

Repeated on BirminghamWired, Pushpi News, MizoramExpress, Paper News, World News, Sain Sunsite, University of Information, Michelmores, Cancer Support Groups



Patients fight bone drug ruling

Cancer groups are to appeal against a ruling which would deny NHS patients in England and Wales a drug for treating life-threatening bone marrow diseases.

The drug, Vidaza, gives patients with myelodysplastic syndromes (MDS) an extra nine months of life on average.

But the National Institute for Health and Clinical Excellence (NICE) ruled that Vidaza is too costly, relative to the benefits it brings.

The drug costs £45,000 a year per patient.

MDS means that the bone marrow does not produce enough of one or more types of blood cells. Most patients need regular blood transfusions.

The average survival of patients with MDS is about 20 months. Nearly a third of patients progress to acute myeloid leukaemia, an aggressive form of leukaemia.

“ How can it be in the patient's best interest to deny them an average of an extra nine months of life? ”

David Hall, MDS UK Patient Support Group

Research has shown that the average survival for higher-risk MDS patients receiving Vidaza (also known as azacitidine) was 24.5 months, compared with 15 months for patients receiving conventional treatments such as supportive care or chemotherapy.

The appeal against the NICE ruling is being lodged by three cancer patient groups - the MDS UK Patient Support Group, the Leukaemia Society and the Rarer Cancers Forum. The manufacturer of Vidaza, Celgene, is also appealing against the decision.

'Significant advance'

David Hall, an MDS patient who is also chairman of the MDS UK Patient Support Group, said Vidaza was the first significant advance in medical treatment for this condition.

Mr Hall, whose group is supported by pharmaceutical companies, including Celgene, said: "The negative recommendation is a return to hopelessness for UK MDS patients," he said.

"The recommendations also create an ethical dilemma for doctors treating this condition. How can it be in the patient's best interest to deny them an average of an extra nine months of life?"

"Some patients have their lives extended by much longer than the average nine months," he added.

The charity argues that the cost for the NHS is unlikely to be significant because there are only around 700 people in the UK with higher risk MDS.

'Not cost effective'

NICE published its draft guidance on Vidaza earlier this month and will now consider the appeal.

Their spokeswoman, Dr Carole Longson, said: "We are disappointed not to be able to recommend this drug."

She said that Vidaza is not a cure for MDS but could potentially prolong the lives of people with these conditions by around nine months longer than standard treatment.

However, she said: "The Appraisal Committee concluded that, relative to the benefits, the price the NHS is being asked to pay for azacitidine is still too high for it to be recommended as a cost-effective use of NHS resources."

Story from BBC NEWS:

<http://news.bbc.co.uk/go/pr/fr/-/1/hi/health/8579915.stm>

Published: 2010/03/23 00:59:26 GMT

© BBC MMX

APM

Patient group challenges NICE guidance, warns litigation against NHS likely if implemented

LONDON, March 22 (APM) - The MDS UK Patient Support Group said it is appealing against NICE's rejection of Celgene's Vidaza for bone marrow diseases, claiming doctors may face disciplinary action and the health service legal action by patients if the 'perverse' guidance is implemented.

Monday's move comes after NICE rejected, in its final appraisal, Vidaza in myelodysplastic syndromes, chronic myelomonocytic leukaemia and acute myeloid leukaemia. ([APMHE 18274](#))

Celgene has already told APM it will appeal and after a scathing attack on the NICE decision, today's move by MDS UK was not unexpected.

NICE has a solid record in defending its decisions and grounds for appeals are limited, although the MDS group has taken all three of the broad categories and backed them up in a seven-page challenge seen by APM.

PROCEDURAL FAILURES

The MDS body said NICE has failed to act fairly and in accordance with the appraisal procedure set out in its own guidance.

Among challenges under this heading are that evidence that would have slanted the cost-benefit of the drug in favour of a 'yes' was ignored.

It said the final appraisal determination (FAD) is "unfair" because the appraisal committee completely ignored evidence available to the MDS UK Patient Support Group on quality of life - in particular fatigue - and patient and carer views of MDS and existing treatments.

It said the ignored evidence "clearly demonstrates that quality of life is improved immensely for patients receiving azacitidine," and "had the committee been provided with this evidence, then we consider that a greater weight would have been applied to the ICERs (Incremental Cost-Effectiveness Ratio) for azacitidine either comparing to best supportive care alone or to chemotherapy, and that it would be recommended for use on the National Health Service."

PERVERSE

The allegation that NICE's appraisal committee did not see all the evidence it needed is followed up by a claim that, while this in itself was perverse, even in the absence of full data, the rejection of the drug is perverse in light of the information NICE did have.

In this case, MDS UK says Vidaza shows 9.5 month extension to life, which is greatly in excess of that required for special consideration to be given under the institute's end-of-life criteria. As such, rejection is perverse.

It also said NICE's insistence on only using best supportive care (BSC) as a price comparator is perverse because chemotherapy is frequently used in additions to BSC.

EXCEEDED POWERS

Under this heading, MDS UK said NICE's recommendations are incompatible with the Human Rights Act 1998 and the General Medical Council's code of ethics with which doctors must comply.

Again noting the 9.5 months of extra life afforded by Vidaza, the patient group said under GMC rules and given the study data on Vidaza, doctors would be expected to prescribe it.

"We suspect that a doctor who does not prescribe azacitidine to appropriate MDS patients may be increasing the risk of negligence claims against the NHS, particularly as the overwhelming evidence is that azacitidine is clinically effective and given the very small numbers of patients with MDS in this country, unlikely to be a significant burden on NHS finances."

In addition, UK MDS patients may be inclined to obtain therapy in France or other countries on a case by case basis, with their physicians' recommendation, it continued.

"To this end, we query whether an appropriate risk assessment has been conducted by NICE in conjunction with the NHS Litigation Authority (which deals with legal claims against the service)."

In the case of human rights, the charity looked at certain articles within the European Convention for the Protection of Human Rights and Fundamental Freedoms and argues NICE's rejection of the Vidaza runs contrary to them.

NICE's full guidance, which would have been issued automatically in the absence of an appeal, will now not be published until the outcome of the appeal - which in itself could end with the appraisal committee starting again.

However, while challengers to NICE via its appeals process have enjoyed some success, the institute is usually the victor and ultimately substantial changes following re-appraisals are rare.

Repeated on freerepublic.com

MailOnline

Cancer support groups appeal ban of bone marrow disorder drug on NHS

23rd March 2010

Three cancer patient support groups today announced they are appealing a decision to reject a drug used to treat a range of bone marrow disorders for use on the NHS.

The National Institute for Health and Clinical Excellence (Nice) ruled in draft guidance earlier this month that azacitidine - or Vidaza - could not be prescribed on the NHS for the treatment of a number of myelodysplastic syndromes (MDS).

MDS UK Patient Support Group, The Leukaemia Society and the Rarer Cancers Forum are all appealing against the Nice guidance.



Appeal: Three cancer patient support groups are opposing a decision to reject Vidaza, a drug used to treat bone marrow disorders, for use on the NHS

Approximately four in 100,000 people in the UK have MDS, a group of debilitating bone marrow diseases that lead to complications such as recurrent or life-threatening infections or bleeding, according to the MDS UK Patient Support Group.

The charity said azacitidine had been proven not only to slow the progress of the disease but also to vastly improve patients' quality of life by freeing them from repeated cycles of blood transfusions.

It described the draft decision as a huge blow to MDS patients, particularly those with the high risk forms of these diseases, for whom the outlook is often bleak.

The MDS UK Patient Support Group cited a study published in *The Lancet Oncology*, which demonstrated that the median overall survival for higher-risk MDS patients receiving azacitidine was 24.5 months, compared with 15 months for patients receiving conventional care such as supportive care or chemotherapy - a difference of 9.5 months.

More...

The charity said a negative recommendation for azacitidine was unreasonable given the 9.5 month average extension to life - more than three times the figure required by Nice's end-of-life criteria.

There are approximately 700 people with higher-risk MDS in the UK so any financial burden on the NHS is unlikely to be significant, the MDS UK Patient Support Group added.

David Hall, chairman of the MDS UK Patient Support Group and an MDS patient, said: 'The negative recommendation is a return to hopelessness for UK MDS patients who have glimpsed hope in the EU-approval of the first therapy for malignant bone marrow disease that is a significant advance in medical treatment for this condition.

'The recommendations also create an ethical dilemma for doctors treating this condition. How can it be in the patient's best interest to deny them an average of an extra nine months of life? Some patients have their lives extended by much longer than the average nine months.

'We believe strongly that the underlying appraisal methodology used by Nice is unfair and lacks transparency.

'We also believe that the recommendations are perverse as the appraisal committee completely ignored evidence presented to it on quality of life and does not understand the nature of this very rare disease.'

Nice said final guidance on the drug, after a period of consultation on the draft ruling, would be issued in May.

The watchdog said that according to the manufacturer's estimates, azacitidine costs around £45,000 per patient.

Dr Carole Longson, of Nice, said: 'Azacitidine is the first drug that has been developed specifically for treating MDS.

'It is not a cure, but could potentially prolong the life of people with these conditions by around nine months longer than standard treatment.

'We are disappointed not to be able to recommend this drug.

'The independent appraisal committee considered all published evidence on the effectiveness of azacitidine and the cost, including the proposed "patient access scheme".

'The appraisal committee concluded that relative to the benefits, the price the NHS is being asked to pay for azacitidine, is still too high for it to be recommended as a cost effective use of NHS resources.'

A Department of Health statement said: 'Nice is an independent body and it would not be appropriate for us to interfere in an ongoing Nice appraisal.

'We recognise that the institute's decisions have serious implications for patients and their carers, which is why Nice operates an open and transparent process in the development of guidance.'

The Pharma Letter

UK cancer patient support groups to appeal negative NICE decision on Celgene's Vidaza

Article | 23 March 2010

The MDS UK Patient Support Group says it has lodged an appeal against the negative recommendation issued earlier this month by the National Institute for Health and Clinical Excellence (NICE) for the use of Celgene's Vidaza (azacitidine) in patients with higher-risk myelodysplastic syndrome (MDS; The Pharma Letter March 4). The Leukaemia Society and the Rarer Cancers Forum are also appealing the NICE guidance.

Approximately four in 100,000 people in the UK have MDS, a group of debilitating bone marrow diseases that lead to complications such as recurrent or life-threatening infections or bleeding. Most MDS patients have to rely on frequent blood transfusions to manage anemia and extreme fatigue. While the average survival of patients with MDS is about 20 months, nearly a third (30%) progress to acute myeloid leukaemia (AML), a very aggressive and resistant form of leukemia with an average survival period of a few months only.

Enjoying this article? Have the leading Biopharma news & analysis delivered daily on email [by signing up for our FREE email newsletter here.](#)

Cost per patient is \$45,000

Vidaza is estimated to cost about £45,000 (\$67,572) per patient. Even with the discount and the more relaxed cost-effectiveness criteria, the drug's most plausible cost-per-QALY (cost per quality-adjusted life year, which is the measure NICE uses to assess cost-effectiveness) would still be £63,000, according to the NICE. That figure is more than double the agency's unofficial threshold of £25,000-£30,000 per QALY.

A study published in The Lancet Oncology demonstrated that the median overall survival for higher-risk MDS patients receiving azacitidine was 24.5 months compared with 15 months for patients receiving conventional care such as supportive care or chemotherapy – a difference of 9.5 months. The study

also showed that at two years, the survival rate for patients receiving azacitidine was just over 50%, nearly double that of patients receiving conventional care (26%).

David Hall, chairman of the MDS UK Patient Support Group and MDS patient said: "The negative recommendation is a return to hopelessness for UK MDS patients who have glimpsed hope in the European Union-approval of the first therapy for malignant bone marrow disease that is a significant advance in medical treatment for this condition. The recommendations also create an ethical dilemma for doctors treating this condition. How can it be in the patient's best interest to deny them an average of an extra nine months of life? Some patients have their lives extended by much longer than the average nine months."

He continued: "We believe strongly that the underlying appraisal methodology used by NICE is unfair and lacks transparency. We also believe that the recommendations are perverse as the appraisal committee completely ignored evidence presented to it on quality of life and does not understand the nature of this very rare disease."

Recommendations contrary to Human Rights Act

The MDS UK also believes the NICE has made recommendations that are incompatible with the Human Rights Act 1998 - particularly in terms of preventing foreseeable loss of life and discrimination against the elderly and newly-diagnosed patients - as well as the General Medical Council's code of ethics. Not being able to offer patients azacitidine and the chance of an extra extension to life flies in the face of doctors' ethical obligations and may lead to negligence claims against the NHS.

The MDS UK Patient Support Group also said it endorses the report released last week (March 15) by the Rarer Cancers Forum (RCF), which stated that the NICE has rejected cancer treatments which could have benefited up to 16,000 patients in spite of its new guidelines for the appraisal of life-extending, end-of-life treatments. The RCF said that the way the NICE is interpreting these guidelines is confusing and runs counter to the spirit of the 2008 report from Professor Mike Richards, the government's national clinical director for cancer: "Improving access to medicines for NHS patients."

Latest News

Appeal launched over Vidaza use

Tuesday 23rd March 2010

The National Institute for Health and Clinical Excellence (NICE) said the Vidaza drug, used to treat patients with myelodysplastic syndromes (MDS), is too expensive to use: it costs around £45,000 a year per patient.

MDS is a disease whereby the bone marrow does not produce enough of one or more types of blood cell.

In around a third of all cases, patients develop acute myeloid leukaemia, a very aggressive form of cancer.

Tests carried out have shown that patients receiving conventional methods of treatment, such as chemotherapy, survive just 15 months.

But those who receive Vidaza survive for around 24.5 months.

NICE published its draft report on the use of the treatment earlier this month, but will reconsider following an appeal by the MDS UK Patient Support Group, the Leukaemia Society and the Rarer Cancers Forum, as well as Celgene, which manufactures the drug.

Copyright Press Association 2010

[NICE](#)

Latest News

NICE to consider Vidaza appeal

Tuesday 23rd March 2010



A ruling that denies NHS patients in England and Wales a treatment for life-threatening bone marrow diseases is to be contested by cancer groups.

Vidaza, which costs £45,000 per person and gives people with myelodysplastic syndromes (MDS) an average of nine months extra to live, is too costly, the National Institute for Health and Clinical Excellence (NICE) decided.

MDS inhibits the bone marrow's ability to produce different types of blood cells, with most sufferers needing regular transfusions to stay alive.

The average survival time of patients with the condition is 20 months. Nearly a third of patients progress to acute myeloid leukaemia, an aggressive form of leukaemia.

The appeal against the ruling will be lodged by the MDS UK Patient Support Group, the Leukaemia Society and the Rarer Cancers Forum, with drug manufacturer Celgene also throwing its weight behind the campaign.

SCRIP

NICE no for Celgene's Vidaza violates Human Rights Act, say patient groups 23
March 20 JO Franceses Bruce

UK patient groups have lodged an appeal against health technology appraisal body NICE'S decision to decline Celgene's anticancer, Vidaza (5-azacitidine), for patients with myelodysplastic syndromes (MDS). The patient groups say the recommendations are "incompatible" with the UK's 1998 Human Rights Act.

Earlier this month NICE said that the drug, which offers on average nine months' life extension, would not be a cost-effective option for treating myelodysplastic syndromes, chronic myelomonocytic leukaemia and acute myeloid leukaemia (scripnews.com, 4 March 2010). The drug would cost the NHS £45,000 per patient per year and NICE decided that the most plausible incremental cost-effectiveness ratio for the drug in the general patient population was £63,000 per quality adjusted life year (QALY) gained.

But the MDS UK Patient Support Group together with the Leukaemia Society and the Rarer Cancers Forum have filed an appeal. They claim that the institute has exceeded its powers by issuing recommendations that are incompatible with the Human Rights Act and the General Medical Council's (GMC) code of ethics.

This could mark the first appeal application to be lodged against NICE on such grounds. Professor Rodney Taylor, deputy chairman of the MDS UK Patient Support Group, told *Scrip* that the group was unaware of any other appeal based on these grounds, NICE declined to comment.

NICE'S final appraisal determination concedes that the drug does give around nine months' life extension compared with conventional treatments. However, the decision to decline the drug for NHS funding means that doctors will be forced to "withhold treatments, which raises serious ethical questions", says Professor Taylor.

The GMC's code of ethics states that doctors should offer "treatments where the possible benefits outweigh any burdens or risks associated with the treatment ... Prolonging life will usually be in the best interests of a patient." The patient groups also argue that stopping doctors from prescribing Vidaza could therefore lead to a rise in negligence claims against the NHS.

The groups argue that NICE'S decision does not meet Article 2 of the European Convention for the Protection of Human Rights and Fundamental Freedoms (transposed

in to the 1998 Human Rights Act). This article states "Everyone's right to life shall be protected by law."

Other Grounds

Furthermore, the patient groups maintain that the institute "failed to act fairly" as it did not give due consideration to quality of life data gathered from 1,000 patients across the world, said Professor Taylor. Because the appraisal committee "completely ignored evidence presented to it on quality of life", the groups are arguing that the recommendations are "perverse", said David Hall, chairman of the MDS UK Patient Support Group, in a statement.

NICE Appeals

25/03/2010

Scrip News - NICE no for Celgene's Vidaza violates Human Rights Act, say patient gr... Page 2 of 2

NICE said it could not comment on "potential appeals", but in a statement, it said these would be "scrutinised by the chair of the appeal committee, who will look and see that they contain the necessary information and that the appeal falls under any one or more of the grounds for appeal". The institute will eventually announce whether it accepts the appeal on its website.

NSCE's appeal panel will not accept challenges unless the grounds for appeal meet the following criteria;

- . The institute has not acted fairly or in accordance with its published procedures as detailed in the guide to the technology appraisal process;
- . A NICE final appraisal determination is "perverse in the light of the evidence submitted"; . The institute has exceeded its powers.

No new evidence of the drug can be submitted as part of an appeal.

Celgene has also filed an appeal, but it was unable to comment for this article.

Nursing Times

Appeal after NICE deems bone marrow drug too expensive

23 March, 2010

Sufferers of bone marrow disease could be denied a drug that prolongs life by an average of nine months, cancer campaigners have said.

A ruling will deny [NHS](#) patients the drug Vidaza, considered by the [National Institute for Health and Clinical Excellence](#) (NICE) as too expensive for the benefits it [offers](#).

Vidaza manufacturer, Celgene, is appealing against the decision on its drug, which costs £45,000 a year per patient.

MDS means that the bone marrow does not produce enough of one or more types of blood cells. Most patients need regular blood transfusions.

Patients with myelodysplastic syndromes (MDS) only survive for 20 months on average, with almost a third developing the aggressive acute myeloid leukaemia.

Patients with MDS, which stops bone marrow producing enough blood cells, usually need frequent blood transfusions.

Vidaza (also known as azacitidine) helped higher-risk MDS patients survive 24.5 months, while those treated with conventional methods like chemotherapy survived for an average of 15 months.

Three cancer groups are opposing the NICE ruling - the Leukaemia Society, the MDS UK Patient Support Group and the Rarer Cancers Forum.

After releasing its draft guidance on the drug in early March, NICE will now consider their appeal.

**CANCER PATIENT SUPPORT GROUPS
APPEAL NHS DRUGS REFUSAL**

By Emma Foster, **Community Newswire**

HEALTH Cancer, 23 Mar 2010 - 10:51

Three cancer patient support groups have today announced they are appealing a decision to reject a drug used to treat a range of bone marrow disorders for use on the NHS.

The National Institute for Health and Clinical Excellence (Nice) ruled in draft guidance earlier in the month that azacitidine (or Vidaza) could not be prescribed on the NHS for the treatment of a number of myelodysplastic syndromes (MDS).

MDS UK Patient Support Group, The Leukaemia Society and the Rarer Cancers Forum are all appealing against the Nice guidance.

Approximately four in 100,000 people in the UK have MDS, a group of debilitating bone marrow diseases that lead to complications such as recurrent or life-threatening infections or bleeding, according to the MDS UK Patient Support Group.

The charity said azacitidine had been proven not only to slow the progress of the disease but also to vastly improve patients' quality of life by freeing them from repeated cycles of blood transfusions.

It described the draft decision as a huge blow to MDS patients, particularly those with the high risk forms of these diseases, for whom the outlook is often bleak.

The MDS UK Patient Support Group cited a study published in *The Lancet Oncology*, which demonstrated that the median overall survival for higher-risk MDS patients receiving azacitidine was 24.5 months, compared with 15 months for patients receiving conventional care such as supportive care or chemotherapy - a difference of 9.5 months.

The charity said a negative recommendation for azacitidine was unreasonable given the 9.5 month average extension to life - more than three times the figure required by Nice's end-of-life criteria.

There are approximately 700 people with higher-risk MDS in the UK so any financial burden on the NHS is unlikely to be significant, the MDS UK Patient Support Group added.

David Hall, chairman of the MDS UK Patient Support Group and an MDS patient, said: "The negative recommendation is a return to hopelessness for UK MDS patients who have glimpsed hope in the EU-approval of the first therapy for malignant bone marrow disease that is a significant advance in medical treatment for this condition.

"The recommendations also create an ethical dilemma for doctors treating this condition. How can it be in the patient's best interest to deny them an average of an extra nine months of life? Some patients have their lives extended by much longer than the average nine months.

"We believe strongly that the underlying appraisal methodology used by Nice is unfair and lacks transparency.

"We also believe that the recommendations are perverse as the appraisal committee completely ignored evidence presented to it on quality of life and does not understand the nature of this very rare disease."

Nice said final guidance on the drug, after a period of consultation on the draft ruling, would be issued in May.

The watchdog said that according to the manufacturer's estimates, azacitidine costs around £45,000 per patient.

Dr Carole Longson, of Nice, said: "Azacitidine is the first drug that has been developed specifically for treating MDS.

"It is not a cure, but could potentially prolong the life of people with these conditions by around nine months longer than standard treatment.

"We are disappointed not to be able to recommend this drug.

"The independent appraisal committee considered all published evidence on the effectiveness azacitidine and the cost, including the proposed 'patient access scheme'.

"The appraisal committee concluded that relative to the benefits, the price the NHS is being asked to pay for azacitidine, is still too high for it to be recommended as a cost effective use of NHS resources."

A Department of Health statement said: "Nice is an independent body and it would not be appropriate for us to interfere in an ongoing Nice appraisal.

"We recognise that the institute's decisions have serious implications for patients and their carers, which is why Nice operates an open and transparent process in the development of guidance."

For more information visit the patient support groups' websites at:
www.mdspatientsupport.org.uk, www.leukaemiasociety.org or
www.rarercancers.org.uk.

Hc2d.co.uk

NICE ruling on Vidaza challenged

23rd March 2010

Cancer support groups have said they will appeal against the National Institute of Health and Clinical Excellence's decision not to treat health service patients with Vidaza.



The drug gives an extra nine months of life, on average, to people suffering from myelodysplastic syndrome (MDS), a form of bone marrow disease.

NICE have decided that Vidaza, which costs £45,000 per patient per year, is too expensive.

The disease causes patients' bone marrow to malfunction and means they require blood transfusions. In a third of patients the disease leads to acute myeloid leukaemia.

The average survival rate after diagnosis is around 20 months. According to research, patients treated with Vidaza survived for 24.5 months in comparison to 15 months for patients treated with chemotherapy.

David Hall, an MDS patient who is also chairman of the MDS UK Patient Support Group, said the drug represented the first step in available medication to treat the disease.

Mr Hall said: "The negative recommendation is a return to hopelessness for UK MDS patients."

"The recommendations also create an ethical dilemma for doctors treating this condition. How can it be in the patient's best interest to deny them an average of an extra nine months of life?"



Patient groups appeal 'perverse' Vidaza ruling

Patient groups have appealed against the negative recommendation by NICE for the use of Vidaza (azacitidine) in patients with higher-risk myelodysplastic syndrome (MDS).

Appeals have been lodged by MDS UK, the Leukaemia Society and the Rarer Cancers Forum.

MDS is a group of debilitating bone marrow diseases that lead to complications such as recurrent or life-threatening infections or bleeding. While the average survival of patients with MDS is about twenty months, nearly a third (30%) progress to acute myeloid leukaemia (AML), a very aggressive and resistant form of leukaemia.

A study published in *The Lancet Oncology* showed that the overall survival for higher-risk MDS patients receiving azacitidine was 24.5 months compared with 15 months for patients receiving conventional care such as supportive care or chemotherapy. The study also showed that at two years, the survival rate for patients receiving azacitidine was just over 50%, nearly double that of patients receiving conventional care (26%).

David Hall, Chairman of the MDS UK Patient Support Group, said: “The negative recommendation is a return to hopelessness for UK MDS patients who have glimpsed hope in the EU approval of the first therapy for malignant bone marrow disease that is a significant advance in medical treatment for this condition.

“We believe strongly that the underlying appraisal methodology used by NICE is unfair and lacks transparency. We also believe that the recommendations are perverse as the appraisal committee completely ignored evidence presented to it on quality of life and does not understand the nature of this very rare disease.”

MDS UK argues that the 9.5-month average extension to life should qualify azacitidine for approval, as it is more than three times the figure required by NICE’s end-of-life criteria and longer than the extensions to life accepted by NICE in the past.

The group adds that there are only approximately 700 people with higher-risk MDS in the UK and any financial burden on the NHS is unlikely to be significant.

In addition, MDS UK believes that NICE ignored strong quality of life evidence gathered from over 1,000 patients worldwide by the MDS Foundation, as well as arguing that NICE’s recommendations are incompatible with the Human Rights Act 1998 and the General Medical Council’s code of ethics.

The appeals follow the release of a report by the Rarer Cancers Forum (RCF), which stated that NICE has rejected cancer treatments which could have benefited up to 16,000 patients.

CELGENE AND THREE UK PATIENT GROUPS APPEAL NICE'S REJECTION OF VIDAZA

The Pink Sheet DAILY 3/22/2010

EXECUTIVE SUMMARY

"The Pink Sheet" DAILY -- Celgene and three UK patient support groups are independently appealing the rejection of Vidaza (azacitidine) for the treatment of myelodysplastic syndrome (MDS), a move that was part of a March 4 draft guidance issued by the UK's National Institute for health and Clinical Excellence (NICE).

