The Sarcoma Policy Checklist

February 2017

Paolo Casali, Istituto Nazionale Tumori, Italy
Nora Drove, Eli Lilly & company
Sarah Dumont, Institut Gustave Roussy, France
Mikael Eriksson, University of Lund, Sweden
Alessandro Gronchi, Istituto Nazionale dei Tumori, Italy and EORTC Soft Tissue and Bone Sarcoma
Bernd Kasper, University of Heidelberg, Germany
Estelle Lecointe, Info Sarcomes, France and SPAEN
Claudia Valverde, University Hospital Vall' Hebron, Spain
Markus Wartenberg, SPAEN & das Lebenshaus e.V., das Wissenhaus GmbH, Germany
Roger Wilson, SPAEN
Shannon Boldon, The Health Policy Partnership
Suzanne Wait, The Health Policy Partnership

Sarcoma Patients EuroNet

Info Sarcomes

Das Lebenshaus

The Sarcoma Policy Checklist was developed by a group of leading representatives from patient organisations, clinical experts and industry. This project was initiated and funded by Lilly.

This document was drafted by the Sarcoma Policy Checklist Expert Group. Drafting and editing of the document as well as day-to-day implementation of the project was coordinated by the Health Policy Partnership Ltd. Eli Lilly & company (Lilly) provided financial support for meeting costs and materials produced by the group. Lilly did not provide any fees to any of the members of the group for their involvement in this project. Lilly provided comments on the document, however the content of the final document reflects consensus from the members of the group who have full editorial control.

References for infographic opposite:

1. Sarcoma UK. The National Sarcoma Survey, Transforming Patient Experience, 2015:35.
2. Fletcher C, Bridge JA, Hogendoorn P, *et al*. WHO Classification of Tumours of Soft Tissue and Bone. Fourth Edition. WHO Classification of Tumours, 2013:468.
3. Sharma S, Takyar S, Manson SC, *et al*. Efficacy and safety of pharmacological interventions in second- or later-line treatment of patients with advanced soft tissue sarcoma: a systematic review. BMC Cancer 2013;13(1):1-21.
4. Lurkin A, Ducimetière F, Vince DR, *et al*. Epidemiological evaluation of concordance between initial diagnosis and central pathology review in a comprehensive and prospective series of sarcoma patients in the Rhone-Alpes region. BMC Cancer 2010;10(1):1-12.
5. Ray-Coquard I, Montesco MC, Coindre JM, *et al*. Sarcoma: concordance between initial diagnosis and centralized expert review in a population-based study within three European regions. Ann Oncol 2012;23(9):2442-9.
6. National Institute for Health and Care Excellence (NICE). Improving Outcomes for People with Sarcoma: the Manual. NICE Guidance on Cancer Services: Developed by the National Collaborating Centre for Cancer, 2006:142.

Executive Summary

Sarcomas in Europe: strengthening the policy response

A family of rare cancers that develop in the connective tissues[1]

Significant heterogeneity

More than **70** **different** types[2]

Can occur **anywhere** **in the body**[2]

As a result:

Healthcare professionals: difficult to acquire specialist training

Patients: late or mis-diagnosis, limited access to specialist care

Research: challenging to find treatments specific to each type of sarcoma

Health care system: high costs due to variability of quality of care

Some of the poorest patient experiences of any cancer type[1]

1% of all cancers,

2% of all cancer deaths[3]

Up to **40%** incorrect diagnosis[4 5]

Only **22%** of patients participate in clinical trials[1]

Up to **1 in 4** patients treated for something else[6]

Significant **inequalities** across Europe in access to available treatments

The Sarcoma Policy Checklist - What is needed to improve sarcoma care?

1. **Designated and accredited** centres of reference for sarcoma in each country
2. Greater **professional training** for all health care professionals involved in sarcoma care
3. A **multidisciplinary approach** to care for every patient with sarcoma
4. Greater **incentives** for research and innovation
5. More **rapid access** to effective treatments

Table of Contents

Part 2:

Introduction

Rare cancers represent 22% of all cancer diagnoses and typically have worse survival than common cancers such as cancers of the breast, colon or prostate.[1] Over the past decade, a number of stakeholders, such as Rare Cancers Europe, have raised awareness of the need for enabling policies to improve availability and patient access to appropriate information, clinical trials, effective treatments and care for all rare cancers.[2] The recent launch of a Joint Action on Rare Cancers may also be seen as a positive development, as it aims to better integrate the needs for rare cancers into national cancer plans.[3]

Despite these efforts, several challenges still exist for rare cancers – and many of these challenges are amplified in the case of sarcomas.

What are sarcomas?

Sarcomas are a heterogeneous family of rare cancers that develop in the connective tissues. There are approximately 70 subtypes of sarcomas and they can occur anywhere in the body.[4]

The two main types of sarcomas are:

1. **Soft tissue sarcomas (STS) - 84%** of sarcomas. These develop in soft tissue or all the supporting tissues in the body except bone (e.g. fat, muscle, blood vessels, nerves, or joints).[5] They include **gastrointestinal stromal tumours (GIST)**, which account for approximately 20% of STS.
2. **Bone sarcomas - 14%** of sarcomas.[6]

Estimates of the actual numbers of sarcomas are uncertain, because registration is not mandatory in most countries and standards of registration are inconsistent.

The heterogeneity of sarcomas challenges medical knowledge, clinical research and policy-making

The numbers of patients with each clinical presentation are extremely low, and subtypes have very different prognosis, features, characteristics and healthcare system demands.[6]

Sarcomas: key facts and figures

Sarcomas represent **1%** of all cancers yet **2%** of total cancer-related mortality.[7]

There are approximately **6** cases per 100,000 population per year, representing **27,908** new cases per year (EU27).[6]

5-year relative survival is 56% for soft tissue sarcomas, 70% for GISTs and 61% for bone sarcomas.[8]

Survival depends on when a sarcoma is diagnosed: For soft tissue sarcomas, the 5-year overall survival rates range from **15%** (for patients with metastatic relapse) to as high as **90%** (for early-stage disease) in both the US and EU.[8 9 10]

What are some of the challenges with sarcomas?

- **Sarcoma patients report some of the poorest experiences of any cancer type.**[11]
- **Patients often lack appropriate information** about their condition, centres of excellence, available treatments and care pathways, and ongoing clinical trials. As a result, they cannot make informed choices about their care.[11]
- **Most physicians have little or no experience in diagnosing or treating sarcomas due to their rarity.** They may not refer patients to a specialist,[8] often leading to delayed or incorrect diagnosis.
- **Inaccurate diagnosis leads to mismanagement in 70% of patients,**[8] with a number of patients receiving treatment for a condition other than sarcoma.[11 12]
- **The culmination of late or misdiagnosis and variable quality of care** has a considerable impact on the quality of life of patients and their families.
- **Some treatments are not reimbursed** and patients may have to travel great distances to receive appropriate care, or pay for treatments out-of-pocket.[13]
- **In some types of sarcomas, such as the first line treatment of advanced soft tissue sarcoma, we have not seen any significant advances in treatment** for over 30-40 years. GIST is an important exception.

About this document

This document was developed by the Sarcoma Policy Checklist expert group, a multi-stakeholder group of experts from the medical, patient advocacy and pharmaceutical industry fields to help policymakers close the gap in access to high quality information and care for sarcoma patients across Europe.

The document is divided into two parts:

1. The **Sarcoma Policy Checklist** describes **five key areas** where policy makers may focus their efforts to make the most impact on care for sarcoma patients.
2. **Six country profiles (for France, Germany, Italy, Spain, Sweden and the United Kingdom)** are then presented to illustrate to what extent these recommendations are implemented in different countries.

The Sarcoma Policy Checklist: What is most needed to improve sarcoma care?

1

Designated and accredited centres of reference for sarcoma in each country

2

Greater **professional training** for all health care professionals involved in sarcoma care

3

A **multidisciplinary approach** to care for every patient with sarcoma

4

Greater **incentives** for research and innovation

5

More **rapid access** to effective treatments

1 Designated and accredited national centres for reference on sarcoma

The policy checklist - what is needed?

- ☑ Each country has at least one national centre of reference, or a clear link to a centre of reference in another country
- ☑ A national accreditation process is in place to designate centres of reference based on clear quality standards
- ☑ Centres of reference are evaluated regularly against these standards to ensure continuous quality of care

Why this is important

- **Because sarcomas are so rare and present in so many forms,** it is difficult for most health professionals to gather sufficient experience in sarcomas.
- **It is therefore recommended that sarcoma care be delivered in designated specialist centres – or centres of reference.**
- **Centralising care within high-volume centres has been shown to improve the overall quality of care for sarcomas,**[14 15] by providing patients with early, accurate and quality diagnosis,[16 17] timely referral for expert second opinion[16] and access to information.[15]

How well are we doing across Europe?

- **Centres of reference for many forms of sarcoma exist in all six countries. However, they are not always formally designated by explicit quality standards,** nor is the quality of care monitored over time.[13 18]
- **This makes it difficult for patients and referring physicians to know where to seek specialist care.** This is a particular issue in the case of surgical expertise in sarcomas.
- **Efforts to establish quality standards for sarcoma at the European level** have recently been led by Sarcoma Patients EuroNet Association (SPAEN)[19] and by the European CanCer Organisation (ECCO).[20] The **UK** has clearly defined national standards for sarcoma, and **Sweden** has a particularly sophisticated monitoring of quality of sarcoma care through its cancer registry for extremity and trunk wall sarcoma.
- **Access to centres of reference for patients living outside of urban areas is often an issue.** For example in **Spain**, patients may have difficulty receiving permission from their region to be transferred to another region for treatment and may not be reimbursed for associated travel or accommodation expenses.
- **Several national sarcoma centres of reference will become part of the emerging European Reference Network (ERN) for rare adult solid tumours, including sarcomas (EURACAN).** However, ERNs are in early stages of implementation and clear processes still need to be established to facilitate collaboration, cross-border referrals and appropriate reimbursement between participating centres.[13 16 18 21]

European Reference Networks –

what advantages may they bring for sarcoma research and care?

- ✓ Greater opportunities for patients to participate in clinical trials and receive optimal treatment and care through cross-border collaboration[14 16]
- ✓ The creation of registries and consolidated collection of real-world data,[13 16 18 21] working to agreed common standards
- ✓ The development of quality assurance mechanisms for laboratory testing[21]
- ✓ Training and education tools for health professionals[21]
- ✓ Accelerated exchange[13 18 22] of information, biological samples, radiological images, other diagnostic materials, and e-tools for telemedicine between participating centres.[15 16]

2 Greater professional training

The policy checklist - what is needed?

- ☑ A national referral protocol for suspected sarcoma patients advises non-specialists of ‘red flag’ symptoms and when to refer patients to centres of reference
- ☑ Training on rare cancers is included in the general medical curriculum
- ☑ Ongoing training on rare cancers is available for all oncologists
- ☑ Specialised training programmes on sarcomas are available for all health care professionals involved in the sarcoma multidisciplinary care team

Why this is important

- **Because sarcomas can occur anywhere in the body, patients with symptoms may present to a wide range of physicians –** General Practitioners (GPs) but also gynaecologists, dermatologists, surgeons, and others.
- **Most of these physicians will never have seen a case of sarcoma, and may not know to which specialist they should refer patients.** This may lead to delayed diagnosis, inappropriate treatment[11 12] and compromised outcomes as a result.[8]
- **Many oncologists have not been trained on how to diagnose or treat sarcomas.**

How well are we doing across Europe?

- **In all six countries studied, medical doctors do not receive any formal training on rare cancers (including sarcomas) as part of their general training.** However, both **Spain** and the **UK** have targeted efforts at improving early diagnosis and referral for sarcoma.
- **Training on rare cancers is also not part of the formal training of oncologists in most countries,** although there are ongoing efforts to change this in some countries.
- **There are several pan-European specialist training programmes on sarcoma.** For example, the European Society of Surgical Oncology (ESSO) has put together a surgical training course on sarcoma, called the **European School of Soft Tissue Sarcoma Surgery.**[15] In addition, specialised courses on the management of sarcomas are available in many countries, often in the form of online training programmes.
- In addition to formal training, **having simple referral guidelines,** such as exist in **Sweden,** has been shown to lead to more rapid and accurate diagnosis and treatment of sarcomas.

3 A multidisciplinary approach to care for every patient

The policy checklist - what is needed?

- ☑ National guidelines exist for the treatment of all sarcomas, for adults and children
- ☑ All sarcoma patients are treated by a dedicated multidisciplinary team (MDT) according to a clear care pathway
- ☑ The minimum composition of the MDT is clearly defined
- ☑ Patients are assigned a dedicated key health worker and given a personalised care plan to help them navigate through their care

Why this is important

- **Support for sarcoma patients from a wide range of professionals** (histopathologists, radiologists, surgeons, medical oncologists, psycho-oncologists, palliative care experts, rehabilitation specialists) is crucial to ensure all of their needs are met over time.[8]

How well are we doing across Europe?

- Most national guidelines recognise that **the organisation of sarcoma care in MDTs** is key to provide high-quality sarcoma care to patients.
- **A MDT approach to care** is also a criteria for centres to become part of the recently initiated ERN for sarcomas.
- **Despite this, implementation of MDTs varies considerably between centres within all countries.** Many centres do not have sufficient resources to implement a systematic MDT approach to sarcoma care.
- **A particular challenge is to include primary and community-based providers within the MDT** to ensure high quality of diagnosis and care across the entire care pathway.
- **What's more, the composition of a specialist sarcoma MDT is often not clearly defined** and appropriately trained personnel may not be available in all centres.

4 Greater incentives for research and innovation

The policy checklist - what is needed?

- ☑ A national sarcoma registry is in place
- ☑ Standardised datasets allow comparable real-world data to be collected and compared across centres of reference
- ☑ There are national research collaborations for sarcoma
- ☑ There are incentives for public-private partnerships focused on rare cancers, including sarcomas

Why this is important

- **There has, traditionally, been a lack of funding for basic research on sarcomas.** As a result, we do not know the causes of many sarcomas[6] and the evidence base on incidence and survival patterns for sarcoma is not strong.[8]
- **Findings from basic research are essential** to drive research efforts towards treatments that may improve patient outcomes.
- **Enrolment of sufficient numbers of patients into sarcoma clinical trials is an ongoing challenge** due to the small number of patients with each specific type of sarcoma.[11 13 16]
- As a result, the **collection of real-world data is critical** to have sufficient patient data to help drive further research efforts as well as improvements in patient care.

How well are we doing across Europe?

- **There are a number of important research initiatives on sarcomas in different countries.** For example, **France** has good availability of data in both their pathology and clinical networks, allowing them to lead many national and international sarcoma research projects. **Sweden** has a National Sarcoma Quality Registry (INCA) that collects sarcoma patient data from all regions, and offers opportunities for real-world data analyses. In the **UK,** a comprehensive survey of sarcoma patients has provided important insights into the experience of patients with sarcoma. **Italy** has led significant efforts in paediatric sarcomas. **Spain** has set up a rare sarcoma project to determine the burden of rare sarcoma and improve treatment pathways for patients.
- **Despite these efforts however, there is still the need for more basic research on sarcomas** – and funding to do so.
- **The development of the ERN is likely to play an important role in encouraging the collection of comparable real-world data across different centres,** as the collection of prospective hospital data will be mandatory for ERN accreditation.

5 More rapid access to effective treatments

The policy checklist - what is needed?

- ☑ Sarcoma patients or their representatives are involved in health technology assessment (HTA) and other access pathways
- ☑ There are special regulatory and access pathways for rare cancers
- ☑ There is alignment between regulatory and reimbursement agencies on evidentiary requirements for sarcoma and other rare cancers
- ☑ A national clinical trial portal listing all ongoing sarcoma clinical trials is available to the public

Why this is important

- **It is not appropriate for data requirements for both registration and reimbursement to be the same for rare cancers as for more common cancers** because of the difficulty of obtaining clinical trial data on rare cancers.
- **Involvement of sarcoma patients in HTA and other access decision-making processes** is key to ensure that **the valuation of new treatments for a rare cancer like sarcoma is based on what matters most to patients** and that priority is given to treatments that may make the greatest difference to patient care. Ideally, patients should also be involved in the design stage of clinical trials.
- **Sarcoma patients are often not aware of the possibility to participate in clinical trials.** For example, a national sarcoma survey in the UK found that the majority of patients (67%) were not asked by their doctor if they wanted to take part in a clinical trial and if they were, uptake was low (22%).[11]

How well are we doing across Europe?

- **The European Medicines Agency recognises the need for greater flexibility in drug regulatory pathways** in order to improve rapid access to innovative treatments to patients for whom few treatment alternatives exist. This includes allowing smaller trials and adapted trial designs for rare cancers,[23] accelerated review, conditional marketing authorisation and adaptive licensing.[13 18 24]
- **Unfortunately, this flexibility is not necessarily matched by reimbursement and HTA agencies in most countries.** This often leads to long delays, or even denial of access to patients in many countries.[8 13 14 18]
- In many countries, **patient groups** are leading efforts to try to reduce existing disparities in access to treatments for rare cancers. However, patients are not involved in HTA or access decisions in any country other than the UK.
- **Although early access or compassionate use programmes exist in many countries, they have not necessarily been applied to sarcoma.**
- **Sarcoma clinical trial information is available to the public in all countries,** however access to clinical trials is often limited for patients who are treated within sarcoma centres of reference.

Conclusion

Despite advances in research, patient management and treatment in recent years, sarcoma patients still report some of the poorest experiences of care among cancer patients.[11]

This report proposes five key areas where policy advances are needed to help redress this situation. All patients with sarcoma should have access to specialised care in designated centres of reference. Links between centres of reference and community providers must be strengthened, so that high-quality diagnosis and care is available to patients across the entire care pathway. Regulatory and access requirements should be adapted to reflect the specificity of sarcomas, for example the difficulty in obtaining data from large clinical trials. And finally, promising new treatments should be made available to patients as rapidly, and equitably, as possible in all European countries.

These recommendations are, to a large extent, also applicable to other rare cancers, and may thus also be extended to improve the situation for other rare cancers. As rare cancer patients represent 22% of all cancer cases in Europe,[1] the urgency to improve care and outcomes for these patients should be a key priority for all European health care systems.

Part 2:
Country Profiles

This section contains 6 country profiles on sarcoma, which have been developed for France, Germany, Italy, Spain, Sweden, and the United Kingdom.

These documents look at how well each country is performing against the five key recommendations set out in the Sarcoma Policy Checklist.

Sarcomas in **France**

Each year, 3,526 people are diagnosed with soft tissue (STS) and visceral sarcoma and 521 people are diagnosed with bone sarcoma (2013 data).[25]

1

There should be at least one designated centre of reference for sarcomas.

There are 28 reference centres for sarcoma.[26] Three are national coordinating centres, nine are designated expert centres and 16 are reference centres.[27] Pathology and oncology have separate reference centres organised in respective networks. Oncology reference centres are organised in the **French Clinical Reference Network for soft tissue and visceral sarcomas (NetSarc)** and the **French Reference Network for bone sarcoma and rare bone tumours (ResOs).**[25 28]

The **NetSarc** model has proven to be successful as patients treated by expert surgeons and oncologists in the NetSarc network have had better outcomes than those treated outside this network.[29] Patient representatives are part of the NetSarc committee and provide input on areas for improvement.

Pathology reference centres are organised in the **French Sarcoma Pathological Reference Network (RRePS).**[28] RRePS ensures a second expert pathological review for confirmation of diagnosis of all soft tissue sarcomas. ResOs is both a clinical and pathology network for bone tumours.[26 28] More than 90% of patients have benefited from a second reading of their pathology report by the RRePS and ResOs networks.[26] Over 40% of first histological diagnoses were modified after the second reading and resulted in an alternative treatment course.[17 30]

Accreditation of reference centres is by self-assessment and an independent external assessment.[25 27] There is no monitoring system in place, and centres can keep their accreditation, for example, if one of their key sarcoma experts decides to leave. Also, accreditation is mostly based on a centre's expertise in oncology, not surgery, and the quality of sarcoma surgery varies considerably between reference centres.

There are few sarcoma surgeons in France, and many sarcoma patients are treated by general surgeons lacking sarcoma expertise. For this reason, there are ongoing discussions to develop a national network for sarcoma surgery to build up the sarcoma surgery community, and ensure that sarcoma patients are only operated upon by specialised sarcoma surgeons in reference centres.[31]

2 All relevant specialists should receive training on sarcomas and training on rare cancers should be included in the general medical curriculum.

Sarcoma treatment guidelines exist in France, however professional awareness of these guidelines is lacking, particularly in rural areas. Radiology and pathology guidelines require that all patients with a suspected sarcoma be sent to a tumour board and treated in a reference centre.[31] There are no official guidelines for sarcoma surgery.

The **general medical school curriculum** does not include training on rare cancers but there are ongoing discussions to do so. Patient groups are working closely with medical institutions to improve training opportunities in sarcoma.

An **e-learning programme** was set up by the Gustave Roussy Institute to better educate radiologists, general practitioners, general surgeons and non-expert surgeons on sarcomas. It includes modules on all sarcoma types, with online theory and technical video lectures, and expert Q&A sessions. Additionally, the school of oncology training (EFEC) offers a professional development course to all healthcare professionals on sarcoma.[32]

A **surgical training** course on sarcomas is also available called **e-surge.** It provides sarcoma and GIST live surgery and classroom training and is hosted by various institutions. It aims to train the expert surgeons within Netsarc.[33]

3 Sarcoma care should be delivered by a multidisciplinary team (MDT) following a managed care pathway.

European Society for Medical Oncology (ESMO) guidelines provide the basis for sarcoma guidelines in France.[25] Multidisciplinary guidelines are also produced and disseminated by NetSarc.[25 34]

There is no national definition of the minimum composition of a MDT, and whether patients formally receive a dedicated key health worker depends on the treatment centre. However, patients receive a personalised care plan, and difficult patient cases are often discussed at the inter-regional expert multidisciplinary committee meetings.[34]

4 Incentives for and investment in research on sarcomas need to be improved.

The national **Sarcoma Database** gathers clinical data from patients discussed on **sarcoma multidisciplinary committees (RCP) in NetSarc centres.**[25 28] The database is used by the **French Sarcoma Group – Bone Sarcoma Study Group (GSF-GETO)** for sarcoma research. Additionally, the **RRePS and RESOS** pathology networks have a tissue bank that allows for translational research.[26]

The above sarcoma databases collect **standardised data** on characteristics of patients and tumours, care and follow-up. Survival data are often not recorded, aside from date of death. A quality assurance programme has been established and an external audit is planned.[25]

Due to the amount of data available through these networks, France currently leads research projects on sarcoma in the international cancer genome projects.[35] For example, there are 142 translational studies that have been started or are on-going in France for rare cancers, and 49% of these studies are within NetSarc (2013 figure).[25]

Info Sarcomes also provides an annual research grant of €15,000 to support sarcoma research.[31]

5 Efforts should be made to improve access to care for sarcoma patients at the national level and reduce inequalities in access to new treatments.

Special regulatory and access pathways for rare cancers have been established. Early access programmes such as the ATU (Autorisation Temporaire d'Utilisation) exist for conditions that are life threatening and/or for which there is no therapeutic alternative. Submissions for medicines which (a) are a new therapeutic modality, (b) address high unmet need, (c) have demonstrated efficacy and tolerability may also be fast-tracked.[36] The ATU agreement can be granted by French CA at a physician's request on an individual patient basis (nominative ATU) or for a defined group of patients (cohort ATU).

Phase two or single arm trial data are not recognised as sufficient evidence to grant reimbursement by national pricing and reimbursement authorities.

Patients are not involved in health technology assessment or access pathway decisions, but patient groups (Info Sarcomes) are trying to gain access to these decisions.

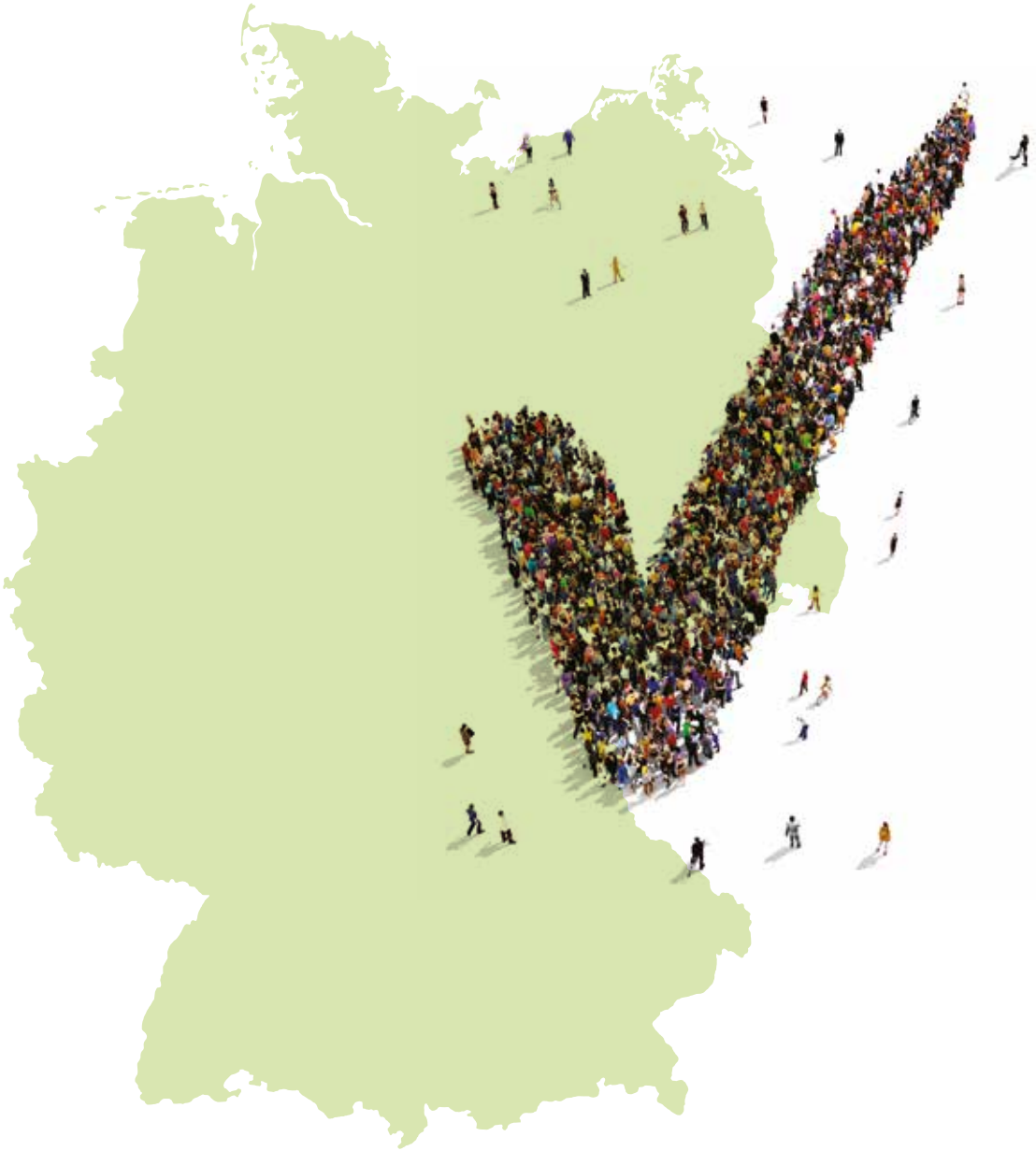
There is a registry of clinical trials organised by the National Institute of Cancer (INCA), called NETSARC clinical trial network.[25] The NetSarc-ResOs network allows for access to national clinical trials. However, there is still limited access to clinical trials for patients who are not treated in a sarcoma reference centre.

Key Links

Info Sarcomes: http://www.infosarcomes.org/

SOS Desmoide: http://www.sos-desmoide.asso.fr/

Sarcomas in **Germany**



There were 3,940 new cases of soft tissue sarcomas (STS), 860 of bone sarcomas and approximately 1,200 of gastrointestinal stromal tumour (GIST) in 2013. There were 385 deaths due to bone sarcomas and 1,575 due to STS in 2013.[37]

1 There should be at least one designated centre of reference for sarcomas.

There are several high-volume centres that treat sarcoma patients in Germany. Two sarcoma centres, in Mannheim and in Essen, have applied to be part of the **European Reference Network** for rare solid tumours including sarcomas.

A formal accreditation system for national reference centres for STS is currently under development. This will be set up under the Deutsche Krebsgesellschaft e.V. and OnkoZert certification process. First accreditations are expected for spring 2018. However, there is no formal accreditation process for designating bone and GIST national sarcoma reference centres. This means that any hospital can establish a sarcoma centre and sarcoma experts may not necessarily work at a sarcoma centre, making it difficult for patients to access the appropriate care.

Due to this, patient organisations have taken an active role in helping patients find national sarcoma treatment centres and specialists. Specifically, **Das Lebenshaus** recommends a list of high-volume centres and experts having a multidisciplinary approach to care.[38]

2 All relevant specialists should receive training on sarcomas and training on rare cancers should be included in the general medical curriculum.

There is no national sarcoma referral protocol that advises health care professionals of 'red flag' symptoms, or identifies steps to confirm initial diagnosis and the need for further referral.

Rare cancers are not part of the general medical curriculum and there is low professional awareness of sarcoma and how to correctly diagnose it. In a German study on the accuracy of sarcoma diagnosis, the error rate of primary diagnosis was over 60% among non-specialised pathology departments.[39] Unfortunately, raising awareness of sarcomas and other rare cancers through training of the healthcare workforce is currently not a national priority.

There is also a lack of specialised sarcoma training opportunities for oncologists and other specialists.

The **Annual Sarcoma Conference** (www.sarkomkonferenz.de) was established in 2011 in Germany to strengthen the existing sarcoma community, by providing a platform for medical experts, industry and patient representatives from the German-speaking sarcoma community (Austria, Germany and Switzerland) to share knowledge and expertise with a focus on education and a view to help change policy priorities at the national level.[40]

3 Sarcoma care should be delivered by a multidisciplinary team (MDT) following a managed care pathway.

National treatment guidelines for the treatment of paediatric STS [41] and uterine sarcomas are available,[42] and national guidelines for the treatment of adult STS are being developed from 2017-2019.

Currently, the minimum composition of a sarcoma MDT is not specified, and organisation of care into MDTs varies across centres. However, treatment by a specialised sarcoma MDT will become a key criterion for accreditation of national sarcoma reference centres in the future.

4 Incentives for and investment in research on sarcomas need to be improved.

There is **no national sarcoma registry** covering all sarcomas. However, there are initial efforts to change this. A national registry focuses on more common tumour types but sarcoma patients are not well documented in it.

There are several national research and study groups for sarcoma. The **Interdisciplinary Working Group on Soft Tissue Sarcoma** of the **German Cancer Society** is involved in producing clinical guidelines, conducting research, encouraging participation in registries, providing training and collaborating with international research groups on sarcoma (especially **Rare Cancers Europe**).[43] The **German Interdisciplinary Sarcoma Group (GISG)** is also involved in all these initiatives, but focuses on the initiation of national clinical trials as well as participating in international clinical trials.[44] The **Sarcoma Working Group of the Arbeitsgemeinschaft Internistische Onkologie,** which consists of medical oncologists, is also involved in conducting and developing clinical trials.

Despite these research efforts, overall, more funding is needed for basic sarcoma research in Germany to better understand the natural history of sarcoma and guide the development of effective treatments.

5

Efforts should be made to improve access to care for sarcoma patients at the national level and reduce inequalities in access to new treatments.

Patient representatives are not involved in the health technology assessment processes. There are no special access pathways for orphan drugs, however, a full reimbursement dossier is not required and orphan drugs are by law granted with an added benefit rating.

The **GISG** publishes ongoing and completed trials in sarcomas online and this information is readily available to patients.[45] Also, the patient support group **Das Lebenshaus** and **SOS Desmoid** provide clinical trial information online.[46] [47] However, patients must be treated in a sarcoma reference centre in order to be recruited for clinical trials, therefore, access can be problematic.[48]

Key Links

Das Lebenshaus e.V.: www.daslebenshaus.org

SOS Desmoid e.V.: www.sos-desmoid.de

German Interdisciplinary Sarcoma Group (GISG): www.gisg.de

AIO Arbeitsgemeinschaft Internistische Onkologie / STS:
www.aio-portal.de/index.php/ueber-uns-294.html

Deutsche Krebsgesellschaft – IAWS Working Group:
www.krebsgesellschaft.de/deutsche-krebsgesellschaft-wtrl/deutsche-krebsgesellschaft/ueber-uns/organisation/sektion-b-arbeitsgemeinschaften/iaws.html

German Sarcoma Conference – Sarkomkonferenz: www.sarkomkonferenz.de

Sarcomas in **Italy**

Sarcomas of all types have an incidence rate of 6 cases per 100,000 per year, and there are an estimated 5883 new cases per year (2015). Of them, 4072 soft tissue sarcomas (STS), 499 bone sarcomas, 386 gastrointestinal stromal tumours (GIST) and 927 Kaposi sarcomas.[49]

1 There should be at least one designated centre of reference for sarcomas.

Approximately 10 sarcoma reference centres were selected in Italy to be part of the European Reference Network (ERN) for rare adult solid tumours including sarcomas.[50]

The Italian Rare Cancer Network is a professional clinical network that connects reference centres for sarcomas. It is based on voluntary collaboration of participating centres, and currently averages 1000 patients each year.[51] The Network currently lacks a formal accreditation framework, however, efforts are ongoing to establish one.[49 51] **For example, the Italian Society of Surgical Oncology (SICO)** is working with different cancer societies to define criteria for reference centres in the Italian Rare Cancer Network and create a set of **quality indicators.** However, the question of who will be responsible for accrediting and evaluating the reference centres in the future remains unanswered.[52]

In 2016, a **working group** was set up involving the **Italian Ministry of Health** and representatives from 20 regions to determine how best to formally incorporate the Italian Rare Cancer Network into the National Health System.[51] Discussions are still ongoing, however the goal is to have the Italian Rare Cancer Network formally recognised within national healthcare objectives.[50]

2 All relevant specialists should receive training on sarcomas and training on rare cancers should be included in the general medical curriculum.

Overall, **clinical practice guidelines** for various types of sarcomas (Ewing sarcoma,[53] bone sarcoma,[54] STS and GIST[55]) recommend referral to specialised centres for appropriate histological diagnosis and treatment.

There is no formal training on rare cancers (including sarcoma) within the **general medical curriculum** or **oncology training,** however efforts are ongoing to create more formalised training programmes.[50 52]

A **specialised surgical training** programme for sarcomas is offered by the **European School of Soft Tissue Sarcomas hosted in Italy.**[56] This is important as, in Italy, surgeons are often the first port of call for patients with sarcoma. A number of other exchange programmes also exist.[50 57] There is also a course offered on musculoskeletal pathology at the Istituto Ortopedico Rizzoli di Bologna.[58]

3 Sarcoma care should be delivered by a multidisciplinary team (MDT) following a managed care pathway.

Currently, multidisciplinary care is not mandatory or monitored in Italy, and the composition of a sarcoma MDT is not clearly defined. However, multidisciplinary care standards and guidelines are being built into the accreditation criteria for designating reference centres in the **Italian Rare Cancer Network.**[52] The organisation of care in MDTs was also a prerequisite for centres to be considered part of European Reference Network (ERN) for rare adult solid tumours including sarcomas, to be operational in 2017.[50]

Patients may receive a personalised care plan and dedicated key health worker depending on hospital resources, but it has not been made mandatory at the national level.

4 Incentives for and investment in research on sarcomas need to be improved.

The **Italian National Association of Cancer Registries (AIRTUM)** collects population-level data on sarcoma (incidence, prevalence and survival) from 40 general population-based cancer registries and 5 specialised cancer registries.[49] It covers 50% of the 2013 Italian population and allows for collaborative studies on cancer epidemiology in Italy.[49] However, the AIRTUM database does not always provide detailed information on pathology and patient outcomes. The **Italian Rare Cancer Network** has its own database of all clinical cases tele-consulted, but this is not representative of all sarcoma cases.[50]

There is therefore the need for **prospective hospital-based data** on the diagnosis, management, and outcomes of sarcoma patients. Currently, only two centres (Milan and Bologna) provide this kind of data. Mandatory prospective data collection is to be built into the accreditation criteria for ERNs.[52]

The **Italian Sarcoma Group** leads research activities and data collection efforts for sarcoma in Italy. There are also significant research efforts in paediatric sarcomas.[59 60]

5 Efforts should be made to improve access to care for sarcoma patients at the national level and reduce inequalities in access to new treatments.

There are no fast-track mechanisms for orphan drugs for rare cancers in Italy. Access to innovative treatments for conditions where no alternative therapy exists is stipulated by a decree (No. 648/96).[61] [62] In theory, there is a 100-day limit on fast-track pricing and reimbursement negotiations, however, this is not always respected. This decree has yet to be used for rare cancer drugs.

Patients are not involved in health technology assessment (HTA) or access pathway decisions in Italy.

National improvement efforts are ongoing to improve access to therapies for rare cancers. **The Association of Italian Medical Oncologists (AIOM)** produced recommendations to improve access to care for sarcoma patients and is currently working with the **Italian Agency for Drugs (AIFA)** to reduce the complexity and time needed to introduce new drugs.[49]

In terms of access to clinical trials, the **Italian Sarcoma Group** provides a list of all ongoing sarcoma trials in Italy and a comprehensive list of all clinical trials is available on the AIOM website.[63] [64]

Key Links

The Italian Sarcoma Group: http://www.italiansarcomagroup.org/

Sarcomas in **Spain**

The incidence of soft tissue sarcoma (STS) in Spain is estimated to be 3.1 cases per 100,000 population (2015).[65]

1

There should be at least one designated centre of reference for sarcomas.

Five sarcoma reference centres have been endorsed by the Spanish Ministry of Health to become part of the **European Reference Network (ERN)** for rare adult solid tumours including sarcomas: two in Catalonia, two in Madrid and one in Seville.[66 67 68]

These centres were selected based on the following criteria: they see a **minimum number of patients** each year (80 cases for soft tissue sarcoma, 10 for retroperitoneal sarcoma, and 10-12 for bone sarcoma);[67] they have appropriate **multidisciplinary structures** in place, including surgeons, radiologists, pathologists and medical oncologists who all contribute to treatment plans; and they have **a pathology department** for diagnosis.[66]

There is **no monitoring system** currently in place. Plans to develop a new accreditation system have been discussed, however it is unclear who will be responsible for carrying out evaluations.[67] What's more, financial barriers are slowing down its implementation (see case study below).

CASE STUDY

Lack of funding for reference centres in Spain

Despite the formalisation of national reference centres for sarcoma in Spain, lack of funding is an ongoing concern, and no additional budget has been allocated to build and maintain multi-disciplinary teams and relevant services within these centres.[66]

Patients must often travel to another region (Comunidad Autonoma) to receive appropriate treatment. Associated costs, for example for travel, are often not reimbursed, leaving patients to pay for them out-of-pocket. Oncologists can apply for their patients to be reimbursed, but this process is often slow, and approval is not guaranteed.[67]

2 All relevant specialists should receive training on sarcomas and training on rare cancers should be included in the general medical curriculum.

There are national Spanish clinical practice guidelines that indicate **'red flag' symptoms for referral** to sarcoma reference centres, and recommend pathways from primary centres to sarcoma specialist centres.[69] However, referral is not mandatory, and patients are often treated outside these centres as a result.

The **general medical school curriculum** in Spain differs by university, therefore not all medical school graduates receive training on rare cancers (including sarcomas), let alone oncology in general.[67] There are a number of **sarcoma training courses** offered in Spain to oncologists and surgeons, and sarcoma patient advocacy groups are working to improve awareness among healthcare professionals (see case study).[70 71]

CASE STUDY

Sarcoma awareness training efforts

The Spanish Sarcoma Patients Association (AEAS) and Fundación Mari Paz Jiménez Casado (FMPJC) are leading sarcoma awareness and training efforts nationally and have initiated an early diagnosis programme for sarcomas aimed at primary care physicians. **AEAS and FMPJC** help to refer patients to experienced sarcoma specialists for second opinion diagnosis[72] and treatment. **FMPJC** provides annual scholarships and grants that promote education of physicians and researchers for sarcoma, awarded on an annual basis.[73 74]

3 Sarcoma care should be delivered by a multidisciplinary team (MDT) following a managed care pathway.

Multidisciplinary care guidelines have been produced for all sarcoma types. The latest update of the soft tissue sarcoma multidisciplinary clinical practice guidelines was published in 2016 by **the Spanish Group for Research on Sarcomas (GEIS)** and **the Spanish Oncology Group (SEOM).**[75] According to the guidelines, **MDTs** should include at a minimum pathologists, radiologists, surgeons, radiation and medical oncologists.[69] GIST and bone sarcoma guidelines are currently being updated.

The allocation of a dedicated health worker and a **personalised care plan** for sarcoma patients is currently not a priority due to limited resources and practice varies between hospitals. Personalised care plans tend to be informal and depend on the healthcare professional.[67]

4 Incentives for and investment in research on sarcomas need to be improved.

There is no national sarcoma registry in Spain and this is unlikely to be a priority for the government due to financial constraints. There is no mandatory data collection among sarcoma centres.

There are, however, national and international research collaborations for sarcoma, led by **GEIS.**[73 76 77] They facilitate national collaboration between medical and research staff in Spain and internationally. There are 70 participant centres spread across Spain, with more than 20 ongoing collaborative clinical trials. GEIS also promotes basic and translational research in sarcoma.[73 76 77]

In addition, the **Rare Sarcoma Project** was set up to determine the burden of rare sarcomas in Spain. Data collected includes epidemiology, type of treatment, tissue samples, pathology and biopsy reports. These data will be used to improve treatment pathways for patients.[67]

5 Efforts should be made to improve access to care for sarcoma patients at the national level and reduce inequalities in access to new treatments.

There are no specific health technology assessment (HTA) criteria for orphan drugs. However, there is a compassionate use application for drugs that have not yet been approved which is available in some hospitals.[67] Generally, access to orphan drugs is limited: a survey looking at 60 orphan drugs found that only 33% were available in Spain as compared to 90% in France, the Netherlands and Denmark.[78]

Patients are not typically involved in HTA and access pathways in Spain, however they have been so for a number of orphan drugs. Patient associations have also created a joint platform and led efforts to help improve access to drugs for sarcoma. For example, the **FMPJC** provides patients with access to a legal team to give advice on access issues they face.[67]

After national approval of price and reimbursement, new treatments must be included in each of 17 regional formularies, and then individual hospitals may or may not also conduct their own evaluation. This system of drug approvals may result in significant delays and regional inequalities in access to new treatments for patients.

Clinical trial information is available to patients on **GEIS website** and **AEAS website** for sarcomas and GIST.[79]

Key Links

The Spanish Sarcoma Patients Association (AEAS): http://www.aeasarcomas.org/

Fundación Mari Paz Jiménez Casado (FMPJC): http://www.fundacionmaripazjimenez.org/

Spanish Group for Research on Sarcoma (GEIS): http://www.grupogeis.org/

Sarcomas in **Sweden**

Approximately 300 patients are thought to be diagnosed with sarcoma each year; however, this number is estimated to be higher (400-500 cases) due to under-registration of gastrointestinal stromal tumour (GIST) cases.[80] There are 70-100 cases of bone sarcomas, 50 cases of visceral and retroperitoneal sarcomas, and approximately 180-250 cases of other soft tissue sarcomas (STS) per year. GIST would be among the visceral cases, but since the incidence of GIST alone has been estimated to about 12/million each year, Sweden has about 120 new cases of this entity. Additionally, sarcomas represent approximately 10% of all cancers affecting children and young adults.[80 81]

1

There should be at least one designated centre of reference for sarcomas.

Sweden has centralised sarcoma care in five out of the six healthcare regions. There are **sarcoma reference centres** in Gothenburg, Linkoping, Lund, Stockholm and Umea regions.[82] Uppsala is the only healthcare region that has not been successful at centralising sarcoma care in a reference centre due to the lack of a multidisciplinary tumour board and of orthopaedic surgeons within the region. Therefore, patients in Uppsala are referred to Stockholm for surgery, and other treatment is decentralised.

While there is no formalised system for **accreditation and monitoring** of reference centres, there is a long-standing tradition of referral of sarcoma patients to these centres.

Monitoring of quality of care for extremity and trunk wall sarcomas is done by online reporting of data. Time from referral to diagnosis, and from diagnosis to treatment, is collected nationally as is information on a multidisciplinary team (MDT) treatment decisions and R0 surgical margins.[83] Further monitoring is also done within each individual sarcoma reference centre. For example, in Lund, a multidisciplinary steering group meets at least three times a year to discuss quality of care issues, and potential solutions for all sarcoma patients (e.g. number of specialists, training, resources, etc.).

2 All relevant specialists should receive training on sarcomas and training on rare cancers should be included in the general medical curriculum.

Training on rare cancers is not a mandatory part of the medical curriculum for oncologists, although there is a mandatory soft and bone tumour course in the curriculum for orthopaedic residents that focuses on warning signs of sarcoma and the initial diagnostic process.

Sweden has led the way in Scandinavia by creating **simple referral guidelines for STS.**[84] These advise health professionals of **'red flag'** symptoms of sarcomas and outline **clear steps for referral** to sarcoma reference centres. If sarcoma is suspected, healthcare professionals are recommended to refer patients directly to a reference centre before biopsy and surgery are performed.

This **referral practice** gives patients rapid access to specialist sarcoma care and has been found to improve referral rates, reduce costs associated with local recurrence and result in better surgical results and patient outcomes.[84 85] Depending on the type of sarcoma, between 80-100% of cases are referred to reference centres before surgery.[86 87]

Unfortunately, referral patterns have been less successful for **abdominal sarcomas.** However, improvements are being made by mandating that all retroperitoneal sarcoma patients be operated on at three specific Swedish hospitals from now on. Similarly, GIST patients often receive surgery and oncological treatments outside of a sarcoma reference centre.

The **Scandinavian Sarcoma Group (SSG)** exists to connect Scandinavian sarcoma professionals and collectively produce protocols, guidelines and recommendations collaboratively with multidisciplinary input.[88 89] Swedish reference centres are linked with reference centres in Norway (Oslo, Bergen, Trondheim and Tromsö) and to a lesser degree with reference centres in Finland and Denmark.

3 Sarcoma care should be delivered by a multidisciplinary team (MDT) following a managed care pathway.

It is estimated that 90% of sarcoma patients are treated by a MDT.

National standardised pathways for sarcomas are currently being developed by the inter-regional boards of the **Regional Cancer Centres (RCC),** in collaboration with the **Swedish Communities and Counties (SKL).** However, implementation is not accompanied by an increase in healthcare workforce to meet targets.

The **Swedish Cancer Registry** collects data on all sarcoma patients in Sweden and includes information on whether or not treatment plans were decided by a **multidisciplinary tumour board,** whether or not the patient was assigned a **contact nurse,** and when the patient was informed about their **initial treatment plan.**[81] Data on referral patterns, lead times, and tumour characteristics are also collected.

4 Incentives for and investment in research on sarcomas need to be improved.

Patient data from all sarcoma reference centres are collected nationally in the INCA sarcoma quality registry as part of the **Swedish Cancer Registry.** The INCA platform has national coverage from all regions. Discussions are underway to try and link Swedish sarcoma patient data with data from other Nordic countries, as they all collect the same data, therefore this can be merged easily. From 2017, a joint Scandinavian annual report on sarcoma care will be presented.

The SSG leads national and international research collaborations on sarcoma in Scandinavia.[90] It has played an important role in improving the quality of sarcoma diagnosis, pathology,[81] treatment and care and creating a platform to coordinate basic research and clinical trials in all participating countries.[88]

5 Efforts should be made to improve access to care for sarcoma patients at the national level and reduce inequalities in access to new treatments.

There are no specific health technology assessment criteria for orphan drugs and patients are not involved in this process. However, there are ongoing discussions if a higher cost per QALY (quality adjusted life year) should be accepted as well as higher degrees of uncertainty in clinical evidence for orphan drugs due to disease severity and high unmet need.

Most hospital medicines (eg. cancer medicines that are infusion-based) are not reimbursed through the National Authority, i.e. the Swedish Pharmaceutical Benefits Agency (TLV). Instead these are evaluated through **a new managed entry pathway,** which aims to reduce inequalities in access across the 21 independent county councils.

This new process pathway is currently under investigation by The Swedish Agency for Health and Care Services Analysis (Vårdanalys). The investigation will evaluate the whole process from a patient perspective (i.e. the transparency, predictability and process efficacy). The conclusions of the the authority report will be fed into the newly announced governmental investigation of pharmaceutical pricing and funding which will have a draft report presented in November 2017 and be finalized in the end of 2018.

Generally, access to clinical trials is good, with some exceptions of the Uppsala region due to lack of reference centres. All reference centres are made aware of all clinical trials initiated by the SSG.

Key Links

The Scandinavian Sarcoma Group: http://www.ssg-org.net/

The Scandinavian Sarcoma Group for Nurses and Physiotherapists:
http://www.ssg-nurses-physiotherapists.org/

Sarcomas in the **United Kingdom**

Each year, **3,800 patients are diagnosed with sarcoma in the United Kingdom,** representing 1% of all cancer diagnoses.[11]

1 There should be at least one designated centre of reference for sarcomas.

Reference centres exist for all types of sarcomas in the UK. There are approximately 15 sarcoma specialist centres in England. South Wales has a specialist centre for soft tissue sarcomas (STS) and all other Welsh sarcoma patients are sent to England for treatment. Northern Ireland has three hospitals that treat sarcoma patients, while Scotland has five in total working as a single network.[91 92]

There is **no formal national accreditation system** for sarcoma reference centres and currently, accreditation is based on Specialist Commissioning. Standards are set by key publications from the **National Institute for Health and Care Excellence (NICE)** "Improving Outcomes for People with Sarcoma: the Manual (2006) and Sarcoma Quality Standards" (2014) (see below).[8 12 93] Each clinical commissioning group (CCG) is responsible for ensuring that reference centres are accessible to every UK individual, and that they operate in accordance with these national standards.[94]

There is **no formalised monitoring** system in place to consistently evaluate quality of care in sarcoma reference centres. However, a Quality Surveillance Programme based on the **Sarcoma Measures** publication by the **National Cancer Peer Review-National Cancer Action Team** is being developed.[93]

NICE Sarcoma Quality Standards dictate quality of care expectations for sarcoma centres.[93]

Statement 1
Sarcoma advisory groups and sarcoma multidisciplinary teams (MDTs) have pathways for referral and diagnosis in place for people with suspected sarcoma.

Statement 2
Adults, children and young people with bone sarcoma and adults with soft tissue sarcoma have their care plan confirmed by a sarcoma MDT and treatment delivered by services designated by the sarcoma advisory group.

Statement 3
Sarcoma MDTs publish information about their shared pathways, activity and patient outcomes, including information on site-specific sarcomas.

Statement 4
People with a retroperitoneal sarcoma are referred before having any treatment to a sarcoma treatment centre with special expertise in managing this type of tumour.

Statement 5
Surgeons performing planned resections of sarcomas are core or extended members of a sarcoma MDT.

Statement 6
People with sarcoma are supported by an allocated key worker with specialist knowledge of sarcomas and their treatment.

2 All relevant specialists should receive training on sarcomas and training on rare cancers should be included in the general medical curriculum.

Referral criteria have been established for all sarcomas that advise health professionals of **'red flag' symptoms of sarcoma** that warrant referral to a reference centre.[91 95 96] Additionally, the importance of early diagnosis for cancer is stressed in several key national publications by NICE.[8 12 97]

The **general medical curriculum** does not include modules on sarcomas or rare cancers, and training on these topics very much depends on each individual medical school. There is, however, very good availability of specialist training on sarcoma in the UK, with many secondments from overseas.

More professional training could benefit sarcoma patients, as delays to diagnosis and misdiagnosis persist in the UK. According to the Sarcoma Patient Survey, only 20% of sarcoma patients were told by their general practitioner (GP) or emergency doctor that they might have sarcoma, and 27% of patients who visited their GP were started on treatment for another condition, or told that their symptoms were not serious.[11]

Two initiatives that were set up to improve awareness among general practitioners of sarcoma symptoms and encourage early referral include the 'On the ball' campaign (see case study) and the 'Awareness & Suspicion for Sarcoma' programme, which is accredited by the British Medical Journal and teaches doctors how to properly diagnose sarcomas

CASE STUDY

Raising awareness of sarcomas with the 'On the ball' campaign by Sarcoma UK

The 'On the ball' public awareness campaign led by Sarcoma UK aims to educate GPs to identify sarcomas earlier.[98]

'On the ball' packs contain a golf ball key ring with the message 'is it sarcoma?' to act as a visual reminder to GPs about 'red flag' signs of sarcoma, as well as a diagnostic toolkit with a clinical information sheet about sarcomas, the 'red flag' signs of sarcoma and the need to refer sarcoma patients directly to specialist centres for diagnosis and treatment.[92 99]

Over 1,600 On the Ball packs have been delivered so far.[99]

3 Sarcoma care should be delivered by a multidisciplinary team (MDT) following a managed care pathway.

Guidelines exist in the UK for treatment of all sarcoma types.[91 95 96] They specify the need for MDTs, key health workers for patients, evidence-based treatment at sarcoma centres, and personalised care plans for patients.[91 95] There is broad recognition of the importance of a MDT approach and it seems to be occurring widely for sarcoma patients. In 2015, 90% of sarcoma patients were treated by a MDT in the UK.[8]

However, there are still improvements to be made to ensure all patients receive personalised care plans. The Sarcoma Patient Survey found that only 48% of patients received a plan that told them everything from start to finish of their treatment and 40% of patients said they did not receive a personalised care plan.[11]

4 Incentives for and investment in research on sarcomas need to be improved.

There is no **UK-wide sarcoma registry.** However, the National Cancer Registration and Analysis Service (NCRAS) coordinates eight regional cancer registries and registration is mandatory for all NHS hospitals but not privately provided treatments. There is a national dataset for sarcoma. Data is collected on treatments, such as chemotherapy use and radiotherapy. All data maps into national data on deaths to give a full picture of when a patient was diagnosed, how they were treated, and their date of death.

There are no governmental incentives for research into rare diseases.[94] However, Sarcoma UK funds different research projects and since 2009 have awarded over £1 million in scientific medical grants to better understand sarcomas.[100]

The voluntary sector investment in sarcoma research is more than £4 million per year and continues to grow. There are a number of **research collaborations** between charitable organisations, universities, clinical units, NGOs (Cancer Research UK, Sarcoma UK, Bone Cancer Research Trust), and the government - with funding available through National Institute for Health Research and Medical Research Council.[94]

5

Efforts should be made to improve access to care for sarcoma patients at the national level and reduce inequalities in access to new treatments.

Special regulatory or access pathways for rare cancers have been established. The NICE ultra-orphan process was developed based on the appraisal of a sarcoma drug, however recent sarcoma treatments have been approved through the Cancer Drugs Fund.[94 101 102] Sarcoma patients can, and have been involved in the NICE drug appraisal and guidance process.[94]

Sarcoma clinical trials, and trials that are open to sarcoma patients are available online through the UK Clinical Trials Gateway.[103] However, sarcoma patients may be **inadequately informed about clinical trial options** - 67% of patients in the Sarcoma Patient Survey reported not being asked if they would like to take part in a clinical trial, and only 22% of patients took part in one.[11]

Key Links

Sarcoma UK: https://sarcoma.org.uk/

References

1. Gatta G, Van der Zwan JM, Casali PG, *et al*. Rare cancers are not so rare: The rare cancer burden in Europe. *European Journal of Cancer* 2011;47(17):2493-511.
2. Rare Cancers Europe (RCE). About rare cancers 2016. Available from: http://www.rarecancerseurope.org/About-Rare-Cancers
3. Rare Cancers Europe (RCE). Joint Action on Rare Cancers Kick Off Meeting 2016. Available from: http://rarecancerseurope.org/Events/Joint-Action-on-Rare-Cancers-Kick-Off-Meeting
4. Fletcher C, Bridge JA, Hogendoorn P, *et al*. WHO Classification of Tumours of Soft Tissue and Bone. Fourth Edition. WHO Classification of Tumours, 2013:468.
5. Cancer Research UK. About Sarcomas 2016. Available from: http://www.cancerresearchuk.org/about-cancer/type/sarcoma/about/about-sarcomas#YxpbR62XBff7D1h4.97
6. Stiller CA, Trama A, Serraino D, *et al*. Descriptive epidemiology of sarcomas in Europe: report from the RARECARE project. *European Journal of Cancer* 2013;49(3):684-95.
7. Sharma S, Takyar S, Manson SC, *et al*. Efficacy and safety of pharmacological interventions in second- or later-line treatment of patients with advanced soft tissue sarcoma: a systematic review. *BMC Cancer* 2013;13(1):1-21.
8. National Institute for Health and Care Excellence (NICE). Improving Outcomes for People with Sarcoma: the Manual. NICE Guidance on Cancer Services: Developed by the National Collaborating Centre for Cancer, 2006:142.
9. Zagars GK, Ballo MT, Pisters PWT, *et al*. Prognostic factors for patients with localized soft-tissue sarcoma treated with conservation surgery and radiation therapy. *Cancer* 2003;97(10):2530-43.
10. Cancer Research UK. Survival statistics for soft tissue sarcomas 2016. Available from: http://www.cancerresearchuk.org/about-cancer/type/sarcoma/treatment/statistics-and-outlook-for-soft-tissue-sarcoma
11. Sarcoma UK. The National Sarcoma Survey, Transforming Patient Experience, 2015:35.
12. National Institute for Health and Care Excellence (NICE). Sarcoma Quality Standards. 2015:38.
13. European Society for Medical Oncology (ESMO). Improving Rare Cancer Care in Europe: Recommendations on Stakeholder Actions and Public Policies. 2010:10.
14. RARECARENet. Country Report Bulgaria 2016. Available from: http://www.rarecarenet.eu/rarecarenet/images/Resources/National_Conferences/RARECARENet_country_report_-_BULGARIA_-_TRANSLATED.pdf
15. European Union Committee of Experts on Rare Diseases (EUCERD). Recommendations on Quality Criteria for Centres of Expertise for Rare Diseases in Member States 2011:13.
16. Rare Cancers Europe (RCE). Rare Cancer Patient Toolkit: Speak up for rare cancer. 2016:19.
17. Ray-Coquard I, Montesco MC, Coindre JM, *et al*. Sarcoma: concordance between initial diagnosis and centralized expert review in a population-based study within three European regions. *Annals of Oncology* 2012;23:2442-49.
18. European Society for Medical Oncology (ESMO). Statement on the council recommendation on rare diseases: ESMO, 2008.
19. Sarcoma Patients EuroNet (SPAEN). Sarcoma Patient Pathway Analysis and Recommendations for Service Development. 2016. Available from: www.sarcoma-patients.eu
20. Andritsch E, Beishonb M, Bielack S, *et al*. ECCO Essential Requirements for Quality Cancer Care: Soft Tissue Sarcoma in Adults and Bone Sarcoma. A critical review. *Critical Reviews in Oncology/Hematology* 2017:94-105.
21. European Union Committee of Experts on Rare Diseases (EUCERD). Recommendations on Rare Disease European Reference Networks (RD ERNS). 2013:18.
22. The Council of the European Union. Council Recommendation of June 8 2009 on an action in the field of rare diseases: Official Journal of the European Union, 2009:7-10.
23. Ashley D, Thomas D, Gore L, *et al*. Accepting risk in the acceleration of drug development for rare cancers. *Lancet Oncology* 2015;16:4.
24. Casali PG, Bruzzi P, Bogaerts J, *et al*. Rare Cancers Europe (RCE) methodological recommendations for clinical studies in rare cancers: a European consensus position paper. *Annals of Oncology* 2015;26(2):300-6.
25. Institut National Du Cancer. French national networks for rare cancers in adults: review and outlook 2014. 2015.
26. Coindre JM, Ducimetiere F, Mathoulin-Pélissier S, *et al*. Management of soft tissue sarcoma in France - a retrospective analysis of the french clinical biological sarcoma database (GSF-GETO) [abstract]. 2015.
27. Dumont S. Epidémiologie des Sarcomes Réseaux de Référence, unpublished data on file. 2016
28. NetSarc-ResOs. Réseaux de référence Cliniques 2016. Available from: https://netsarc.sarcomabcb.org/
29. Blay JY, Le Cesne A, Penel N, *et al*. Improved Sarcoma Management in a National Network of Reference Centers: Analysis of the NetSarc Network on 13,454 Patients Treated Between 2010 and 2014. *Journal of Clinical Oncology* 2016;34.
30. Lurkin A, Ducimetière F, Vince DR, *et al*. Epidemiological evaluation of concordance between initial diagnosis and central pathology review in a comprehensive and prospective series of sarcoma patients in the Rhone-Alpes region. *BMC Cancer* 2010;10(1):1-12.
31. Dumont S. Stakeholder interview with Boldon S, 2016.

32. Ecole de Formation En Cancérologie. Liste offre formations cancerologie 2017. Available from: http://www.efec.eu/wp-content/uploads/2016/10/EFEC-2017-LISTE-OFFRE-FORMATIONS-CANCEROLOGIE-DPC_Web.pdf
33. Connective Tissue Oncology Society (CTOS). Programme e-surge 2016. Available from: https://www.ctos.org/Portals/0/PDF/Programme%20e-surge%20-%202016%20Final.pdf
34. Ligier K, Maynou C, Leroy X, *et al*. Improvement of the initial management of sarcomas after the dissemination of evidence-based guidelines depends on the primary sarcoma location: a population-based study. *BMC Cancer* 2015;15:218.
35. International cancer genome consortium. Cancer genome projects - France 2016. Available from: https://icgc.org/icgc/cgp/60/355/1002113
36. Tordrup D. Autorisations temporaires d'utilisation (ATU). *RARE Journal* 2014;1(3):83-97.
37. Center for Cancer Registry Data - Federal Statistical Office. Report on Cancer in Germany 2016: Number of Cancer Neoplasms and Deaths in Germany 2013.P21.
38. Das Lebenshaus. Interdisziplinäre Sarkom-Zentren: Denn Sarkome gehören in Experten-Hände! Wissenswert, 2010:19-23.
39. Lehnhardt M, Daigeler A, Homann HH, *et al*. Importance of specialized centers in diagnosis and treatment of extremity-soft tissue sarcomas. Review of 603 cases. *Chirurg* 2009;80(4):341-7.
40. Sarkom Konferenz. Sarkomkonferenz 2016. Available from: http://www.sarkomkonferenz.de/index.php/sarkomkonferenz-2017/zertifizierung
41. Gesellschäft für Pädiatrische Onkologie und Hämatologie. Weichteilsarkome. Leitlinie der Gesellschäft für Pädiatrische Onkologie und Hämatologie, 2011.
42. Deutsche Gesellschaft für Gynäkologie und Geburtshilfe. Leitlinienprogramm- Uterine Sarkome, 2015.
43. Deutsche Krebsgesellschaft. Interdisziplinäre AG Weichteilsarkome 2016. Available from: https://www.krebsgesellschaft.de/deutsche-krebsgesellschaft-wtrl/deutsche-krebsgesellschaft/ueber-uns/organisation/sektion-b-arbeitsgemeinschaften/iaws.html
44. German Interdisciplinary Sarcoma Group (GISG). German Interdisciplinary Sarcoma Group. 2016.
45. German Interdisciplinary Sarcoma Group (GISG). Studien unter GISG. 2016.
46. Das Lebenshaus. Krebserkrankungen erforschen 2015. Available from: http://www.daslebenshaus.org/studienallgemein
47. SOS desmoid. 2016. Available from: http://www.sos-desmoid.de/
48. Rüsseler A, Brors B, Fischer T, *et al*. Molekularpathologie von Sarkomen- Erste Ergebnisse des Sarkomforschungsverbundes KoSar. *Der Pathologe* 2010;Suppl 2(31):211-14.
49. Busco S, Buzzoni C, Mallone S, *et al*. Italian cancer figures report 2015: The burden of rare cancers in Italy. *Epidemiologia & Prevenzione* 2016;40(1 Suppl 2):1-120.
50. Casali P. Stakeholder interview with Boldon S, 2016.
51. Trama A, Mallone S, Ferretti S, *et al*. The burden of rare cancers in Italy: the surveillance of rare cancers in Italy (RITA) project. *Tumori* 2012;98(5):550-8.
52. Gronchi A. Stakeholder interview with Wait S, 2016.
53. Italian sarcoma group and alleanza contro il cancro. Linea Guida per il Trattamento del Sarcoma di Ewing 2010. Available from: http://lnx.samosoftware.com/clienti/isg/wp-content/uploads/2014/03/linee.guida_.ewing_.pdf
54. Italian sarcoma group and alleanza contro il cancro. Linea Guida per il Trattameno dell'Osteosarcoma 2009. Available from: http://lnx.samosoftware.com/clienti/isg/wp-content/uploads/2014/03/linee.guida_.osteosarcoma.pdf
55. Associazione Italiana di Oncologia Medica (AIOM). Linee Guida per il trattamento di sarcomi dei tessuti molli e GIST, 2015.
56. European Society of Surgical Oncology (ESSO). European School of Soft Tissue Sarcoma 2016. Available from: http://www.essoweb.org/european-school-of-sts/
57. Fondazione IRCCS Istituto Nazionale dei Tumori. Struttura Semplice Chirurgia dei Sarcomi, Attività Didattica 2016. Available from: http://www.istitutotumori.mi.it/modules.php?name=Content&pa=showpage&pid=792
58. Istituto Ortopedico Rizzoli B, Italy. Course on Musculoskeletal Pathology 2016. Available from: http://www.italiansarcomagroup.org/wp-content/uploads/2015/12/Preliminary-Program-XXIX-COURSE-2016.pdf
59. Associazione Italiana Ematologia Oncologia Pediatrica. Gruppi di lavoro - sarcomi delle parti molli 2016. Available from: http://www.aieop.org/web/index.php?q=node/385
60. Associazione Italiana Ematologia Oncologia Pediatrica. Gruppi di lavoro, sarcomi ossei 2016. Available from: http://www.aieop.org/web/index.php?q=node/386
61. The Italian Medicines Agency (AIFA). Pricing and reimbursement 2016. Available from: http://www.agenziafarmaco.gov.it/en/content/pricing-and-reimbursement
62. The Italian Medicines Agency (AIFA). Legge 648/96 2016. Available from: http://www.agenziafarmaco.gov.it/it/content/legge-64896
63. Italian sarcoma group. Studi clinici 2016. Available from: http://www.italiansarcomagroup.org/studi-clinici/
64. The Associazione Italiana di Oncologia Medica (AIOM). studi clinici 2016. Available from: http://www.aiom.it/studi-clinici/1,108,1,
65. Nersesyan K, Robinson D, Pomerantz D. Epidemiology and treatment of soft tissue sarcoma in the EU5. ISPOR 18th Anuual European Congress 7-11 November 2015. Milan, 2015.
66. Valverde C. "Surgery remains the key in addressing sarcomas" Gaceta Medica; 2016. Available from: http://www.gacetamedica.com/portada/la-cirugia-sigue-siendo-la-clave-en-el-abordaje-de-los-sarcomas-HD489889#printAcUrlSection
67. Valverde C. Stakeholder interview with Boldon S, 2016.
68. The Spanish Group for Research on Sarcomas (GEIS). Centres, Services and Units Reference (CSUR), 2016. Available from: http://www.grupogeis.org/index.php/quienes-somos/centros-de-referencia

69. Garcia del Muro X, de Alava E, Artigas V, *et al*. Clinical practice guidelines for the diagnosis and treatment of patients with soft tissue sarcoma by the Spanish group for research in sarcomas (GEIS). *Cancer chemotherapy and pharmacology* 2016;77(1):133-46.
70. The Spanish Group for Research on Sarcomas (GEIS). Advanced Course Sarcomas. 2016
71. The Spanish Group for Research on Sarcomas (GEIS). Master's Degree in Musculoskeletal Tumors 2016. Available from: http://www.grupogeis.org/index.php/actividad-cientifica/formacion/master-de-sarcomas
72. Sarcoma Patients Euronet (SPAEN). 20th of September - Sarcoma National Day in Spain SPAEN; 2014. Available from: http://www.sarcoma-patients.eu/index.php/28-spaen/home/259-20th-of-september-sarcoma-national-day-in-spain
73. The Spanish Group for Research on Sarcomas (GEIS). Mission, vision and values 2016. Available from: http://www.grupogeis.org/index.php/quienes-somos/mision-vision-y-valores
74. Angeles Diaz M. Together we stand, we learn, strengthen us. LillyPad; 2016. Available from: https://lillypad.es/entry.php?e=162&n=entre-todos-nos-apoyamos-nos-informamos-nos-fortalecemos#
75. López-Pousa A, Martin Broto J, Martinez Trufero J, *et al*. SEOM Clinical Guideline of management of soft-tissue sarcoma (2016). *Clinical and translational oncology* 2016;18:1213–20.
76. The Spanish Group for Research on Sarcomas (GEIS). GEIS structure 2016. Available from: http://www.ctu.mrc.ac.uk/euramos/main_site_content_pages/images_and_documents/Bone%20Planning%20Meeting%20ESF/Presentations/Day2_b_GroupStructures/GEIS_Structure_MartinBroto.pdf
77. The Spanish Group for Research on Sarcomas (GEIS). Spanish Sarcoma Research Group Portal 2016. Available from: http://www.geisgroup.org/
78. Tordrup D, Tzouma V, Kanavos P. Orphan drug considerations in Health Technology Assessment in eight European countries. *International Journal of Public Health* 2014;1(3):15.
79. The Sarcoma Patients Spanish Association (AEAS). Clinical Trials 2016. Available from: http://www.aeasarcomas.org/es/ensayos-clinicos
80. Styring E. Sarcoma Cancercentrum; 2016. Available from: http://www.cancercentrum.se/samverkan/cancerdiagnoser/sarkom/
81. Styring E. Manual. The national quality register for Sarcomas of the extremeties and trunk wall.: Regional Cancercentrum I Samverkan, 2015.
82. Bauer HCF, Alvegard TA, Berlin O, *et al*. The Scandinavian Saroma Group Register 1986-2001,. *Acta Orthopaedica Scandinavica* 2009
83. OCSYD. Kvalitetsuppföljning, RCC Syd, senast uppdaterad 2016-12-07 ocsyd; 2016. Available from: http://www.ocsyd.se/kvalitetsdata/webrapport/
84. Styring E, Billing V, Hartman L, *et al*. Simple guidelines for efficient referral of soft-tissue sarcomas: a population-based evaluation of adherence to guidelines and referral patterns. *The Journal of Bone & Joint Surgery* 2012;94(14):1291-6.
85. Trovik CS. Local recurrence of soft tissue sarcoma. A Scandinavian Sarcoma Group Project. *Acta Orthopaedica Scandinavica* 2001;72(300):1-31.
86. Alvegård TA, Bauer H, Blomqvist C, *et al*. The Scandinavian Sarcoma Group—background, organization and the SSG Register—the first 25 years. *Acta Orthopaedica Scandinavica* 2004;75(sup311):1-7.
87. Rydholm A. Centralization of soft tissue sarcoma. The southern Sweden experience. *Acta Orthopaedica Scandinavica* 1997;273:4-8.
88. Alvegard T, Sundby Hall K, Bauer H, *et al*. The Scandinavian Sarcoma Group: 30 years' experience. *Acta Orthopaedica Scandinavica* 2009;80(334):1-104.
89. Scandinavian Sarcoma Group (SSG). Treatment protocols and recommendations - sarcoma 2016. Available from: http://www.ssg-org.net/treatment-protocols-and-recommendations/ongoing
90. Scandinavian Sarcoma Group (SSG). BYLAWS 2016. Available from: http://www.ssg-org.net/om
91. Grimer R, Athanasou N, Gerrand C, *et al*. UK Guidelines for the Management of Bone Sarcomas 2010. Available from: https://sarcoma.org.uk/sites/default/files/bsg_bone_guideline_in_sarcoma.pdf
92. Sarcoma UK. Sarcoma Specialist Centres 2016. Available from: https://sarcoma.org.uk/health-professionals/sarcoma-specialist-centres
93. National Cancer Peer Review-National Cancer Action Team. Sarcoma Measures. In: Health, ed., 2011.
94. Wilson R. Stakeholder interview with Wait S, 2016.
95. Grimer R, Judson I, Peake D, *et al*. Guidelines for the management of soft tissue sarcomas 2010. Available from: http://www.swscn.org.uk/wp/wp-content/uploads/2014/11/UK_Guidelines_For_The_Management_of_Soft_Tissue_Sarcomas.pdf
96. Reid R, Bulusu R, Carroll N, *et al*. Guidelines for the Management of Gastrointestinal Stromal Tumours (GIST) 2009. Available from: http://www.augis.org/wp-content/uploads/2014/05/GIST_Management_Guidelines_180809.pdf
97. National Institute for Health and Care Excellence (NICE). Suspected cancer: recognition and referral [Guidelines]. 2015. Available from: https://www.nice.org.uk/guidance/ng12/resources/suspected-cancer-recognition-and-referral-1837268071621
98. Sarcoma UK. About 'On The Ball' 2016. Available from: https://sarcoma.org.uk/get-involved/other-ways-get-involved/order-ball-pack/about-ball
99. Sarcoma UK. Order your 'On the Ball' pack 2016. Available from: https://sarcoma.org.uk/get-involved/other-ways-get-involved/about-ball/order-your-ball-pack
100. Sarcoma UK. Research 2016. Available from: https://sarcoma.org.uk/research
101. Tordrup D. NICE highly specialised technologies guidance. *RARE Journal* 2014;1(3):83-97.
102. NHS Commissioning Board. Interim Commissioning Policy: Individual funding requests, 2016.
103. National Institute for Health Research. UK Clinical Trials Gateway 2016. Available from: https://www.ukctg.nihr.ac.uk

The Sarcoma Policy Checklist was developed by a group of leading representatives from patient organisations, clinical experts and industry. This project was initiated and funded by Lilly.

For a copy of the report, go to: **www.sarcoma-patients.eu/**