

Imatinib for chronic myeloid leukaemia

**Understanding NICE guidance –
information for people with chronic
myeloid leukaemia, their families and
carers, and the public**

This guidance replaces technology appraisal
guidance on this topic that was issued in
June 2002

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The use of imatinib for chronic myeloid leukaemia

Understanding NICE guidance – information for people with chronic myeloid leukaemia, their families and carers, and the public

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To order copies

Copies of this booklet can be ordered from the NHS Response Line; telephone 0870 1555 455 and quote reference number N0337. A version in Welsh and English is also available, reference number N0338. Mae fersiwn yn Gymraeg ac yn Saesneg ar gael hefyd, rhif cyfeirnod N0338. The NICE technology appraisal on which this information is based, *Imatinib for Chronic Myeloid Leukaemia*, is available from the NICE website (www.nice.org.uk). Copies can also be obtained from the NHS Response Line, reference number N0340.

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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 and quoting reference number N0340.

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.



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