PRESS RELEASE

Cancer patient support groups express extreme disappointment at NICE final decision on Vidaza

London, 4 March 2010 - A group of UK cancer patient support organisations is disappointed and angered by the decision announced today by the National Institute for Health and Clinical Excellence (NICE) not to make Vidaza (azacitidine) available through the NHS to patients with a range of life-threatening bone marrow diseases.

The Final Appraisal Determination issued by NICE states that azacitidine is not recommended as a treatment option for people with intermediate-2 or high risk myelodysplastic syndromes (MDS), nor is it recommended for patients with forms of chronic myelomonocytic leukaemia or acute myeloid leukaemia. While NICE recognised that azacitidine was clinically effective and that it should be seen as a life-extending end-of-life treatment, it decided that it should not be provided through the NHS on cost-effectiveness grounds.

Approximately four in 100,000 people in the UK have MDS, a debilitating bone marrow disease that leads to complications such as recurrent or life-threatening infections or bleeding. Most MDS patients have to rely on frequent blood transfusions to manage anaemia and extreme fatigue. 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 with an average survival period of a few months only.

David Hall, Chairman of the MDS UK Patient Support Group and MDS patient said, “This is a huge blow to MDS patients, particularly those with the high risk forms of these diseases for whom the outlook is often bleak. Azacitidine is the only licensed drug available specifically to treat MDS and has been proven not only to slow the progress of the disease but also vastly improve patients’ quality of life by freeing them from repeated cycles of blood transfusions. A total of only 700 patients a year in England and Wales would require treatment with azacitidine so we do not believe that providing this life-extending treatment would make a huge impact on the NHS budget.”

Kathy Heptinstall, Operating Director of the Myelodysplastic Syndromes Foundation (the only international organization devoted to these diseases) said about the decision, “This
negative appraisal of Vidaza from NICE denies MDS and AML patients access to the only
drug proven to prolong their lives. It is an affront to all patients with haematological
malignancies and devalues their very existence. In addition to prolonging life, Vidaza has also
been shown to improve the quality of life for MDS patients, many of whom have no other
treatment options.”

In supporting the patient groups, Professor Ghulam Mufti, Professor of Haematology/
Oncology, at King’s College Hospital and Chairman of the UK MDS Forum said, “Vidaza,
and other drugs of this family, have been shown, in randomised multicentre clinical trials, to
prolong the survival of MDS patients, particularly those with high risk of early death or
leukaemic transformation. These drugs are in routine use in the USA following the approval
by FDA, and most, if not all, the countries in the EU. It is disconcerting to note that we seem
to be always last in licensing effective medications, particularly in relatively rare disorders. It
should be noted that Vidaza is the only effective drug for these patients, who have a high risk
of considerable morbidity and mortality. “

North Devon MP Nick Harvey, who has a number of MDS patients in his constituency has
also reacted with disappointment to the NICE decision. “This is extremely upsetting news for
MDS suffers, considering the lack of alternative treatments available. The positive effects of
Vidaza are clear and it is shocking that yet again the Government has reinforced its record of
being the last to license new and effective medications. This rare and debilitating disease
should not be ignored by the NHS on the grounds of cost-effectiveness. The NICE’s decision
raises considerable questions about whether the NICE Health Technology Appraisal is an
appropriate mechanism for measuring the true value to patients of new and innovative
medicines for rarer cancers.”

Janet Hayden, Myeloid Clinical Nurse Specialist at the Kings College Hospital has treated
over a hundred patients with azacitidine: “In clinical trials, we have seen very positive results
in many patients, especially in regards to improving survival and quality of life. Outside our
trials, applications to the PCT for funding have largely been rejected. The majority of patients
are not transplant candidates and this ruling means that we are now no longer able to offer the
only drug we currently have that has any hope of improving quality of life and survival
outcome.”

A petition calling on the Prime Minister to ensure that people experiencing MDS receive care
comparable with that available in most other European countries has attracted over 300
signatures and is available at http://www.ukmdsforum.org/petition.php

**Response from the Patient Groups:**
MDS UK Patient Support Group
Rodney Taylor, Deputy Chair and MDS patient
"My personal experience has been a most dramatic transformation from profound debilitation back to leading a totally normal life without the need for blood transfusions or any other treatment. We believe the existing NICE appraisal process, specifically the mathematical analysis is inappropriate in evaluating innovative treatments for rarer conditions. Trying to apply a common benchmark to small numbers of patients will inevitably lead to delays and constraints within the NICE processes – all resulting in refusals to recommend crucial drugs. We advocate allowing patient access to licensed medicines for rarer cancers in advance of a NICE appraisal to allow a longer period to gather evidence on survival and benefit of increased quality of life."

Macmillan Cancer Support
Mike Hobday, Head of Campaigns:
“We are extremely disappointed NICE has rejected the only drug available proven to give patients with myelodysplastic syndromes and acute myeloid leukaemia more time and a better quality of life. Azacitidine is just one of many drugs for rarer cancers to be rejected by NICE in the past year. The current system is failing people with rarer cancers. It’s time for a more flexible approach – one that doesn’t rule you out if you have the wrong kind of cancer.”

Leukaemia CARE
Tony Gavin, Chief Executive:
“Denying this innovative treatment to MDS patients and those progressing to leukaemia, will sentence them to a poorer quality of life and an earlier than necessary death. Further to a review of the economic and cost-effectiveness criteria used by NICE, increased consideration must be given to quality of life issues in appraising innovative treatment for rarer diseases.”

Leukaemia & Lymphoma Research
Kenneth Campbell, Clinical Information Officer:
“In cases such as MDS where there is no effective existing therapy, comparisons tend to be with very cheap, but ineffective therapies. This distorts the economic analysis as compared with an otherwise equivalent analysis where there is an expensive existing treatment, which is less clinically effective than the drug being appraised. In the latter case, a treatment which will cost the NHS much more than Vidaza for a similar gain will be ruled cost effective. This is inequitable as it means that the patients with the most desperate clinical need (as they have no effective treatment) face the highest barrier to approval of a treatment.”

The CML Support Group UK
Sandy Craine, Director
“As patients and advocates we remain angry, frightened and very frustrated by what many see 
as cynical decision-making by NICE, which seems to have more to do with cost cutting than 
with a rational assessment of effective therapies for British citizens suffering from life 
threatening diseases.”

**Rare Disease UK**

**Alastair Kent, Chair:**

“This is another example of the difficulties therapies for rare conditions face in satisfying 
NICE’s criteria. Due to the small market to recoup the significant research and development 
costs and the difficulty in collecting data on effectiveness, therapies for rare conditions are 
often expensive and at the high end or beyond what NICE considers to be cost effective using 
the ICER/QALY analysis. We believe the Department of Health and NICE need to work 
together to develop a new system of appraising therapies for rare conditions.”