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*Together We Can Make A Difference
For Those Affected By Sarcomas!*

Sarcoma Patient
Pathway Analysis
and Recommendations
for Service Development

Version III – 10/2016

POLICY PAPER





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SARCOMA PATIENT PATHWAY ANALYSIS AND RECOMMENDATIONS FOR SERVICE DEVELOPMENT

by

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*Version 3
31/10/2016*

This paper has been prepared in response to the growing number of requests for sarcoma patient views on such matters as expert treatment centres, reference centres, rare cancer protocols etc. Our objective with this paper is to give a clear statement of what we expect sarcoma treatment to look like, how we expect service structures to develop to respond to patient needs, and how national and international referral practice should evolve.

The paper is based on a patient pathway which is neither typical nor ideal but which is based on the kinds of treatment options that arise at different times with the majority of sarcoma tumour sub-types.

Sarcomas are a heterogeneous group of cancers affecting connective soft tissue and bone. Some are more common than others. They include invasive non-malignant Desmoid tumours. Sarcomas can affect any age group and occur in almost any part of the body, although some sites are more common than others. There are over 50 sub-types, a number which grows as genetic analysis identifies distinctions between tumours which at one time were regarded as the same. The wide variations in patient response to different treatments can increasingly be explained by this analysis but at the same time the relative scarcity of new treatments to address such differences is a reality that must be addressed.

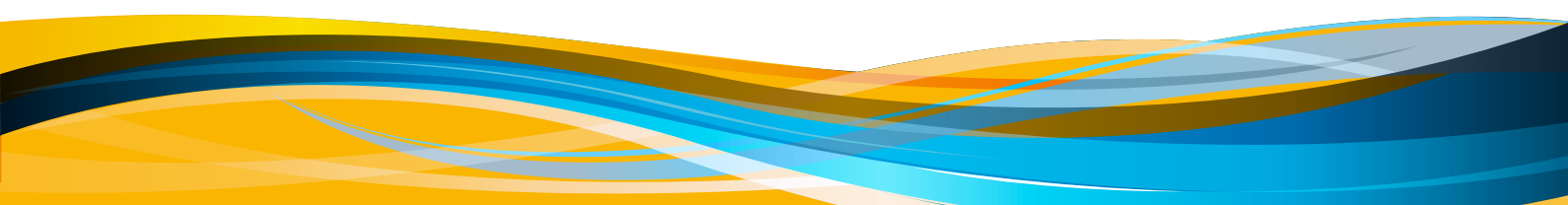
Achieving an accurate diagnosis is the first challenge a new patient faces. Because these cancers are rare (appr. 60 per 1 Mio. of population) family doctors and those practicing in local hospitals rarely see a case. Misdiagnosis, inappropriate and non-expert treatment are common. Even when diagnosed correctly or partially correctly (i.e. soft tissue sarcoma but not sub-type) many patients experience non-expert surgery which can disadvantageously affect outcome.

Approximately 50% of sarcoma patients will die within five years. Death is usually but not always from metastatic spread of the disease, usually to lungs or liver but also known in other organs. A significant number of patients are diagnosed with metastases at first presentation with the disease, particularly those with emergency room admission to hospital. This group of patients has a median life expectancy of less than a year.

Even with good primary surgery recurrences are known although low-grade tumours are more likely to recur late and locally (even after 10 years) while high grade lesions are more likely to have metastatic recurrence within 2-3 years.

Some sub-types seem more ready to progress than others. For some situations there is no known effective treatment once advanced disease is established. For a few there is no explanation for positive response to treatment when a 'poor prognosis' was anticipated.

It is against this background that this pathway has been prepared. It is not intended to be definitive for all sarcomas, that is why expertise gained by specialist doctors from years of practice and sharing with national and international colleagues is so valuable for sarcoma patients.



DIAGNOSTIC PATHWAY

Pathway Overview

Pathway Expanded View

Recommendations

Patient first presents with suspicious lump

OBJECTIVE: prompt presentation by patient, suspicion by primary care practitioner, prompt and accurate referral to specialist centre/diagnostics.

May be several visits to family doctor/general practitioner before a referral for full diagnostics.

Patients must seek a diagnosis, not settle for symptom management.

Limb or trunk tumours may be 'shelled out' as cysts. They may remain undiagnosed until they recur.

Emergency presentation is common, especially with teenagers.

Patients may self-refer to cancer centre and be triaged to appropriate diagnostics.

First visit to competent diagnostic centre

OBJECTIVE: accurate diagnosis with imaging, pathological and clinical information describing grade, histotype, stage etc. which enables a treatment plan to be defined.

For limb/trunk tumours diagnosis is clinical examination, MRI scan, and biopsy. Confirmed diagnosis must be followed with CT to check for metastasis. Diagnosis should be confirmed by pathology second opinion. Stage should be defined. **1**

For retroperitoneal sarcomas a biopsy is advised. This should be taken by the sarcoma surgeon guided by CT imaging. The same may apply for abdominal and pelvic tumours. **2**

Confirmed diagnosis should result in discussion at specialist sarcoma MDT prior to commencing any treatment.

GIST, Gynae, Head & Neck and Skin sarcomas tend to be diagnosed in site specific centres, often following surgery. Local arrangements should be made to ensure that patients are diagnosed correctly and receive appropriate treatment. **3**

Data regarding all patients should be entered to a relevant sarcoma registry.

Patients should have access to all the information they need, whether through traditional paper media or new media.

As earlier diagnosis is one of the factors leading to improved survival, specialist centres should work with local patient advocacy groups to consider what joint activity might help create speedier patient presentation at specialist diagnostic and treatment centres.

Every healthcare system should:

1. Endeavour to raise awareness of sarcoma among primary care doctors and secondary care medical and nursing staff.
2. Have a referral protocol in place to advise of 'red light' symptoms, advise on taking initial diagnostic steps, and referral.
3. Referral advice should include locally invasive non-malignant fibromatosis (Desmoid tumours).
4. A secondary **OBJECTIVE** is that the right diagnosis is arrived at without any need for the patient to search for advice/opinions from other countries.

Healthcare systems should ensure that:

5. They have a network of specialist sarcoma centres. To have centres which treat approx. 250 new patients each year would suggest a centre for every 4 million of population (and pro rata).
6. Healthcare systems must have processes in place to ensure that sarcoma patients are only treated in these centres.
7. Every MDT has all required surgical and oncology disciplines. **4**
8. Accurate pathology is critical to good care. Histopathologists should be members of a quality assurance scheme which allows second opinions to be routine practice.
9. Each MDT has referral protocols for consultation with relevant local site specific centres even when patients are to continue to be treated in those centres.
10. A sarcoma registry is established, using data fields which can be aggregated with other registries. Data entry should be mandatory.

PRIMARY TREATMENT PATHWAY

Pathway Overview

Pathway Expanded View

Recommendations

Primary treatment

OBJECTIVE: curative treatment using evidence-based approaches observing published protocols and with guidance where appropriate from recognised individual specialists.

Where possible and available all patients will be offered treatment within a clinical study. **5**

Patients should be asked to consent to tissue donation and arrangements should be made to collect tissue. **6**

The proposed treatment plan should be shared with the patient (and parents when paediatric) along with any additional information which they may need or request. The lead doctor should be available to answer any questions.

Bone sarcomas will have a mix of chemotherapy, radiotherapy and surgery, possibly with additional therapy aimed at effective disease-free maintenance. **7**

Most soft tissue tumours will have surgery, with radiotherapy prior to or following surgery, or both, according to local protocol. Some chemo-sensitive sub-types may also have adjuvant or neo-adjuvant chemotherapy. **7**

Every centre should have a referral network allowing access to treatment technology such as Isolated Limb Perfusion, proton beam radiotherapy etc. **8**

Patients with site specific tumours have individual requirements. For example: GIST patients may be eligible for adjuvant imatinib following surgery, gynae patients should not have radiotherapy and chemotherapy is of unproven value. Expertise is required here and that may require both site specific and sarcoma experts. **3**

Paediatric patients will have treatment determined by age, tumour type and stage whether bone or soft tissue. **9**

Follow-up on completion of primary treatment should be determined based on the risk of recurrence for each individual patient. This risk will include assessment of surgical margins (R0 etc.), grade (mitotic count), histology confirmed by post-surgical pathology, staging and clinical judgment.

Follow-up practice should be reviewed to ensure it meets patient needs, not clinical perceptions of need. **10**

Each MDT should have:

11. Evidence based protocols for treatment of all sarcoma types and all age groups. These protocols may be developed in collaboration locally, nationally or internationally.
12. A tissue bank which collects, stores and makes tissue available to approved laboratory studies. The tissue bank should be approved by ethical regulators and tissue should only be released to ethically approved studies.
13. Referral network should be in place for use of specialist high-cost technology not locally available.
14. A programme of research to build and share new evidence about treatment. Studies may be local, national or international, in any medical or other clinical discipline and relevant to any stage in the disease pathway.
15. An attitude of mind which says that when a doctor wishes to treat off-protocol that doctor can make a rational case, consult peers and colleagues, and receive open-minded judgement.

Quality of Life issues for patients following primary treatment centre around treatment related side-effects and follow-up practice. An MDT should be asking "what makes a great cancer experience?" and acting accordingly to examine what could be done better.

This is the first time when patients need support. Rehabilitation may be necessary. The scale of physical support may range from prosthetics to simple physiotherapy. The range of psychological support can run from counselling (talking therapy) to the ability to share experience with others in a mutual support group.

Healthcare systems must:

16. Put in place structures which allow a rehabilitation programme to be individualised for each patient.

ADVANCED DISEASE PATHWAY

Pathway Overview

Pathway Expanded View

Recommendations

- Patient presents with recurrent lump
- Patient presents with indicative symptoms
- Patient presents with vague symptoms
- Recurrence identified in follow-up clinic

OBJECTIVE: prompt treatment of recurrence, whether localised or metastatic, with the aim of providing a high quality of life together with duration of survival, always respecting patient's wishes.

A primary care practitioner or secondary care triage must refer any patient presenting with a lump close to the site of a previously resected lump urgently to specialist centre. Diagnosis is critical and patients should not settle for symptomatic treatment. If symptoms are indicative of metastasis this should be stated on referral. Vague symptoms should always be referred for investigation.

Localised recurrence should follow primary treatment route. Patients should be assessed for potential metastasis.

Suspected metastasis should include check for localised or nodal disease. PET/CT may offer value in some cases.

An inconclusive but suspicious diagnosis should be referred for a second opinion.

MDT discussion on appropriate route for the patient. All metastatic patients should receive expert symptom management, whether being actively treated or in palliative care.

Where possible and available all patients will be offered treatment within a clinical study.

4

The treatment plan should be shared with the patient (and parents when paediatric) along with any additional information which they may need or request. The lead doctor should be available to answer any questions.

Surgery or ablative therapy may be appropriate. This may involve consulting additional specialists as a protocol can only be advisory.

Chemotherapy, targeted therapy or other drug treatment may be indicated. Self-managed treatments should have treatment adherence information and support.

No further treatment may be advised because of age, physical capability, nature of disease, patient choice.

Patient treatment data should be recorded in the registry.

A strong context of patient consent is applicable. Disease will be incurable and prognosis will be unique to the individual. The patient may not wish to proceed with active treatment which affects quality of life. At the point where the patient decides to end active treatment palliative care should already be available and continue until end-of-life.

All patients with advanced stage sarcoma should be able to access support. Treatment can have side effects and while clinical support will help advice from others is valuable too. Psychological support should be offered, this can run from counselling (talking therapy) to having the opportunity to share experience with others in a mutual support group.

Every healthcare system should:

17. Provide guidance to primary care on 'red warning' symptoms for sarcoma recurrence including both nodal and distant metastasis.
18. Healthcare systems and research funders should take note of the clinical need for reliable and accurate quality of life data which patients can interpret and understand.
19. Drug treatment options for advanced/metastatic disease are limited. Therefore access to innovative treatments should be made available.
20. Registration should continue, recording treatments until death.

These steps may be repeated as further recurrences or relapses are diagnosed following therapy.

Notes to “Pathway Diagram” *(the red number spots)*

1. Patients may be diagnosed with distant metastasis at first presentation. The eventual outcome is unlikely to be affected by treating the primary tumour. Treatment for metastasis is most likely to be chemotherapy as a palliative measure. Prognosis is generally poor for these patients.
2. Retroperitoneal, abdominal and pelvic sarcomas should involve the treating surgeon in their diagnosis. A biopsy, if performed, should be undertaken by that surgeon so that the path of any needle penetrating the tumour, or the tissue surrounding any tumour incision, can be safely excised during the main surgery.
3. Site specific centres in this context means a surgical specialist in GI, gynaecology, head & neck or dermatology. Diagnosis prior to surgery is not always possible. These sarcomas may be excised with positive margins by a surgeon who is not aware that this is a cancer. This can create complications and threaten outcomes. It is essential that a sarcoma pathologist reviews tumour tissue and a specialist oncologist is involved in decision-making once a diagnosis has been confirmed. Agreed treatment may continue with the site specialist, subject to continued involvement of the sarcoma team when decisions need to be taken, or be transferred to the sarcoma specialist team.
4. The skills which an MDT should have as core membership are: surgeon(s), oncologists (medical, radiation, paediatric), histopathologist(s), diagnostic/imaging radiologist, clinical nurse specialist, physiotherapist. Further members committing significant time to sarcoma patients will be: specialist surgeon(s), palliative care specialist (doctor or nurse), interventional radiologist, rehabilitation specialists (e.g. prosthetist), clinical psychologist, psychotherapists.
5. Every MDT should be conducting research. Major treatment questions will be international studies which the local MDT can adopt. A full and up-to-date listing of all major treatment studies should be maintained so that patients can be matched to a study. It is accepted that some patients will not wish to enter a study not being run by their local MDT; however, they should be given the chance.
6. Sarcoma tissue and accompanying data should be collected according to a standard procedure operated by an approved tissue bank to ensure quality. The tissue bank should work to legally and ethically approved standards for tissue storage and for data management. Studies which have been scientifically peer reviewed and are ethically approved should be supported to have access to tissue and data.
7. In most healthcare systems surgeons specialise to some extent. This means that when specialist skills are required (e.g. reconstruction, bone endoprostheses) they may need to be brought into the MDT.
8. High cost specialist equipment tends to be introduced initially in a few centres. Proton beam radiotherapy is a classic example. Isolated Limb Perfusion is more widely available but still not in every treatment centre. Other new radiotherapy technology (e.g. stereotactic cyberknife) is more widely available but still not in every centre. Each MDT should have an established link to centres which can provide such services so that e.g. teenage girls with pelvic Ewing sarcoma, or adults with large volume lower limb tumours, can receive the highest standards of treatment.
9. Treatment of paediatric sarcomas is an important sub-speciality. Tumours with similar histology to an adult tumour can behave differently from the adult version. Bone tumours form a higher proportion of the total. Expert surgery is critical. The use of endoprostheses for bone replacement requires lifetime specialist maintenance.
10. Follow-up can be a burden for healthcare systems, clinicians and patients. Practice should be continually reviewed. Expressed patient needs include: seeing the same doctor each time, a full explanation of what the follow-up is meant to achieve, time for questions to be answered and empathy.

Summary Recommendations *(the red boxes)*

A. Awareness.

Every Healthcare System Should:

1. Endeavour to raise awareness of sarcoma among primary care doctors and secondary care medical and nursing staff.

FURTHER COMMENT: patient advocacy groups can work with governments and healthcare systems to raise awareness. It is difficult to promote sarcoma awareness in the general public. Targeting doctors and healthcare practitioners who may be in a position to suspect sarcoma should help raise levels of earlier diagnosis. Such targeting may be through education programmes, conferences, and in-practice training.

2. Have a referral protocol in place to advise of 'red light' symptoms, advise on taking initial diagnostic steps, and referral.

FURTHER COMMENT: there is no question that the single biggest factor in improving the overall survival with sarcoma is early access to specialist treatment. This starts with better awareness among potential patients but we recognize the difficulty of doing this. The best route is better awareness among the medical staff who are the first to be consulted by sarcoma patients, about the indicative symptoms and the optimum referral route to specialist care.

3. Desmoid tumours (locally invasive but non-malignant fibromatosis) will usually be treated within the sarcoma speciality and referral information should include them.

FURTHER COMMENT: differences in treatment between sarcomas and Desmoid tumours are a clinical issue and not dealt with in this document. The sarcoma treatment structure should allow paediatric patients, whether sarcoma or Desmoid, to progress to adulthood with continuity of expert care.

4. A secondary OBJECTIVE is that the right diagnosis is arrived at without any need for the patient to search for advice/opinions from other countries.

FURTHER COMMENT: while this seems self-explanatory the intention is that healthcare systems should adopt a target of providing all diagnoses to the highest standards. A diagnosis referred to a specialist unit outside the country should be seen as a system failure requiring action to improve local standards.

B. Diagnosis.

Healthcare Systems Should Ensure That:

5. They have a network of specialist sarcoma centres. To have centres which treat approx. 250 new patients each year would suggest a centre for every 4 million of population (and pro rata).

FURTHER COMMENT: this ratio is based on the UK analysis which indicates this as a level at which the costs of the MDT are most efficiently employed. A better ratio would maximise opportunities for patient care.

6. Healthcare systems must have processes in place to ensure that sarcoma patients are only treated in these centres.

FURTHER COMMENT: we recognise that this is easier to achieve in a centralised healthcare system than in an insurance centred system. Withholding of fees, recovery of payments for inappropriate treatment etc. are steps of which we would approve.

7. Every MDT has all required surgical and oncology disciplines.

FURTHER COMMENT: the healthcare system and the MDT should work together on succession planning. Ill health, maternity and holiday cover provide opportunities to bring in professionals looking to specialise in treating sarcoma. Training fellowships should be used to create a national pool of familiarity with sarcoma to provide this cover. In this way retirements etc. for MDT members can be planned for.

8. Accurate pathology is critical to good care. Histopathologists should be members of a quality assurance scheme which allows second opinions to be routine practice.

FURTHER COMMENT: the biggest factor in accelerating patients to the correct mode of treatment is accurate pathological diagnosis. It is reported that where patients are referred into specialist care with a diagnosis from non-experts more than one-third are inaccurate.

9. Each MDT has referral protocols for consultation with relevant local site specific centres even when patients are to continue to be treated in those centres.

FURTHER COMMENT: it is not acceptable to patients that a patient with a sarcoma cannot be treated by a sarcoma specialist. Current practices across Europe protect non-sarcoma specialists discovering a sarcoma from referring the patient on. This must stop.

10. A sarcoma registry is established, using data fields which can be aggregated with other registries. Data entry should be mandatory.

FURTHER COMMENT: centralised patient registration and analysis of the data collected contributes to improving clinical practice. Being able to aggregate data from all registries will provide data from significant numbers of patients and permit real-life experience to contribute to service improvements.

C. Primary Treatment. Each MDT Should Have:

11. Evidence based protocols for treatment of all sarcoma types and all age groups. These protocols may be developed in collaboration locally, nationally or internationally.
12. A tissue bank which collects, stores and makes tissue available to approved laboratory studies.
13. Referral network should be in place for use of specialist high-cost technology not locally available.
14. A programme of research to build and share new evidence about treatment. Studies may be local, national or international, in any medical or other clinical discipline and relevant to any stage in the disease pathway.

FURTHER COMMENT: patients believe that research into nursing practices and the contribution nurses make to sarcoma patient care needs to be undertaken. We believe that the nursing role is central to a good holistic outcome, as opposed to a more simplistic medical outcome.

15. An attitude of mind which says that when a doctor wishes to treat off-protocol that doctor can make a rational case, consult peers and colleagues, and receive open-minded judgement.

FURTHER COMMENT: hospitals and regulatory bodies have well-proven systems for ensuring patient safety and we do not want to over-ride these. However, we do want to encourage innovation. We also want to open the way for those patients with a very rare sarcoma to have access to treatments which are rationally identified, even if not indicated by strict conventional methods. Full information prior to patient consent will be a primary condition of approval.

D. Rehabilitation. Healthcare Systems Must:

16. Put in place structures which allow a rehabilitation programme to be individualised for each patient.

FURTHER COMMENT: A proper assessment of the social care needs of every patient is a priority. Meeting those needs will depend on national and local conditions.

E. Advanced Disease. Every Healthcare System Should:

17. Provide guidance to primary care on 'red warning' symptoms for sarcoma recurrence including both nodal and distant metastasis.

FURTHER COMMENT: as with the first diagnosis getting an early referral into specialist care provides the best opportunity for a patient with recurrence to have a good outcome.

18. Healthcare systems and research funders should take note of the clinical need for reliable and accurate quality of life data which patients can interpret and understand.

FURTHER COMMENT: the treatment for advanced sarcoma is full of uncertainties which need clarifying. This will eventually come from large-scale data analysis because the challenge of getting prospective data has proved impossible to meet. An important component for the patient seeking to make a decision is trustworthy quality-of-life information. This too is currently missing but requires prospective study.

19. Drug treatment options for advanced/metastatic disease are limited. Therefore access to innovative treatments should be made available.

FURTHER COMMENT: while clinical studies can meet this requirement they are not always available for a specific sub-type. Where a rational scientifically led analysis indicates that an off-label treatment, or a non-patent re-purposed treatment may be beneficial for a patient it must be considered. Similarly healthcare systems should have processes in place to ensure rapid access to innovative treatments with fast decisions made on reimbursement.

20. Registration should continue, recording treatments until death.

Summary Recommendations *(the red boxes)*

F. The Bigger Picture:

Sarcoma Patients EuroNet (SPAEN) has a membership which extends beyond Europe. We recognise that this Pathway paper has an European focus and is largely based on western European experience supported by published evidence from healthcare systems in the wealthier regions of the world. We do believe that the standards implied in this paper are what sarcoma patients everywhere should be entitled to receive. We recognise that this can only be an ambition in much of the world but we hope that healthcare systems everywhere can work towards this objective.

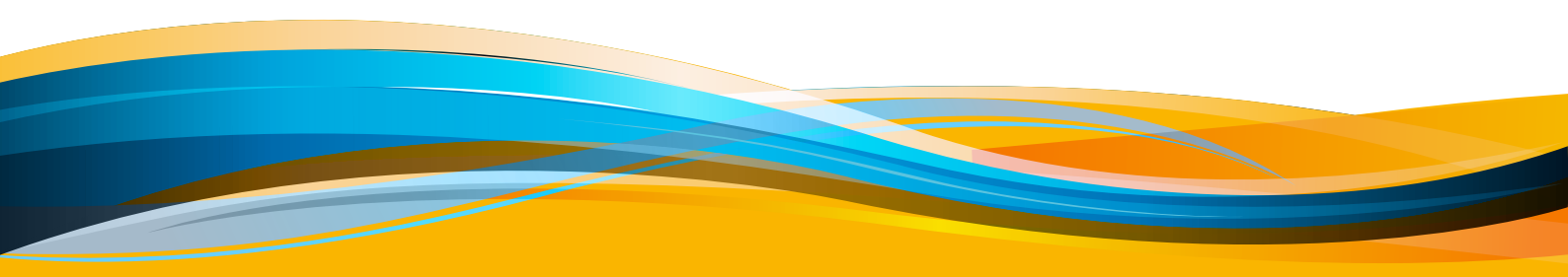
Nominating specialist MDTs/specialist treatment centres in European countries will be easier in some countries than in others. Even with pan-EU agreement on the principles this will be an area for disagreement, especially from clinicians who regularly treat sarcomas but whose centres do not meet whatever qualifying criteria are applied. Nominated centres will also need to be ‘inspected’ regularly to ensure that qualifying criteria are still being met.

We have particular concerns with regard to countries of Eastern Europe which are unable to provide the quality of expert treatment and care we believe that sarcoma patients deserve. SPAEN regularly receives requests for guidance on obtaining a second opinion. While a pathology opinion may be relatively easily achieved a second opinion for treatment raises the question of obtaining recommended treatment if it cannot be provided within the patient’s own healthcare system, especially if relevant expertise is not available locally. We believe there are three needs which must be met to help address this issue:

- A minimum of one sarcoma specialist centre in each country
- Liaison between new/developing sarcoma centres and established providers in Western Europe
- Structures which provide training through professional meetings, extended fellowships, lecture series etc. to build expertise

One idea which deserves exploring is that of new / developing specialist centres being ‘adopted’ by one or more established centres which can provide training, visiting experts, second opinions and the structures for acting on second opinions (subject to funding arrangements).

Patients fully support the challenges to clinical research and regulatory practices indicated in the papers from Rare Cancers Europe, ESMO, ECCO and other organisations/networks.



Sarcoma Patients EuroNet e.V.

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