

YOU HAVE BEEN GIVEN THIS LEAFLET BECAUSE YOUR DOCTOR CONSIDERS THAT A DRUG CALLED MEPACT IS AN APPROPRIATE TREATMENT FOLLOWING SURGERY FOR OSTEOSARCOMA

The NHS uses a variety of methods to decide whether to make treatments available to patients. Decisions for the treatment of osteosarcoma are mostly made at a national level and are covered by blanket policies so that individual patients all receive the same standard of care.

The new drug Mepact (mifamurtide) is not included in this national policy. The National Institute of Health and Clinical Excellence (usually known as NICE) is reviewing Mepact and we are hopeful that national approval will be given before long so that doctors who wish to prescribe it for their osteosarcoma patients can do so.

However until that approval is given each time a doctor wants to prescribe Mepact he must apply to the Primary Care Trust (PCT) which is responsible for funding that individual patient's care. The process is known as an 'individual funding request'.

What follows is based on the assumption that the doctor responsible for the treatment has decided that Mepact is appropriate treatment for the patient, that the patient/parents/guardians (as appropriate) have been fully informed about the drug and have consented to the course of treatment, and that the doctor is proposing to make an application for funding.

The PCT has complete discretion to make the decision to fund Mepact or not. The doctor will complete an application form and submit evidence from clinical trials to support the application. The PCT's initial decision can be appealed if it refuses to fund the treatment but our experience of appeal processes is that they take a lot of time and cause a lot of stress.

So, our suggestion is that the doctor's request to the PCT is supported by letters from:

- the patient
- parents/guardians (as relevant)
- the patient's GP
- his/her school or college
- other appropriate local voices

We have attached some ideas about what these letters might say, indicated who they should be sent to, and give below some further advice about the process.

ABOUT OSTEOSARCOMA

Osteosarcoma is the commonest primary bone cancer and the third most common cancer in children. However, it is very rare. The incidence is around 2 to 3 cases per million of population each year which means there are about 150 cases a year in the UK of which about half are more elderly people. It is commoner in males than females.

Surgery for osteosarcoma is nationally funded by the NHS through five hospitals in England serving England and Wales (arrangements for Scotland and Northern Ireland are slightly different). Patients have a period of induction chemotherapy with the aim of getting the tumour under control, then surgery, then follow-up (adjuvant) chemotherapy to try and ensure that any remaining tumour cells are destroyed. The nature of the surgery will differ according to the location of the tumour. It occasionally requires an amputation.

Patients under the age of 30 and with a complete removal of the tumour confirmed by pathology following the surgery, may be eligible for Mepact. In trials Mepact has shown a reduction in death of almost one-third. This was measured over ten years for a group of patients treated in a clinical trial. Mepact must be given in combination with chemotherapy and it works by accelerating the cell death of the micro-metastases which can cause a recurrence.

We estimate (data are not available) that this group of patients numbers 50-60 per annum in the UK.

The marketing licence for Mepact issued by the European Commission says the following:
MEPACT is indicated in children, adolescents and young adults for the treatment of high-grade resectable non-metastatic osteosarcoma after macroscopically complete surgical resection. It is used in combination with post-operative multi-agent chemotherapy. Safety and efficacy have been assessed in studies of patients 2 to 30 years of age at initial diagnosis.

WHAT TO DO NOW ...

STEP 1

Identify your Primary Care Trust and note their main office address. Your hospital doctor may not immediately know which PCT is responsible but your GP will know and the receptionist should be able to give you the name and full address. If you have access to the internet you will also be able to access the PCT's own website. The PCT can also be identified through the alphabetical list at <http://tinyurl.com/yhuncrg> which gives the main address and also has links to the website. If possible identify the name of the Chief Executive. This is not always easy.

Name of Chief Executive:

Name of PCT:

Telephone number:

Address:

STEP 2

Contact the PCT. The Patient Advice and Liaison (PALS) office should be able to send you details of the procedure for applying for funding, which will already have been made available to your doctor. They will also be able to advise you of the timetable you need to keep to for sending any letters to the PCT.

They should also give you information about what the PCT's committee will be looking for if it is going to award funding. Every PCT is different so it is not possible here to give a complete breakdown.

Under the NHS Constitution you have the right to expect local decision-making about funding treatments to be made rationally following proper consideration of the evidence. If they decide not to fund a treatment you and your doctor believe is the right one for the patient, they are required to give you a full explanation of that decision.

STEP 3

Contact your GP and ask for a letter of support to be sent to the PCT requesting that Mepact is funded. Your GP may not know about the treatment so might want further information. This is readily available on the internet and can also be obtained from the manufacturer, Takeda UK Ltd on 01628 537 900. Ask for a copy of the GP's letter to the PCT.

The principle research reference is:

Osteosarcoma: The Addition of Muramyl Tripeptide to Chemotherapy Improves Overall Survival – A Report from the Children's Oncology Group
Myers et al. Journal of Clinical Oncology Vol 26 No4 February 2008

STEP 4

If the patient normally attends school contact the school's headteacher and ask if the school would be willing to send a letter of support. The best way for that to be done will vary according to how the school normally works. It may involve Governors, teachers, or pupil friends of the patient. If the patient is a college student contact the Principal to ask for a letter of support.

Give them the PCT address and suggest that the letter is addressed to the Chief Executive.

Ask the school/college to keep a copy of its letter to the PCT.

The same approach may be used with organisations and clubs of which the patient is a member (eg religious organisations, Scouts/Guides, sports clubs, choir/orchestra etc). Again try and ensure that copies of any letters are kept.

STEP 5

Write a simple history of the progress of the diagnosis and treatment which has already happened, and which is planned.

Date of first visit to GP

How many visits before referral to consultant

Date of first visit to consultant

Date of first visit to specialist osteosarcoma unit

Date of confirmed diagnosis

Location of the tumour

Date of start of chemotherapy

Date of surgery

Nature of surgery (eg bone replacement, amputation)

Start date for post-operative chemotherapy

STEP 6

Draft letters which will be sent from patient and parents (if relevant). One aim of the letter(s) is to identify that this request for funding is an exceptional situation. We have some suggestions for paragraphs which can be used to help explain why this is the case.

A personal letter from the patient should be in his/her own words and might describe how they are feeling, having come part of the way through treatment, but knowing that more is needed. They can say how they know that the doctors have done their best to cure the disease but that this new treatment has shown a big difference in preventing recurrence, and the PCT can make that difference a real chance for personal long term survival. Talk about ambitions, getting fully mobile again, and getting back to living a normal life

A parental letter can concentrate more on emphasising the exceptional nature of osteosarcoma and this new treatment but should also refer to the role the youngster plays in society, helping neighbours, school/sport/clubs etc. The relationship with brothers/sisters (if relevant) is also important to mention.

SOME SUGGESTED PARAGRAPHS FOR LETTERS

(name of patient) is one of very few patients each year who will be eligible for treatment with Mepact. Firstly osteosarcoma is rare and any cancer in children and teenagers is rare. We have been told the number of people in the UK who will have a complete resection and therefore be eligible for this treatment is between 50 and 60. This is therefore an exceptional situation, made the more exceptional by the long term benefit which clinical trials have shown for this small group of patients – almost a one-third reduction in the risk of dying from the disease.

There are no alternatives to treatment with Mepact. If funding is refused (name of patient) will have been refused the chance to benefit from a treatment which reduces the risk of death by almost one-third.

Summarise the diagnosis/treatment pathway you have recorded earlier in this leaflet. Emphasise the amount of time it has taken from first having symptoms (first visit to GP) to now. Treatment with Mepact lasts for up to 36 weeks so by the time it finishes most patients will have been in active treatment for well over a year. Emphasise the family's commitment to supporting their patient through everything, as they have done so far, so this treatment is important to them too.

KEEP COPIES OF ALL LETTERS

WHAT HAPPENS WHEN EVERYTHING HAS BEEN SENT

The information you have from the PCT should tell you when the committee will meet to decide on the funding.

Some PCTs have been known to invite patient/parent to the meeting and it is a good thing to go if at all possible. Prepare a few words to say about the importance of the treatment for your patient and family – write them down and be happy to read them if necessary. Be ready for some questions, they will know how nervous you will be and they will not rush you, so take your time answering, give them the information you want them to know.

The PCT will contact your doctor shortly after the decision is made, and they should also contact you direct. If funding is awarded your doctor will immediately start to make the necessary preparations to start treatment.

Experience in recent years has shown that about two-thirds of all requests for cancer treatment through this route are awarded funding. A significant percentage of those refused funding are then awarded funding following an appeal. Few treatments for children's cancer have yet been subject to the 'individual funding request' route so we have no way of knowing whether PCT's will be more or less sympathetic.

If funding is refused we recommend an appeal. [Please contact us at the addresses below.](#)

You should immediately ask the PCT for copies of the minutes of the meeting, copies of any advice they were given, and a full explanation of the discussion and decision in line with the NHS Constitution.

The best source of immediate advice at this point is at <http://www.pamelanorthcottfund.org.uk/>

It will also be appropriate to inform the local press of the PCT's decision. The copies you have of all the letters supporting the funding request will be valuable here.

We also recommend contacting your MP – their name and address can be found at <http://www.parliament.uk/mpslordsandoffices/index.cfm> or from your local council.

Other reading:

The Rarer Cancers Forum leaflet "Getting the Treatment You Need" can be obtained from the Rarer Cancers Forum on 01227 738279

Contacts if an appeal is necessary:

Kate Spall	Pamela Northcott Fund	kate@pamelanorthcottfund.org.uk
Roger Wilson	Sarcoma UK	info@sarcoma-uk.org
Kate Connor	Bone Cancer Research Trust	kate.connor@bcrt.org.uk

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