

PRESS RELEASE -

Latest NICE guidance offers new hope for people with chronic myeloid leukaemia

The National Institute for Clinical Excellence (NICE) has today (22nd October) issued guidance on the use of imatinib for chronic myeloid leukaemia (CML) within the NHS in England and Wales.

The Institute's key recommendations are :

- that imatinib should be the first treatment considered for an adult with the Philadelphia-chromosome (the most common) type of CML in the chronic phase (the early stage of the disease when it is developing more slowly),
- that imatinib should also be an option for an adult who is diagnosed with the Philadelphia-chromosome type of CML in the accelerated or blast crisis phase (when the disease is becoming more aggressive).
- if a person in the chronic phase of CML is already taking interferon-alpha (an alternative treatment option for CML), the choice about whether to change to imatinib should be made by the doctor and patient after a discussion about the person's circumstances, the options and the side effects of the treatments.

Andrew Dillon, NICE Chief Executive and Executive Lead says, "This appraisal is another example of NICE working to ensure that new treatments that are clinically and cost effective are made available to patients across the NHS. The process NICE follows is genuinely consultative and involves bringing all of the evidence together and engaging with professionals and patients. Today's guidance should lead to a faster and more uniform uptake of this important new treatment."

Marc Stowell, Director of Operations at Leukaemia CARE says: “Whilst not a cure, this drug is set to revolutionise the treatment of chronic myeloid leukaemia and is already leading to increased survival rates and improvements to the quality of life in those currently receiving the treatment. Leukaemia CARE is delighted that NICE has approved imatinib for use in the NHS as a first line treatment for CML as now many more patients can benefit from it. As a patient organisation we very much appreciate the fact that the quality of life benefits for patients who receive imatinib have been taken into account within the NICE appraisal process. This guidance will be well accepted by many patients and carers who will have the knowledge of increased survival and will provide an immeasurable emotional benefit for those people who are directly affected.”

NICE issued guidance on the use of imatinib for second line treatment in adults with chronic phase CML who are intolerant of interferon-alpha therapy or in whom interferon-alpha is deemed to have failed to control the disease, and as a first line treatment in adults with CML in the accelerated phase or blast-crisis in September 2002. Following changes to the licensed indications of this drug, the Institute was subsequently asked by the Department of Health to develop the guidance on the use of imatinib for first line treatment in adults with chronic phase CML issued today.

Ends

Notes to Editors

1. A Question and Answer Document accompanies this release which explains why and how NICE developed its recommendations. It also answers some common questions.
2. The Institute’s guidance on the use of imatinib for first line treatment in adults with chronic phase CML can be found at <http://www.nice.org.uk/Docref.asp?d=89864> from 22nd October.
3. The Institute’s guidance on the use of imatinib for second line treatment in adults with chronic phase CML who are intolerant of interferon-alpha (IFN-a) therapy or in whom IFN-a is deemed to have failed to control the disease, and as a first line treatment in adults with CML in the accelerated phase or blast-crisis issued in September 2002 can be found at <http://www.nice.org.uk/cat.asp?c=37604>.

About NICE

4. NICE is part of the NHS. It is the independent organisation responsible for providing national guidance on treatments and care for those using the NHS in England and Wales. Its guidance is for healthcare professionals and patients and their carers to help them make decisions about treatment and healthcare. For further information about NICE you can visit www.nice.org.uk.
5. NICE produces guidance in three areas of health:

- the use of new and existing medicines and treatments within the NHS in England and Wales – technology appraisals.
 - the appropriate treatment and care of patients with specific diseases and conditions within the NHS in England and Wales – clinical guidelines.
 - whether interventional procedures used for diagnosis or treatment are safe enough and work well enough for routine use – interventional procedures.
 - NICE also funds three enquiries that undertake research into the way patients are treated to identify ways of improving the quality of care (the investigations are known as confidential enquiries).
6. NICE guidance and recommendations are prepared by independent groups that include professionals working in the NHS and people who are familiar with the issues affecting patients and carers.

About technology appraisals

5. Technology appraisals are recommendations on the use of new and existing medicines and treatments within the NHS in England and Wales, such as:
- medicines (for example, drugs)
 - medical devices (for example, hearing aids or inhalers)
 - diagnostic techniques (tests used to identify diseases)
 - surgical procedures (for example, repairing hernias)
 - health promotion activities (for example, patient education models for diabetes).
5. Our technology appraisal recommendations are prepared by an independent Committee, who include healthcare professionals working in the NHS and people who are familiar with the issues affecting patients and carers. The Committee considers the evidence on the clinical and cost effectiveness of the technology – this includes hearing the views of, and evidence from, clinical health professionals, experts and patients.
6. NHS organisations in England and Wales have to make the resources and facilities available to enable NICE guidance to be implemented. In January 2002 the Government announced a legal obligation for the NHS to provide funding for treatments and drugs recommended by NICE as a part of its technology appraisals work programme.

About Leukaemia CARE

7. Leukaemia CARE is a national Charity that provides CARE and support to patients, their families and carers during the difficult journey through the diagnosis and treatment of leukaemia or an allied blood disorder. Leukaemia CARE provides the only dedicated free phone CARE Line for patients and their families that enables people to discuss their feelings, concerns and emotions at such a difficult period of time.

For more information or to arrange an interview with someone who has CML and has been treated with imatinib call Marc Stowell on 01905 330003 or 07811 446 287. Website www.leukaemiaCARE.org.uk

The use of imatinib for chronic myeloid leukaemia – some common questions

What is NICE guidance?

The National Institute for Clinical Excellence (NICE) is part of the NHS. It produces guidance (recommendations) on the use of medicines, medical equipment, diagnostic tests and clinical and surgical procedures within the NHS in England and Wales.

To produce this guidance, NICE looks at how well the medicine, equipment or procedure works and also how well it works in relation to how much it costs. This process is called an appraisal. The appraisal process involves the manufacturer of the medicine or equipment for which guidance is being produced and the organisations that represent the healthcare professionals, patients and carers who will be affected by the guidance.

NICE was asked to look at the available evidence on the medicine imatinib and provide guidance that would help the NHS in England and Wales decide when it should be used.

What is chronic myeloid leukaemia?

Normally, a person's blood contains a balanced number of the different types of blood cell. The main types of blood cell are:

- the red blood cells, which carry oxygen around the body
- the white blood cells, which fight infection
- the platelets, which are involved in forming blood clots.

The 'starter' cells (known as stem cells) that develop into these types of blood cell are produced in the bone marrow, which is found inside bones. The different types of blood cell are produced by rounds of division (one cell divides to form two cells, which divide to form four cells and so on). This process is normally controlled so that the marrow produces the right numbers of the different types of cell. Once the cells have developed and matured, they enter the blood.

Chronic myeloid leukaemia (often shortened to CML) is a cancer of one of the main types of white cell. With CML, the normal processes of division and repair get out of control and too many immature and mature white blood cells are produced. The result is that the bone marrow becomes so full of these white blood cells that the production of the other types of blood cell suffers. Another effect is that immature white blood cells enter the blood but are not able to fight infection properly.

These effects explain some of the common symptoms of CML. A person with CML may become tired and breathless because there aren't enough red blood cells carrying oxygen round the body (because the bone marrow can't produce the normal healthy supply of red blood cells). People with CML are also more likely to suffer from infections because their white blood cells, which would normally fight off infections, aren't working properly.

Different stages of CML

There are three main stages ('phases') of CML:

- the chronic phase
- the accelerated phase
- the blast crisis phase.

During the chronic phase, the leukaemia is developing quite slowly. After a time (4–5 years on average), the person moves into the accelerated phase. In this phase, the cancer is developing more quickly – the immature white cells are filling up the bone marrow and preventing the production of healthy levels of other cells. After some months, the person moves into the blast crisis phase, in which the bone marrow is really struggling to make the other cells that the body needs. Some people with CML move from the chronic phase to the blast crisis phase without going through the accelerated phase.

What causes CML?

In most people with CML (nine out of ten), the problem lies in one of their chromosomes. Chromosomes are the bundles of genes found in all cells – we have 46 chromosomes. In most people with CML, parts of two of the chromosomes in their stem cells have become swapped over to give rise to an abnormal chromosome called the Philadelphia chromosome. The presence of the Philadelphia chromosome

means that stem cells carrying an abnormal protein occur in the bone marrow. It is the presence of the abnormal protein that causes the normal processes of cell division and repair to become out of control. So the abnormal stem cells divide to give very large numbers of abnormal white blood cells.

What is imatinib?

Imatinib is a medicine that recognises the cells that carry the abnormal protein and reduces the ability of the abnormal protein to disrupt the normal processes of division and repair.

It is currently licensed in the UK for the treatment of adults with the Philadelphia-chromosome type of CML who:

- have just been diagnosed, if a stem cell transplant isn't an appropriate first option, or
- are in the chronic phase, if another type of treatment called interferon-alpha has been unsuccessful, or
- are in the accelerated or blast crisis phase.

What has NICE recommended on the use of imatinib?

NICE has made the following recommendations about the use of imatinib to treat chronic myeloid leukaemia within the NHS in England and Wales.

NICE has recommended that imatinib should be the first treatment considered for an adult with the Philadelphia-chromosome type of CML in the chronic phase.

It should also be an option for an adult who is diagnosed with the Philadelphia-chromosome type of CML in the accelerated or blast crisis phase.

Imatinib should also be an option for an adult who:

- was diagnosed with the Philadelphia-chromosome type of CML when he or she was in the chronic phase, and
- has since gone on to the accelerated or blast crisis phase, but
- has not previously had imatinib.

If a person has been taking imatinib for CML while in the chronic phase, but still goes on to the accelerated or blast crisis phase, NICE has recommended that imatinib

treatment is continued only as part of a research study. This means that information should be collected on the treatment and the effect on the patient's CML. This information can then be looked at when NICE reviews its guidance in 2006.

If a person in the chronic phase of CML is already taking interferon-alpha, the choice about whether to change to imatinib depends to some extent on how well the interferon-alpha is working and how well the person is feeling. The decision about changing treatment should be made by the doctor and patient after a discussion about the person's circumstances, the options and the possible side effects and problems with imatinib.

What should I do next?

Imatinib is usually prescribed by hospital specialists. If you are unsure about whether this guidance applies to you, then you should discuss it with your doctor/specialist at your next appointment.

Will NICE review its guidance?

Yes. The guidance will be reviewed in September 2006.

Further information

The NICE website (www.nice.org.uk) has further information on NICE and the full guidance on the use of imatinib in CML that has been issued to the NHS. The guidance can also be requested from the NHS Response Line by phoning 0870 1555 455.

If you have access to the Internet, you can find more information on leukaemia on the NHS Direct website (www.nhsdirect.nhs.uk). You can also phone NHS Direct on 0845 46 47.

Leukaemia CARE is a national Charity that provides CARE and support to patients, their families and carers during the difficult journey through the diagnosis and treatment of leukaemia or an allied blood disorder. For more information please contact Leukaemia CARE on 01905 330003. Website www.leukaemiaCARE.org.uk