



Ipilimumab for previously treated advanced (unresectable or metastatic) malignant melanoma: *Factor50* response to NICE Appraisal Consultation Document

About Factor50:

Factor50 is a patient support group working with The Christie Hospital in Manchester. We campaign for greater awareness of the dangers of malignant melanoma, and also raise money to conduct research into the disease. We work closely with patients, offering support and guidance to those coping with this aggressive and destructive cancer. Factor 50 was formed following the realisation that there were very limited options for advanced melanoma patients other than standard treatments, which were clearly inadequate.

Our response:

We would firstly like to thank NICE for giving us the opportunity to respond to its Appraisal Consultation Document (ACD) on Ipilimumab as a treatment for advanced (unresectable or metastatic) melanoma. In particular we thank the Committee for its recognition that Ipilimumab “may represent a potentially valuable new therapy and that the mechanism of action was novel”.

However, it is for this very reason that we are particularly disappointed by the Appraisal Committee’s decision, which we believe is misguided and which we would urge you to reconsider and overturn. As a patient support group we are acutely aware of the impact that this negative decision will have on the lives of the patients and families we help. We feel this decision is particularly short-sighted given the fact that the sharp rise in the incidence of melanoma is set to continue in the coming years and that the Cancer Drugs Fund - currently the only hope for advanced melanoma patients - is set to end in just two years. We are gravely concerned for the future of melanoma patients and also for the doctors who have to treat these patients. They are unable to offer their patients anything other than standard treatments, such treatments that are widely acknowledged as ineffective. We feel it is an unfair and unnecessary burden on our doctors.

Are the provisional recommendations sound and a suitable basis for guidance to the NHS?

Factor50 does **not** believe that the provisional recommendations are either sound or a suitable basis for guidance to the NHS.

NICE has acknowledged that this drug represents a real step change in the treatment of patients with advanced melanoma since it is the first licensed treatment in over 30 years to actually extend life expectancy in this area. The ACD recognised that Ipilimumab addresses a “significant unmet need for effective therapies in this patient population”, as existing NHS treatments for advanced melanoma are outdated and ineffective. In view of the recognition of the innovation in cancer treatment that Ipilimumab offers, Factor50 feels the Committee’s decision is extremely disappointing and comes as a devastating blow to patients in England and Wales.

Costs and benefits

Factor50 accepts that Ipilimumab is a high-cost treatment. However, we strongly believe that it is a high *value* treatment that offers genuine survival benefits to patients and hope in a disease area which has seen no such breakthrough for over 30 years.

Factor50 acknowledges that unfortunately Ipilimumab cannot be said to provide a cure for patients with advanced melanoma. However, where patients can currently expect to survive for between 6 and 9 months after being diagnosed with this aggressive disease, trials showed that 46% of patients were alive after a year of treatment with Ipilimumab. Furthermore, as a result of our work with patients, at Factor50 we know there are many examples of patients that have achieved significantly longer-term survival benefits through treatment with the drug. We would urge the Committee to remember the evidence it heard directly in September from Richard Jackson, a melanoma patient who, over three years on from completing his course of treatment with Ipilimumab, has been able to maintain a normal family and working life. By his own admission Richard suffered severe side effects but has stated that for the glimmer of hope that this drug gave to him, he was ready to accept any risks and indeed, patients who do not receive any treatment are likely to suffer serious effects in any event.

We welcome the Committee’s assessment that Ipilimumab is a life-extending, end-of-life treatment and that the trial evidence presented for consideration was robust. We call on the Committee to recognise just how priceless any significant extension of life is for patients

suffering with this aggressive disease. At Factor50, we regularly see the devastating impact that a diagnosis of advanced melanoma has, particular when - as is sadly so often the case - it is young patients with young families who receive that diagnosis. The extra months and as Richard's case showed, years, that can be provided by a treatment such as Ipilimumab not only gives patients the opportunity to arrange their affairs but, crucially, can mean the difference between seeing their children's first day at school or first birthday. As Joanne, a melanoma patient aged just 30, told us recently: "I need to live. I *have* to live for my children. I just want a few more years so that my boys will remember me."

The average age of diagnosis for this disease is just 50 and treatments like Ipilimumab can offer a greater amount of time to these patients' family members to come to terms with a diagnosis. This not only benefits those individuals but also society as a whole. NICE decisions on treatments for more long-term degenerative illnesses have take account of the cost to society of the emotional and economic toll taken on family members acting as carers for patients suffering with these diseases. Yet in its decision on Ipilimumab we believe the Committee has failed to take into account the impacts felt across the same areas by individuals struggling to deal with the quick and unexpected loss of a loved one as a result of a disease such as melanoma.

Innovation

We are also concerned that NICE has failed to appreciate the long-term implications of refusing to recommend Ipilimumab at a time when the incidence of melanoma in the UK is rising alarmingly, a trend that is expected to continue and even accelerate over the next 20 years.

A study published this month in the British Journal of Cancer, *Cancer in the United Kingdom: projections to the year 2030*, claims that though overall cancer rates are projected to be stable over the next 20 years, melanoma incidence is set to rise by 52% in both men and women by 2030. The study projects the disease will become the fourth most common cancer in men and the fifth most common in women over the period.

In the light of this information, the Chief Executive of Cancer Research UK, Harpal Kumar, has stated: "As we develop ever more sophisticated ways to detect and treat cancer successfully, health planners must deploy resources more effectively to enable all patients to benefit from the latest developments and cutting edge treatments."

Factor50 believes that the development of Ipilimumab represents an opportunity for NICE and the NHS to make these words a reality. Factor50 is committed to improving awareness of the dangers of melanoma and improving prevention and early diagnosis but it is equally vital to take action on treatment now if we are to improve patient outcomes from a disease whose burden on the NHS will only rise in the coming years. The arrival of Ipilimumab is the first time we have been able to offer real hope to patients. Furthermore, through its novel process of 'immunopotentialisation', Ipilimumab could provide a basis for the NHS to use future new effective treatments alone or in combination.

Are there any aspects of the recommendations that need particular consideration to ensure NICE avoids unlawful discrimination against any age group of people on the grounds of gender, race, disability, age, sexual orientation, religion or belief?

Factor50 believes that the Committee has failed to fully acknowledge in its report the disproportionate incidence of advanced melanoma in patients aged between 15 and 34 years, a demographic in which it is the second most common cancer in the UK. We believe that a more accurate reflection of the value this drug could bring to the NHS would be achieved by giving a more appropriate weight to this factor within NICE's decision-making process.

Factor50 notes with interest the words of NICE Chief Executive, Sir Andrew Dillon, who said in announcing NICE's decision in 2010 not to recommend the bone cancer drug mifamurtide: "We understand a diagnosis of cancer is very distressing, and especially so when children and young adults are affected. With this in mind, we are disappointed that the evidence for mifamurtide is not stronger. It is important to remember, though, that other, effective treatments are available in the NHS for treating this condition."

This may be true when assessing bone cancer treatment, but unfortunately, the same cannot be said in this case. There simply are no licensed effective treatments in advanced melanoma, a disease which also affects young adults and young people who should have their whole lives ahead of them, and yet NICE has come to the same decision.

Each death from advanced melanoma results in an average of 22 years of life lost. It is a disease which often affects people in the prime of their life. In Ipilimumab, patients have been offered for the first time the hope and expectation of a minimum of several additional extra months spent with their loved ones. Factor50 urge NICE to think again before removing that hope.

In closing, we believe it would be of great benefit if NICE and the manufacturer were able to work together to ensure that the future of melanoma treatment does not grind to a halt.