Access to newly licensed medicines

Melanoma Action Support Scotland

We welcome the opportunity to provide the experience of patients suffering from advanced malignant melanoma to the committee.

MASScot is a small charity run by patients for patients. We have no employees. We provide support and prevention and skin awareness education. We campaign on all skin cancer issues.

We thank you for taking on the challenging task of examining the issues regarding the approval process for newly licensed medicines and the system of Individual Patient Treatment Requests (IPTRs) in Scotland. It causes us great concern. We appreciate that the workload will be considerable and will therefore use bullet points as much as possible. We will be happy to expand as required.

Licensed products for treatment of advanced melanoma and our limited experience of them:

Dacarbazine (DTIC) licensed 1970s effective in around 10% of patients. Now generic and cheap, given by intravenous infusion in three weekly pulses. DTIC has worked first time around for some at first treatment but not for further tumours. We know three long-term survivors, 1 x 8yrs and 2 x 30+yrs. Side effects are variable but not intolerable in our experience. We appreciate that we are very unusual in having this long-term result.

Ipilimumab (Yervoy) licensed 13th July 2011, intravenous infusion three weekly pulses x four. Costs around £45K per course – price we are told offered to SMC. Ipilimumab is used after DTIC or a similar trial product, as part of a trial in all but one case. Side effects can be severe requiring admission. One lady, who required treatment at a time when there was no trial, opted to pay £90k (full price) for the product. She was very active until two weeks ago. She is now dying of brain mets.

Vemurafenib (Zelboraf) licensed 17th February 2012 oral taken twice a day continuously. Costs around £45k per year. Vemurafenib has seemed like a miracle in the fast return to health it provided. This only lasted 6-18 months after which there was fast deterioration. It has only been given as part of a trial. Fewer than 50% of our members tested had the B-RAF V600 mutation which this drug specifically treats.

Only DTIC is available to prescribe in Scotland. IPTRs have not been allowed as far as we are aware for any of the new drugs.

In England 231 people have been treated with ipilimumab of the 18,500 treatments via the cancer drugs fund (www.mediacentre.dh.gov.uk/2912/08/cancersdrugsfund/usage). We pay the
same NI contributions. We would rather pay for prescriptions – affordable, than sell our home to pay for life preserving therapy. Health is a devolved power; this does not bode well for independence.

We do not want a cancer drugs fund; treatment is required for other life limiting diseases too.

Our concerns are also for those who treat us. We have conscientious, caring doctors. They must suffer frustration knowing there are products available to extend and improve life we are concerned that as vacancies arise in oncology departments, they will not attract the calibre of staff that we benefit from currently. Training will suffer; research opportunities will dry up and with it the savings that the NHS makes on the free trial drugs, comparator drugs, equipment and staff.

Pharmaceutical companies will not invest in studies or infrastructure in Scotland if they are not to have a market here. Cancer research units offer hope but we suggest that they are often charity-funded units within the NHS, where the patient is a guinea pig and the product is research from which we all gain.

Melanoma stats from isd Scotland 2010.

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<th>Males</th>
<th>Females</th>
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<tr>
<td>Rank – incidence</td>
<td>7</td>
<td>5</td>
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<tr>
<td>Rank – mortality</td>
<td>15</td>
<td>18</td>
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<tr>
<td>Percentage frequency of all cancers</td>
<td>3.7%</td>
<td>4.0%</td>
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<tr>
<td>Number of new cases diagnosed in 2010</td>
<td>524</td>
<td>617</td>
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<tr>
<td>Number of deaths recorded in 2010</td>
<td>114</td>
<td>81</td>
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<tr>
<td>Change in incidence from 2000 to 2010</td>
<td>66.2%</td>
<td>59.8%</td>
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<tr>
<td>Change in mortality from 2000 to 2010</td>
<td>40.4%</td>
<td>28.2%</td>
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<td>1 year relative survival for patients diagnosed between 2003 and 2007</td>
<td>96.0%</td>
<td>98.9%</td>
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<td>5 year relative survival for patients diagnosed between 2003 and 2007</td>
<td>87.8%</td>
<td>95.5%</td>
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Melanoma is the most common cancer in Scotland in the 15 to 34 age range and 5 people in this age range died of it 2010. “The unusually young age distribution for an adult cancer emphasises the importance of its prevention and early diagnosis to avert the potential loss of many years of life. On average 20 years of life are lost for each melanoma death” Prof B Diffey.

Of the 195 people who died of melanoma in 2010, 5 were under 34 years of age, 23 were under 50 and 97 were under 70. By age 70 people often have other medical problems which would prevent them from having these
therapies. We are therefore pleading for treatment for around 125 people a year.

Please consider the total cost, not just the cost of drugs. Tax income is lost and the costs of supporting a family where a parent has died can be considerable. One-parent families are reportedly less likely to be successful. How much worse is suffering when money might have changed the outcome? Parents of melanoma patients may have expected that they would be cared for by their child, the cost to the State in providing this care is worth taking into account.

No money is being spent on melanoma prevention or early detection in Scotland despite the increases and the evidence that early detection usually means treatment by minor surgery alone.

I append some individual patient stories; they demonstrate that delay costs lives.

**Patient 1**

I had a small birthmark mole on my shoulder which a few years ago started to change in shape and grew into a lump. I attended my GP on a few occasions to have him look at it and he said it was a fatty lump. Eventually it became so large he referred me to dermatology at my local general hospital.

The lump was cut out and the doctor said you will have a bit of a scar but you will not die from it. They would send it to pathology just to be sure. 10 days later I received a call to say I had skin cancer—melanoma—and I had an appointment to see a plastic surgeon.

My world felt as if it had fallen apart. No support and no counselling. Just left on my own with my wife and two boys to deal with this news.

I had a skin graft and was told of the high risks of the skin cancer returning. During a six monthly scan, while checking the size of some nodes on my lung, they found the cancer had spread to my liver and it was inoperable.

The message was cold, I was left to fill in the gaps and unbelievably we left the consultant’s room with that stark message and went and sat in the car park not believing what we had just heard. Our world had changed in 2 minutes and it would never be the same again.

Again no support, counselling or treatment but there might be trials I could get a place on.

Telling our two boys that the cancer had returned and was inoperable was just heart breaking. They were looking for answers that we just couldn’t provide. The prognosis was that I would live for maybe a year maybe a bit longer and
maybe a bit less. This was in March 2011. You can’t get your head around that news.

We decided as a family to make a bucket list and do as much as we could while we could. Over the next year we did some great stuff and friends and family were wonderful in their support. We lived as normally as possible for our son’s sake, I carried on working.

Then out of the blue I was asked to attend the clinic, where I was told there was a trial I might be eligible for. In preparation for that I had another scan, the first in a year. To my surprise I was told that the tumour in my liver had shrunk – this was thought to be due to the previous trial I had been on – but had had to abandon as it was upsetting my diabetes too much. This news was such a relief to us and made us realise that medicine is not an exact science.

I was then offered a trial of ipilimumab and have just had the final infusion of this trial. I have not had a post treatment scan as yet so do not know what it has done if anything but I feel ok and have been able to carry on working – as I have done throughout the journey. This treatment is not allowed to patients on cost grounds so a trial was my only hope.

Without this trial, I would have had no hope of survival. I am 48 years old and have two teenage sons at university. They still need their Dad and I want to be here to see them mature.

When told there is nothing that can be done for you and you have maybe a year to live, it is totally devastating When you then discover from the internet that there is not one but two drugs for advanced melanoma in use and available in England and other parts of the world, but that the Scottish Government or the SMC have said it was too expensive it makes you feel angry. As a tax and NI payer I don’t remember any time being told that there was a limit to treatment costs on the NHS. Had we been told we would have had a chance to make provision in case. If we cannot afford life saving drugs, why do we have free prescriptions? Seems worth asking?

**Patient 2**

The Treatment of Malignant Melanoma has remained virtually unchanged for 30 years but the arrival of the Immuno-drug Ipilimumab, which encourages the body’s own immune system to fight the cancer, has radically altered the approach taken for those suffering advanced skin cancers like me, a 64 year old from Lanarkshire, I have advanced stage of melanoma known as metastatic with tumours now reaching both my lungs, back and arms.
I was told the drug was available to me if I had the money to pay for it as I didn’t qualify under Individual Patient Treatment Request and no trials were running at that time on the drug. If you try the few drugs that are available with no effect then the only option is to try to purchase the drug at £90,000 this will give you a course of 4 cycles over 12 weeks.

With great difficulty, financial hardship, and some borrowing from family and friends I was able to receive this drug, with the course just finished on 4th May. I will have a CT scan in due course to get an outcome from my treatment.

For me the choice was easy, I was warned about the possibility of the severe side-effects but none could be worse than dying.

First post treatment scan showed improvement but a subsequent scan showed one remaining tumour had started to grow again. She then developed brain mets – September- and is now dying. Until a short time ago this fit lively lady had been attending Zumba classes.

Patient 3

I was extremely fortunate (if fortunate can be used in conjunction with the dire situation many of us find ourselves in with melanoma) to successfully meet the criteria to gain entry onto the Vemurafenib Trial through the Beatson Cancer Research Unit.

When I was originally diagnosed as having Metastatic Melanoma back in January, I was battling with daily pain and discomfort of a massively swollen abdomen which had originally been treated as some form of IBS by my GP practice for the previous few weeks as process of elimination. I was struggling to take on board fluids or food due to my internal organs being crushed by what turned out to be ascites fluid in the abdomen so weight loss was becoming more and more apparent, tiredness was becoming a problem and my complexion was becoming more grey as the days passed. At this stage I was using paracetamol and ibuprofen for pain relief in addition to the mixture of IBS treatments. When the diagnosis was eventually confirmed and I had my first oncology appointment, I was put on Tramadol for the pain relief, but as much as this fought the pain better, I found myself in a world where I was unable to function, motor skills were affected, I was unable to hold conversations and would more often than not just fall asleep until the medication wore off. My life was revolving around sleeping in my bed and then during the day moving to the sofa in a Tramadol based stupor before moving back to bed at night having not eaten or drank much at all during the day. After travelling to Glasgow to sign the consent forms, I had an emergency admission to the western general hospital in Edinburgh where they investigated and tried many options before finally inserting a drain and
having 5.5 litres of fluid removed from my abdomen giving me instant relief and the ability to eat. Unfortunately, this was to be short lived as when I was given IV fluids, it seemed to go straight back into the abdominal cavity where it had originally been drained. A second drain was inserted and removed another 5 litres. My oncologist informed me that I had two options available which were the standard dicharbozine chemotherapy which was the standard form of chemotherapy for melanoma or that there was potentially a drug trial that I may qualify for which had seen some great results in improving quality of life whilst also extending life by several months; possibly even years! Luckily I qualified and successfully made it onto the trial a week before the trial closed to new candidates. Within days, even though I was still hospitalised, I was beginning to feel better than I had in weeks. I couldn't put my finger on exactly what way I was feeling better but I just felt that something was changing. I still had to have fluid drained from my abdomen as this was still a side effect of where/ how melanoma had affected me. Within weeks, I was back on my feet and back on the golf course. Something I thought I'd never manage again after having been such an avid sportsperson between cycling, football, gym, running, golf etc. Since then, in between regular visits and stays as an in-patient at the Beatson Unit for secondary symptoms I have been to Barcelona for a city trip, I have been to both the Scottish and English FA cup finals and a Manchester United v Everton Premiership football match at Old Trafford. I have also been to Deep Sea World and Edinburgh Zoo in a bid to create lasting memories for myself and my family. I have also planned to be at T in the Park this year and also had a flying lesson booked for my birthday earlier this week but both were cancelled due to the weather but I will make every effort to reschedule or replace each of these events with something the same or similar in the near future. I have also undertaken a fundraiser to raise awareness and funds for the Beatson West of Scotland Cancer Centre as a way of saying thank you for all the care and support they have given me over the last 5 months under their care. This took form of 4 rounds of golf at Dalmahoy Country Club in Edinburgh on the one day. Having tee'd off at 5:45am; we finally putted out on the 18th green for the last time at 22:30 at night in near darkness. There is no way I could even have completed a 9 hole putting green before being on the Vemurafenib drug never mind completing 17 hours of nonstop golf on the day. My scan results, as determined by my agreement to be on the trial have showed an initial 20% reduction in tumour sizes followed by the last set of results which showed that everything had remained around the same which indicates that the melanoma in under control, which for something that is such an aggressive disease is still a good sign. All in all, since using the Vemurafenib drug, I have been able to squeeze in so many lifetime memories with friends and family that I would never have dreamed of being able to make before using the drug. To me and all who know me ...... these memories are priceless!!
Sponsorship for the golf day raised £16,000.00 for the Beatson.

Patient 3 died at the end of August.

Patient 4

The undernoted are excerpts from emails showing the dates when each new event occurred.

8\textsuperscript{th} Feb 11  I’ve been diagnosed with melanoma, 4.1mm deep in my lower hip. I’ve been told I’m category 2C.  
16\textsuperscript{th} February I had a wide excision.

3/3/11 Offered Avastin M trial. Also got great news, the results from my latest leg surgery came back all clear.

12 April11 Clear CT scan results at the Beatson today. I have to go back for all sorts of other tests next Tuesday for the drug trial. I’m like a lab rat! (Avastin M trial)

26 April 11  I got a total clean bill of health today at the Beatson and have been picked for the drug trial. I start my Avastin-M treatment on May 6

13 May 11  I am currently a bit worked up. My lymph node on my dodgy leg has swollen up/gone hard. Seen by surgeon and put on waiting list for surgery. 

(The result of this was to be removed from the +ve arm of the trial as no longer eligible, but continue to be followed up without any therapy). 

8\textsuperscript{th} July  I’m now a stage 3b. They removed eight lymph nodes and two had melanoma. The casing of one of them had burst, allowing cancer to spread into the local area. There are two options – wait and see if anything comes up in terms of lumps and moles or radiotherapy. Dr said there’s little evidence that this works with melanoma so is only offering it to me, not insisting that I take it. (Surgeon advised against radiotherapy – so a third opinion sought.

29\textsuperscript{th} July I got the second opinion from Prof X from London and have been advised not to have radiotherapy.

\textbf{Wedding 12\textsuperscript{th} August}. Limping a bit but able to dance my first dance with my husband.

12\textsuperscript{th} September  I’ve found a mole on my leg this morning. It’s a mole they’ve warned me about before and I’ve spotted a slight change in it.

23 Jan The two lumps removed from my bad leg were indeed melanoma but the mole on my left was OK. However Mr X spotted a new lump on my bad leg that will require more surgery in two weeks or so.
15th Feb 12  I had my op on Monday and they removed five nodules, we expected it just to be two. Limping about like I’ve been shot, but otherwise fine, it’s not bleeding. I also got my CT scan results on Tuesday. They’ve found a tiny suspect area on my liver and two on my lung. We’re going back in today to sign up for a trial of Vemurafenib, as long as the tissue samples from the Royal show that I’m compatible. The area is too small to do a reliable biopsy on so at least they’ve caught it early if it is melanoma.

1st March – The results of the gene tests show I do not have the mutation. Back to the Beatson again tomorrow and hope for ipi. I hope going to that dinner helps! (Dinner in Parliament 14th Sept 2011)

Patient 4 was offered a trial of two unlicensed products to be given together and had opted for that in the hope of surviving and being fit enough for the ipilimumab trial in the summer.

She died on the 5th August one week before her first wedding anniversary aged 36. She was an only child. She had been super-fit and in a very demanding well-paid post prior to noticing a change in a mole.

Had we had drugs available she would have been given ipilimumab in February immediately after the first drug given had failed.

She was a lovely very much loved girl, a new wife and an only child.

Investment by the State would have included schooling, university education, health care including all immunisations. She was a taxpayer in a very good full time post with unsocial hours so her tax bill would have been considerable and would have cared for her parents in their old age. What is the financial cost to the State of that?

We do not blame the SMC but we feel the remit of the committee requires scrutiny and a faster method of negotiating with drug companies. They will try to get the best price but they will gain nothing if the drug is not used. I cannot believe they will not be willing to reach an amicable settlement.

Melanoma Action Support Scotland
11 September 2012.