

# Sarcoma UK

Third Edition  
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**Sarcoma UK**

## Developing Sarcoma Treatment Guidelines

### Making our Voice Heard

Our front page story is about the development of sarcoma guidelines by NICE the National Institute of Clinical Excellence). Three sarcoma patients (one each with soft tissue, bone and GIST) are involved in the Guidelines Development Group, as equals with clinicians and administrators.

Two sarcoma patients serve on the National Cancer Research Institute's clinical study group for sarcoma. At a recent workshop on the new National Cancer Tissue Resource, hosted by Macmillan there were four sarcoma patients and carers involved.

Sarcoma UK is working with GIST Support UK and Cancer Bacup on representations to NICE over the technical appraisal of Glivec for the treatment of advanced GIST. Sarcoma UK, as a patient organisation, has also made representations to the European Commission about Yondelis (ET743).

Tell us what you are doing locally, regionally, or about any national work. We are starting to create a voice for sarcoma. Lets be heard !

*Roger Wilson*

The National Institute for Clinical Excellence (NICE) has begun work on service guidelines for the treatment of sarcoma in the NHS. The guidance development group includes clinicians from all the disciplines involved, nurses and healthcare professionals, patients and NHS service managers. The guidance will cover service organisation, referral and clinical support. It will be in place by mid-2005. It is expected work on full guidelines for the treatment of sarcoma will then be developed as well.

At present the organisation and structure of sarcoma services is left to each Cancer Network to decide. In England there are 34 networks, there are 3 in each of Wales and Scotland, and one covering Northern Ireland.

The service picture across the country is variable. The number of centres offering specialist multi-disciplinary treatment is small and they are mostly centred in teaching hospitals. The route which patients take to reach these tertiary centres has improved significantly in recent years and the specialist clinicians are seeing an increase in the number of sarcoma patients they treat. However there is no consistent picture on important issues such as to which consultant a GP

should refer a suspected sarcoma patient, or guidance about when a patient should be referred from a local hospital to a specialist centre.

Scotland has service guidance already and NICE will deliver the guidelines for England and Wales next year. There will be an implementation period before the recommendations are fully in place. The period will vary around the country.

While it seems a long time before NICE moves on to tackle treatment guidance the leading specialist clinicians are taking steps to publish guidelines on treatment standards themselves. These will be based on evidence from clinical research and clinical trials undertaken all around the world. While it will not be a requirement on any doctor to follow these guidelines once they are in place they will describe best practice and they will be continually revised as new treatments are developed.

The creation of the British Sarcoma Group – a professional association of clinicians and health professional specialising in sarcoma treatment (see page 5) – will give these guidelines greater force and this work could shorten the time taken by NICE to reach agreement on formal treatment guidance to which doctors would be expected to conform.

Inside:

A Pink Rolls for Penny  
The British Sarcoma Group

The Glivec Story  
Lee & the Lottery

## HOPE FOR YONDELIS CONTINUES

The Spanish bio-technology company Pharmamar is continuing its commitment to the innovative drug Yondelis (also known as ET743, or trabectedin) despite being refused marketing permission by the European medicines regulator EMEA.

An appeal to EMEA against the refusal failed by a narrow margin (14 to 12 with 1 abstention). Despite strong representations from clinicians and patient groups seeking to have the EMEA's recommendation overturned the European Commission has endorsed the EMEA decision. The main sticking point has been that there has been no randomised controlled clinical trial comparing Yondelis with today's standard drugs.

The requirement to have a large scale comparative trial before any marketing is licensed presents a problem. Such trials in soft tissue sarcoma, even on a pan-European basis, take a long time. Pharmamar's application was based on limited trials with patients suffering from advanced disease and the licence request was only for use by patients who have failed all standard therapies, so it was a doubly disappointing decision.

Pharmamar is running a clinical trial in the USA. This trial is showing responses in about 10% of patients with advanced disease, but more importantly a larger number of patients have seen their disease stabilised for a period of time. Yondelis is not a cure but it is helping patients with otherwise untreatable sarcoma to live longer.

Yondelis is the only new drug on the horizon for the treatment of advanced and metastatic soft tissue sarcoma although small Phase 1 clinical trials of possible new treatments happen regularly.

## PINK ROLLS FOR PENNY

In 2003 Penny Tong attracted attention by raising funds for sarcoma research at the Christie Hospital leading a sponsored walk with the help of a Centurion tank. This spring the walk was led by a pink Rolls Royce with Penny, who was too unwell to walk herself, encouraging the walkers from an armchair in the boot.

The walk wends its way between six pubs in the remote west of Shropshire and was supported by a hundred or more local people walking, cycling, and stopping off to sample the delights of the various hostelrys.

If you want to raise funds for research Sarcoma UK is starting a research fund and a number of leading sarcoma doctors have agreed to advise us on making grants.



# FINDING OUT

**The first stop for information about your case and your treatment should be your doctors. The hospital specialist will have the latest information and will be able to tell you most about the treatment appropriate for you. Your GP will have some information but remember that on average a GP will only ever have one sarcoma patient in his whole career ! Sarcoma is that rare !**

General sarcoma information is available from Cancer Bacup. They have good booklets, which have recently been revised, on Soft Tissue Sarcoma and on Understanding Primary Bone Cancer. There are also good booklets on treatments such as Radiotherapy and Chemotherapy. Your hospital information centre may have them, if not call Cancer Bacup (phone number below) and they will post them to you straight away. Some hospitals also have produced their own information which may be available from a nurse specialist or their information centre.

If you have access to the Internet there is a wide range of information available, from around the world. Even if you cannot access the Internet yourself there may be someone who can do it for you and print out the pages they find. It could be quite a lot of paper !

Information can help you decide what questions you want to ask. Doctors or nurses don't mind being asked, they would rather hear all the questions you want answered and they will spend time answering them. Their focus is on you, the patient.

If you have been diagnosed with advanced sarcoma, information is just as important as it is for the newly diagnosed, though it may be of a different kind. Treatments such as chemotherapy have a lot of mystique about them and raise fears of their own. Rather than listening to old stories about side effects it is much more valuable to hear about how treatments have changed and how modern medicine, and some complementary therapies too, can help you through a demanding treatment.

It is also important to understand that if you do not want to learn too much about your disease you don't have to. The choice is yours.

## Contact numbers and websites

### CANCER HELP UK

Cancer Help is Cancer Research UK's website for patients. Excellent sarcoma material including "Questions for Your Doctors". Click the link to 'Specific Cancers' and also use the search function as there are further references within the site

<http://www.cancerhelp.org.uk>

### CANCERBACUP

Cancer Bacup's free helpline is staffed by experienced nurses who can answer many questions. The web link leads to booklets on sarcoma and other cancer related issues - all available free by phoning Bacup.

<http://www.cancerbacup.org.uk/catalogue/sarcomas.htm>

**freephone helpline**  
**0808 800 1234**

**publications**  
**020 7696 9003**

### MACMILLAN

Macmillan works to improve the quality of life for people living with cancer. It offers information and practical advice over the phone and its web site is extensive. Macmillan also has information on local support groups.

<http://www.macmillan.org.uk>

**freephone helpline**  
**0808 808 2020**

### UK CHILDREN'S CANCER STUDY GROUP

The UKCCSG is a main contact point for professional treating children's cancer. Support materials and excellent downloadable newsletter are on the website.

<http://www.ukccsg.org>

### SARCOMA UK

We are the new kid on the block and our web presence is being worked on. The main page offers links to a range of further Internet resources and you can download our first two editions if you missed them.

<http://www.sarcoma-uk.org>

### INDUSTRY SITES

**Novartis** manufactures Glivec®, the radical new treatment for GIST, an intestinal sarcoma.

<http://www.novartisoncology.com/home.jsp>

### CLINICAL TRIALS IN THE UK

Large scale Phase 3 trials available in the UK. Click Database button and check Sarcoma.

<http://www.ncrn.org.uk/portfolio/database.asp>

### EARLY PHASE TRIALS IN UK

follow link to Experimental Trials Register

<http://www.ntrac.org.uk/>

### GIST SUPPORT UK

The UK's GIST support group with online information about Glivec.

<http://www.gistsupportuk.com>

### LIFERAFT GROUP

The worldwide Liferaft Group offers support and information to GIST patients receiving Glivec.

<http://www.liferaftgroup.org>

Be cautious when using the web to look for information on any cancer. Just because a website exists it does not mean that those responsible have any expertise. Information can only be general and can not be specific to a particular patient.

# THE BRITISH SARCOMA GROUP



BSG President **Professor Ian Judson**

The UK's sarcoma clinicians have created the British Sarcoma Group. It is a response to growing demand from the multidisciplinary teams involved in the management and treatment of sarcomas for action to address issues of patient management, which they describe as "somewhat disorganised due, at least in part, to the low incidence of this disease."

The overall objective of the BSG will be to improve significantly the UK organisation of sarcoma patient management, ensuring equal access to the best standards of care for all

patients across the UK. The association will represent the specialists' interests with regulatory bodies and other professional groups, help the UK focus on collaborative international research into sarcoma treatment, and raise standards in the treatment of these rare cancers.

The inaugural meeting in May was chaired by Mr Rob Grimer of Birmingham's Royal Orthopaedic Hospital. Professor Ian Judson (Royal Marsden Hospital) was elected as the BSG's first president. The meeting heard a keynote presentation from Dr Henrik Bauer, Chairman of the Scandinavian Sarcoma Group, whose twenty-one years of achievement has helped provide an inspiration for the formation of its British counterpart. A similar group was set up in Italy a few years ago and a sarcoma research group has recently been formed in the USA.

The BSG is open to membership from all doctors, nurses and healthcare professionals involved in the treatment of sarcoma.

## USING CANCER TUMOURS FOR RESEARCH

The government's Human Tissue Bill is working its way through Parliament. Although much of what it covers is about organ donation in response to the tragedy of the Alder Hey and Bristol children's organ warehousing scandals the Bill also covers the retention for research purposes of cancer tumours removed during surgery.

The concept of 'informed consent' lies at the heart of the proposed changes. Samples from tumours are already taken for diagnostic purposes and they are usually kept in case information on that patient's cancer is important for future treatment. In the case of rare cancers like sarcomas, taking additional samples of the tumour for use by researchers is important to help the progress of research.

The government originally proposed an informed consent process which created a lot of concern because many doctors and nurses feared it would be a burden on scarce time and resources. The experience of cancer patients also suggests that during the stressful time before surgery adding another administrative process is far from helpful. A study in Peterborough showed that most patients are happy to know that their excised cancer can be used in research and have no concerns about its being kept for that purpose. There is also a strong wish from patients not to create tasks which further overload NHS resources.

The government's final proposals do not remove the need for consent but the onus is placed on patients to opt-out if they do not want their tumours used for research, rather than to opt-in. There are strong safeguards in place protecting the identity of donors and guaranteeing anonymity throughout the research use of any tissue.

Organ donation will still require a full informed consent process with donors, or their close relatives, 'opting in'.

# THE GIST AND GLIVEC STORY



Gastrointestinal stromal tumour (GIST) is a soft tissue sarcoma affecting the stomach and gastrointestinal tract. It is one of the more difficult cancers to diagnose, often being painless until quite advanced. Advanced GIST has been difficult to treat, the only effective approach was surgery and when tumours were inoperable there was little doctors could do.

In 2000 a drug called Glivec, originally developed to treat chronic myeloid leukaemia, was tested on a GIST patient. Clinical trials started very shortly afterwards and the drug gained licensing approvals in the USA and Europe in record times. Patients who once had weeks to live are now alive years later. Although not every GIST patient can benefit from Glivec, after two years 72% of patients have their disease controlled.

Glivec is not a chemotherapy. It is a targeted molecular therapy taken daily in the form of capsules or pills. It has some side effects although generally these are tolerated well. Patients do not suffer hair loss or the risks of infection that come with chemotherapy. Glivec does not act the same way as chemotherapy either. CT scans may not show tumour reduction even when patients are feeling much better so expert examination of CT scans is necessary to confirm a patient's response.

Some GIST patients do not respond to Glivec, some find the side effects difficult and, sadly, some patients build up resistance to the drug. New compounds are in development and the first of these, SU011248, is now in clinical trials in the USA. Sadly the action of Glivec is very specific to GIST. There have been rare instances when patients with other sarcomas have shown a limited response but until medical science understands which of the biological factors that respond to Glivec are present in which kinds of tumour, trials cannot start.

We are now awaiting the final details of a Technology Appraisal by the National Institute of Clinical Excellence (NICE) which will decide the conditions for Glivec to be made available on the NHS in England and Wales. In Scotland Glivec is already available on the NHS for GIST patients with advanced disease.

The story of Glivec is a symbol of new hope for cancer treatments in the coming years. While it is too



much to expect many (or indeed any) new drugs to have the same dramatic effect that Glivec has achieved with GIST, the fact that a daily pill can control a cancer has now been demonstrated.

GIST Support UK is a small group of GIST patients which keep in contact through a website and by email. There have also been two meetings – one in London and one in Leeds – with patients attending from all over the UK (see picture). What brings them together is that most of them take Glivec.

Sarcoma UK has been working closely with GIST Support UK to represent a patient voice to NICE in the Technology Appraisal of Glivec.

There are two internet based international support groups for patients with GIST. The Liferaft Group has a strong commitment to clinical research and has undertaken patient oriented research into the side-effects of Glivec. It is taking a similar approach with new compounds too as they enter trials. GIST Support International also offers support to patients and their families. Both have email support lists. See page three for Internet addresses.



**GSI** Gist Support International



## YOU'RE MORE LIKELY TO WIN THE LOTTERY!

**I was once told that statistically you are more likely to win the lottery than to be diagnosed with a sarcoma. So how come I've never won more than a tenner, yet hit the damn jackpot with the sarcoma!**

My name is Lee and in July 2002, at the age of 34, was diagnosed with osteosarcoma in my right leg. The shock of that still hits me every day. I had to undergo eight months of intensive chemotherapy, interrupted only to have major surgery – an endoprosthesis replacement of the right femur (a metal knee and thigh bone to you and me) – and a great deal of discomfort - In summary, a year of sheer hell. The good news is that having recently passed the one year milestone since finishing my treatment I am still here and doing very well. When looking back I remember there were some funny moments. One was when I was told by a consultant that surgery would leave me unable to run, but able to climb a ladder. "Blimey" I said. . . . "It cures vertigo!" Another was when, as a side effect to drinking loads of water during a cycle of the drug methotrexate, I had to visit the toilet 14 times in 12 hours. This clearly wasn't funny, but when complaining to my nurse she simply smiled and said "At least your kidneys are functioning!" "Thanks for the sympathy" I replied as we both laughed out loud.

Has sarcoma changed my life – you bet it has. My attitude to life has changed in many ways. The things that used to irritate me no longer do and I often think to myself "why worry about that, it's not a matter of life or death!" I honestly believe that in some ways I'm now a better, more centred person than I was two years ago.

The last year has been an extremely busy one. I was determined that my illness wouldn't rule my life anymore than it had to and I returned to work full time in July 2003, and haven't had a single sick day off since. My wife Debbie and I have bought a new car, a fantastic Mini Cooper, moved house and have both been promoted at work.

I know the remission period is a long one but I've always believed that a positive attitude is vital in beating cancer and is indeed at least as effective as any medical treatment, and I know I'm going to make it to a ripe old age.

Finally I'd like to wish you all good luck and good health, and remember to stay positive. Oh, by the way, please can I win the lottery next time!!

**Lee Harcus**

Lee has been keeping a full diary of his treatment and his feelings. It is an inspiring read. Have a look at his HealthCheck Information Website at [www.webpageit.co.uk](http://www.webpageit.co.uk)

## ANDREW'S STORY

**My problem was discovered at Easter time 2002 when I had appendicitis. A routine ultra-sound scan displayed irregularities in my liver. Were they birth marks, scar tissue or what? This led to a CT scan which confirmed the liver problem and identified a tumour in my stomach about the size of my fist.**

A subsequent biopsy eventually concluded GIST with secondaries in the liver and following further surgery in Liverpool in May, to explore the lower bowel and find the route forward, I was advised I was a good match for Glivec.

Medication began on 31st May and continued until 9th October 2003. It was stopped prior to surgery on 22nd October. The first routine scan after I started taking Glivec indicated significant reduction in the stomach and liver tumours. The second scan showed a further marginal reduction in the stomach tumour. A welcome surprise then came when the surgeon revised his opinion regarding my liver. He advised that in fact no reduction seemed to have taken place with the liver markings and it was now his view that a 50/50 chance existed that the liver may, after all, be healthy and the damage may have been simply birthmarks not a tumour.

Surgery was aimed at removing the affected part of my stomach (and liver if necessary). Any damage to my liver would be assessed during surgery by sending samples to pathology for analysis.

I awoke from surgery to be told there was a good chance all the stomach tumour had been removed but all the stomach had gone with it due to difficulties in working around a main artery. The appendix had also gone (good riddance) and the liver, on the face of it, seemed OK. So, no secondary cancer although a few apparently unrelated puzzles which were being examined.

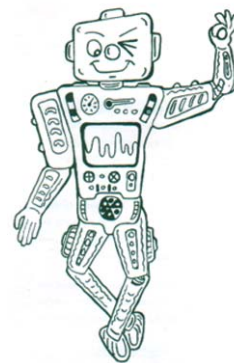
I realise I have probably been a lot more fortunate than many others. Firstly, by pure accident, I was discovered relatively early when appendicitis was diagnosed and a routine ultrasound scan was performed. Secondly, if I hadn't had "freakish" birthmarks on my liver, the original CT scan, which led to discovery of the stomach tumour wouldn't have taken place. Thirdly I was a good match for Glivec which, in my case, produced minimal side-effects. Finally, after getting over the initial shock of discovering I had cancer I had plenty of opportunity for discussion, to rationalise my thinking process and to get as fit as possible prior to surgery which was a worthwhile investment. I've also had fantastic support from my family and friends and a great medical team to help me.

## ROHBTS

The only national support group for sarcoma patients was founded nearly fifteen years ago in Birmingham. ROHBTS – the Royal Orthopaedic Hospital Bone Tumour Service has a focus on the young people and their families who make up an important proportion of the patients at the ROH although the adult patients can be members too.

As the hospital is one of the UK's principle centres for the treatment of bone sarcomas many patients have had radical surgery involving artificial replacement bones or amputation. The challenge of re-adjusting to a normal life is well illustrated by Lee's story (opposite page).

ROHBTS operates a telephone tree support network, provides information to patients, raises funds to support the oncology wards in the hospital, and helps patients and their families with short breaks and holidays at a mobile home kept in mid-Wales.



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# Sarcoma UK

This newsletter is published free of charge to sarcoma patients, carers, friends and those who treat this group of rare cancers.

If you require additional copies of Sarcoma UK or would like to distribute it through hospital, support group, charity etc please let us know by email of the number of copies required and the mailing address. Email to: [info@sarcoma-uk.org](mailto:info@sarcoma-uk.org) or to [sarcoma@dflair.demon.co.uk](mailto:sarcoma@dflair.demon.co.uk)

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## ABOUT SARCOMA UK

The response to the first editions of this newsletter has been overwhelming. Thank you to everyone who has written to us.

Sarcoma UK is now incorporated as a company limited by guarantee with charitable objectives:

- To provide information and support to sarcoma patients, their carers and families
- To support the development of a coherent national network of specialist centres to treat sarcoma in line with the best identified clinical practice
- To support research which leads to better treatment and better outcomes for sarcoma patients

We have been generously supported in the development of Sarcoma UK with unrestricted grants from Novartis Oncology and Pharmamar SA.

Our immediate aim is to continue publishing this newsletter as a means of creating contact with sarcoma patients and their families and carers. We are also preparing a booklet for newly diagnosed patients and looking at how other published information needs can be met.

There is an email support list now running and about 30 patients and carers subscribe already. If you would like to know more, or would like to register as a subscriber, please contact us at [info@sarcoma-uk.org](mailto:info@sarcoma-uk.org).

We have already received a small number of requests for information on how to support sarcoma research. We are setting up a research fund and establishing a clinical advisory group so that we can ensure proper use of donations. If anyone wishes to support research into sarcoma we would be happy to hear from you.

Now we have a structure for the newsletter we would also be glad of any donations to support the costs. We are also developing patient information material and our website at [www.sarcoma-uk.org](http://www.sarcoma-uk.org) is also being re-launched with a wider and better range of information.

To stay in touch with us please complete the form at the centre of the newsletter or contact us at the address above. You are already on our mailing list if you received this copy of the newsletter direct by post.

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