoma and Ewing's Sarcoma, and to improving patient outcomes.
In the 3 years December 2005 – 2008, the Trust has awarded over £400,000 for twelve research projects in the United Kingdom.

Personal experience as a parent carer.
Guy was diagnosed with OS in February 1997, at the age of 17. He received treatment through a combination of chemotherapy and surgery with the removal of most of his left femur and the top of his tibia, and to his lungs. He went into remission for 3 years and commenced studies at the University of Teesside; but in 2000 the OS returned, this time to his neck vertebrae C1 & C2. Again, chemotherapy was given prior to and after the complete removal of these bones in what is now recognised as unique, pioneering surgery in the UK. Again, he went into remission, this time for 9 months, but for a third time the OS returned to envelop vertebrae C3 & C4. Neither chemotherapy nor surgery was now appropriate, and he died 12 days after diagnosis.
During this long 5 year period, my wife and I acted as principal carers both during the numerous stays in hospital and at home. This included maintaining a rota of family members on the Ward, not just at home. This was because of the expectations and encouragement by staff that we should be part of the caring team in hospital. This was greatly valued by Guy and by us, rather than having restricted visiting times – but it did add to travel and time commitments.
This involved several thousand of pounds expenditure in travel and accommodation expenses whilst attending hospitals & clinics in Birmingham and Leeds from our home in York, as well as coping with a reduction in our salary incomes. It included the disruption of family life, dependency on other family members and neighbours, additional stress at work when we were able to be there and a strain on our relationship. We later became more aware of the lack of attention we had given to our 21 year old daughter, and to the trauma she was experiencing as a close sibling.
Whilst we do not for one moment regret our commitment to our son's care and the financial burden thereof, it was an extremely stressful and emotional time and its effect has changed us and our lives.
The view of the Bone Cancer Research Trust (BCRT).
Today, I represent the BCRT whose core membership includes not only patients and former patients, but also their parents, families and friends. Bereaved families also count as a significant group whose motivation is to honour their loved ones by striving to improve the outcomes for those undergoing treatment.
The Trust recognises that within the PBC categories, OS has the largest number of

patients. In the UK, approximately 400 new cases of PBC will be diagnosed in any one year; some 90% of these new cases will be in children and, particularly, in teenagers. OS accounts for some 50% of all new PBC cases, and is now the second largest cancer group in teenagers for any cancer. OS peaks at between the ages of 15 and 20 years.

We recognise that OS is a rare cancer, and is regarded as an Ultra Orphan Disease. With there being less than 200 new cases of OS in any year, it follows that there will never be a large number of patients to undergo clinical trials. Perhaps because of this, there has been extremely little research into the causes and treatment of OS, which could account for the significant fact that there has been no change in the survival rate for OS patients over the last 20 years. This contrast very badly with the improvement in survival rate for virtually all other childhood, teenage and young adult cancers. Consequently, the failure to improve the OS survival rate flies in the face of the Cancer Reform Strategy.

On 7th. January 2009, the British Journal of Cancer (Cancer Research UK) published a paper lead by Dr. Richard McNally of the Institute of Health and Society, University of Newcastle, which concluded that survival for childhood bone cancer is lower in the UK than any other Western European country. It stated that, although 5-year survival from childhood cancers in the UK had now reached 75% (with some at over 90%), Osteosarcoma had not improved in the last 20 years, remaining at about 60%. On closer evaluation of this paper, the statistical information covered the age range 0 – 14 years – i.e. short of the peak age for OS of 15 – 20 years within the total age band of 0 – 24 years; when this is factored in, the overall 5-year survival rate for 0 – 24 years drops to around 55%. This last figure compared with 65% for 0 – 14 years in other Western European countries.

Figures from North America show an improvement on the Western European figures at a 5-year survival rate for OS of 70%. The Trust has been made aware that clinical trials in North America using Mifamurtide indicate an improvement to 78% - a 42% improvement in the chance of survival beyond 5-years on the UK figures or, in terms of lives prolonged, some 46 more children/teenagers/young adults very year – or in more straightforward terms, by using Mifamurtide the numbers improve from a 5-year survival of 5 or 6 in 10, to 7 or 8 in 10.

The Trust has reviewed the Paul Meyers *et al* Trial (Children's Oncology Group 1993 – 97) in North America covering 178 hospitals and are impressed by the results of such a large survey. If the introduction of Mifamurtide is deferred in the UK, so that a further clinical trial can take place in the UK, the numbers available in the UK are so small compared with other cancer groups, and also would not be reported until 2020. Such a delay would deprive some 500 young people of the chance of absolute survival.

The Trust feels that the studies to date indicate that:

- there is no uncertainty about the survival benefits of Mifamurtide;
- that there is little variation of advanced side effects between metastatic and non-metastatic cases, and therefore should be available to all patients;
- that there are no life-threatening risks associated with Mifamurtide;
- and that its use significantly reduces the risk of death from OS without increasing the risk of morbidity or mortality.

What patients and carers want.

The only thing that matters is survival.

This mean long term survival comparable with the life expectancy of a child/teenager/young adult without OS.

Limited period survival is not acceptable.
So the notion that OS can be measured in terms of "disease free survival" is rejected – it must be "absolute survival".
Quality of life during treatment and beyond is important but for the vast majority of patients and carers, the requirement to slightly extend treatment time if Mifamurtide were to be used would be regarded as nothing compared with the benefits of improved survival expectancy. In fact, the first trial in the last 20 years shows real therapeutic benefits.
I have not doubt that my son, given the opportunity, would have readily agreed to try Mifamurtide. He found it very difficult to believe that little or no research had been done on OS, or was planned to be done, especially given the young ages of the overwhelming number of OS patients.
That lack of research was the great motivator to his establishing his own Research Fund.

Cost benefits.
The Trust acknowledges that cost benefit analyses are an important factor. Because OS is a young person's disease, the loss of the potential of that young person has to be factored in.
An increase in their life expectancy will achieve a national benefit to the UK as a whole.
Throughout their childhood, teenage and young adult life, the UK taxpayer through Government welfare and education schemes will have been funded. The expectation for everyone beyond the age of 24 is that they will be, through employment, an income tax contributor for some 40+ years. At a time when the post 65-years population is growing and requires substantial underpinning funding by the working population, this is extremely important.
Given this, the costs of providing Mifamurtide to a group of young people so that they can survive to play a full role of employment income generation, should not be ignored.

Conclusion.
The Trust asks NICE to consider positively the advice of the Committee for Medical Products for Human Use (European Medicines Agency) of 18th. December 2008, in that there is a favourable benefit to risk balance for this product, and then to recommend the use and funding of this product by the NHS.
Thank you.