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

Rarer Cancers:

Your constituent, their treatment and how you can help.

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MP Rarer Cancers Toolkit
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1. Introduction

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The MDS UK Patient Support Group was established by patients and clinicians to provide help and support to people with myelodysplastic syndromes (MDS), a rare form of blood cancer. The group provides a network of support for those diagnosed with MDS; organises meetings and events; provides information about where to get specialist treatment and campaigns to ensure that people with MDS get the best treatment and care available.

This **'MP Rarer Cancers Toolkit'** is designed to help MPs and their staff answer questions from constituents about rarer cancers. It also provides an overview of the barriers to treatment access for people with rarer cancers which have become big political issues in recent years.

As many as 50% of all cancers can be described as 'rare'. This includes all except the 'big four' tumour types of bowel, breast, lung and prostate. Yet The All Party Parliamentary Group on Cancer 'Inquiry into Inequalities in Cancer' [i]published in 2009 revealed that there are significant inequalities in access to diagnostics and treatment for people with rarer cancers that needs to be addressed. A recent survey of MPs published by MDS UK revealed that 8 out of 10 Members agreed that patients with rarer forms of cancer should have the same access to new treatments in the NHS as patients with more common tumour types, which is very encouraging. Less heartening was that MP knowledge about the institutions and processes by which cancer treatments are made available in the NHS is quite poor. MDS UK have produced this Toolkit to help increase understanding about the challenges faced by people with rarer cancers but also provide practical information about where to direct constituents with questions and concerns about their disease.

My role in the MDS UK Patient Support Group is as chairman, but I am also a physician who has MDS. I believe that it is absolutely essential that MPs, and others in positions of power in directing healthcare resources, should understand what rarer cancers are and what MDS means to patients, families and carers. That is the reason that we are providing you with this toolkit.

If you have questions or comments, please contact us at MDS UK on 020 7733 7558 or by emailing mds-uk@mds-foundation.org.

2. What are rarer cancers and blood cancers?

Rarer cancers

Almost 50% of all new cases of cancer diagnosed each year in the UK can be described as 'rare'. These are those outside the 'big four', most prevalent cancers including breast, lung, bowel and prostate. The term 'rarer cancers' therefore can be used to describe hundreds of sorts of cancers, some with much small patient populations than others.

Rarer cancers are not always the less common tumour sites. As research continues and a deeper understanding of the more common cancers has developed, smaller subgroups are being identified that have different treatment requirements and prognoses too, so the concept of rarer cancers becomes increasingly important.

Rarer cancers can be treated but access to diagnostic tests and treatment can be difficult. Research and development costs of newer treatments for rarer cancers are often very high making treatment often prohibitively costly.

Myelodysplastic Syndromes

- Myelodysplastic syndromes (MDS) are a group of rarer cancers of the blood which are **caused by failure of the bone marrow to produce normal blood cells.** The cells that are produced do not function properly and have a greatly reduced life.

- The consequences of this are that **patients become anaemic with associated fatigue** due to the inadequacies of red blood cells. They are also more prone to infection because of impairment of white blood cells and they have a tendency to bruise and to bleed because their platelets do not function effectively.

- **MDS affects 2,000-3,000 new patients each year in the UK**, which is around five in each Parliamentary constituency and less than twenty in an average District General Hospital. This is an incidence of around 1 in 20,000-30,000 people, meaning that the average GP might see a new case once or twice in a working career.

- **Life expectancy depends on the characteristics of the disease in the individual patient**. In many cases, MDS progresses to acute myeloid leukaemia (AML), a very aggressive form of leukaemia with an average survival period of just six to twelve months. Median survival, depending on risk level, ranges from a matter of months to five years or so.

- Until February 2011, when NICE approved the use of azacitidine for the NHS, there were no treatments available to treat MDS. **Azacitidine is the only active treatment** for MDS that has been proven to prolong life.

Blood Cancers

➢ Blood cancers encompass a group of disorders that affect the body's ability to produce blood cells. The major abnormality in cancer of the blood is the failure of blood cells, produced in bone marrow, to mature fully.

➢ There are three main types of blood cells and a correct balance between each of their populations must be maintained for normal health:

Red cells: which carry oxygen to all tissues of the body

White cells: which fight infection

Platelets: which help clotting of the blood

➢ In leukaemia, normal control mechanisms break down and the bone marrow starts to produce large numbers of abnormal cells of one type. The large number of abnormal white cells can result in a severe drop in normal white cell production.

Symptoms

➢ Blood cancers, especially chronic leukaemias, are often difficult to diagnose. The symptoms can be slow to develop and vary depending on type of diseases and advanced it is.

➢ Early signs are flu like symptoms and also tiredness, breathlessness and pale skin due to anaemia caused by the reduction in number of red blood cells.

Causes

The exact causes of blood cancers aren't known, although there are some risk factors that can increase the chances of developing it including:

- A weakened immune system
- Age – some leukaemias are more common in people over 40
- Gender - slightly more men than women are affected by leukaemia
- Smoking
- Certain genetic conditions, such as Down's syndrome
- Other blood disorders, such as aplastic anaemia - a rare condition where the bone marrow fails to produce any of the blood cells.

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‘Orphan’ and ‘Ultra Orphan’ Diseases

- The term ‘orphan disease’ is often used to describe conditions that affect only a small number of patients.
- The term is derived from the legal classification for ‘orphan drugs’ used in EU legislation to describe a drug indicated for disease with a prevalence of less than 5 patients per 10,000 of the EU population.
- ‘Orphan disease’ and ‘orphan drug status’ are not classifications recognised in the UK. Treatments given ‘orphan drug status’ by the European Medicines Authority still go through the same NICE process as treatments with larger patient populations.
- The wide range of conditions that fall within the definition of ‘orphan diseases’ has led to the emergence of an informal subcategory, called ‘ultra-orphan diseases’, to describe extremely rare conditions. The term has no formal legal definition but treatments for these very rare diseases, commonly those that affect fewer than 1 case per 50,000 of the population do not go through the NICE process before being used in the NHS. Instead they go through National Commissioning and funds are allocated for their use at a national level, instead of through PCTs.
- National Commissioning recognises that the high cost of treating a very small number of patients with very rare conditions should not be shouldered locally by PCTs, but taken from a national pot.

3. The Blood Cancer Patient Pathway

This is the typical journey that a person with a blood cancer will travel from presentation of symptoms to treatment in a specialist centre.

A. Referral

Some patients may be identified by the GP or in a hospital clinic on the basis of a 'routine' blood test. Others may present with symptoms of fatigue, a proneness to infection or a tendency to bruise and bleed. All should be referred to a consultant haematologist who might then refer the patient on to a 'centre of excellence' with a special interest in their particular condition.

B. Diagnosis

A haematologist will undertake blood samples which will be tested for deficiencies of normal blood cells and for the presence of abnormal white blood cells. Depending on the results, further tests may be needed such as:

- Bone marrow biopsy, to examine all the developing blood cells under a microscope and to examine the structure of the bone marrow.
- Analysis of the chromosomes and DNA of the abnormal cells found in the blood and bone marrow (cytogenetics).
- Analysis of the antigens present on the leukemic cells (immunophenotyping).
- CT, MRI, X-ray or ultrasound scans to look for enlarged lymph nodes, an enlarged spleen or other tumours.

C. Treatment

How well treatment works depends on the type and stage of the disease. Acute leukaemia often goes into remission. This is when the symptoms go away and the disease is under control, but not necessarily cured. Unfortunately, for many people with acute leukaemia, remission is followed by a relapse.
Initial treatment may include antibiotics, blood transfusions, platelet transfusions and growth factor injections. Then, depending on the type of leukaemia, treatment may involve:

- **Chemotherapy:** Medicines that destroy cancer cells
- **Radiotherapy:** X-rays that target cancer cells
- **Bone marrow or stem cell transplant:** Transferring another person's (preferably a close relatives') healthy bone marrow or stem cells into the body after treatment to reduce the risk of the body rejecting the transplanted cells.
- **Monoclonal antibody therapy:** Medicines that are designed to recognise and target markers that are commonly carried by cancer cells.

4. Access to treatments for rarer cancers:

A guide for assisting with constituent enquiries

This section provides background information and answers to frequently asked questions about the application of NICE guidance and the methods by which it is formulated. It also includes information on the options for patients should a treatment they have been prescribed not be recommended for use in the NHS.

What is NICE?

- The National Institute for Health and Clinical Excellence (NICE) makes recommendations to the NHS about the clinical and cost effectiveness of new treatments and procedures.
- They evaluate almost all cancer medicines licensed for use in the UK, normally within six months of them becoming available. NICE's value for money judgements are then used by PCTs to decide whether they will make money available to fund these therapies on the request of clinicians writing the prescriptions.
- NICE guidance primarily only applies to NHS organisation in England. However some guidance is followed in Scotland on the advice of NHS Quality Improvement Scotland. NICE guidance supersedes advice from the All Wales Medicines Strategy Board and is also followed in Northern Ireland on the advice of the Regulation and Quality Improvement Authority (RQIA).

NICE to know

- PCT's are obliged to make funds available for new treatments within three months of NICE issuing a positive recommendation.
- Patients with rarer cancers who are eligible for treatment with licensed medicines, recommended by NICE, should not be refused access where their clinician has prescribed it.
- Prior to NICE completing their evaluation, PCTs are not allowed to use the lack of NICE guidance as a reason for turning down requests for new treatments or to anticipate the outcome of their appraisal.
- Before NICE has issued guidance, many PCTs follow the guidance of their Cancer Network on whether or not to make new treatments available. Collaborations of networks such as the London New Cancer Drugs Group (LNCDG) undertake their own analysis of new cancer treatments which Cancer Networks in London as well as others, use to advise PCTs prior to NICE issuing their final guidance.

How NICE makes recommendations

- The NICE Health Technology Assessment (HTA) methodology makes an assessment of the cost-effectiveness of new technologies compared to current standard practice. It was introduced to ensure that NHS funds are focused on those treatments that improve the quality and/or length of someone's life and also represent an effective use of NHS resources.

- Expert review groups, comprising both health professionals and patients, examine evidence on how well a drug works and whether it provides good value for money.

- The calculation used by NICE to measure the **clinical effectiveness** of new treatments is quality-adjusted life years or 'QALY'. A QALY gives an idea of how many extra months or years of life of a reasonable quality a person might gain as a result of treatment.

- **Cost effectiveness** is calculated by looking at how much a drug or treatment costs per quality adjusted life year gained. Put another way- the cost of using the drugs to provide a year of the best quality of life available.

- Even though each drug is considered on a case-by-case basis, generally, if a treatment in above the £20,000-30,000 per QALY range, then it is not be considered cost effective for use in the NHS.

NICE HTA and Rarer Cancers

Innovative treatments for small patient groups that address a currently unmet need and have no equivalent treatment to compare it to, will often have a cost/QALY above NICE's £30,000 cost effectiveness threshold and are not customarily recommended for use in the NHS.

This means that PCTs are not obliged to make funding available when a clinician prescribes treatment however they can still make decisions on a case by case basis through Individual Funding Requests

Individual funding requests (IFR)

An individual funding request (IFR) is a request to a PCT to fund an episode of healthcare that is not routinely provided at present. This normally occurs where a patient falls outside an existing generic or treatment-specific policy or where patients have a very rare clinical condition.

The process for IFRs can vary between PCTs. It is therefore important to check the information made available each PCT's website or from the Patient Advice and Liaison Service (PALS). This will outline the local procedure for making an IFR and the time in which patients can expect their request to be considered.

When making an IFR, a patient will be asked to provide information including:

- Medical history
- Information about alternative treatment options
- Cost and length of treatment
- Expected benefit of treatment

A patient's clinician will be able to help with the submission.

Exceptional Cases

Exceptional cases procedure is used where a PCT has a policy in place that rules against the funding of a particular medicine. An Exceptional Funding Request made by a clinician, on behalf of a patient, to the PCT must demonstrate the exceptional reasons why a patient should receive treatment.

Appeals

If a PCT turns down an IFR or Exceptional Funding Request, patients have the right to appeal against the decision. The appeal will consider whether the procedure followed during the original IFR was fair and rational. Where new information is made available, the PCT is likely to require a new IFR rather than an appeal.

- To make an appeal, a patient will need to:
- Find out the grounds under which your PCT will allow an appeal
- Find out who is on the appeal panel
- Obtain copies of all written documentation associated with your IFR. If your PCT does not make this readily available, your constituent can access this with a Freedom of Information request.

Impact on Patients

The IFR and Exceptional Cases procedure can be a prolonged and very stressful ordeal for patients awaiting treatment. The NHS National Prescribing Centre (NPC) provides advice and guidance to PCTs on how to manage the process swiftly while ensuring that patients are informed of the outcome of their hearing in a timely and compassionate way.

Further information can be found at www.npc.co.uk

Cancer Drugs Fund

The Cancer Drugs Fund was introduced by the Government to fund new drug treatments and support treatment for patients eligible for care under the NHS, who have been unable to access clinical treatments recommended by their oncologist.

The full fund with a budget of up to £200m a year has been available since April 2011. Prior to that, and in recognition of the need for some patients to access treatment now, the Government has introduced an Interim Cancer Drugs Fund of £50m available from 1st October 2010. The fund is being allocated by new clinical advisory boards in SHAs.

It is worth noting that the CDF does not cover Northern Ireland – and that patients there experience severe problems accessing life extending drugs. Northern Ireland is supposed to create their own CDF to overcome this clear disadvantage compared to the rest of the country.

Interim Cancer Drugs Fund

- The scheme did not replace the standard IFR process but was intended to complement it.
- SHAs have to put in place transparent arrangements that are acceptable and equitable to patients, clinicians and commissioners and provide equity and the best use of the resources available.
- The criteria for access to the fund should be based upon evidence of clinical effectiveness and deliver measurable outcomes such as overall survival, progression-free survival and improved quality of life.
- The processes should be simple to administer and the use of drugs monitored/audited to ensure resources are used in accordance with the agreed arrangement and required outcomes are delivered.

Early evidence showed that the Interim CDF was effective in enabling some patients to access medicines where NICE has yet to provide guidance or where the treatment is not recommended for use but the patient falls within the licensed indication and their oncologist feels that they would benefit from treatment.

However – later evidence showed that the full budget dedicated to each SHA was not use to the full for the intended purpose. Many SHA's underused the ICDF and the funds were not recuperated.

Currently – a minority of SHA's are still trying to limit the use of some drugs by requesting staff to complete additional IFR forms or CDF forms.

Value Based Pricing

The Cancer Drugs Fund has been introduced as a stop-gap to enable cancer patients to access innovative and high cost medicines not currently recommended for use in the NHS by NICE, before Value Based Pricing (VBP) is introduced in 2014.

A Value Based Pricing mechanism would replace the profit and price control elements of the current system, whilst allowing negotiation of prices based on their clinical value. Therefore new and innovative medicines that demonstrate significant benefits to patients, over those products already on the market, will be valued higher in the NHS. The price of a product will therefore be linked directly to its value, enabling more treatments for rarer cancers that provide a currently unmet need, to be made available in the NHS.

Moving to a Value Based Pricing System

The government intends to implement a common pricing system across the UK for new active licensed treatments placed on the market from 1 January 2014 called value based pricing. VBP is about setting out how, in valuing any new medicine, other factors beyond clinical effectiveness and price can be taken into account. VBP is therefore an attempt to redefine how value is assessed. A broader assessment of 'value' will be used to consider the wider societal and economic benefits of different treatments before arriving at a price.

4. VBP policy: purpose and aims

According to the Department of Health, the purpose of VBP is to "improve NHS patients' access to effective and innovative drugs by ensuring they are available at a price that reflects the value they bring" (DH, Dec 2010: 11). The central aims of the policy are listed as follows:

- improve outcomes for patients through better access to effective medicines;
- stimulate innovation and the development of high value treatments;
- improve the process for assessing new medicines, ensuring transparent, predictable and timely decision-making;
- include a wide assessment, alongside clinical effectiveness, of the range of factors through which medicines deliver benefits for patients and society;
- ensure value for money and best use of NHS resources.

5. Core components of VBP

The detail of how any new system may operate has not been set out fully either in the initial consultation in Dec 2010, or in the government's response in July 2011. Despite the lack of detail, the DH has made it clear that we can expect to see the following four key components within any new system.

5.1. Burden of illness

This refers to the seriousness of the condition. The DH have commissioned research (EEPRU, 2012) aimed at understanding how society values various health states to inform how calculations and weightings could be applied to the QALY to capture 'burden of illness'. How burden of illness will be measured or weighted under VBP is not known.

5.2. Innovation

There will be a therapeutic innovation and improvement (TII) weighting applied to those treatments considered to be 'innovative'. Treatments that may fall under this category may have a more favourable mode of action, fewer side effects or improve patient experience. Kennedy attempted to capture what 'innovation' may mean in the context of a NICE appraisal; he attempted to define what an innovative medicine would look like and came up with a potential list (Kennedy, 2010: 24). The DH are clear that, "innovation is only meaningful in terms of the benefits that a product provides to patients and society" (DH, 2011: 20). It is intended that in assessing innovation, the government is encouraging companies to develop treatments in those areas of high unmet need. How innovation will be measured or weighted under VBP is not known.

5.3. Societal benefits

It is possible that under VBP, the societal impact of a treatment will form an explicit part of any assessment of a new treatment. This was widely supported in the consultation responses the DH received. This would include the impact on carers, and the potential for a treatment to allow an individual to return to work. Again, calculations and weightings to be applied to the QALY are dependent on the outcomes of research commissioned by the DH.

5.4. Threshold

As part of value based pricing there would be a basic cost effectiveness threshold, directly reflecting the health gains displaced when new treatments are funded. This would set the maximum that the government was prepared to pay for medicines that offered no additional value in terms of innovation, tackling diseases with a high burden of illness or wider societal benefits. Currently the threshold is set at £30,000 with the exception of end of life treatments, where the threshold has been raised to £40,000. Under VBP, it is possible that the threshold could change subject to New evidence; speculation would suggest anynew threshold will be lower, and not higher, and around £20,000 mark.

It is likely that the DH will publish a further consultation on VBP before the end of the year.

Impact on NICE

In their submission to the DH on VBP, NICE suggest rather than implementing a whole new system of valuing medicines, the DH should instead consider building on the current NICE technology appraisal process to develop a single integrated process (NICE, 2011). NICE fundamentally believe that their current approach can be extended to incorporate all dimensions proposed on the consultation document. They envisage a new system to work as follows:

- Submission of a 'value dossier'
- Expert panels to assess and weight burden of illness
- An appraisal committee that will assess burden of illness, therapeutic innovation and wider societal values
- Final guidance to the NHS published by NICE

5. Getting your constituent the help they need

It is important to note that this toolkit is intended to provide a overview of the main issues, terminology and organisations involved in the rarer cancers treatment pathway, not a definitive guide to your local services. PCTs and SHAs are responsible for commissioning cancer services and making funding available for treatments and therefore it is important that you contact your local services to get up-to-date information on their policies and procedures.

Patients should first and foremost be encouraged to discuss their treatment decisions with their clinician. However, your constituents may contact you to help with any number of problems associated with rarer cancers and you may be able to help them by:

- **Encouraging** your constituent to speak to their clinician to advise them on the best possible treatment for their cancer.
- **Exploring** the possibility of an IFR with their clinician if treatment is not available on the NHS and encourage them to find out more about this from their PCT.
- **Contacting** a patient support group who will be able to provide advice and support to your constituent throughout their treatment.
- **Seeking advice** from Citizen's Advice and Macmillan Cancer Support to discuss the other aspects of help that they might benefit from after their diagnosis for example legal or financial support, travel insurance or child care advice.
- **Writing** to the Department of Health and/or NICE where you have concerns about the treatment that your constituent has received.
- **Provide** your constituent with advice on your PCT's complaints procedure if they feel that their case has not been given full or prompt consideration. You may also wish to write to your PCT to urge them to deliver a timely response or to reconsider their decision.

Resources:

- You can find out if a treatment has been licensed for use in the UK by going to the European Medicines Agency Website.
- You can find out if a treatment has been recommended for use in the NHS via the 'Published Appraisals' page of the NICE website.

References:

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http://www.nice.org.uk/newsroom/features/measuringeffectivenessandcosteffectivenesstheqaly.jsp

iv NHS London Strategic Health Authority, 'Interim Cancer Drugs Funding Arrangement for London' (NHS London SHA, October 2010), p2
v Office of Fair Trading, 'The Pharmaceutical Price Regulation Scheme: an OFT market study' (OFT, February 2007), http://www.oft.gov.uk/shared_oft/reports/comp_policy/oft885.pdf

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