MDS UK
Supporting Patients with Bone Marrow Failure
www.mdspatientsupport.org.uk

Patrons
[redacted]
Chairman
[redacted]
Chief Executive
[redacted]
Tel [redacted]

NICE
Attn of Committee C, Frances Sutcliffe, Meindert Boyson, Nicole Fisher
Level 1A, City Tower
Piccadilly Plaza
Manchester M1 4BT

04/06/14

Re. Lenalidomide for the treatment of myelodysplastic syndromes associated with a deletion 5q cytogenic abnormality in people with red blood cell transfusion dependence [ID480]

Dear Committee C, Frances, Meindert, Nicole,

Please see below our response to the latest ACD for this appraisal. As we are addressing a variety of issues and sending further documents with our response, we have summarised the different points below.

A. ACD Comments

1. Survival Benefit issue (ACD 4.3, 4.5, 4.9, 4.10, 4.12, 4.15)
2. PAS administration issue (ACD 4.13)
3. Sub-group of patients for lenalidomide
4. Iron chelation treatment (ACD 3.37)

B. Additional comments and documents

1. Additional patient statements – Appendix 1
2. Northern Ireland Assembly - MP David McIlveen
3. Small population issue
4. Scotland – SMC - Regional discrepancy in UK

C. Process issue

1. Insufficient involvement with non-pharma consultees
2. NICE Patient Expert Report 2012

A. 1. Survival Benefit Issue

We were very surprised to read the NICE press release regarding the draft guidance:
*"Commenting on the draft guidance, **Sir Andrew Dillon, NICE Chief Executive, said:** "The committee heard from clinical experts that lenalidomide is an effective therapy. However, the data provided by the company showed uncertainty about whether lenalidomide actually extended lives."*

Firstly - as mentioned in our original submission, and in the azacitidine STA, for such MDS patients , the quality of life gained through transfusion independence is of equal importance to survival – if not more important for some patients, due to their older age.
As per our main submission and patient statements, lenalidomide is a drug that, through transfusion independence, drastically improves the quality of life of this group of del5q patients, who would otherwise be destined for supportive care only, and therefore be extremely limited in their daily activities.
Therefore – placing the emphasis on survival, and not approving the drug because it may not increase survival, is not a valid argument in this group of patients.

Secondly, the French randomised trial has shown a survival benefit for responders to the treatment.
The trial shows a background transformation rate to AML of about 25% and less transformation and better survival for cytogenetic responders and those who become transfusion independent on lenalidomide.

The topic of survival was previously discussed in the FAD, where the Committee seemed to agree on a survival benefit due to transfusion independence:

FAD - http://www.nice.org.uk/nicemedia/live/13555/65538/65538.pdf

3.42 *The manufacturer submitted the results of a systematic literature review to support its rationale that transfusion dependence is a prognostic factor for overall survival and rate of progression to AML. Sixteen of the 17 studies (mainly retrospective case series or registry populations, also one study describing the results of a randomised controlled trial and one post-hoc analysis of randomised controlled trial data – 5000 patients in total) meeting the inclusion criteria reported statistically significant associations between transfusion status and overall survival. This association was explained by:*

- *transfusion dependence and anaemia leading to increased non-leukaemic death (particularly cardiac death)*
- *transfusion dependence and anaemia leading to increased risk of AML and leukaemic death*
- *transfusion independence after dependency at baseline improving overall survival because of reduced complications from chronic anaemia.*

The manufacturer also cited literature that examined the relationship between AML and both transfusion status and erythroid response, arguing that lenalidomide triggers programmed cell death in the deletion (5q) clone, and that the MDS-004 trial showed significant reductions in progression to AML for patients whose condition responded to lenalidomide

3.44 The ERG considered the additional information submitted by the manufacturer. It agreed that this information, combined with the results of the MDS-004 trial, suggested that it was reasonable to assume a 2-step relationship, first between lenalidomide response and transfusion independence, and then transfusion independence and overall survival.

3.46 and 4.6 of the FAD – "*Overall the Committee concluded that it was reasonable to assume a relationship between lenalidomide and transfusion independence, and transfusion independence and overall survival, although there remained uncertainty about the exact strength of the relationship and whether the relationship diminished over time.*"

We read the many sources of evidence in Table 1 in Evidence 03, which seem to have been overlooked by the committee? http://www.nice.org.uk/nicemedia/live/13555/67708/67708.pdf

We also noted that NICE recognises and values the benefit of transfusion independence on QOL and potentially survival – in the following Guidance from April 2014:

National Institute for Health and Care Excellence- Appraisal consultation document – Erythropoiesis-stimulating agents (epoetin and darbepoetin) for treating anaemia in people having cancer treatment (including review of TA142) - Issue date: April 2014

Current practice

4.3.1Clinical need of patients, including the availability of alternative treatments
The Committee heard from the patient expert that symptomatic anaemia is associated with fatigue and the inability to perform everyday tasks. It also heard from the clinical specialists that standard treatment for anaemia in people having cancer treatment includes blood transfusions, which could worsen quality of life and potentially shorten survival.
The Committee concluded that people with anaemia who have cancer treatment need treatment options that potentially reduce the need for a blood transfusion and which improve quality of life.

4.3.3 Estimate of the size of the clinical effectiveness including strength of supporting evidence
The Committee concluded that ESAs were effective in increasing haemoglobin concentrations, improving haematological responses and reducing the need for blood transfusions.

4.3.4 Based on the available evidence, the Committee concluded that it could not assume that ESA treatment either prolonged or shortened survival compared with treatments that did not include ESAs.

4.3.5 The Committee concluded that the available evidence suggests that ESA treatment improves health-related quality of life compared with treatment without ESAs.

4.3.9 Uncertainties around and plausibility of assumptions and inputs in the economic model. The Committee was concerned that the Assessment Group did not include disutilities associated with adverse reactions in the QALY calculation given that most adverse reactions occurred more frequently in the ESA arms. However, it recognised that there would be minimal effect on the incremental cost effectiveness ratios (ICERs) given that the adverse reactions in the study were rare.

4.3.9 The Committee concluded that the QALY gain with ESAs may have been underestimated given that the potential benefits of ESAs associated with avoiding blood transfusions and reducing the need for hospital visits were not captured in the QALY calculation.

Reference to the fact that the drug may progress transformation to AML should no longer be relevant. The EMA having licensed the drug for del 5q as a single abnormality should be evidence enough that this topic is no longer an issue.

A further paper, based on a subset of the MDS 004 trial also highlights the following results amongst the isolated del 5q patients, demonstrating longer overall survival in this group of patients:

Results: Rates of red blood cell-transfusion independence (RBCTI) ≥182 days were higher in the lenalidomide 10 mg (57.4%; P < 0.0001) and 5 mg (37.2%;P = 0.0001) groups versus placebo (2.2%). Cytogenetic response rates (major + minor responses) were 56.8% (P < 0.0001), 23.1% (P = 0.0299), and 0%, respectively. Two-year cumulative risk of acute myeloid leukaemia progression was 12.6%, 17.4%, and 16.7% in the lenalidomide 10 mg, 5 mg, and placebo groups, respectively. In a 6-month landmark analysis, overall survival was longer in lenalidomide-treated patients with RBC-TI ≥182 days

Progression to AML by randomized treatment group is presented in Figure 2A. The estimated 2-year cumulative risk of progression to AML was 12.6% (95%CI: 5.4–27.7), 17.4% (95%CI: 8.7–33.3), and 16.7% (95%CI: 8.3–32.0) in the lenalidomide 10 mg, lenalidomide 5 mg, and placebo groups, respectively.

The estimated 4-year cumulative risk of progression to AML was 30.6% (95%CI: 18.1–48.8), 35.4% (95%CI: 21.4–54.6), and 43.3% (95%CI: 27.6–63.1) in the lenalidomide 10 mg, lenalidomide 5 mg, and placebo groups, respectively.

Overall survival

Median OS was 4.0 years (95%CI: 2.5–NR), 3.5 years (95%CI: 1.7–4.8), and 2.9 years (95%CI: 2.2–4.2) in the lenalidomide 10 mg, lenalidomide 5 mg, and placebo groups, respectively (Figure 3A). Of the 7 patients randomized to placebo who did not crossover to lenalidomide 5 mg, 6 (85.7%) died. In a 6-month landmark analysis, median OS was significantly longer in patients who achieved RBC-TI ≥182 days (5.7 years; 95%CI: 3.2–NR) compared with non-responders (2.7 years; 95%CI: 1.5–4.8; log-rank test P = 0.0072.

Median OS was similar in patients who achieved a major or minor cytogenetic response (4.3 years; 95%CI: 2.3–NR) compared with non-responders (3.7 years; 95%CI: 2.0–4.7; logrank test P = 0.4073; Figure 3C).

In a study of untreated RBC transfusion-dependent patients with IPSS-defined Low- or Int-1-risk MDS and del(5q), median OS was approximately 3.7 years; 82% of the patients had isolated del(5q) (5). In the same study, RBC transfusion dependency was associated with shorter OS (HR 2.260; P = 0.001) in a Cox proportional hazards model of MDS patients with isolated del(5q). In the current analysis, median OS was significantly longer in patients who achieved RBC-TI ≥182 days compared with non-responders. Although OS by cytogenetic response was not significantly different between responders and non-responders, this lack of significance may have been due to the limited number of events and patients in this subset analysis.

Extracts from "Outcomes in RBC transfusion-dependent patients with Low-/Intermediate-1-risk myelodysplastic syndromes with isolated deletion 5q treated with lenalidomide: a subset analysis from the MDS-004 study."

European Journal of Haematology, Article first published online: 9 JUN 2014, DOI: 10.1111/ejh.12380

2. PAS Scheme ACD 4.13

4.13 The Committee questioned whether all the savings would be realised in practice for every patient because of the administration process involved. The Committee concluded that this added to the uncertainty of the cost reductions associated with the patient access scheme

Based on the official remit of PASLU, they are responsible for assessing the feasibility and administration burden of all patient access schemes for the NHS, not the committees, nor the ERG.
We believe this topic should not have been raised.
The PAS for lenalidomide was recommended as feasible, given the level of administration it requires.

As per NICE documentation, PASLU is described as issuing robust advice:
"1.6 The process for advising whether implementing a patient access scheme is feasible in the NHS in England and Wales has been specifically designed to provide robust advice to the Department of Health. This advice will inform ministerial decisions on whether NICE should consider a patient access scheme as part of its technology appraisals programme.

1.9 Most of the evidence that the PASLU needs to advise on whether a scheme is feasible is submitted by the manufacturer or sponsor of the technology. A review of the submitted information is produced by the PASLU, and if necessary the PASLU asks for further clarification (see 2.4.2). Further input is provided by the members of the independent Expert Panel and co-optees to the panel (see table 1). The PASLU asks for advice from within or outside NICE when necessary.

Schemes should be clinically robust, clinically plausible, appropriate and monitorable (e.g. if it is a responder scheme, there must be a relatively straightforward way to measure a patient's clinical response). "

The description of the PASLU remit adds:
"The more systematic use of such schemes will need to be reviewed in light of experience. The timing of such a review will be jointly agreed but will be initiated not later than 2 years after the commencement of this agreement. "

We would welcome such a timed review, which should then show the drug in practice, further clinical benefits and the benefits for the NHS.

3. Potential sub-group of patients for lenalidomide:

A portion of del5q patients are known (those with a P53 mutation) to have a sub-optimal response to lenalidomide. 18% of del5q patients have this mutation, based on paper referenced below.
We suggest investigating further with MDS experts to check whether looking at only non P53 del5q patients would help reduce the cost-effectiveness "uncertainty" in this appraisal.

Reference paper:
J Clin Oncol. 2011 May 20;29(15):1971-9. doi: 10.1200/JCO.2010.31.8576. Epub 2011 Apr 25.
TP53 mutations in low-risk myelodysplastic syndromes with del(5q) predict disease progression.
Jädersten M1, Saft L, Smith A, Kulasekararaj A, Pomplun S, Göhring G, Hedlund A, Hast R, Schlegelberger B, Porwit A, Hellström-Lindberg E, Mufti GJ.

"The probability of a complete cytogenetic response to lenalidomide was lower in mutated patients (0 of 7 v 12 of 24; P = .024).

CONCLUSION: Importantly, mutations were present years before disease progression and were associated with an increased risk of leukemic evolution. Our findings indicate a previously unrecognized heterogeneity of the disease which may significantly affect clinical decision making."

4. Iron Chelation treatment (ACD 3.37)
Deferiprone was added to the cost calculations by the ERG.
However this drug is not a licensed medication in this setting – this should have been checked with MDS experts before being added to the model.

B.

1. Additional patient statements, highlighting specifically:

A - Length of use of the drug
B – Treatment Interruptions
C – Other treatments/transfusions needed whilst on Lenalidomide
D – Any other comment

Fiona Pirilla – Lenalidomide patient in UK
A - How long you have been using the drug (in months or cycles please – or years):
I am currently on cycle 54 of treatment this time round. I originally started on Lenalidomide on a drug trial at Kings College from 2006 – 2007 and took 17 cycles of the drug with a great response. Then the trial ended and the drug supply stopped.

B - Whether you had any treatment interruptions (treatment postponed for any reasons) – and for how long (months) ?
As highlighted above after my initial trial finished, so did my drug treatment.
I was left a bit high and dry with no access to the drug for over 2 years – effectively because of the cost.
I applied to my local primary care trust (as it was then) but they would not fund the drug as it was not licenced. It was not until January 2010 that I was able to have access to the drug again, by which time my condition had noticeably deteriorated again. I have now been taking it continuously since then till the present date and as stated am now on cycle 54.

C - Whether you needed any other treatments alongside lenalidomide (such as transfusions, iron chelation) and for how long?
Both on the initial drug trial and now I have to take GCSF injections to boost my white cell and neutrophil counts. I currently use an injection every three days. I also take an aspirin daily to avoid potential blood clotting.

D - Please feel free to make any other comment regarding the benefits of the drug:
Given the length of time I have taken the drug I have been totally transfusion free which is a massive benefit to me. I have also been able to resume normal levels of exercise due to the normal counts of my HGB. I would not be achieving this without this treatment, so my quality of life is massively improved.

[redacted] – Lenalidomide patient in UK
A - I started Lenalidomide in January 2010 and I am currently on cycle 54.

B - I had no interruptions since I started my first cycle.
I was on 10mg for a few months and then the dose was lowered to 5mg which is what I have been on since and doing really well.

C - From the very first cycle, I have not required any blood transfusions which I had been having every 6 weeks, nor have I required any other treatment alongside lenalidomide.

D - I have been transfusion free since I started the drug.
The transfusions barely did anything but with the drug, the quality of my life is just as good as before anaemia set in, bringing my HB down to 7.
I started running and training about 9 months after starting on the drug and it was great to be able to do it (I would get out of breath just walking up the road prior to taking the drug).
I can now run over 8k which is no small feat for me!
Being healthy also enabled my husband and I to adopt another child which was simply amazing.

I was also able to go back to work 4 days a week.
Prior to my treatment, it was a struggle getting up, going to work or doing very much, and I had awful headaches.
The chronic anaemia affected my personality too as I had no energy.
I could fill up a whole page on the benefits of the drug to my life but I will leave it at that.

[redacted] – Lenalidomide patient treated in Spain, also overseen in France
A and B - I am taking REVLIMID 10g/day 3weeks and one week rest since August 2005, without interruption.
C - I didn't need any other treatment. I received red cells transfusions before I began the treatment , but nothing since I have Revlimid. My haemoglobin has been between 12,5 and 13,5 since then.
No side effects, no complications in my case.
D - Of course, I am very happy with my treatment.

[redacted] – lenalidomide patient in Northern Ireland.

A - I am mid way through the 6th cycle of lenalidomide having the drug on 13/12/2013. The first four cycles were provided by Celgene on a compassionate basis and as a consequence of my good response I have been granted funding for one year, IFR's having been turned down on numerous previous occasions.

B - My treatment was stopped for 2 weeks to allow my platelets to rise from 24x109/l to 51x109/l, to allow me to have some dental work done.

C - My last blood transfusion was on 6/2/2014 when my Hb was 71g/l. My Hb has now risen to 123g/l. (I had previously required transfusions every 4-5 weeks).

D - After struggling with the side-effects of the drug for 8 – 10 weeks I began to feel the benefits of the drug during the 4th cycle and now with my Hb at 123g/l, I feel like a new person!
My wife is treating me to a river cruise on the Danube in a few weeks' time to celebrate my 70th birthday; something which would not be possible without lenalidomide.

Further patient statement – in direct response to ACD:

My name is and I am 69 years old. I was first diagnosed with MDS 5q minus in 2008 and at the time my haemoglobin was 12. I was advised that I had a good prognosis and it was possible that the MDS would lay dormant, possibly never affecting my life. The Hb remained constant through to 2010 when it started to fall and by early 2013 it was down to 7.

I had always maintained a healthy and fit lifestyle, running regularly, playing tennis, walking and swimming. By 2013 I was unable to do any of these leisure activities and found great difficulty in walking 100 yards on an incline. I was totally fatigued, could no longer concentrate and it became very difficult for me to maintain my business activity. At this time , the at , applied to the Cancer Drug Trust, to have Lenalidomide-Revlimid. During this period, I was having regular blood transfusions on a monthly basis. They helped a little but for no more than two weeks of the month and at no time did my Hb climb into double figures. Furthermore, the blood transfusions led to me having antibodies within my blood.

Eventually, referred me to at who succeeded in getting me onto a trial with Revlimid which I started taking in October 2013. After three months, I began to experience a remarkable transformation with my haemoglobin rising each month and last month it was in excess of 12. I am now able to lead a very normal and active life, running, walking, swimming and playing tennis regularly. I work a full day without any problems and I am now able to lead a normal fulfilling life with my wife, children and grandchildren. For this, I will be forever grateful to Lenalidomide and and his team of Consultants.

Through my occupation, I have been able to talk with senior Haematologists in the US, Asia and Australia, all of whom have the highest regard for the drug and prescribe Revlimid to those who suffer MDS 5q minus. It would be tragic if this drug was not maintained as available in this country for all those in need, both present and future and I sincerely hope that in due course NICE approve Lenalidomide for use in England.

Further patient statement – in direct response to ACD:

[redacted] – Northern Ireland statement

Unlike the rest of the United Kingdom, Northern Ireland does not have a cancer drug fund. Instead application for funding is made by means of Individual Funding Requests which are submitted by the consultant with the approval of the trust. Over the last 2-3 years at least 4 IFR's have been submitted on my behalf but have been refused because my consultant was not able to show exceptionality. This was very puzzling since 5q- is very rare in a population the size of Northern Ireland (I have yet to meet another patient with 5q- in NI which I think is pretty exceptional). Knowing that had I lived anywhere else in the British Isles (GB and ROI) I would have had access to the drug was very frustrating. Having been proud to work 40 years in the NHS, I could not believe the service would let me down when I needed it most. The unfairness of this situation led to a mixture of feelings such as bewilderment, disillusionment even anger and depression.

Thanks to the work of my [redacted] at [redacted] and my [redacted] [redacted] at [redacted] [redacted] [redacted] who would not take "no" for an answer I have at last acquired lenalidomide, as a result of 4 cycles provided by Celgene leading to funding for 1 year, which has completely turned my life around. Without the drug I was struggling with fatigue, loss of appetite, continually feeling cold, depression and loss of self esteem. I was a keen golfer but had to resign from the golf club. My quality of life was enhanced every 4 weeks by the wonderful blood donors of the United Kingdom who freely donate their blood to help others.

My wife has been absolutely wonderful throughout my illness, looking after the garden, mowing the lawns, driving the car, all areas which I felt were my responsibility but which I was largely unable to cope with. Recently she said to me "I know you have had a hard time, but you have no idea what I have been going through". So it is not just the patient who suffers but their loved ones as well. And knowing there is a drug capable of reversing all of this, (as I now benefit from), but is not available because it has not been given NICE approval is totally incomprehensible. Thanks to this drug my Hb level has increased by 50g/l and I feel I have been given my life back.

I have attached a copy of Hansard for the NI Assembly (6/5/2014) where under Private Member's Business Cancer Drugs Funding [redacted] has used my case history to highlight the human side of the lack of funding for rare cancers in Northern Ireland.

Regards

[redacted]

2. Northern Ireland

Northern Ireland MP [redacted] recently took part in a Northern Ireland Assembly presentation about access to treatment for rarer cancers.
He called on ministers to address the issue of the severe lack of crucial drugs for cancer patients in this part of the country which does not benefit from the Cancer Drug Fund.
We urge NICE and the committee to take these views into consideration.

Private Members' Business – Cancer Drugs: Funding
Topic: The difficulties faced by patients obtaining funding for cancer drugs; concern about the varying policy positions throughout the UK and Ireland regarding the accessibility of cancer drugs; calls on the Minister of Health, Social Services and Public Safety to undertake a review into the practices in Northern Ireland to provide greater clarity for patients trying to obtain funding for cancer drugs.

Comment extracted:
"[redacted]: Thank you. I welcome the intervention. It is a point that I will expand on in just a moment. In England, as we are aware, a cancer drugs fund (CDF) is in operation that provides direct access for cancer sufferers to cancer drugs. In Scotland, just this month, the peer-approved clinical system has been put in place. That has replaced the IFR model and the clear inequalities in access that it was producing. In Wales, the All Wales Medicines Strategy Group is in place to appraise medicines before NICE. Consequently, that, too, improves access for cancer patients. However, a recent study by Bristol University uncovered that cancer sufferers in England are seven times more likely to receive the drug than those in Wales. There are varying journeys towards greater access to cancer drugs in the UK. We, however, are not yet at the starting line. When the SDLP has asked the Minister about that inequality, he has deflected it by bringing up the viability of the IFR process or even the issue of prescription charges or welfare reform.
The Rarer Cancers Foundation and others have undertaken research about the cost of a CDF model here. It ranges between £5 million and £6 million. That is significantly less than the amounts needed in England, and it is feasible, we argue, if the Minister wants it to be. It is also important to note that, due to sophisticated molecular testing, improved diagnosis is available. Clinicians are available to accurately pinpoint which drug will work for a particular individual. So a cancer drugs fund model will cost more but will be much more refined and accurate than initially imagined."

Northern Ireland and Wales are at an incredible disadvantage – as the Cancer Drug Fund is not available to those regions, therefore creating a post-code lottery – yet again.
This issue also creates severe problems of cross regional referrals – causing a further and artificial strain on the NHS.

3. Small population size

This group of patients in this appraisal is extremely small – and therefore additional evidence from abroad must be considered to help reduce the uncertainty the Committee still struggles with.

We strongly request that the Committee engages with as many MDS experts as possible, in order to gather further clinical evidence, as opposed to requesting additional data from the manufacturer.

To date, only one MDS expert was consulted internally at NICE, which we insist is neither satisfactory, nor objective or conclusive.

We request specifically that the 31.9% survival estimate at 26 cycles of treatment be validated by several experts in the UK and abroad. We strongly believe this would assist in reducing uncertainty.

Previous NICE appraisals benefitted from the "small population criterion" as in the HTA below.

We believe that lenalidomide fits in with this particular concept.

http://publications.nice.org.uk/pemetrexed-maintenance-treatment-following-induction-therapy-with-pemetrexed-and-cisplatin-for-ta309/consideration-of-the-evidence

4.21 The Committee discussed the small patient population criterion. It heard from NICE that, for treatments for small groups of patients, higher prices, and therefore reduced cost effectiveness, were more likely to be justified given the need to recoup costs of development of the product if the licensed indications only apply to a small potentially eligible patient population. It further heard that the case for reduced cost effectiveness weakens as the potential total population for a product increases. Therefore, taking into account the cumulative population covered by all the indications in the marketing authorisation needs to be considered. The Committee understood that the small patient population criterion was intended to recognise the long-term benefits to the NHS of innovation.

4. Scotland and SMC appraisal

The SMC was satisfied with the data provided for their appraisal of this same drug and was able to approve the drug for Scotland. The process and work by this HTA group was just as rigorous as NICE.

Following the approval in Scotland, patients are once again facing a post-code lottery.

This is again increasing issues of regional cross referrals.

C.

1. Insufficient involvement with Patient group consultees.

Since the publication of the FAD, we requested more ample involvement, in order to assist with the validation of clinical data. Despite regular communication, this did not take place and we as patient groups, nor the clinical expert groups were fully informed about the additional data requested from the manufacturer.

This unfortunately also reflects the experience gathered in the recent NICE Patient Expert Report 2012, which was released recently – extracts below.

We would again urge NICE and committees to take this opportunity to engage more fully with patient group consultees at every stage of the appraisal.

2. Report – NICE Patient Expert Survey

The results of a NICE survey amongst patient experts conducted in 2012 shows that unfortunately, two years later, NICE committees have still not taken up the opportunity to work more closely with patient experts, and use their knowledge and experience to improve and assist in the appraisals.

We find ourselves facing similar issues in this lenalidomide appraisal.

National Institute for Health and Care Excellence - Technology Appraisal Patient Expert Survey 2012 Report.
By Lizzie Amis and Heidi Livingstone - Public Involvement Programme
Experiences during the committee meeting - overall experience

"Not only did the patient experts find the slide presentation more difficult to understand, but also only just under half the patient experts (48%) thought that the slides addressed the key patient issues, with over a third (33%) saying that they thought they were not addressed.
"The slide presentation did not properly address patient needs and the reasons why one of the technologies appraised was more beneficial to some patients than others. This had to be explained by the clinical experts."
"There was patient experience as far as goes side effects etc. however the patient experience is far more than this. My overriding feeling with NICE TAs is that it is hard science driven, and the wider patient issues have little consideration/value to the determination."
"The cost effectiveness Committee 'lead team' member addressed issues that could not be described as 'key patient issues' but were rather (broadly) NHS resources issues. There are generally a large number of slides in his presentation
Only 65% of patient experts were aware that there is a lay member of the committee responsible for that particular appraisal topic.
The comments about the lay members and the lay lead role vary and show that there could be an improvement in awareness of and understanding of the lay members of the committee and the lay lead role.
There is considerable room for improvement; only about 40% said that they understood the slide presentations and could follow and participate in the discussions. Just under half thought that the patient issues were represented in the slides.
There were comments about the patients presence being 'tokenistic' and that the patient experience has little to do with the committee's overall determination
Although 65% knew that there was a lead lay member responsible for their topic, there was a lack of clarity about what their role is.
Other patient experts thought that although some of the key issues were addressed some still were not and point out what they see as the flaws regarding NICE's methodology for including patient issues.
"Obviously some key patient issues were addressed but not all. I fail to understand how NICE can be a partner in an initiative like the Technology Strategy Board (TSB) 'Stratified Medicine Innovation Platform' which

accepts that randomised clinical trial (RCT) evidence is not exhaustive, although not without value, whilst simultaneously elevating RCT evidence via inclusion/exclusion criteria, to be the sole clinical evidence on which economic models are built. The key patient issue is that clinically speaking not all patients, even though they have the same diagnosis, are the same. The modelling effectively shuts out evidence that might offer redress to that relied upon."

What is most important to patient experts?

The most important issues for patients are:

1. Being able to raise relevant issues at the committee meeting

2. Feeling that their contribution made a difference

Feeling that their contribution made a difference

This is one of the top two issues of important to patient experts and thus another key area for improvement. Although the majority of the patient experts felt their contribution made a difference, there were issues of: a feeling of tokenism; the organisational statement being more relevant than the patient expert presence; feeling that not all patient issues were addressed; feeling that patient issues and evidence are not viewed equally with the RCT evidence and the health economics.

Final comments

We firmly believe there is a possibility to reduce the uncertainty in this STA by involving all stake holders and consultees, given the issues we raised in our comments, and the information we provided – if used by the committee.

We urge NICE to pursue this consultation further to come to an acceptable outcome in order to make the drug available – and therefore remove the current unacceptable regional discrepancy.

We remain at the Committee's disposal for any additional information.

We look forward to hearing from you.

Kindest regards,

[redacted] - [redacted] - MDS UK Patient Support Group

[redacted] – [redacted] - MDS UK Patient Support Group

Leukaemia CARE (Joint Consultee with MDS UK)